As confidentially submitted to the Securities and Exchange Commission on July 26, 2024, as Amendment No. 1 to the draft registration statement confidentially submitted on June 14, 2024. Amendment No. 1 to this draft registration statement has not been publicly filed with the Securities and Exchange Commission and all information contained herein remains strictly confidential.

Registration No. 333-

UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
Washington, D.C. 20549

FORM S-1
REGISTRATION STATEMENT
UNDER
THE SECURITIES ACT OF 1933

CAMP4 Therapeutics Corporation
(Exact name of registrant as specified in its charter)

Delaware	**2834**	**81-1152476**
(State or other jurisdiction of Incorporation or organization)	(Primary Standard Industrial Classification Code Number)	(I.R.S. Employer Identification No.)

One Kendall Square
Building 1400 West, 3rd Floor
Cambridge, Massachusetts 02139
(Address, including zip code, and telephone number, including area code, of registrant's principal executive offices)

Josh Mandel-Brehm
Chief Executive Officer
One Kendall Square, Building 1400 West, 3rd Floor
Cambridge, Massachusetts 02139
Telephone: (617) 651-8867
(Name, address, including zip code, and telephone number, including area code, of agent for service)

Copies to:

Marc A. Rubenstein **Thomas J. Danielski** **Ropes & Gray LLP** **Prudential Tower** **800 Boylston Street** **Boston, Massachusetts 02199** **(617) 951-7000**	**Siavosh Salimi** **William A. Magioncalda** **Paul Hastings LLP** **The MetLife Building** **200 Park Avenue** **New York, New York 10166** **(212) 318-6000**

Approximate date of commencement of proposed sale to the public:
As soon as practicable after this Registration Statement is declared effective.

If any of the securities being registered on this form are to be offered on a delayed or continuous basis pursuant to Rule 415 under the Securities Act of 1933, check the following box. ☐

If this form is filed to register additional securities for an offering pursuant to Rule 462(b) under the Securities Act, check the following box and list the Securities Act registration statement number of the earlier effective registration statement for the same offering. ☐

If this form is a post-effective amendment filed pursuant to Rule 462(c) under the Securities Act, check the following box and list the Securities Act registration statement number of the earlier effective registration statement for the same offering. ☐

If this form is a post-effective amendment filed pursuant to Rule 462(d) under the Securities Act, check the following box and list the Securities Act registration statement number of the earlier effective registration statement for the same offering. ☐

Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer, a smaller reporting company, or an emerging growth company. See the definitions of "large accelerated filer," "accelerated filer," "smaller reporting company," and "emerging growth company" in Rule 12b-2 of the Exchange Act.

Large accelerate filer ☐	Accelerated filer ☐
Non-accelerated filer ☒	Smaller reporting company ☒
	Emerging growth company ☒

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 7(a)(2)(B) of the Securities Act. ☐

The Registrant hereby amends this Registration Statement on such date or dates as may be necessary to delay its effective date until the Registrant shall file a further amendment which specifically states that this Registration Statement shall thereafter become effective in accordance with Section 8(a) of the Securities Act of 1933, as amended, or until the Registration Statement shall become effective on such date as the Securities and Exchange Commission, acting pursuant to said Section 8(a), may determine.

Subject to completion, dated , 2024

Preliminary prospectus

shares

Common stock

This is an initial public offering of shares of common stock by CAMP4 Therapeutics Corporation.

We are offering shares of our common stock. The initial public offering price is expected to be between $ and $ per share.

Prior to this offering, there has been no public market for our common stock. We have applied to list our common stock on the Nasdaq Global Market under the trading symbol "CAMP," and this offering is contingent upon the listing of our common stock on the Nasdaq Global Market.

We are an "emerging growth company" and a "smaller reporting company" as defined under the U.S. federal securities laws and, as such, will be subject to reduced public company reporting requirements for this prospectus and future filings. See "Prospectus summary—Implications of being an emerging growth company and a smaller reporting company."

Investing in our common stock involves a high degree of risk. See "Risk factors" beginning on page 15 to read about factors you should consider before buying shares of our common stock.

Neither the Securities and Exchange Commission nor any state securities commission has approved or disapproved of these securities, or passed upon the accuracy or adequacy of this prospectus. Any representation to the contrary is a criminal offense.

	Per share	Total
Initial public offering price	$	$
Underwriting discounts and commissions(1)	$	$
Proceeds before expenses, to us	$	$

(1) See the section titled "Underwriting" for additional information regarding underwriting compensation.

We have granted the underwriters an option for a period of 30 days to purchase up to additional shares of common stock from us at the public offering price less the underwriting discount.

The underwriters expect to deliver the shares against payment on or about , 2024.

J.P. Morgan Leerink Partners Piper Sandler William Blair

, 2024

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Neither we nor the underwriters have authorized anyone to provide any information or to make any representations other than those contained in this prospectus or in any free writing prospectus prepared by or on behalf of us or to which we have referred you. We and the underwriters take no responsibility for, and can provide no assurance as to the reliability of, any other information that others may provide you. We and the underwriters are not making an offer to sell these securities in any jurisdiction where the offer or sale is not permitted. You should assume that the information contained in this prospectus is accurate only as of the date on the front cover of this prospectus, regardless of the time of delivery of this prospectus or of any sale of our common stock. Our business, financial condition, results of operations and prospects may have changed since that date.

For investors outside of the United States: neither we nor any of the underwriters have done anything that would permit this offering or possession or distribution of this prospectus in any jurisdiction where action for that purpose is required, other than the United States. Persons outside of the United States who come into possession of this prospectus must inform themselves about, and observe any restrictions relating to, the offering of the shares of common stock and the distribution of this prospectus outside of the United States.

Presentation of financial information

Pursuant to the applicable provisions of the Fixing America's Surface Transportation Act, we are omitting our unaudited consolidated financial statements as of and for the three months ended March 31, 2024 and 2023 because they relate to historical periods that we believe will not be required to be included in the accompanying prospectus at the time of the contemplated offering. We intend to amend the registration statement to include all financial information required by Regulation S-X under the Securities Act of 1933, as amended, at the date of such amendment before distributing a preliminary prospectus to investors.

Prospectus summary

This summary highlights, and is qualified in its entirety by, information contained elsewhere in this prospectus. This summary does not contain all of the information you should consider before investing in our common stock. You should read this entire prospectus carefully, especially the sections titled "Risk factors," "Business," and "Management's discussion and analysis of financial condition and results of operations" and our consolidated financial statements and the related notes appearing elsewhere in this prospectus, before making an investment decision. As used in this prospectus, unless the context otherwise requires, references to "we," "us," "our," "the company," "CAMP4" and "CAMP4 Therapeutics" refer to CAMP4 Therapeutics Corporation.

CAMP4 is the final camp before the summit of Mount Everest. It is also home to a climbing haven in Yosemite National Park where the world's greatest climbers gather to push the boundaries for what is thought to be possible. Like these elite climbers, we are pushing the boundaries of biology to discover and develop new and potentially life changing therapeutics.

Overview

We are a clinical-stage biopharmaceutical company pioneering the discovery and development of regulatory RNA-based therapeutics with the goal of upregulating gene expression and restoring healthy protein levels to treat a broad range of genetic diseases. Regulatory RNAs, or regRNAs, play a central role in the regulation of every protein-coding gene by contributing to gene activation and suppression. Our approach is designed to amplify messenger RNA, or mRNA, expression by harnessing the power of regRNAs that form localized complexes with transcription factors and regulate gene expression. Our proprietary RNA Actuating Platform, or RAP Platform, allows us to rapidly and systematically identify and characterize the active regulatory elements controlling every expressed gene and tens of thousands of druggable enhancer and promoter regRNA sequences that control protein-coding genes. Once a disease-associated target gene is identified, we apply our RAP Platform to identify the controlling regRNA and rapidly generate novel antisense oligonucleotide, or ASO, candidates, which we also refer to as RNA Actuators. These ASOs are designed to bind to the identified regRNA and amplify the expression of the target gene in a specific and controllable way. We are initially focused on metabolic and central nervous system, or CNS, diseases with validated disease biology, and we believe our RAP Platform allows us to address a broad range of genetic diseases in which a modest increase in protein expression can be clinically meaningful.

Based on our preclinical studies, we believe our lead product candidate, CMP-CPS-001, has the potential to be the first disease-modifying therapy for the treatment of the most prevalent urea cycle disorders, or UCDs. UCDs are a group of severe, inherited metabolic diseases caused by mutations in the genes that encode one or more of the eight enzymes and transporters necessary to convert ammonia into urea. The inability of the body to properly metabolize ammonia leads to the accumulation of toxic levels in circulation, ultimately resulting in severe health outcomes, such as neurologic disability, seizure and death. CMP-CPS-001 is designed to improve urea cycle activity by amplifying expression of carbamoyl phosphate synthetase 1, or CPS1, an enzyme that catalyzes the first step of the urea cycle, by binding to a CPS1-specific regRNA. Our preclinical studies have demonstrated that modulating the activity of the target regRNA increases expression of the *CPS1* gene, resulting in increased CPS1 enzyme levels, which allows for more ammonia to be converted into urea, thereby lowering ammonia levels to normal, healthy ranges. These preclinical studies also demonstrated that CMP-CPS-001 can increase the level of, or upregulate, the production of multiple enzymes responsible for converting ammonia into urea, potentially allowing us to address more than 85% of patients with UCDs, which we refer to as our pan-UCD approach. We are in the early stages of development and are evaluating CMP-CPS-001 in an ongoing Phase 1 clinical trial in healthy volunteers and expect to report data from the single ascending dose, or SAD, portion of the trial in and from the multiple ascending dose, or MAD, portion of the trial in . We are also leveraging our RAP Platform to advance two preclinical programs for the treatment of both heterozygous familial hypercholesterolemia, or FH, and synaptic Ras GTPase activating protein 1, or SYNGAP1,-related disorders. We expect to initiate final Good Laboratory Practice, or GLP, toxicology studies in our FH and SYNGAP1 programs in to enable the filing of clinical trial applications.

The transcription of DNA into mRNA, the molecular template that is then translated into protein, is a complex yet carefully coordinated cellular process involving numerous components. Only a small portion of the DNA in the human genome is transcribed into RNA that codes for proteins. The vast majority of the transcriptome originates from non-coding regions of DNA, a portion of which, referred to as enhancers and promoters, perform a crucial role in determining the specificity, timing and level at which a particular gene is expressed. RegRNAs are non-coding RNAs that are transcribed by these enhancer and promoter DNA regions that form localized complexes with transcription factors to control the expression of protein-coding genes, either increasing or decreasing their expression within natural physiological ranges. The approximately 20,000 genes that code for mRNA in the human genome are controlled by hundreds of thousands of DNA enhancers and their associated regRNAs.

Deficient protein levels characterize over a thousand diseases. Haploinsufficient diseases are dominantly inherited conditions in which inadequate gene expression is driven by a mutation in a single allele, or gene copy, and results in reductions of protein levels by as much as 50%. Numerous other genetic conditions are caused by recessive mutations that result in diminished gene activity. Data from our preclinical studies and research reports published by third parties demonstrate that increasing expression of disease-associated genes by modest amounts can restore healthy protein levels and provide therapeutic benefit in these disorders. Therefore, modest increases in protein expression have the potential to be clinically meaningful in both haploinsufficient and recessive partial loss-of-function disorders, of which there are more than 1,200. Our RAP Platform has the potential to identify the regRNA associated with all of these diseases, which we believe enables us to design RNA Actuators to address the underlying biology of these diseases. We aim to leverage our RAP Platform to develop product candidates designed to regulate transcription in a gene-specific manner to restore healthy protein levels and remedy these diseases. However, our approach is unproven and may not lead to successful efforts to develop and commercialize our product candidates and to identify and discover additional potential product candidates.

Our RAP Platform

We believe our RAP Platform can unlock the potential of the human genome and have broad applications across a range of diseases caused by sub-optimal levels of protein expression. Our technology is based upon the pioneering work in transcription regulation conducted by our co-founders, Richard Young, PhD and Leonard Zon, MD. We have built our RAP Platform to identify and characterize every regRNA that controls protein-coding genes and to develop novel ASO-based therapeutics to modulate regRNA activity to increase the expression of protein-coding genes of interest and thereby address the underlying cause of genetic diseases. Based on our proprietary mapping of regRNAs and screening and optimizing of ASOs, we have established a leadership position in regRNA-targeting therapies. Our goal is to be the preeminent company focused on discovering, developing and delivering regRNA-targeting therapeutics to patients. We believe that the ability to upregulate genes selectively through targeting regRNA could provide a new way to treat a wide range of human diseases and has the potential to become a class of new medicines.

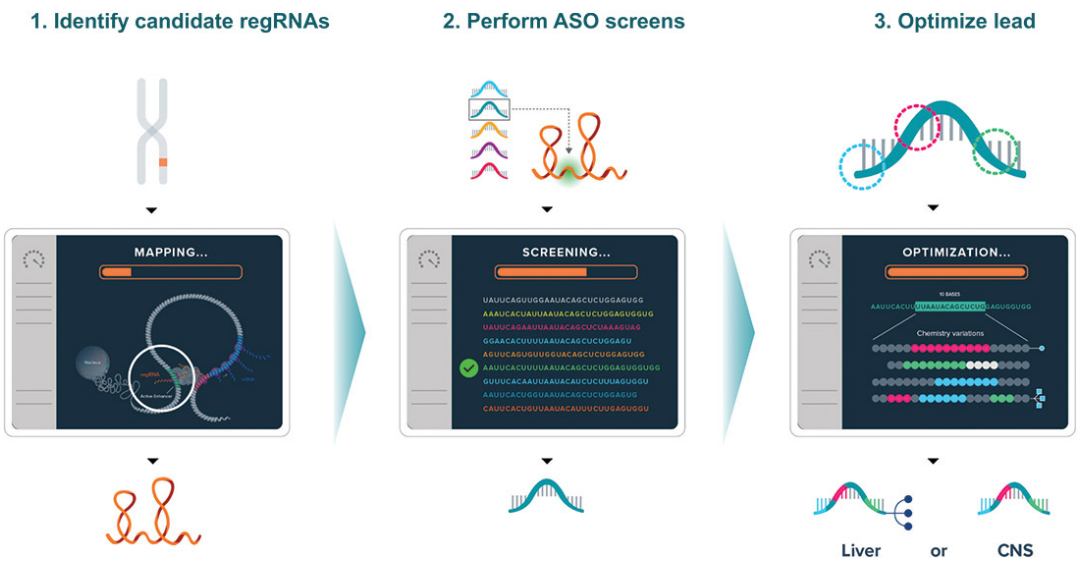
At present, very few regRNAs are described in public genomic databases, as they are often expressed at low levels and their importance was not fully understood. Our RAP Platform utilizes next-generation sequencing technologies and custom sequence analyses to map the active regulatory elements controlling every expressed gene. These data empower our proprietary machine learning algorithm, known as EPIC, to identify the specific control elements that regulate any gene of interest in the most specific manner, including elements that may restrict gene expression to a particular cell type. This enables us to identify the exact sites of regRNA synthesis and ultimately map the complete sequence of every candidate regRNA to target for therapeutic gene control. To date, we have mapped multiple cell types in as little as three months, comprising a number of potentially addressable diseases in the liver, CNS, heart, skeletal muscle and immune system. Our in-house development and application of this technology has enabled us to identify tens of thousands of enhancer and promoter regRNA sequences and their key biological properties, resulting in what we believe to be the most robust regRNA dataset available.

We combine our RAP Platform with ASO chemistry that has been utilized and validated in U.S. Food and Drug Administration, or FDA,-approved products to develop programmable RNA Actuators that are designed to precisely

upregulate gene expression at the transcriptional level. Once a target gene is nominated, our RAP Platform rapidly identifies the controlling regRNA sequence, and we perform ASO screens to identify regions where ASO binding results in optimal upregulation of that target gene. Further rational design is applied to the ASOs identified in the screen. Our proprietary technology enables us to design RNA Actuators that optimize for specificity by avoiding binding to regRNAs that act on more than one gene and any other similar sequences found elsewhere in the transcriptome. As a result, our sequence-specific approach enables us to precisely target regRNA transcripts to increase gene expression. Our approach is designed to enable the efficient and systematic creation of RNA Actuators to target regRNAs of interest. Building upon the power of this technology, our RNA Actuators can be programmed to engage regRNA targets, producing tunable increases in protein expression. While other ASOs have received regulatory approval, no regulatory authorities to date have approved ASOs that are directed towards regRNAs and, as a result, there is uncertainty as to the safety and efficacy profile of our product candidates compared to currently approved ASOs.

The key steps involved in our RAP Platform are illustrated below:

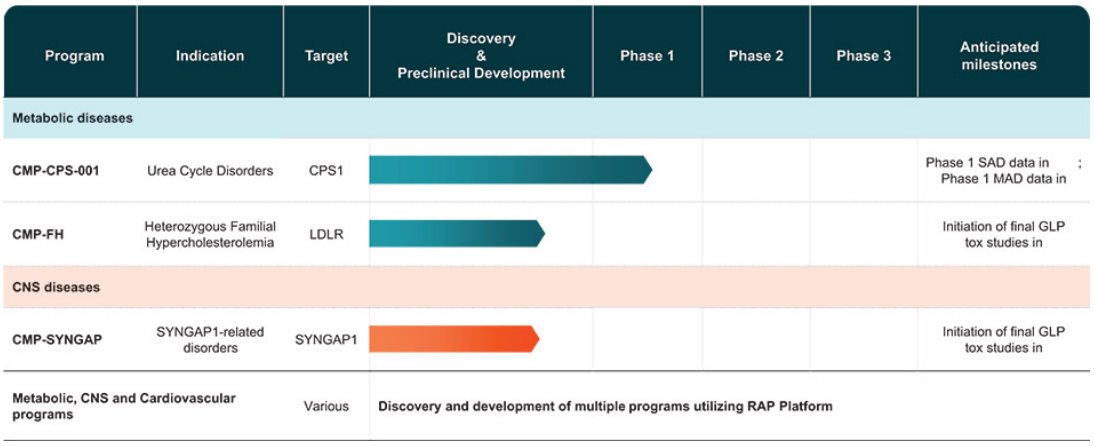
Our proprietary RAP Platform



We design RNA Actuators to leverage existing oligonucleotide delivery approaches to enable drug delivery to specific types of tissues throughout the body. We believe our RAP Platform can address any disease where a modest increase in protein expression has the potential to be clinically meaningful, including haploinsufficient diseases or recessive loss-of-function diseases. Furthermore, as we continue to map regRNAs and conduct ASO screens in more cell types, the data generated will improve the algorithms we use to identify the candidate regRNAs to specifically control gene expression. We believe the knowledge and learnings from our initial programs will significantly expedite selection of lead candidates and position us to rapidly expand our pipeline.

Our pipeline

We are leveraging our RAP Platform to advance a pipeline of programs initially focused on metabolic and CNS disorders with validated disease biology and attractive potential market opportunities due to the significant unmet need of affected patients. We retain exclusive, worldwide development and commercialization rights to all of our product candidates and discovery programs.



CMP-CPS-001: Potential treatment for urea cycle disorders

Based on our preclinical studies, we believe our lead product candidate, CMP-CPS-001, has the potential to be the first disease-modifying therapy for the treatment of the most prevalent UCDs. UCDs are a group of severe, inherited metabolic diseases caused by mutations in the genes that encode one or more of the eight enzymes and transporters necessary to convert ammonia into urea, which is then excreted from the body. The inability of the body to properly metabolize ammonia leads to the accumulation of toxic systemic levels in circulation, ultimately resulting in severe health outcomes, such as neurologic disability, seizure and death. UCDs occur across all age groups, from infants to adults, and mild symptoms may go unnoticed until a stressor, such as illness, surgery, protein consumption or environmental stress, overwhelms compensatory functions, resulting in hyperammonemic crisis, or extremely high levels of ammonia. The prevalence of UCDs is estimated to be approximately 3,700 patients in the United States, of which we estimate are late onset, defined as having severe symptom onset after one month of life, and 96% of these late onset patients have enzyme deficiencies we can address. The incidence of UCDs in the United States is estimated to be approximately 1 in 35,000 births, with similar prevalence and incidence estimated for Europe. The most common UCD, accounting for approximately 60% of UCD diagnoses, is ornithine transcarbamylase, or OTC, deficiency, caused by mutations in the *OTC* gene. The next two most common genetic subtypes are caused by mutations in the genes coding for the enzymes argininosuccinate lyase, or ASL, and argininosuccinate synthetase, or ASS1, deficiencies which affect approximately 16% and 14% of UCD patients, respectively.

There are no FDA-approved, disease-modifying therapies to treat the most prevalent UCDs. The standard of care is supportive in nature and intended to reduce the frequency of, but not eliminate, hyperammonemic crises. Current protocols for patients involve efforts to lower plasma ammonia levels. Reduction in plasma ammonia is achieved through nitrogen scavengers to remove excess nitrogen, along with the dosing of supplemental citrulline. These nitrogen scavenger agents carry an onerous pill regimen and significantly diminish the quality of life for patients. Longer-term maintenance regimens involve strict adherence to a low-protein diet along with the prophylactic use of nitrogen scavenger agents. When necessary, hemodialysis is used to reduce ammonia concentrations. The existing supportive measures are not sufficient, with many patients suffering neurological disability and premature death. Therapies currently in development are targeting only a select subgroup of patients with UCDs, which includes those with OTC deficiency and patients 12 years and older. We have designed CMP-CPS-001 to be broadly applicable to UCD patients and to overcome the limitations of the current standard of care as well as programs in development for the treatment of late onset UCDs by using an established ASO modality and convenient once-monthly subcutaneous administration in order to provide UCD patients with the potential for a safe and efficacious treatment option. We are initially targeting our development of CMP-CPS-001 in the most prevalent late-onset patients (those with OTC, ASL and ASS1 deficiencies, which together constitute more than 80% of patients with UCDs) and we may expand into additional groups of patients with less common forms of UCD.

CMP-CPS-001 is designed to improve urea cycle activity by amplifying expression of CPS1, a key enzyme that catalyzes the first step of the urea cycle, by binding to a CPS1-specific regRNA. CMP-CPS-001 is a subcutaneously injected ASO conjugated to N-acetylgalactosamine, or GalNAc, a ligand that enables targeted delivery to the liver, designed to be administered monthly. Increasing *CPS1* expression enhances the metabolism of ammonia and upregulates multiple urea cycle enzymes, including OTC, resulting in elevated urea cycle activity. Our RAP Platform enabled us to (i) identify the key enhancer modulating *CPS1* expression, (ii) screen ASOs directed to the regRNAs expressed by this enhancer, and (iii) generate a lead RNA Actuator designed to increase *CPS1* expression.

Our preclinical studies have demonstrated that modulating the activity of the target regRNA increases expression of the *CPS1* gene, resulting in increased CPS1 enzyme levels, which allows for more ammonia to be converted into urea, thereby lowering ammonia levels to normal, healthy ranges. This includes studies in a mouse model where we demonstrate that increasing *Cps1* expression can overcome a partial loss of function mutation in the urea cycle enzyme, *Otc*, and improve ammonia clearance. These preclinical studies also demonstrated that CMP-CPS-001 can upregulate the production of multiple enzymes responsible for converting ammonia into urea, which supports our pan-UCD approach. In non-human primate, or NHP, studies, the administration of CMP-CPS-001 increased the synthesis of urea, commonly referred to as ureagenesis. In these NHP studies, labeled sodium acetate was used as part of a ureagenesis rate test, or URT, to measure the metabolic output of the urea cycle. Carbaglu, approved for ultra-rare *N*-acetylglutamate synthesase, or NAGS-deficient patients, utilized the URT in healthy volunteers and showed that minimal increases in ureagenesis translated to substantial ammonia reductions in NAGS-deficient patients. Rates of ureagenesis were found to exceed those achieved by placebo in a statistically significant manner. This assay is also being used in our Phase 1 clinical trial. An increase in the metabolic output of the urea cycle, as indicated by an increase in the amount of labeled sodium acetate metabolized, is expected to correlate with an increase in the amount of ammonia metabolized. Although we believe that an increase in ureagenesis in our Phase 1 clinical trial may correspond with clinically meaningful improvements in ammonia metabolism in UCD patients, ureagenesis is not an established clinical endpoint and the URT results obtained in our Phase 1 clinical trial in healthy adult volunteers should not be interpreted as evidence of efficacy of CMP-CPS-001. For a further discussion of our use of this assay, please see “Risk factors—The outcome of preclinical studies and earlier-stage clinical trials may not be predictive of future results or the success of later preclinical studies and clinical trials.” We are evaluating CMP-CPS-001 in a randomized, double-blind and placebo-controlled Phase 1 clinical trial to evaluate safety, tolerability and pharmacokinetics in healthy volunteers in Australia. We expect to report Phase 1 clinical trial data from the SAD portion in and from the MAD portion in .

CMP-FH: Program for the treatment for heterozygous familial hypercholesterolemia

Our CMP-FH program is developing an RNA Actuator as a disease-modifying therapy to lower LDL cholesterol, or LDL-c, levels for the treatment of FH. FH is a group of genetic disorders that lead to reduced levels of low-density lipoprotein, or LDL, receptor, or LDLR, and/or impaired receptor function in the liver, thereby diminishing liver-mediated removal of LDL. The most common genetic cause of FH is due to mutations in the *LDLR* gene, accounting for an estimated 85% to 90% of all FH cases and is a significant contributor to early-onset cardiovascular disease. Heterozygous FH is caused by LDLR haploinsufficiency and is a common genetic disorder affecting approximately one in 200 to one in 300, or over 3 million patients in the United States and Europe, in the aggregate.

In addition to dietary and lifestyle modifications, patients with heterozygous FH are often treated first with statins. However, up to 60% of patients on statins alone still remain above recommended LDL-c levels and at increased risk for cardiovascular events. Furthermore, patient adherence to the required daily dosing schedule is often poor and statin intolerance is estimated to affect approximately 10% of patients. Monoclonal antibodies and short-interfering RNAs, or siRNAs, have emerged as a promising therapeutic approach, but despite the availability of these treatments, a significant unmet medical need remains in up to 40% of patients on combinations of statins and these therapies who may seek an alternative because they still remain above recommended LDL-c levels or their disease is unresponsive to the use of statins and existing therapies.

Our CMP-FH program utilizes a GalNAc-conjugated, subcutaneously delivered ASO designed to increase the expression of LDLR, a well-validated target that directly lowers LDL-c levels. Leveraging our RAP Platform, we (i) identified the key regRNAs that modulate *LDLR* expression, (ii) screened ASOs targeting the regRNAs and (iii) generated multiple lead RNA Actuators that increase LDLR-encoding mRNA. Our preclinical studies have demonstrated that increased transcription of *LDLR* led to a meaningful increase in LDLR protein synthesis and cellular uptake of LDL-c, which provides evidence of our therapeutic approach. These preclinical studies have further demonstrated that increases in LDLR mRNA levels up to 100% were sufficient to reduce circulating LDL-c levels by approximately 25%. This effect represents a clinically meaningful decrease, which we believe has the potential to reduce the risk of serious coronary events. Moreover, we have shown that the increase of *LDLR* expression results in approximately 50% increase in plasma high-density lipoprotein, or HDL, levels. These data indicate that our LDLR regRNA-targeting ASOs can reduce LDL-c that contributes to atherosclerotic cardiovascular disease while increasing HDL, which promotes reduction of circulating cholesterol and is inversely correlated with disease. *In vitro* assessments have also suggested that use of one of our lead candidate RNA Actuators may work in a complementary, additive fashion with statins. We expect to initiate final GLP toxicology studies in to enable the filing of a clinical trial application.

CMP-SYNGAP: Program for the treatment for SYNGAP1-related disorders

Our initial CNS development program, CMP-SYNGAP, aims to address the underlying cause of SYNGAP1-related disorders. SYNGAP1-related disorders are a group of neurodevelopmental conditions caused by pathogenic variants in the *SYNGAP1* gene leading to a haploinsufficient state that reduces SYNGAP protein levels by as much as 50%. SYNGAP plays a critical role in the development of cognition and proper synaptic function. Epilepsy is a common characteristic of these disorders and nearly all patients present with some degree of developmental delay and cognitive impairment. Patient estimates for SYNGAP1-related disorders vary significantly. We estimate that 5,000 individuals have been diagnosed with these disorders in the United States, though we believe many more with mild symptoms remain undiagnosed and are not included in this estimate. Incidence estimates of SYNGAP1-related disorders range from 1 to 40 in 100,000 individuals and the disorder is reported to represent 0.5% to 1.0% of all intellectual disability cases.

There are no FDA-approved, disease-modifying therapies for SYNGAP1-related disorders. There is also no definitive treatment protocol, which is dependent on seizure type and severity and other neurological characteristics. Treatment is often limited to supportive physical, occupational and speech therapy. A combination of non-specific anti-seizure medications may be prescribed to treat seizures, though SYNGAP1-related disorders have proven difficult to control with available therapeutics. As many as 50% of patients do not adequately respond to medication, in which case implantable devices, such as those for vagus nerve stimulation, may offer incremental therapeutic benefit.

We are advancing our CMP-SYNGAP program to address the significant unmet need for these patients by targeting the direct cause of SYNGAP1-related disorders, haploinsufficiency, which we believe is amenable to targeting through regRNAs. Our CMP-SYNGAP program is a novel approach that targets the *SYNGAP1* gene at the transcriptional level to restore SYNGAP function and improve symptoms, by utilizing an intrathecally delivered ASO. We have identified specific regRNA sequences involved in *SYNGAP1* transcription and leverage our RAP Platform to generate ASOs that function to increase *SYNGAP1* transcription. Upregulation of *SYNGAP1* gene expression may increase SYNGAP protein levels in amounts sufficient to yield therapeutic benefit. Our preclinical studies demonstrated a dose-dependent increase in SYNGAP1 mRNA levels accompanied by a reduction in *SYNGAP1* expression. We expect to initiate final GLP toxicology studies in to enable the filing of a clinical trial application.

Our team

Our management team brings a depth of experience and knowledge in platform research, drug discovery and development and commercialization. Our team is led by our President and Chief Executive Officer Josh Mandel-Brehm, who brings over 18 years of leadership experience with life sciences companies, including business development and operational experience from his time at Biogen, Sanofi and Genzyme; David Bumcrot, PhD, our

Chief Scientific Officer, an industry expert who was responsible for the initial therapeutic initiatives utilizing CRISPR technology at Editas Medicine and the start of RNAi therapeutic development at Alnylam Pharmaceuticals; Yuri Maricich, MD, our Chief Medical Officer, who led clinical, regulatory, quality and medical affairs functions as a member of the executive team of several early-stage biopharmaceutical companies, including Pear Therapeutics; and Kelly Gold, our Chief Financial Officer, who was previously part of the corporate finance and business planning groups at Biogen and the healthcare investment banking group of Deutsche Bank.

Our technology is based on the pioneering work in transcription regulation conducted by our distinguished co-founders, Richard Young, PhD, of the Whitehead Institute for Biomedical Research and the Massachusetts Institute of Technology, and Leonard Zon, MD, who is affiliated with Boston Children's Hospital and the Harvard Medical School.

Since our inception, we have raised $188.3 million. Our investor group includes entities affiliated with 5AM Ventures; AH Bio Fund I, L.P.; Everest Aggregator, LP, an affiliate of Enavate Sciences; entities affiliated with the Kaiser Permanente Group Trust; entities affiliated with Northpond Ventures, LLC; entities affiliated with Polaris Partners; and SMRS-TOPE LLC. Prospective investors should not rely on the investment decisions of our existing investors, as these investors may have different risk tolerances and strategies and have purchased their shares in prior offerings at prices lower than the price offered to the public in this offering. In addition, some of these investors may not be subject to reporting requirements under Section 16 of the Securities Exchange Act of 1934, and, thus, prospective investors may not necessarily know the total amount of investment by each of the prior investors and if and when some of the prior investors decide to sell any of their shares. See the sections titled "Certain relationships and related person transactions" and "Principal stockholders" for more information on prior purchases by and current holdings of these stockholders.

Our strategy

Our mission has been to decode the rules of human gene expression to develop a new class of medicines that can transform the treatment paradigm for a wide range of genetic-based diseases. To accomplish this, we leverage our proprietary RAP Platform to map cells and discover regRNAs that regulate protein-coding genes in diseases characterized by sub-optimal levels of protein expression where modest increases in protein production can have a clinically meaningful therapeutic effect on patients. The key elements of our strategy include:

- Advance our lead candidate, CMP-CPS-001, through clinical trials and become the first approved disease-modifying therapy for UCDs.
- Rapidly advance our disease-modifying candidates for heterozygous familial hypercholesterolemia and SYNGAP1-related disorders into clinical development.
- Leverage our RAP Platform to expand our pipeline in metabolic, CNS and other disease areas characterized by sub-optimal levels of protein expression.
- Leverage validated modalities to efficiently advance programs through clinical development and regulatory approval.
- Pursue strategic partnerships to maximize the value of our product candidates and RAP Platform.
- Build a leading regRNA-targeting therapeutic company.

Risks associated with our business

Our business is subject to numerous risks that you should be aware of before making an investment decision. These risks are described more fully in the "Risk factors" section of this prospectus immediately following this prospectus summary and include, among others:

- We have incurred significant losses since our inception, have no products approved for sale and we expect to incur losses for the foreseeable future;

- Even if this offering is successful, we will require substantial additional capital to finance our operations, and a failure to obtain this necessary capital when needed on acceptable terms, or at all, could force us to delay, limit, reduce, or terminate our development programs, commercialization efforts or other operations;
- Raising additional capital may cause dilution to our stockholders, restrict our operations or require us to relinquish rights to our technologies or product candidates;
- Our independent registered public accounting firm has expressed substantial doubt about our ability to continue as a going concern in its report on our audited financial statements included in this prospectus;
- We are early in our development efforts. Our product candidates are in varying stages of preclinical and clinical development and we have not completed a clinical trial of any product candidate. As a result, it will be many years before we commercialize a product candidate, if ever. If we are unable to identify and advance product candidates through preclinical studies and clinical trials, obtain marketing approval and ultimately commercialize them, or experience significant delays in doing so, our business will be materially harmed;
- Our business is highly dependent on our lead product candidate, CMP-CPS-001, as our sole clinical-stage program, and we must complete clinical testing before we can seek regulatory approval and begin commercialization of any of our other product candidates. If we are unable to obtain regulatory approval for, and successfully commercialize, CMP-CPS-001, our business may be materially harmed and such failure may affect the viability of our other product candidates;
- Drug development is a lengthy and expensive process, and preclinical and clinical testing is uncertain as to the outcome. We may encounter substantial delays in the commencement, enrollment or completion of our clinical trials and may never advance to clinical trials, or we may fail to demonstrate safety and effectiveness to the satisfaction of applicable regulatory authorities, which could prevent us from advancing or commercializing our product candidates on a timely basis, if at all;
- If any of our current or any future product candidates cause undesirable side effects or have other unexpected adverse properties, such side effects or properties could delay or prevent regulatory approval, limit the commercial potential or result in significant negative consequences following any potential marketing approval;
- We face substantial competition, which may result in others discovering, developing or commercializing products before us or more successfully than we do;
- We may enter into collaborations with third parties for the research, development and commercialization of certain of the product candidates we may develop. If any such collaborations are not successful, we may not be able to capitalize on the market potential of those product candidates;
- Our future success depends on our ability to retain key executives and to attract, retain and motivate qualified personnel;
- We may encounter difficulties in managing our growth and expanding our operations successfully;
- We currently depend on third-party suppliers for the manufacture of our product candidates. The loss of these or future third-party suppliers, or their inability to provide us with sufficient supply, could harm our business;
- Our rights to develop and commercialize our product candidates are subject, in part, to the terms and conditions of licenses granted to us by third parties. If we fail to comply with our obligations under these arrangements or otherwise experience disruptions to our business relationships with our current or any future licensors, we could lose such intellectual property rights that are important to our business;
- Third parties may initiate legal proceedings alleging that we are infringing, misappropriating or otherwise violating their intellectual property rights, the outcome of which would be uncertain and could harm our business;

- If we or our licensors are unable to obtain, maintain, enforce and adequately protect our intellectual property rights with respect to our product candidates and technology, or if the scope of any patent or other intellectual property protection obtained is not sufficiently broad, our competitors could develop and commercialize products and technology similar or identical to ours, and our ability to successfully develop and commercialize our product candidates and technology may be adversely affected. Further, we do not currently own or in-license any issued patents directed to the composition of matter, or methods of use, of our product candidates; if we fail to obtain such patents, our competitors may be able to develop, make or market products identical to our product candidates after expiration of any applicable regulatory exclusivities;
- We rely, and intend to continue to rely, on third parties to perform some of our preclinical studies and conduct our clinical trials. If these third parties do not successfully carry out their contractual duties, fail to comply with applicable regulatory requirements, or do not meet expected deadlines, our development programs may be delayed or subject to increased costs or we may be unable to obtain regulatory approval for or commercialize our product candidates;
- There has been no public market for our common stock. An active, liquid, and orderly market for our common stock may not develop, or we may in the future fail to satisfy the continued listing requirements of Nasdaq, and investors may not be able to resell their common stock at or above the initial public offering price or at all; and
- The trading price of the shares of our common stock could be highly volatile, and purchasers of our common stock could incur substantial losses.

If we are unable to adequate address these and other risks we face, our business, results of operations, financial condition and prospects may be harmed.

Implications of being an emerging growth company and a smaller reporting company

We qualify as an “emerging growth company” as defined in the Jumpstart Our Business Startups Act of 2012, as amended, or the JOBS Act. For so long as we remain an emerging growth company, we may take advantage of relief from certain reporting requirements and other burdens that are otherwise applicable generally to public companies. These provisions include:

- reduced obligations with respect to financial data, including only being required to present two years of audited financial statements, in addition to any required unaudited interim financial statements with correspondingly reduced “Management’s discussion and analysis of financial condition and results of operations” disclosure;
- an exception from compliance with the auditor attestation requirements of Section 404 of the Sarbanes-Oxley Act of 2002, as amended;
- reduced obligations with respect to disclosure about our executive compensation arrangements in our periodic reports, proxy statements and registration statements;
- exemptions from the requirements of holding non-binding advisory votes on executive compensation or golden parachute arrangements; and
- an exemption from compliance with the requirements of the Public Company Accounting Oversight Board regarding the communication of critical audit matters in the auditor’s report on financial statements.

We may take advantage of these provisions until the last day of the fiscal year ending after the fifth anniversary of this offering or such earlier time that we no longer qualify as an emerging growth company. We will cease to qualify as an emerging growth company on the date that is the earliest of: (i) the last day of our fiscal year following the fifth anniversary of the date of the completion of this offering, (ii) the last day of the fiscal year in which we have more than $1.235 billion in total annual gross revenues, (iii) the date on which we are deemed to be a “large accelerated filer” under the rules of the U.S. Securities and Exchange Commission, which means the

market value of our common stock that is held by non-affiliates exceeds $700 million as of the prior June 30th, or (iv) the date on which we have issued more than $1.0 billion of non-convertible debt over the prior three-year period. We may choose to take advantage of some but not all of these reduced reporting burdens. We have taken advantage of certain reduced reporting requirements in this prospectus. Accordingly, the information contained herein may be different than you might obtain from other public companies in which you hold equity interests.

In addition, under the JOBS Act, emerging growth companies can delay adopting new or revised accounting standards until such time as those standards apply to private companies. We have elected to take advantage of the extended transition period to comply with new or revised accounting standards and to adopt certain of the reduced disclosure requirements available to emerging growth companies. As a result of the accounting standards election, we will not be subject to the same implementation timing for new or revised accounting standards as other public companies that are not emerging growth companies until such time that we either (i) irrevocably elect to "opt out" of such extended transition period or (ii) no longer qualify as an emerging growth company, which may make comparison of our financials to those of other public companies more difficult. As a result of these elections, the information that we provide in this prospectus may be different than the information you may receive from other public companies in which you hold equity interests. In addition, it is possible that some investors will find our common stock less attractive as a result of these elections, which may result in a less active trading market for our common stock and higher volatility in our share price.

We are also a "smaller reporting company," meaning that the market value of our shares held by non-affiliates plus the proposed aggregate amount of gross proceeds to us as a result of this offering is less than $700 million and our annual revenue was less than $100 million during the most recently completed fiscal year. We may continue to be a smaller reporting company after this offering if either (i) the market value of our shares held by non-affiliates is less than $250 million or (ii) our annual revenue was less than $100 million during the most recently completed fiscal year and the market value of our shares held by non-affiliates is less than $700 million. If we are a smaller reporting company at the time we cease to be an emerging growth company, we may continue to rely on exemptions from certain disclosure requirements that are available to smaller reporting companies. Specifically, as a smaller reporting company, we may choose to present only the two most recent fiscal years of audited financial statements in our Annual Report on Form 10-K and, similar to emerging growth companies, smaller reporting companies have reduced disclosure obligations regarding executive compensation. We may continue to be a smaller reporting company until the fiscal year following the determination that we no longer meet the requirements necessary to be considered a smaller reporting company.

Corporate information

We were originally incorporated under the laws of the State of Delaware in 2015 under the name Marauder Therapeutics, Inc. and began operations in 2016. We changed our name to CAMP4 Therapeutics Corporation in March 2018. Our principal executive offices are located at One Kendall Square, Building 1400 West, 3rd Floor, Cambridge, Massachusetts 02139 and our telephone number is (617) 651-8867. Our website address is www.camp4tx.com. The information contained on, or accessible through, our website is not incorporated by reference into this prospectus. We have included our website in this prospectus solely as an inactive textual reference.

"CAMP4," "RAP Platform," "RNA Actuator" and our other registered or common law trademarks, trade names or service marks appearing in this prospectus are the property of CAMP4 Therapeutics Corporation and are registered as trademarks in the United States and other countries. This prospectus also contains references to trademarks belonging to other entities. Solely for convenience, trademarks and trade names referred to in this prospectus, including logos, artwork and other visual displays, may appear without the ® or ™ symbols, but such references are not intended to indicate in any way that we will not assert, to the fullest extent under applicable law, our rights or the rights of the applicable licensor to these trademarks and trade names. We do not intend our use or display of other entities' trade names, trademarks or service marks to imply a relationship with, or endorsement or sponsorship of us by, any other entity.

The offering

Common stock offered by us	shares.
Underwriters' option to purchase additional shares	We have granted the underwriters an option for a period of 30 days from the date of this prospectus to purchase up to additional shares of our common stock.
Common stock to be outstanding immediately after this offering	shares (or shares if the underwriters exercise in full their option to purchase additional shares).
Use of proceeds	We estimate that our net proceeds from this offering will be approximately $ million (or approximately $ million if the underwriters exercise in full their option to purchase up to additional shares of common stock), assuming an initial public offering price of $ per share, which is the midpoint of the price range set forth on the cover page of this prospectus, and after deducting the estimated underwriting discounts and commissions and estimated offering expenses payable by us. We currently intend to use the net proceeds from this offering, together with our existing cash and cash equivalents, to and the remainder for working capital and other general corporate purposes. See the section titled "Use of proceeds" for additional information.
Risk factors	You should read the section titled "Risk factors" for a discussion of factors you should consider carefully, together with all the other information included in this prospectus, before deciding to invest in our common stock.
Proposed Nasdaq Global Market symbol	"CAMP"

The number of shares of our common stock to be outstanding immediately following the completion of this offering is based on shares of our common stock outstanding as of , 2024, after giving effect to the automatic conversion of all outstanding shares of our convertible preferred stock and convertible preferred stock warrants, including shares of our Series A Prime convertible preferred stock and shares of our Series B convertible preferred stock, into an aggregate of shares of our common stock, based on an assumed initial public offering price of $ per share, which is the midpoint of the price range set forth on the cover page of this prospectus, immediately prior to the completion of this offering. The number of shares of our common stock to be outstanding after this offering excludes:

- shares of our common stock issuable upon the exercise of stock options outstanding as of , 2024 pursuant to our Amended and Restated 2016 Stock Option and Grant Plan, or the 2016 Plan, with a weighted-average exercise price of $ per share;
- shares of our common stock reserved for future issuance under the 2016 Plan as of , 2024, which shares will cease to be available for issuance at the time our 2024 Equity Incentive Plan, or the 2024 Plan, becomes effective in connection with this offering;
- shares of our common stock issuable upon the exercise of warrants outstanding at , 2024 at a weighted-average exercise price of $ per share;

- shares of our common stock reserved for future issuance under the 2024 Plan, which will become effective in connection with this offering, as well as any automatic increases in the number of shares of common stock reserved for future issuance under the 2024 Plan; and
- shares of our common stock reserved for future issuance under our 2024 Employee Stock Purchase Plan, or the ESPP, which will become effective in connection with this offering, as well as any automatic increases in the number of shares of common stock reserved for future issuance under the ESPP.

Unless otherwise indicated or the context otherwise requires, all information in this prospectus, including the number of shares of common stock that will be outstanding after this offering, reflects and assumes the following:

- the filing and effectiveness of our amended and restated certificate of incorporation, or Restated Charter, and the adoption of our amended and restated bylaws, or Restated Bylaws, each of which will occur immediately prior to the completion of this offering;
- the automatic conversion of all outstanding shares of our convertible preferred stock into an aggregate of shares of our common stock, based on an assumed initial public offering price of $ per share, which is the midpoint of the price range set forth on the cover page of this prospectus, immediately prior to the completion of this offering;
- the conversion of outstanding warrants to purchase 1,602 shares of our Series A Prime convertible preferred stock into warrants to purchase shares of common stock immediately prior to the completion of this offering;
- no vesting or exercise of the outstanding stock options or warrants described above subsequent to , 2024; and
- no exercise by the underwriters of their option to purchase up to an additional shares of common stock in this offering.

Summary consolidated financial data

The following tables set forth our summary consolidated financial data for the years ended December 31, 2023 and 2022. We have derived the statement of operations and comprehensive loss data for the years ended December 31, 2023 and 2022 from our audited consolidated financial statements included elsewhere in this prospectus.

You should read the following summary consolidated financial data together with our consolidated financial statements and the related notes appearing elsewhere in this prospectus and the section of this prospectus titled “Management’s discussion and analysis of financial condition and results of operations.” The summary financial data in this section are not intended to replace our financial statements and are qualified in their entirety by our financial statements and related notes included elsewhere in this prospectus. Our historical results are not necessarily indicative of the results that should be expected in the future.

	Year ended December 31,	
(In thousands, except share and per share data)	**2023**	**2022**
Revenue:		
Research and collaboration revenue	$ 350	$ —
Operating Expenses:		
Research and development	40,616	34,771
General and Administrative	11,613	10,230
Total operating expenses	52,229	45,001
Loss from operations	(51,879)	(45,001)
Other income (expense), net:		
Interest income	2,808	904
Other (expense)	(220)	(95)
Total other income (expense), net	2,588	809
Net Loss	$ (49,291)	$ (44,192)
Net loss per share attributable to common stockholders, basic and diluted(1)	$ (11.13)	$ (12.61)
Weighted average shares of common stock outstanding, basic and diluted(1)	4,429,564	3,503,242
Pro forma net loss per share attributable to common stockholders, basic and diluted(2)	$ (0.33)	
Pro forma weighted average shares of common stock outstanding, basic and diluted(2)	135,079,592	

(1) See Note 2 to our financial statements appearing elsewhere in this prospectus for further details on the calculation of basic and diluted net loss per share attributable to common stockholders and the weighted average number of shares used in the computation of the per share amounts.

(2) The pro forma basic and diluted net loss per share for the year ended December 31, 2023 has been computed to give effect to the automatic conversion of all outstanding shares of our convertible preferred stock and convertible preferred stock warrants into shares of common stock. The unaudited pro forma basic and diluted net loss per share for the year ended December 31, 2023 was computed using the weighted average number of shares of common stock outstanding, including the pro forma effect of the conversion of all outstanding shares of our preferred stock and preferred stock warrants into shares of common stock, as if the conversion had occurred on the later of the first day of the period presented or the original issuance dates of the respective preferred stock.

(in thousands)	As of December 31, 2023 Actual	Pro forma(2)	Pro forma as adjusted(3)
Balance Sheet Data:			
Cash and cash equivalents	$ 38,380	$	$
Restricted cash	1,624		
Working capital(1)	32,206		
Total assets	54,946		
Convertible preferred stock	162,147		
Total stockholders' (deficit) equity	(123,730)		

(1) We define working capital as current assets less current liabilities. See our financial statements and the related notes included elsewhere in this prospectus for further details regarding our current assets and current liabilities.

(2) The pro forma balance sheet data give effect to (i) the automatic conversion of all outstanding shares of our convertible preferred stock and convertible preferred stock warrants into an aggregate of shares of our common stock, based on an assumed initial public offering price of $ per share, which is the midpoint of the price range set forth on the cover page of this prospectus, immediately prior to the completion of this offering, and (ii) the filing and effectiveness of our Restated Charter, which will be effective immediately prior to the completion of this offering.

(3) The pro forma as adjusted balance sheet data gives effect to (i) the pro forma adjustments described in footnote (2) above, and (ii) the issuance and sale of shares of our common stock offered in this offering at an assumed initial public offering price of $ per share, which is the midpoint of the price range set forth on the cover page of this prospectus, after deducting the estimated underwriting discounts and commissions and estimated offering expenses payable by us.

The pro forma and pro forma as adjusted information discussed above is illustrative only and will change based on the actual initial public offering price and other terms of this offering determined at pricing. A $1.00 increase or decrease in the assumed initial public offering price of $ per share, which is the midpoint of the price range set forth on the cover page of this prospectus, would increase or decrease, as applicable, the pro forma as adjusted amount of each of cash, working capital, total assets and total stockholders' (deficit) equity by approximately $ million, assuming that the number of shares offered by us, as set forth on the cover page of this prospectus, remains the same and after deducting the estimated underwriting discounts and commissions and estimated offering expenses payable by us. Similarly, each increase or decrease of 1,000,000 shares in the number of shares offered by us, as set forth on the cover page of this prospectus, would increase or decrease, as applicable, the pro forma as adjusted amount of each of cash, working capital, total assets and total stockholders' (deficit) equity by approximately $ million, assuming no change in the assumed initial public offering price per share and after deducting the estimated underwriting discounts and commissions and estimated offering expenses payable by us.

Risk factors

Investing in our common stock involves a high degree of risk. Before deciding to invest in our common stock, you should consider carefully the risks and uncertainties described below, together with all of the other information in this prospectus, including in the section titled "Management's discussion and analysis of financial condition and results of operations" and in our audited consolidated financial statements and related notes. The risks and uncertainties described below are not the only ones we face. Additional risks and uncertainties that we are unaware of, or that we currently believe are not material, may also become important factors that affect us. If any of the following risks are realized, our business, financial condition, results of operations and prospects could be materially and adversely affected. In that event, the price of our common stock could decline, and you could lose part or all of your investment.

Risks related to our financial position and need for additional capital

We have incurred significant losses since our inception, have no products approved for sale and we expect to incur losses for the foreseeable future.

We are a clinical-stage biopharmaceutical company in the early stages of development with a limited operating history. Since our inception, we have focused primarily on developing our proprietary RNA Actuating Platform, or RAP Platform, identifying, developing and progressing our product candidates through preclinical and clinical development, organizing and staffing our company, research and development activities, establishing and protecting our intellectual property portfolio, and raising capital. Investment in biopharmaceutical product development is highly speculative because it entails substantial upfront capital expenditures and significant risk that any potential product candidate will fail to demonstrate adequate effect or an acceptable safety profile, gain regulatory approval and become commercially viable. We are still in the early stages of development of our product candidates and our lead product candidate is only in a Phase 1 clinical trial. We have no products licensed for commercial sale and have not generated any revenue to date, and we continue to incur significant research and development and other expenses related to our ongoing operations. As a result, we are not profitable and have incurred losses in each period since our inception. For the years ended December 31, 2023 and 2022, we reported net losses of $49.3 million and $44.2 million, respectively. As of December 31, 2023, we had an accumulated deficit of $160.0 million. We expect to continue to incur significant losses for the foreseeable future, and we expect these losses to increase as we continue the research and development of, and seek regulatory approvals for, our lead product candidate CMP-CPS-001 for the treatment of urea cycle disorders, or UCDs, along with any other current or future product candidates we may develop.

We anticipate that our expenses will increase substantially if and as we:

- advance our lead product candidate, CMP-CPS-001, through clinical trials;
- finalize preclinical development for our programs for familial hypercholesterolemia, or FH, and SYNGAP1-related disorders;
- conduct preclinical studies and clinical trials of any future product candidates;
- expand the capabilities of our RAP Platform and seek to identify and develop additional product candidates;
- seek to identify additional product candidates;
- seek marketing approvals for any product candidates that successfully complete clinical trials;
- obtain, expand, maintain, defend and enforce our intellectual property portfolio;
- hire additional clinical, regulatory and scientific personnel;
- contract with manufacturing sources for preclinical and clinical development of any future product candidates we may develop and commercial supply with respect to any such product candidates that receive regulatory approval;

- ultimately establish a sales, marketing and distribution infrastructure to commercialize any products for which we may obtain marketing approval; and
- add operational, legal, compliance, financial and management information systems and personnel to support our research, product development and future commercialization efforts, as well as to support our operations as a public company.

Even if we obtain regulatory approval for, and are successful in commercializing, one or more of any of our current and any future product candidates, we will continue to incur substantial research and development and other costs to develop and market additional product candidates. We may encounter unforeseen expenses, difficulties, complications, delays and other unknown factors that may adversely affect our business. The size of our future net losses will depend, in part, on the rate of future growth of our expenses and our ability to generate revenue. Our prior losses and expected future losses have had and will continue to have an adverse effect on our stockholders' equity and working capital.

We have never generated revenue from product sales and may never achieve or maintain profitability.

Our product candidates are in varying stages of preclinical and clinical development. To date, we have not generated any revenue. We have not completed a clinical trial of any product candidate, and we expect that it will be several years, if ever, before we have a product candidate ready for commercialization. To become and remain profitable, we must succeed in developing, obtaining the necessary regulatory approvals for, and eventually commercializing a product or products that generate significant revenue. The ability to achieve this success will require us to be effective in a range of challenging activities, including:

- identifying product candidates and completing preclinical and clinical development of any product candidates we may identify;
- obtaining regulatory approval for any of our current or future product candidates;
- manufacturing, marketing and selling any products for which we may obtain regulatory approval;
- achieving market acceptance of any products for which we obtain regulatory approval as a viable treatment option; and
- satisfying any post-marketing requirements.

Many of the factors listed above are beyond our control, and could cause us to experience significant delays or prevent us from obtaining regulatory approvals or commercialize our product candidates. We are in the preliminary stages of many of these activities. We may never succeed in these activities and, even if we do, we may never generate revenues that are significant enough to achieve profitability. Because of the numerous risks and uncertainties associated with product development, we are unable to accurately estimate or know the nature, timing or costs of the efforts that will be necessary to complete the preclinical and clinical development and commercialization of any of our current or future product candidates or when, or if, we will be able to generate revenues or achieve profitability.

If we are successful in obtaining regulatory approval to market one or more of our products, our revenue will be dependent, in part, upon the size of the markets in the territories for which we gain regulatory approval, the accepted price for the product, the ability to obtain coverage and reimbursement, and whether we own the commercial rights for that territory. If the number of our addressable patients is not as significant as we estimate, the indication approved by regulatory authorities is narrower than we expect, or the treatment population is narrowed by competition, physician choice or treatment guidelines, we may not generate significant revenue from sales of such products, even if approved.

Even if we do achieve profitability, we may not be able to sustain or increase profitability on a quarterly or annual basis. Our failure to become and remain profitable could impair our ability to raise capital, maintain our research and development efforts, expand our business or even continue our operations. A decline in the value of our company could also cause our stockholders to lose all or part of their investment.

Even if this offering is successful, we will require substantial additional capital to finance our operations, and a failure to obtain this necessary capital when needed on acceptable terms, or at all, could force us to delay, limit, reduce, or terminate our development programs, commercialization efforts or other operations.

Our operations have consumed substantial amounts of cash since inception. We expect our expenses to substantially increase in connection with our ongoing activities, particularly as we conduct our ongoing and planned clinical trials and preclinical studies and potentially seek regulatory approval for our product candidates and any future product candidates we may develop. If we obtain regulatory approval for any of our product candidates, we also expect to incur significant commercialization expenses related to product manufacturing, marketing, sales, and distribution. Because the outcome of any clinical trial or preclinical study is highly uncertain, we cannot reasonably estimate the actual amount of capital necessary to successfully complete the development and commercialization of our product candidates. Furthermore, following the completion of this offering, we expect to incur additional costs associated with operating as a public company.

Based on our current operating plan, we believe that the net proceeds from this offering, together with our existing cash and cash equivalents, will be sufficient to fund our operating expenses and capital expenditure requirements into . We have based these estimates on assumptions that may prove to be wrong, and we could deplete our capital resources sooner than we currently expect. Our operating plans and other demands on our cash resources may change as a result of many factors currently unknown to us, and we may need to seek additional funds sooner than planned. The net proceeds of this offering, together with our existing capital, may not be sufficient to complete development of any of our product candidates, or any future product candidates we may identify, and after this offering, we will require substantial capital to advance our product candidates through clinical trials, regulatory approval, and commercialization. Accordingly, we will need to obtain substantial additional funding in connection with our continuing operations. Our ability to raise additional funds may be adversely impacted by global economic conditions, disruptions to, and volatility in, the credit and financial markets in the United States and worldwide, and diminished liquidity and credit availability. If the equity and credit markets deteriorate, it may make any necessary debt or equity financing more difficult, more costly, and more dilutive. We cannot be certain that additional funding will be available on acceptable terms, or at all. If we are unable to raise capital when needed or on attractive terms, we could be forced to delay, reduce, or eliminate our research and development programs or any future commercialization efforts, or even cease operations. We expect to finance our cash needs through public or private equity or debt financings or other capital sources, including potential collaborations, licenses, and other similar arrangements. In addition, we may seek additional capital due to favorable market conditions or strategic considerations even if we believe we have sufficient funds for our current or future operating plans. Attempting to secure additional financing may divert our management from our day-to-day activities, which may adversely affect our ability to develop our product candidates.

Our future capital requirements will depend on many factors, including, but not limited to:

- the scope, timing and progress of our ongoing CMP-CPS-001 clinical trial;
- the initiation, type, number, scope, progress, expansions, results, costs and timing of preclinical studies and clinical trials of our product candidates and any future product candidates we may choose to pursue, including the costs of modification to clinical development plans based on feedback that we may receive from regulatory authorities and any third-party products used as combination agents in our clinical trials;
- the costs and timing of manufacturing for our product candidates, including commercial manufacturing at sufficient scale, if any product candidate is approved;
- the costs, timing and outcome of regulatory meetings and reviews of our product candidates or any future product candidates, including requirements of regulatory authorities in any additional jurisdictions in which we may seek approval and any future product candidates;
- the costs of obtaining, maintaining, enforcing and protecting our patents and other intellectual property and proprietary rights;

- our efforts to enhance operational systems and hire additional personnel to satisfy our obligations as a public company, including enhanced internal control over financial reporting;
- the costs associated with hiring additional personnel and consultants as our clinical and preclinical activities increase and as we operate as a public company;
- the timing and payment of milestone, royalty or other payments we must make pursuant to our existing and potential future license or collaboration agreements with third parties;
- the costs and timing of establishing or securing sales and marketing capabilities if our product candidates or any product candidate is approved;
- our ability to achieve sufficient market acceptance, coverage, and adequate reimbursement from third-party payors and adequate market share and revenue for any approved products;
- patients' ability and willingness to pay out-of-pocket for any approved products in the absence of coverage and/or adequate reimbursement from third-party payors;
- the terms and timing of establishing and maintaining collaborations, licenses, and other similar arrangements; and
- costs associated with any products or technologies that we may in-license or acquire.

Conducting clinical trials and preclinical studies and discovering potential product candidates using our RAP Platform is an expensive and uncertain process, and we may never generate the necessary data or results required to obtain regulatory approval and commercialize our product candidates. In addition, our product candidates, if approved, may not achieve commercial success. Our commercial revenue, if any, will initially be derived from sales of products that we do not expect to be commercially available for many years, if at all.

Raising additional capital may cause dilution to our stockholders, restrict our operations or require us to relinquish rights to our technologies or product candidates.

Until such time, if ever, as we can generate substantial product revenues, we expect to finance our cash needs through a combination of equity offerings, debt financings, collaborations, strategic alliances and/or licensing arrangements. We do not have any committed external source of funds. To the extent that we raise additional capital through the sale of equity or convertible debt securities, our stockholders' ownership interests will be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect the rights of common stockholders. Any debt financing or preferred equity financing, if available, may involve agreements that include covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, selling or licensing our assets, making capital expenditures, declaring dividends or encumbering our assets to secure future indebtedness.

If we raise additional funds through collaborations, strategic alliances or licensing arrangements with third parties, we may have to relinquish valuable rights to our technologies, future revenue streams, research programs or product candidates or grant licenses on terms that may not be favorable to us. If we are unable to raise additional funds through equity or debt financings or other arrangements when needed or on terms acceptable to us, we may be required to delay, limit, reduce or eliminate some or all of our research and development programs, pipeline expansion or future commercialization efforts or grant rights to develop and market product candidates that we would otherwise prefer to develop and market ourselves.

Our independent registered public accounting firm has expressed substantial doubt about our ability to continue as a going concern.

Based on our current operating plans, we do not have sufficient cash and cash equivalents to fund our operating expenses and capital expenditures for at least the next 12 months from the filing date of this prospectus. In its report accompanying our audited financial statements for the years ended December 31, 2023 and 2022, our independent registered public accounting firm included an explanatory paragraph stating that our recurring losses

from operation raise substantial doubt about our ability to continue as a going concern. Our future viability is dependent on our ability to generate cash from our operating activities or to raise additional capital to finance our operations. There is no assurance that we will succeed in obtaining sufficient funding on terms acceptable to us to fund continuing operations, if at all. The perception that we might be unable to continue as a going concern may also make it more difficult to obtain financing for the continuation of our operations on terms that are favorable to us, or at all, and could result in the loss of confidence by investors, suppliers and employees. If we are unable to continue as a going concern, we may have to liquidate our assets and may receive less than the value at which those assets are carried on our financial statements and it is likely that our investors will lose all or a part of their investment.

Risks related to the research and development of our product candidates

We are early in our development efforts. Our product candidates are in varying stages of preclinical and clinical development and we have not completed a clinical trial of any product candidate. As a result, it will be many years before we commercialize a product candidate, if ever. If we are unable to identify and advance product candidates through preclinical studies and clinical trials, obtain marketing approval and ultimately commercialize them, or experience significant delays in doing so, our business will be materially harmed.

We are early in our development efforts and our lead product candidate is only in a Phase 1 clinical trial. We have focused our efforts to date on developing our RAP Platform, identifying our programs and commencing the preclinical and clinical development of our product candidates. Our ability to generate product revenue, which we do not expect will occur for many years, if ever, will depend heavily on the successful development and eventual commercialization of our product candidates, which may never occur. We currently generate no revenue from sales of any product, and we may never be able to develop or commercialize a marketable product.

We are currently conducting a Phase 1 clinical trial of CMP-CPS-001 in Australia. Clinical trials conducted in Australia using “unapproved therapeutic goods,” or those that have not yet been evaluated by the Therapeutic Goods Association, or TGA, for quality, safety and efficacy, must occur pursuant to either the Clinical Trial Notification Scheme or the Clinical Trial Approval Scheme. In each case, the trial is supervised by a Human Research Ethics Committee, or HREC, an independent review committee set up under the guidelines of the Australian National Health and Medical Research Council that reviews, approves and provides continuing oversight of trial protocols and amendments, and of the methods and material to be used in obtaining and documenting informed consent of the trial subjects. Commencing clinical trials in the United States is subject to acceptance by the U.S. Food and Drug Administration, or the FDA, of an investigational new drug, or IND, application and finalizing the trial design based on discussions with the FDA and other regulatory authorities. In the event that the FDA or TGA requires us to complete additional preclinical studies or we are required to satisfy other requests prior to commencing clinical trials, the start of any future clinical trials may be delayed. Even after we receive and incorporate guidance from the FDA, an applicable HREC or the TGA, such authorities could disagree that we have satisfied their requirements to commence any clinical trial or continue or change their position on the acceptability of our trial design or the clinical endpoints selected, which may require us to complete additional preclinical studies or clinical trials or impose stricter approval conditions than we currently expect, which could delay the start or completion of such clinical trials or require more capital resources than we currently anticipate to start or complete such clinical trials.

We anticipate that for one or more of our product candidates, clinical trials will need to be conducted utilizing sites and patients in the European Union and the United Kingdom. Similar processes and risks are applicable to clinical trial applications, or CTAs, in the European Union as well as the United Kingdom as exist in other regions. Regulators for the European Union and/or for local countries may request additional preclinical studies or may reject the request to initiate clinical trials in humans. Requests for additional preclinical studies prior to commencing clinical trials may result in the delay of future clinical trials. Even after we receive and incorporate guidance from EU and/or local country regulators, the regulatory authorities may disagree with our position that we have satisfied their requirements, require additional preclinical studies or clinical trials, or refuse to approve the product candidate.

Commercialization of any of our current or future product candidates will require preclinical and clinical development; regulatory and marketing approval issued by regulators in any jurisdiction where we seek to commercialize such product candidates, such as the FDA, TGA and the European Commission, or EC, following a favorable assessment performed by the European Medicines Agency, or EMA; manufacturing supply, capacity and expertise; a commercial organization; and significant marketing efforts. The success of any of our current or future product candidates will depend on many factors, including the following:

- timely and successful completion of preclinical studies;
- acceptance of INDs or comparable foreign applications that allow commencement of clinical trials or future clinical trials for any product candidates we may develop;
- successful enrollment and completion of clinical trials, including under the FDA's current Good Clinical Practices, or GCPs, current Good Laboratory Practices, or GLPs, and any additional regulatory requirements from foreign regulatory authorities;
- positive results from our clinical trials that support a finding of safety and effectiveness and an acceptable risk-benefit profile in the intended populations;
- receipt of marketing approvals from applicable regulatory authorities;
- establishment of arrangements through our own facilities or with third-party manufacturers for clinical supply and, where applicable, commercial manufacturing capabilities;
- establishment, maintenance, defense and enforcement of patent, trademark, trade secret and other intellectual property protection or regulatory exclusivity for any product candidates we may develop;
- commercial launch of any product candidates we may develop, if approved, whether alone or in collaboration with others;
- obtaining and maintaining third-party coverage and adequate reimbursement;
- effectively competing against other therapies;
- acceptance of the benefits and use of our product candidates we may develop, including method of administration, if and when approved, by patients the medical community and third-party payors; and
- maintaining a continued acceptable safety profile of our products following regulatory approval.

If we do not succeed in one or more of these factors in a timely manner or at all, we could experience significant delays or an inability to successfully commercialize any product candidates we may develop, which would materially harm our business. If we are unable to advance our product candidates into and through clinical development, obtain regulatory approval and ultimately commercialize our product candidates, or experience significant delays in doing so, our business will be materially harmed.

Our business is highly dependent on our lead product candidate, CMP-CPS-001, as our sole clinical-stage program, and we must complete clinical testing before we can seek regulatory approval and begin commercialization of any of our other product candidates. If we are unable to obtain regulatory approval for, and successfully commercialize, CMP-CPS-001, our business may be materially harmed and such failure may affect the viability of our other product candidates.

There is no guarantee that any of our product candidates will proceed in preclinical or clinical development or achieve regulatory approval. The process for obtaining marketing approval for any product candidate is very long and risky and there will be significant challenges for us to address in order to obtain marketing approval as planned, if at all.

There is no guarantee that the results obtained in our ongoing Phase 1 clinical trial of CMP-CPS-001 or our planned future clinical trials will be sufficient to obtain regulatory approval. In addition, because CMP-CPS-001 is our most advanced product candidate, and because our future product candidates will be based on our RAP Platform and antisense oligonucleotide, or ASO, technology, if our lead product candidate encounters safety or

efficacy problems, developmental delays, regulatory issues, or other problems, our development plans and business related to our other future product candidates could be significantly harmed. A failure of our lead product candidate may affect the ability to obtain regulatory approval to continue or conduct clinical programs for our other or future product candidates.

Our approach to the discovery and development of product candidates based on our RAP Platform is unproven, and we may not be successful in our efforts to develop and commercialize our product candidates and to identify and discover additional potential product candidates.

The success of our business depends upon our ability to identify, develop and commercialize products based on our proprietary RAP Platform. All of our product candidates are still in varying stages of preclinical and clinical development. Our research programs may fail to identify additional product candidates for clinical development for a number of reasons. Our RAP Platform may be unsuccessful in identifying additional potential product candidates and our potential product candidates may be shown to have harmful side effects. In addition, our product candidates may not show promising signals of therapeutic effect in such experiments or studies or they may have other characteristics that may make the product candidates impractical to manufacture, unmarketable or unlikely to receive marketing approval. Further, because all of our product candidates and programs are based on our RAP Platform, adverse developments with respect to one of our product candidates and programs may have a significant adverse impact on the actual or perceived likelihood of success and value of our other product candidates and programs.

In addition, we have not completed a clinical trial of any product candidate or successfully developed any product candidates, and our ability to identify and develop additional product candidates may never materialize. The process by which we identify and develop product candidates may fail to yield additional product candidates for clinical development for a number of reasons, including those discussed in these risk factors. In addition:

- we may not be able to assemble sufficient resources to acquire or discover product candidates;
- competitors may develop alternatives that render our product candidates obsolete or less attractive;
- product candidates we develop may nevertheless be covered by third parties' patents or other intellectual property rights;
- product candidates may, on further study, be shown to have harmful side effects, toxicities or other characteristics that indicate that they are unlikely to be products that will receive marketing approval and achieve market acceptance;
- product candidates may not be effective in treating their targeted diseases or disorders;
- the market for a product candidate may change so that the continued development of that product candidate is no longer reasonable;
- a product candidate may not be capable of being produced in commercial quantities at an acceptable cost, or at all; or
- the regulatory pathway for a product candidate may be too complex and difficult to navigate successfully or economically.

If we are unable to identify and discover suitable product candidates for clinical development, this would adversely impact our business strategy and our financial position and share price and could potentially cause us to cease operations.

Drug development is a lengthy and expensive process, and preclinical and clinical testing is uncertain as to the outcome. We may encounter substantial delays in the commencement, enrollment or completion of our clinical trials, or we may fail to demonstrate safety and effectiveness to the satisfaction of applicable regulatory authorities, which could prevent us from advancing or commercializing our product candidates on a timely basis, if at all.

The risk of failure in developing therapeutic product candidates is high. This elevated risk exists even when preclinical studies in animal models demonstrate positive data. It is impossible to predict when or if any product

candidate would prove effective or safe in humans or will receive regulatory approval. Before obtaining marketing approval from regulatory authorities for the sale of any product candidate, we must complete preclinical development, obtain regulatory authorization to commence clinical trials, and then conduct extensive clinical trials to demonstrate the safety and efficacy of product candidates in humans.

Clinical trials may fail to demonstrate that our product candidates are safe for humans and effective for indicated uses, and earlier results, both preclinical and clinical, may not be indicative of future clinical trial results. Even if the clinical trials are successful, changes in marketing approval policies during the development period, changes in or the enactment or promulgation of additional statutes, regulations or guidance, varying interpretations of clinical data or changes in regulatory review for each submitted product application may cause delays in the approval or rejection of an application.

Before we can commence clinical trials for a product candidate, we must complete extensive preclinical testing and studies that support clearance of our regulatory filings, including IND applications to the FDA in the United States and other similar regulatory filings in other jurisdictions, including with respect to the TGA in Australia and the national competent authorities, or NCAs, in the European Union. We cannot be certain if the outcome of our preclinical studies and clinical trials will ultimately support further development of our product candidates or future programs or whether the FDA, TGA, NCAs or comparable foreign regulatory authorities will accept our proposed clinical programs or whether the outcome of our preclinical testing and studies will ultimately support the further development of our product candidates. Conducting preclinical testing is a lengthy, time-consuming and expensive process. The length of time may vary substantially according to the type, complexity and novelty of the program, and often can be several years or more per program. As a result, we cannot be sure that we will be able to submit INDs, CTAs and other similar regulatory filings for our preclinical programs on the timelines we expect, if at all, and we cannot be sure that submission of such regulatory filings will result in the FDA, TGA, NCAs or comparable foreign regulatory authorities allowing clinical trials to begin.

Clinical testing is expensive, is difficult to design and implement, can take many years to complete and is uncertain as to outcome. We cannot guarantee that any clinical trials will be conducted as planned or completed on schedule, or at all. A failure of one or more clinical trials can occur at any stage of testing, which may result from a multitude of factors, including, but not limited to, flaws in trial design, dose selection issues, patient enrollment criteria, operational challenges, site implementation challenges, biostatistical plans, and failure to demonstrate favorable safety or efficacy traits.

Other events that may prevent successful or timely completion of clinical development include:

- delays in reaching a consensus with regulatory authorities on trial design;
- delays in reaching agreement on acceptable terms with prospective clinical research organizations, or CROs, and clinical trial sites;
- delays in opening clinical trial sites or obtaining required approval from institutional review boards, or IRBs, HRECs or independent ethics committees, or the equivalent review groups for sites outside the United States or Australia, at each clinical trial site;
- imposition of a clinical hold by regulatory authorities as a result of a serious adverse event or manufacturing concerns or after an inspection of our clinical trial operations or trial sites;
- negative or inconclusive results observed in clinical trials, including failure to demonstrate statistical significance, which could lead us, or cause regulators to require us, to conduct additional clinical trials or abandon product development programs;
- failure by us, any CROs we engage or any other third parties to adhere to clinical trial requirements;
- failure to perform in accordance with the FDA's GCPs, Good Manufacturing Practices, or GMP, regulations or those of other regulatory authorities, including, but not limited to, Australia's GMP requirements;
- failure by physicians to adhere to delivery protocols, leading to protocol deviations and variable results;

- inappropriate storage or failure of storage facilities or storage equipment of preclinical or clinical trial samples;
- delays in the testing, validation, manufacturing and delivery of our product candidates to the clinical sites, including delays by third parties with whom we have contracted to perform certain of those functions;
- failure of our third-party contractors to comply with regulatory requirements or to meet their contractual obligations to us in a timely manner, or at all;
- inability to recruit patients to participate in a clinical trial, including as a result of competition with other pharmaceutical and biotechnology companies and the patient population size for our product candidates;
- delays in having patients complete participation in a clinical trial or return for post-treatment follow-up;
- clinical trial sites or patients dropping out of a trial;
- selection of clinical endpoints that require prolonged periods of clinical observation or analysis of the resulting data, or clinical endpoints that have broad variability or inconsistency, resulting in negative or indeterminable results;
- occurrence of serious adverse events associated with a product candidate in development by another company, which are viewed to outweigh its potential benefits, and which may negatively impact the perception of our current or future product candidates due to a similarity in technology or approach;
- changes in regulatory requirements and guidance that require amending or submitting new clinical protocols;
- changes in the legal or regulatory regimes domestically or internationally related to patient rights and privacy;
- lack of adequate funding to continue the clinical trial; or
- lack of diminished revenue potential of the programs due to competition.

Clinical trials must be conducted in accordance with the legal requirements, regulations or guidelines of the FDA, TGA, EC, NCAs and other applicable regulatory authorities, and are subject to oversight by these governmental agencies and IRBs, HRECs or ethics committees at the medical institutions where the clinical trials are conducted. We could encounter delays if a clinical trial is suspended or terminated by us, by the data safety monitoring board for such trial or by the FDA, TGA, EMA or any other regulatory authority, or if the IRBs or HRECs of the institutions in which such trials are being conducted suspend or terminate the participation of their clinical investigators and sites subject to their review. Such authorities may suspend or terminate a clinical trial due to a number of factors, including failure to conduct the clinical trial in accordance with regulatory requirements or our clinical protocols, inspection of the clinical trial operations or trial site by the FDA, TGA, EMA or other regulatory authorities resulting in the imposition of a clinical hold, unforeseen safety issues or adverse side effects, failure to demonstrate a benefit from using a product candidate, changes in governmental regulations or administrative actions or lack of adequate funding to continue the clinical trial.

In addition, we may face challenges associated with clinical testing in pediatric populations, which we currently intend to pursue with respect to CMP-CPS-001, and which could increase our clinical development timelines and operational costs, delay regulatory approval and commercialization for such pediatric indications or expose us to additional liability. For example, finding qualified clinical sites that have access to sufficient pediatric populations and that are willing to participate in our clinical trials may take more time than would be required for the assessment of CMP-CPS-001 in adult patient populations. There may be fewer eligible pediatric patients with the UCD enzyme deficiencies we are targeting for the development of CMP-CPS-001, or with conditions applicable to other product candidates we may develop and assess in future clinical trials. We may also be required to modify the formulation or other aspects of our product candidate, as compared to the comparable product candidate intended for adult patient populations, make manufacturing changes, modify the route of administration and conduct additional clinical trials, such as bridging studies and additional safety studies, before we can commence

our clinical trials in pediatric populations. The FDA or other comparable regulatory authorities may require us to complete studies in adults prior to initiating testing in children. Any delays in our planned clinical development activities for pediatric patients could have an adverse effect on our business operations.

Moreover, principal investigators for our clinical trials may also serve as our scientific advisors or consultants to us from time to time and receive compensation in connection with such services. Under certain circumstances, we may be required to report some of these relationships to the FDA, TGA, EC, NCAs or comparable foreign regulatory authorities. The FDA, TGA, NCAs or comparable foreign regulatory authority may conclude that a financial relationship between us and a principal investigator has created a conflict of interest or otherwise affected interpretation of the trial. The FDA, TGA, EMA or comparable foreign regulatory authority may therefore question the integrity of the data generated at the applicable clinical trial site and the utility of the clinical trial itself may be jeopardized. This could result in a delay in approval, or rejection, of our marketing applications by the FDA, TGA, EMA or comparable foreign regulatory authority, as the case may be, and may ultimately lead to the denial of marketing approval of our product candidates.

Any inability to successfully complete preclinical studies and clinical trials could result in additional costs to us or preclude or impair our ability to generate revenues from product sales, regulatory and commercialization milestones and royalties. In addition, if we make manufacturing or formulation changes to our product candidates, we may need to conduct additional studies to bridge our modified product candidates to earlier versions. Clinical trial delays also could shorten any periods during which we may have the exclusive right to commercialize our product candidates or allow our competitors to bring products to market before we do, which could impair our ability to successfully commercialize our product candidates and may harm our business, financial condition, results of operations and prospects.

Further, conducting clinical trials in foreign countries, such as our ongoing Phase 1 clinical trial of CMP-CPS-001 for the treatment of UCDs, which is being conducted in Australia, presents additional risks that may delay completion of our clinical trials. These risks include the failure of enrolled patients in foreign countries to adhere to clinical protocol as a result of differences in healthcare services or cultural customs, managing additional administrative burdens associated with foreign regulatory schemes, as well as political and economic risks relevant to such foreign countries.

Additionally, if the results of clinical trials are inconclusive or if there are safety concerns or serious adverse events associated with our product candidates, we may:

- be delayed in obtaining marketing approval for product candidates, if at all;
- obtain approval for indications or patient populations that are not as broad as intended or desired;
- obtain approval with labeling that includes significant use or distribution restrictions or safety warnings;
- be subject to changes in the way our product candidates are administered;
- be required to perform additional clinical trials to support approval or be subject to additional post-marketing testing requirements;
- have regulatory authorities withdraw, or suspend, their approval of the product or impose restrictions on its distribution in the form of a Risk Evaluation and Mitigation Strategy, or REMS;
- be subject to the addition of labeling statements, such as warnings or contraindications;
- be subject to litigation; or
- experience damage to our reputation.

Any of these events could prevent us from achieving or maintaining regulatory approval or market acceptance of our product candidates or could substantially increase commercialization costs and expenses, which in turn could delay or prevent us from generating significant revenue from the sale of our product candidates, if approved.

Interim, topline, and preliminary data from our clinical trials and preclinical studies that we announce or publish from time to time may change as more patient data become available and are subject to audit and verification procedures that could result in material changes in the final data.

From time to time, we may publicly disclose interim, topline, or preliminary data from our clinical trials and preclinical studies, which is based on a preliminary analysis of then-available data, and the results and related findings and conclusions are subject to change following quality assurance, audit, and/or a more comprehensive review of the data related to the particular study or trial. We also make assumptions, estimations, calculations, and conclusions as part of our analyses of data, and we may not have received or had the opportunity to fully and carefully evaluate all data. As a result, the interim, topline, or preliminary results that we report may differ from future results of the same studies or trials, or different conclusions or considerations may qualify such results once additional data have been received and fully evaluated. Topline and preliminary data also remain subject to audit and verification procedures that may result, in the final data being materially different from the topline or preliminary data we previously published. As a result, topline and preliminary data should be viewed with caution until the final data are available. Interim data from clinical trials that we may complete are further subject to the risk that one or more of the clinical outcomes may materially change as patient enrollment continues and more patient data become available. Adverse differences between interim, topline, or preliminary data and final data could significantly harm our business prospects.

In addition, others, including regulatory authorities, may not accept or agree with our assumptions, estimates, calculations, conclusions, or analyses or may interpret or weigh the importance of data differently, which could impact the value of the particular program, the approvability or commercialization of particular product candidate and our company in general. Moreover, the information we choose to publicly disclose regarding a particular study or clinical trial is based on what is typically extensive information, and you or others may not agree with what we determine is material or otherwise appropriate information to include in our disclosure, and any information we determine not to disclose may ultimately be deemed significant with respect to future decisions, conclusions, views, activities or otherwise regarding a particular product candidate or our business. If the interim, topline, or preliminary data that we report differ from actual results, or if others, including regulatory authorities, disagree with the conclusions reached, our ability to obtain approval for, and commercialize our product candidates may be harmed, which could harm our business, operating results, prospects, or financial condition.

The outcome of preclinical studies and earlier-stage clinical trials may not be predictive of future results or the success of later preclinical studies and clinical trials.

We are in the early stages of development of our programs and have initiated a Phase 1 clinical trial of our lead product candidate, CMP-CPS-001, in healthy adult volunteers in Australia, but we have not yet completed or received clearance for IND- or CTA-enabling activities for our other product candidates or advanced any other product candidates into clinical development. As a result, our belief in the capabilities of our platform and potential success of our product candidates is based on early research and preclinical studies. However, the results of preclinical studies may not be predictive of the results of later preclinical studies or clinical trials, and the results of any early-stage clinical trials may not be predictive of the results of later clinical trials. In addition, initial success in clinical trials may not be indicative of results obtained when such trials are completed. Moreover, preclinical and clinical data are often susceptible to varying interpretations and analyses, and many companies that have believed their product candidates performed satisfactorily in preclinical studies and clinical trials have nonetheless failed to obtain marketing approval of their product candidates. Our clinical trials may not ultimately be successful or support further clinical development of our product candidates.

Our choices with respect to the design and implementation of our clinical trials will be a significant factor in our ability to successfully and timely complete clinical development with respect to our product candidates. Our Phase 1 clinical trial being conducted in Australia for CMP-CPS-001 utilizes a ureagenesis rate test, or URT, which is an assay that evaluates flux through the urea cycle based on the rate at which an isotope is converted into labeled urea. The assay can be used to measure baseline and post-treatment urea rates and was previously shown to be able to measure ureagenesis in normal healthy volunteer studies and ureagenesis increases in specific UCD patient subtypes using carglumic acid. More specifically, Carbaglu, approved for ultra-rare NAGS-deficient patients,

utilized the URT in healthy volunteers and showed that minimal increases in ureagenesis translated to substantial ammonia reductions in NAGS-deficient patients. Although URTs have experienced expanded use in research and clinical studies and have been shown to correlate with responses in patients, making them a valuable pharmacodynamic tool, they are not an established clinical endpoint and not routinely used for clinical care. As such, it is possible that variability in the results of the assay could render interpretation difficult. While we believe that an increase in ureagenesis as measured by the URT in our Phase 1 clinical trial may correspond with clinically meaningful improvements in ammonia metabolism in UCD patients, there is no guarantee that an increase in ^{13}C-sodium acetate metabolism, as measured by the URT, will correlate to an increase in ammonia metabolism, or that such data will be predictive of positive results with respect to the established clinical endpoints that we expect to use in our later-stage clinical trials, and our use of the URT to measure changes in ureagenesis in our Phase 1 clinical trial should not be interpreted as evidence of the efficacy of CMP-CPS-001.

There is a high failure rate for product candidates proceeding through clinical trials. A number of companies in the pharmaceutical and biotechnology industries have suffered significant setbacks in clinical development even after achieving encouraging results in earlier studies. Any such setbacks in our clinical development could materially harm our business and results of operations.

Additionally, some or all of our planned clinical trials may utilize an “open-label” trial design. An “open-label” clinical trial is one where both the patient and investigator know whether the patient is receiving the investigational product candidate or either an existing approved drug or placebo. Most typically, open-label clinical trials test only the investigational product candidate and sometimes may do so at different dose levels. Open-label clinical trials are subject to various limitations that may exaggerate any therapeutic effect as patients in open-label clinical trials are aware when they are receiving treatment. Open-label clinical trials may be subject to a “patient bias” where patients perceive their symptoms to have improved merely due to their awareness of receiving an experimental treatment. In addition, open-label clinical trials may be subject to an “investigator bias” where those assessing and reviewing the physiological outcomes of the clinical trials are aware of which patients have received treatment and may interpret the information of the treated group more favorably given this knowledge. The results from an open-label trial may not be predictive of future clinical trial results with any of our product candidates when studied in a controlled environment with a placebo or active control.

Additionally, some of our planned clinical trials may utilize a “placebo” and/or blinded clinical trial design. A placebo controlled clinical trial is one where both the participant and the investigator may and/or should not know whether the participants have received the product candidate or placebo. In studies utilizing placebo and/or blinded control, there exists the phenomenon of “placebo response” where participants assigned to the placebo may experience a benefit given their participation in the study. This placebo response in the control group at times may limit or prevent the detection of a numerical and/or a statistical difference between the treatment group and the placebo group.

Certain of the disorders we seek to treat, including UCDs and SYNGAP1-related disorders, have low prevalence and it may be difficult to identify and enroll patients with these disorders. If we experience delays or difficulties in the enrollment and/or maintenance of patients in clinical trials, our receipt of necessary regulatory approvals could be delayed or prevented.

Identifying and qualifying patients to participate in clinical trials of any of our current or future product candidates is critical to our success. Patient enrollment, a significant factor in the timing of clinical trials, is affected by many factors, including the size and nature of the patient population and competition for patients with other trials. Genetic diseases generally, and especially the rare diseases for which some of our current and any future product candidates are targeted, have low incidence and prevalence. For example, the incidence of UCDs in the United States is estimated to be approximately 1 in 35,000 births, with similar incidence estimated for Europe, and accordingly it may be difficult for us to identify and timely recruit a sufficient number of eligible patients to conduct our clinical trials. Further, the pediatric population is an important patient population for CMP-CPS-001 and our addressable patient population estimates include pediatric populations. However, it may be more challenging to conduct studies in this population, and to locate and enroll pediatric patients. Additionally, it may be challenging to ensure that pediatric or adolescent patients adhere to clinical trial protocols. We may not be able

to identify, recruit and enroll a sufficient number of patients, or those with required or desired characteristics, to complete our clinical trials in a timely manner. Patient enrollment and trial completion is affected by factors including:

- size of the patient population, in particular for rare diseases, including the diseases on which we are initially focused, and the process for identifying patients and screening patients;
- design of the trial protocol;
- eligibility and exclusion criteria;
- perceived risks and benefits of the product candidate under study;
- availability of competing therapies and clinical trials;
- severity of the disease or disorder under investigation;
- proximity and availability of clinical trial sites for prospective patients;
- ability to obtain and maintain patient consent;
- risk that enrolled patients will drop out before completion of the trial;
- patient referral practices of physicians; and
- ability to monitor patients adequately during and after treatment.

Our inability to enroll a sufficient number of patients for clinical trials would result in significant delays and could require us to abandon one or more clinical trials altogether. Enrollment delays in these clinical trials may result in increased development costs for our product candidates, which would cause the value of our company to decline and limit our ability to obtain additional financing. Furthermore, we rely on and expect to continue to rely on CROs and clinical trial sites to ensure the proper and timely conduct of our clinical trials and we will have limited influence over their performance.

Furthermore, certain of our planned clinical trials may utilize a “placebo” and/or blinded clinical trial design, which may in some situations cause additional enrollment difficulty. In clinical trials of patients, patients may decline to enroll out of concern of being assigned into the placebo group. This concern may be higher in rare diseases and may increase if other treatments become available to patients during the clinical trial or clinical development.

Even if we are able to enroll a sufficient number of patients for our clinical trials, we may have difficulty maintaining patients in our clinical trials. For example, patients who end up receiving placebo may perceive that they are not receiving the product candidate being tested, and they may decide to withdraw from our clinical trials to pursue other alternative therapies rather than continue the trial with the perception that they are receiving placebo. If we have difficulty enrolling or maintaining a sufficient number of patients to conduct our clinical trials, we may need to delay, limit or terminate clinical trials, any of which would harm our business, financial condition, results of operations and prospects.

If any of our current or any future product candidates cause undesirable side effects or have other unexpected adverse properties, such side effects or properties could delay or prevent regulatory approval, limit the commercial potential or result in significant negative consequences following any potential marketing approval.

We have not completed a clinical trial of any product candidate. It is impossible to predict when or if any of our current or future product candidates will prove safe in humans. There can be no assurance that our product candidates will not cause undesirable side effects.

Although other ASOs have received regulatory approval, no regulatory authorities to date have approved ASOs that are directed towards the type of RNA (regulatory RNAs) that our product candidates target. As a result, there is uncertainty as to the safety profile of any of our current or future product candidates compared to currently approved ASOs.

If any product candidates we develop are associated with serious adverse events, undesirable side effects or unexpected characteristics, we may need to abandon their development or limit development to certain uses or subpopulations in which the serious adverse events, undesirable side effects or other characteristics are less prevalent, less severe or more acceptable from a risk-benefit perspective, any of which would have a material adverse effect on our business, financial condition, results of operations and prospects. Many product candidates that initially showed promise in early-stage testing have later been found to cause side effects that prevented further clinical development of the product candidates.

If in the future we are unable to demonstrate that such side effects were caused by factors other than our product candidates, the FDA, the TGA, EC, NCAs or other regulatory authorities could order us to cease further development of, or deny approval of, any product candidates for any or all targeted indications. Even if we are able to demonstrate that any future serious adverse events are not product-related and regulatory authorities do not order us to cease further development of our product candidates, such occurrences could affect patient recruitment or the ability of enrolled patients to complete the trial. Moreover, if we elect, or are required, to delay, suspend or terminate any clinical trial of any product candidate, the commercial prospects of such product candidates may be harmed and our ability to generate product revenues from any of these product candidates may be delayed or eliminated. Any of these occurrences may harm our ability to develop other product candidates, and may harm our business, financial condition and prospects significantly.

We may develop CMP-FH, and potentially future product candidates, in combination with other therapies, which exposes us to additional risks.

We may develop CMP-FH, and may develop future product candidates, for use in combination with one or more currently approved therapies. Even if any product candidate we develop was to receive marketing approval or be commercialized for use in combination with other existing therapies, we would continue to be subject to the risks that the FDA or similar foreign regulatory authorities could revoke approval of the therapy used in combination with our product candidate or that safety, efficacy, manufacturing or supply issues could arise with these existing therapies. This could result in our own products being less successful commercially.

If the FDA or similar foreign regulatory authorities do not approve these other drugs or revoke their approval of, or if safety, efficacy, manufacturing, or supply issues arise with, the drugs we choose to evaluate in combination with CMP-FH or any product candidate we develop, we may be unable to obtain approval of or market CMP-FH or any product candidate we develop.

We may expend our limited resources to pursue a particular program, product candidate or indication and fail to capitalize on programs, product candidates or indications that may be more profitable or for which there is a greater likelihood of success.

Because we have limited financial and managerial resources, we focus on research programs and product candidates that we identify for specific indications that we believe can be addressed by our technology among many potential options. As a result, we may forego or delay pursuit of opportunities with other product candidates or for other indications that later prove to have greater commercial potential, or we may choose to focus our efforts and resources on a potential product candidate that ultimately proves to be unsuccessful. Our resource allocation decisions may cause us to fail to capitalize on viable commercial products or profitable market opportunities. Our spending on current and future research and development programs and product candidates for specific indications may not yield any commercially viable products. If we do not accurately evaluate the commercial potential or target market for a particular product candidate, we may relinquish valuable rights to that product candidate through collaboration, licensing or other royalty arrangements in cases in which it would have been more advantageous for us to retain sole development and commercialization rights to such product candidate. Any such event could have a material adverse effect on our business, financial condition, results of operations and prospects.

We are conducting and intend to conduct certain of our clinical trials globally. However, the FDA and other foreign equivalents may not accept data from such trials, in which case our development plans will be delayed, which could materially harm our business.

We intend to continue conducting certain of our clinical trials globally. The acceptance by the FDA or other regulatory authorities of study data from clinical trials conducted outside their jurisdiction may be subject to certain conditions or may not be accepted at all. In cases where data from foreign clinical trials are intended to serve as the sole basis for marketing approval in the United States, the FDA will generally not approve the application on the basis of foreign data alone unless (i) the data are applicable to the U.S. population and U.S. medical practice; (ii) the trials were performed by clinical investigators of recognized competence and pursuant to GCP regulations; and (iii) the data may be considered valid without the need for an on-site inspection by the FDA, or if the FDA considers such inspection to be necessary, the FDA is able to validate the data through an on-site inspection or other appropriate means.

In addition, even where the foreign study data are not intended to serve as the sole basis for approval, the FDA will not accept the data as support for an application for marketing approval unless the clinical trial is well-designed and well-conducted in accordance with GCP requirements and the FDA is able to validate the data from the trial through an onsite inspection if deemed necessary. Many foreign regulatory authorities have similar approval requirements. In addition, such foreign trials would be subject to the applicable local laws of the foreign jurisdictions where the trials are conducted. There can be no assurance that the FDA or any comparable foreign regulatory authority will accept data from trials conducted outside of the United States or the applicable jurisdiction. If the FDA or any comparable foreign regulatory authority does not accept such data, it would result in the need for additional trials, which could be costly and time-consuming, and which may result in current or any future product candidates that we may develop not receiving approval for commercialization in the applicable jurisdiction.

Conducting clinical trials outside the United States also exposes us to additional risks, including risks associated with additional foreign regulatory requirements; foreign exchange fluctuations; compliance with foreign manufacturing, customs, shipment and storage requirements; cultural differences in medical practice and clinical research; diminished protection of intellectual property in some countries; and interruptions or delays in our trials resulting from geopolitical events, such as war or terrorism.

Changes in the methods of manufacturing or formulation of our product candidates may result in additional costs or delay.

As our product candidates progress through clinical trials to regulatory approval and commercialization, it is common that various aspects of the development program, such as manufacturing methods and formulation, may be altered along the way in an effort to optimize safety, efficacy, yield, and manufacturing batch size, minimize costs, and achieve consistent quality and results. There can be no assurance that any future manufacturing or formulation changes will achieve their intended objectives. These changes and any future changes we may make to our product candidates may also cause such candidates to perform differently and affect the results of future clinical trials conducted with the altered materials. Such changes or related unfavorable clinical trial results could delay initiation or completion of additional clinical trials, require the conduct of bridging studies or clinical trials or the repetition of one or more studies or clinical trials, increase development costs, delay or prevent potential regulatory approval, and jeopardize our ability to commercialize our product candidates, if approved, and generate revenue.

If the market opportunities for any product candidates we develop are smaller than we believe they are, our revenue may be adversely affected, and our business may suffer. Because the target patient populations of certain of our programs are small, and the addressable patient population even smaller, we must be able to successfully identify patients and capture a significant market share to achieve profitability and growth.

Certain of our research and product development initiatives are focused on treatments for rare diseases. Given the small number of patients who have the diseases that we are initially targeting, including UCDs and SYNGAP1-related disorders, it is critical to our ability to grow and become profitable that we continue to successfully

identify patients with these rare diseases. Our projections of both the number of people who have these diseases, as well as the subset of people with these diseases who have the potential to benefit from treatment with any product candidates we may develop, are based on our beliefs and estimates. These estimates have been derived from a variety of sources, including the scientific literature, surveys of clinics, patient foundations or market research that we conducted, and may prove to be incorrect or contain errors. New studies may change the estimated incidence or prevalence of these diseases. The number of patients may turn out to be lower than expected. The effort to identify patients with diseases we seek to treat is in early stages, and we cannot accurately predict the number of patients for whom treatment might be possible. Additionally, the potentially addressable patient population for each of our product candidates may be limited or may not be amenable to treatment with our product candidates, and new patients may become increasingly difficult to identify or gain access to, which would adversely affect our results of operations and our business. Further, even if we obtain significant market share for our product candidates, because the potential target populations are very small, we may never achieve profitability despite obtaining such significant market share.

Risks related to regulatory approval and commercialization

Even if we complete the necessary preclinical studies and clinical trials, the marketing approval process is expensive, time-consuming and uncertain and may prevent us from obtaining approvals for the commercialization of any product candidates we may develop. If we are not able to obtain, or if there are delays in obtaining, required regulatory approvals, we will not be able to commercialize, or will be delayed in commercializing, product candidates we may develop, and our ability to generate revenue will be materially impaired.

Any product candidates we may develop and the activities associated with their development and commercialization, including their design, testing, manufacture, safety, efficacy, potential confirmatory studies, recordkeeping, labeling, storage, approval, advertising, promotion, sale and distribution, are subject to comprehensive regulation by the FDA and other regulatory authorities in the United States and by comparable authorities in other countries, including the TGA in Australia, the EC and the NCAs in the European Union, and by the Medicines and Healthcare products Regulatory Agency, or MHRA, in the United Kingdom. Failure to obtain marketing approval for a product candidate we may develop will prevent us from commercializing the product candidate in a given jurisdiction. We have not received approval to market any product candidates from regulatory authorities in any jurisdiction. We have no experience as a company in filing and supporting the applications necessary to gain marketing approvals and expect to utilize or rely on third-party experts, CROs, and other competent groups and/or individuals to assist us in this process. Securing regulatory approval requires the submission of extensive preclinical and clinical data and supporting information to the various regulatory authorities for each therapeutic indication to establish the product candidate's safety and efficacy. Securing regulatory approval also requires the submission of information about the product manufacturing process to, and inspection of manufacturing facilities by, the relevant regulatory authority. Any product candidates we may develop may not be effective, may be only moderately effective or may prove to have undesirable or unintended side effects, toxicities, or other characteristics that may preclude our obtaining marketing approval or prevent or limit commercial use.

The process of obtaining marketing approvals, both in the United States and abroad, is expensive, may take many years if additional clinical trials are required, if approval is obtained at all, and can vary substantially based upon a variety of factors, including the type, complexity and novelty of the product candidates involved. Of the large number of product candidates in development, only a small percentage successfully complete the FDA or foreign regulatory approval processes and are commercialized. Even if any product candidates we may develop demonstrate safety and efficacy in clinical trials, the regulatory agencies may not complete their review processes in a timely manner, or we may not be able to obtain regulatory approval. Additional delays may result if an FDA Advisory Committee or other regulatory authority recommends non-approval or restrictions on approval. Changes in marketing approval policies during the development period, changes in or the enactment of additional statutes or regulations, or changes in regulatory review for each submitted product application, may cause delays in the approval or rejection of an application. The FDA and comparable authorities in other countries have substantial discretion in the approval process and may refuse to accept any application or may decide that our

data is insufficient for approval and require additional preclinical, clinical or other studies. In addition, varying interpretations of the data obtained from preclinical and clinical testing could delay, limit or prevent marketing approval of a product candidate. If we experience delays in obtaining approval or if we fail to obtain approval of any product candidates we may develop, the commercial prospects for those product candidates may be harmed, and our ability to generate revenues will be materially impaired.

Further, under the Pediatric Research Equity Act, or the PREA, a new drug application, or NDA, or supplement to an NDA for certain drugs must contain data to assess the safety and effectiveness of the drug in all relevant pediatric subpopulations and to support dosing and administration for each pediatric subpopulation for which the product is safe and effective, unless the sponsor receives a deferral or waiver from the FDA. A deferral may be granted for several reasons, including a finding that the product or therapeutic candidate is ready for approval for use in adults before pediatric trials are complete or that additional safety or effectiveness data needs to be collected before the pediatric trials begin. The applicable legislation in the EU also requires sponsors to either conduct clinical trials in a pediatric population in accordance with a Pediatric Investigation Plan approved by the Pediatric Committee of the EMA, or to obtain a waiver or deferral from the conduct of these studies by the Pediatric Committee of the EMA. For any of our product candidates for which we seek regulatory approval in the United States or the EU, we cannot guarantee that we will be able to obtain a waiver or alternatively complete any required studies and other requirements in a timely manner, or at all, which could result in associated reputational harm and subject us to enforcement action.

Even if we eventually complete clinical testing and receive approval of an NDA or foreign marketing application for any product candidates, the FDA or the applicable foreign regulatory agency may grant approval or other marketing authorization contingent on the performance of costly additional clinical trials, including post-market clinical trials. The FDA or the applicable foreign regulatory agency also may approve or authorize for marketing a product candidate for a more limited indication or patient population that we originally request, and the FDA or applicable foreign regulatory agency may not approve or authorize the labeling that we believe is necessary or desirable for the successful commercialization of a product candidate. Any of these restrictions or commitments could render an approved product not commercially viable, which would materially adversely impact our business and prospects.

We may attempt to seek approval from the FDA or comparable foreign regulatory authorities, where applicable, under the accelerated approval pathways. We may fail to obtain approval under such accelerated approval pathways. Moreover, these pathways may not lead to a faster development, regulatory review or approval process and do not increase the likelihood that our product candidates will receive marketing approval.

We may in the future seek accelerated approval, where applicable, under the FDA's accelerated approval pathway. A product candidate may be eligible for accelerated approval if it treats a serious or life-threatening condition, generally provides a meaningful advantage over available therapies, and demonstrates an effect on a surrogate endpoint that is reasonably likely to predict clinical benefit or on a clinical endpoint that can be measured earlier than irreversible morbidity or mortality, or IMM, that is reasonably likely to predict an effect on IMM or other clinical benefit. As a condition of accelerated approval, the FDA likely would require that we perform adequate and well-controlled post-marketing clinical trials to confirm the product's clinical benefit. These confirmatory trials must be completed with due diligence. Under Food and Drug Omnibus Reform Act of 2022, or FDOR, the FDA is permitted to require, as appropriate, that a post-approval confirmatory study or studies be underway prior to approval or within a specified time period after the date of accelerated approval was granted. FDORA also requires sponsors to send updates to the FDA every 180 days on the status of such studies, including progress toward enrollment targets, and the FDA must promptly post this information publicly. FDORA also gives the FDA increased authority to withdraw approval of a drug granted accelerated approval on an expedited basis if the sponsor fails to conduct such studies in a timely manner, send the necessary updates to the FDA, or if such post-approval studies fail to verify the drug's predicted clinical benefit. Under FDORA, the FDA is empowered to take action, such as issuing fines, against companies that fail to conduct with due diligence any post-approval confirmatory study or submit timely reports to the agency on their progress. Even if we seek to utilize the accelerated approval pathway, we may not be able to obtain accelerated approval and, even if we do, we may not

experience a faster development, regulatory review or approval process for that product. In addition, receiving accelerated approval does not ensure that the product's accelerated approval will eventually be converted to a full approval.

In the EU, under the centralized procedure, the EMA's Committee for Medicinal Products for Human Use may perform an accelerated assessment of a marketing authorization application. Applicants requesting an accelerated assessment procedure must justify that the product candidate is expected to be of major public health interest, particularly from the point of view of therapeutic innovation. Prior to seeking accelerated approval for any of our product candidates, we intend to seek feedback from the FDA or similar foreign regulatory authorities and will otherwise evaluate our ability to seek and receive accelerated approval. There can be no assurance that after our evaluation of the feedback and other factors we will decide to pursue or submit an NDA or similar application for accelerated approval or any other form of expedited development or review. Similarly, there can be no assurance that after subsequent FDA or similar foreign regulatory authorities feedback we will continue to pursue or apply for accelerated approval or any other form of expedited development or review, even if we initially decide to do so. Furthermore, if we decide to submit an application for accelerated approval or other expedited development or review for our product candidates, there can be no assurance that such submission or application will be accepted or that any expedited development or review will be granted on a timely basis, or at all. The FDA or other comparable foreign regulatory authorities could also require us to conduct further studies prior to considering our application or granting approval of any type. A failure to obtain accelerated approval or any other form of expedited development or review for our product candidate would result in a longer time period to commercialization of such product candidate, if any, could increase the cost of development of such product candidate, and could harm our competitive position in the marketplace.

We may seek one or more designations or expedited programs for one or more of our product candidates, but we might not receive such designations or be allowed to proceed on expedited program pathways, and even if we do and proceed on such expedited program pathways in the future, such designations or expedited programs may not lead to a faster development or regulatory review or approval process, and each designation does not increase the likelihood that any of our product candidates will receive marketing approval in the United States

We may seek fast track designation for certain of our product candidates. If a drug is intended for the treatment of a serious or life-threatening condition and nonclinical or clinical data for the drug demonstrates the potential to address an unmet medical need for such a condition, the drug sponsor may apply for fast track designation. The FDA has broad discretion whether or not to grant this designation, so even if we believe a particular product candidate is eligible for this designation, we cannot assure you that the FDA would decide to grant it for any of our other product candidates. Even with fast track designation, we may not experience a faster development process, review or approval compared to conventional FDA procedures. The FDA may withdraw fast track designation if it believes that the designation is no longer supported by data from our clinical development program. Fast track designation alone does not guarantee qualification for the FDA's priority review procedures.

We may seek a breakthrough therapy designation for some of our product candidates. A breakthrough therapy is defined as a drug that is intended, alone or in combination with one or more other drugs, to treat a serious or life-threatening disease or condition, and preliminary clinical evidence indicates that the drug may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development. For product candidates that have been designated as breakthrough therapies, interaction and communication between the FDA and the sponsor of the trial can help to identify the most efficient path for clinical development while minimizing the number of patients placed in ineffective control regimens. Product candidates designated as breakthrough therapies by the FDA may also be eligible for priority review and accelerated approval. Designation as a breakthrough therapy is within the discretion of the FDA. Accordingly, even if we believe one of our product candidates meets the criteria for designation as a breakthrough therapy, the FDA may disagree and instead determine not to make such designation. In any event, the receipt of a breakthrough therapy designation for a product candidate may not result in a faster development process, review or approval compared to therapies considered for approval under conventional FDA procedures and does not assure ultimate approval by the FDA. In addition, even if one or more of our

product candidates qualify as breakthrough therapies, the FDA may later decide that such product candidates no longer meet the conditions for qualification or decide that the time period for FDA review or approval will not be shortened.

If the FDA determines that a product candidate offers a treatment for a serious condition and, if approved, the product would provide a significant improvement in safety or effectiveness, the FDA may designate the product candidate for priority review. A priority review designation means that the goal for the FDA to review an application is six months, rather than the standard review period of ten months. We may request priority review for our product candidates. The FDA has broad discretion with respect to whether or not to grant priority review status to a product candidate, so even if we believe a particular product candidate is eligible for such designation or status, the FDA may decide not to grant it. Moreover, a priority review designation does not necessarily result in an expedited regulatory review or approval process or necessarily confer any advantage with respect to approval compared to conventional FDA procedures. Receiving priority review from the FDA does not guarantee approval within the six-month review cycle or at all.

We intend to pursue orphan drug designation for certain of our product candidates, but we may not be able to obtain such designation, or obtain or maintain the benefits of such designation including orphan drug exclusivity, and even if we do, that exclusivity may not prevent regulatory authorities from approving other competing products.

We intend to seek orphan drug designation for certain of our product candidates; however, we may never receive such designations. Under the Orphan Drug Act, the FDA may designate a product candidate as an orphan drug if it is a drug intended to treat a rare disease or condition, defined as a patient population of fewer than 200,000 in the United States, or a patient population greater than 200,000 in the United States where there is no reasonable expectation that the cost of developing the drug will be recovered from sales in the United States Orphan drug designation must be requested before submitting an NDA. A similar regulatory scheme governs orphan products in the EU and the United Kingdom based on, among others, prevalence of the disease or condition of less than 5 in 10,000.

Orphan drug designation entitles a party to financial incentives such as opportunities for grant funding towards clinical trial costs, tax advantages and application fee waivers. After the FDA grants orphan drug designation, the generic identity of the product candidate and its potential orphan use are disclosed publicly by the FDA. In addition, if a product candidate with an orphan drug designation subsequently receives the first marketing approval for the indication for which it has such designation, the product is entitled to a period of marketing exclusivity, which precludes the FDA from approving another marketing application for the same product for the same therapeutic indication for seven years.

Even if we obtain orphan drug exclusivity for a product, that exclusivity may not effectively protect the product from competition because different products can be approved for the same condition. In addition, even after an orphan drug is approved, the FDA can subsequently approve the same product for the same condition if the FDA concludes that the later product is clinically superior in that it is shown to be safer, more effective or makes a major contribution to patient care. Orphan drug exclusivity may also be lost if the FDA determines that the request for designation was materially defective or if the manufacturer is unable to assure sufficient quantity of the product to meet the needs of the patients with the rare disease or condition. Further, even if we obtain orphan drug designation, we may not be the first to obtain marketing approval for any particular orphan indication due to the uncertainties associated with developing pharmaceutical products.

The FDA may further reevaluate the Orphan Drug Act and its regulations and policies. We do not know if, when, or how the FDA may change the orphan drug regulations and policies in the future, and it is uncertain how any changes might affect our business. Depending on what changes the FDA may make to its orphan drug regulations and policies, our business could be adversely impacted.

Obtaining and maintaining marketing approval or commercialization of our product candidates in the United States does not mean that we will be successful in obtaining marketing approval of our product candidates in other jurisdictions. Failure to obtain marketing approval in foreign jurisdictions would prevent any product candidates we may develop from being marketed in such jurisdictions, which, in turn, would materially impair our ability to generate revenue.

In order to market and sell any product candidates we may develop in the European Union and many other foreign jurisdictions, we or our collaborators must obtain separate marketing approvals and comply with numerous and varying regulatory requirements. The approval procedure varies among countries and can involve additional testing. The time required to obtain approval may differ substantially from that required to obtain FDA approval. The regulatory approval process outside the United States generally includes all of the risks associated with obtaining FDA approval. In addition, in many countries outside the United States, it is required that the product candidate be approved for reimbursement before the product can be approved for sale in that country. We or these third parties may not obtain approvals from regulatory authorities outside the United States on a timely basis, if at all. Approval by the FDA does not ensure approval by regulatory authorities in other countries or jurisdictions, and approval by one regulatory authority outside the United States does not ensure approval by regulatory authorities in other countries or jurisdictions or by the FDA. We may not be able to file for marketing approvals and may not receive necessary approvals to commercialize our product candidates in any jurisdiction, which would materially impair our ability to generate revenue.

In addition, foreign regulatory authorities may change their approval policies and new regulations may be enacted. For instance, the EU pharmaceutical legislation is currently undergoing a complete revision, in the context of the Pharmaceutical Strategy for Europe initiative, launched by the European Commission in November 2020. The European Commission's proposals for revision of several fundamental legislative instruments related to medicinal products, which may reduce the duration of regulatory data protection and revise the eligibility for expedited pathways in addition to other changes, was published on April 26, 2023. The proposed revisions are yet be finalized by the European Parliament and European Council through the co-decision legislative process and the proposals may therefore be substantially revised before adoption, which is not anticipated before early 2026. On April 10, 2024, the European Parliament adopted its position on the Commission proposal to reform. The revisions will however have a significant impact on the pharmaceutical industry and our business in the long term.

Any delay in obtaining, or an inability to obtain, any marketing approvals would prevent us from commercializing any product candidates in the United Kingdom and/or the European Union and restrict our ability to generate revenue and achieve and sustain profitability. If any of these outcomes occur, we may be forced to restrict or delay efforts to seek regulatory approval in the United Kingdom and/or the European Union for any product candidates we may develop, which could significantly and materially harm our business.

Even if we obtain regulatory approval for any of our product candidates, we will still face extensive and ongoing regulatory requirements and obligations, which may result in significant additional expense.

Any product candidate for which we obtain marketing approval, along with the manufacturing processes, post-approval clinical data, labeling, packaging, distribution, adverse event reporting, storage, recordkeeping, export, import, and advertising and promotional activities for such product, among other things, will be subject to extensive and ongoing requirements of and review by the FDA and other regulatory authorities. These requirements include submissions of safety and other post-marketing information and reports, establishment registration and drug listing requirements, continued compliance with GMP requirements relating to manufacturing, quality control, quality assurance and corresponding maintenance of records and documents, requirements regarding the distribution of samples to physicians and recordkeeping and GCP requirements for any clinical trials that we conduct post-approval.

Even if marketing approval of a product candidate is granted, the approval may be subject to limitations on the indicated uses for which the product candidate may be marketed or to the conditions of approval, including a requirement to implement a REMS. If a product candidate receives marketing approval, the accompanying label may limit the approved indicated use of the product, which could limit sales of the product. The FDA may also

require costly post-marketing studies or clinical trials and surveillance to monitor the safety or efficacy of a product. The FDA closely regulates the post-approval marketing and promotion of drugs to ensure drugs are marketed only for the approved indications and in accordance with the provisions of the approved labeling. The FDA imposes stringent restrictions on manufacturers' communications regarding off-label use, and if we market our products for uses beyond their approved indications, we may be subject to enforcement action for off-label marketing. Violations of the Federal Food, Drug, and Cosmetic Act, relating to the promotion of prescription drugs, may lead to FDA enforcement actions and investigations alleging violations of federal and state healthcare fraud and abuse laws, as well as state consumer protection laws.

If we fail to comply with applicable regulatory requirements following approval of any product candidates we may develop, a regulatory agency may:

- issue a warning letter asserting that we are in violation of the law;
- seek an injunction or impose civil or criminal penalties or monetary fines;
- suspend or withdraw regulatory approval;
- suspend any ongoing clinical trials;
- refuse to approve a pending NDA or supplements to an NDA submitted by us;
- seize product; or
- refuse to allow us to enter into supply contracts, including government contracts.

Any government investigation of alleged violations of law could require us to expend significant time and resources in response and could generate negative publicity. The occurrence of any event or penalty described above may inhibit our ability to commercialize any product candidates we may develop and generate revenues.

In addition, later discovery of previously unknown problems with our products, manufacturers or manufacturing processes, or failure to comply with regulatory requirements, may yield various results, including:

- restrictions on such products, manufacturers or manufacturing processes;
- restrictions on the labeling or marketing of a product;
- restrictions on the distribution or use of a product;
- requirements to conduct post-marketing clinical trials;
- receipt of warning or untitled letters;
- withdrawal of the products from the market;
- refusal to approve pending applications or supplements to approved applications that we submit;
- recall of products;
- fines, restitution or disgorgement of profits or revenue;
- suspension or withdrawal of marketing approvals;
- suspension of any ongoing clinical trials;
- refusal to permit the import or export of our products;
- product seizure; and
- injunctions or the imposition of civil or criminal penalties.

Any government investigation of alleged violations of law could require us to expend significant time and resources in response and could generate negative publicity. The occurrence of any event or penalty described above may inhibit our ability to commercialize any product candidates we develop and adversely affect our business, financial condition, results of operations and prospects.

Even if any product candidate that we may develop receives marketing approval, it may fail to achieve the degree of market acceptance by physicians, patients, third-party payors and others in the medical community necessary for commercial success.

If any product candidate we may develop receives marketing approval, it may nonetheless fail to gain sufficient market acceptance by physicians, patients, third-party payors and others in the medical community. Sales of medical products depend in part on the willingness of physicians to prescribe the treatment, which is likely to be based on a determination by these physicians that the products are safe, therapeutically effective and cost-effective. In addition, the inclusion or exclusion of products from treatment guidelines established by various physician groups and the viewpoints of influential physicians can affect the willingness of other physicians to prescribe the treatment. We cannot predict whether physicians, physicians' organizations, hospitals, other healthcare providers, government agencies or private insurers will determine that our product is safe, therapeutically effective and cost-effective as compared with competing treatments. Efforts to educate the medical community and third-party payors on the benefits of any product candidates we may develop may require significant resources and may not be successful. If any product candidates we may develop do not achieve an adequate level of acceptance, we may not generate significant product revenues and we may not become profitable. The degree of market acceptance of any product candidates we may develop, if approved for commercial sale, will depend on a number of factors, including:

- the efficacy and safety of such product candidates as demonstrated in clinical trials;
- the potential advantages and limitations compared to alternative treatments;
- the effectiveness of sales and marketing efforts;
- the cost of treatment in relation to alternative treatments;
- the clinical indications for which the product is approved;
- the convenience and ease of administration compared to alternative treatments;
- the willingness of the target patient population to try new therapies and of physicians to prescribe these therapies;
- the strength of marketing and distribution support;
- the timing of market introduction of competitive products;
- the availability of third-party coverage and adequate reimbursement;
- the prevalence and severity of any side effects; and
- any restrictions on the use of our products, if approved, together with other medications.

The pricing, insurance coverage and reimbursement status of newly approved products is uncertain. Failure to obtain or maintain adequate coverage and reimbursement for our product candidates, if approved, could limit our ability to market those products and decrease our ability to generate product revenue.

Certain of the initial target indications in our pipeline are indications with small patient populations. For product candidates that are designed to treat smaller patient populations to be commercially viable, the reimbursement for such product candidates must be higher, on a relative basis, to account for the lack of volume. Accordingly, we will need to implement a coverage and reimbursement strategy for any approved product candidate that accounts for the smaller potential market size. If we are unable to establish or sustain coverage and adequate reimbursement for any approved product candidates from third-party payors, the adoption of those product candidates and sales revenue will be adversely affected, which, in turn, could adversely affect the ability to market or sell those product candidates, if approved.

We expect that coverage and reimbursement by third-party payors will be essential for most patients to be able to afford these treatments. Accordingly, any future sales of our product candidates, if approved, will depend substantially, both domestically and internationally, on the extent to which the costs of such product candidates

will be paid by health maintenance, managed care, pharmacy benefit and similar healthcare management organizations, or will be reimbursed by government authorities, private health coverage insurers and other third-party payors. Even if coverage is provided, the approved reimbursement amount may not be high enough to allow us to establish or maintain pricing sufficient to realize a sufficient return on our investment.

There is significant uncertainty related to the insurance coverage and reimbursement of newly approved products. In the United States, the principal decisions about reimbursement by government authorities for new products are typically made by the Centers for Medicare & Medicaid Services, or CMS, since CMS decides whether and to what extent a new product will be covered and reimbursed under Medicare. Private payors tend to follow CMS to a substantial degree. However, one payer's determination to provide coverage for a product does not assure that other payors will also provide coverage for the drug product. Further, a payer's decision to provide coverage for a drug product does not imply that an adequate reimbursement rate will be approved. Reimbursement agencies in the European Union may be more conservative than CMS.

Outside the United States, international operations are generally subject to extensive governmental price controls and other market regulations, and we believe the increasing emphasis on cost-containment initiatives in Europe, Canada and other countries has and will continue to put pressure on the pricing and usage of therapeutics such as any product candidates we may develop. In many countries, particularly the countries of the European Union, the prices of medical products are subject to varying price control mechanisms as part of national health systems. In these countries, pricing negotiations with governmental authorities can take considerable time after the receipt of marketing approval for a product. As a result, we might obtain marketing approval for a product in a particular country, but then be subject to price regulations that delay or might even prevent our commercial launch of the product, possibly for lengthy periods of time. To obtain reimbursement or pricing approval in some countries, we may be required to conduct a clinical trial that compares the cost-effectiveness of our product candidate to other available therapies. In general, the prices of products under such systems are substantially lower than in the United States. Other countries allow companies to fix their own prices for products but monitor and control company profits. Additional foreign price controls or other changes in pricing regulation could restrict the amount that we are able to charge for product candidates. Accordingly, in markets outside the United States, the reimbursement for any product candidates we may develop may be reduced compared with the United States and may be insufficient to generate commercially reasonable revenues and profits.

Moreover, increasing efforts by governmental and third-party payors, in the United States and internationally, to cap or reduce healthcare costs may cause such organizations to limit both coverage and level of reimbursement for new products approved and, as a result, they may not cover or provide adequate payment for any product candidates we may develop. We expect to experience pricing pressures in connection with the sale of any product candidates we may develop due to the trend toward managed healthcare, the increasing influence of certain third-party payors, such as health maintenance organizations, and additional legislative changes. The downward pressure on healthcare costs in general, particularly prescription drugs and surgical procedures and other treatments, has become very intense. As a result, increasingly high barriers are being erected to the entry of new products into the healthcare market. There have been instances in which third-party payors have refused to reimburse treatments for patients for whom the treatment is indicated in the FDA-approved product label. Even if we are successful in obtaining FDA approvals to commercialize our product candidates, we cannot guarantee that we will be able to secure reimbursement for all patients for whom treatment with our product candidates is indicated.

In addition to CMS and private payors, professional organizations, such as the American Medical Association, can influence decisions about reimbursement for new products by determining standards for care. In addition, many private payors contract with commercial vendors who sell software that provide guidelines that attempt to limit utilization of, and therefore reimbursement for, certain products deemed to provide limited benefit to existing alternatives. Such organizations may set guidelines that limit reimbursement or utilization of our product candidates. Even if favorable coverage and reimbursement status is attained for one or more product candidates

for which we or our collaborators receive regulatory approval, less favorable coverage policies and reimbursement rates may be implemented in the future.

If we are unable to establish sales, marketing and distribution capabilities or enter into sales, marketing and distribution agreements with third parties, we may not be successful in commercializing any product candidates we may develop if and when they are approved.

We do not have a sales or marketing infrastructure and have no experience in the sale, marketing or distribution of pharmaceutical products. To achieve commercial success for any product for which we have obtained marketing approval, we will need to establish a sales, marketing and distribution organization, either ourselves or through collaborations or other arrangements with third parties.

In the future, we may build a sales and marketing infrastructure to market some of the product candidates we may develop if and when they are approved. There are risks involved with establishing our own sales, marketing and distribution capabilities. For example, recruiting and training a sales force is expensive and time-consuming and could delay any product launch. If the commercial launch of a product candidate for which we recruit a sales force and establish marketing capabilities is delayed or does not occur for any reason, we would have prematurely or unnecessarily incurred these commercialization expenses. These efforts may be costly, and our investment would be lost if we cannot retain or reposition our sales and marketing personnel.

Factors that may inhibit our efforts to commercialize our products on our own include:

- our inability to recruit, train and retain adequate numbers of effective sales, marketing, coverage or reimbursement, customer service, medical affairs and other support personnel;
- the inability of sales personnel to educate adequate numbers of physicians on the benefits of such product candidates;
- the inability of reimbursement professionals to negotiate arrangements for formulary access, reimbursement and other acceptance by payors;
- the inability to price our products at a sufficient price point to ensure an adequate and attractive level of profitability;
- restricted or closed distribution channels that make it difficult to distribute our products to segments of the patient population;
- the lack of complementary products to be offered by sales personnel, which may put us at a competitive disadvantage relative to companies with more extensive product lines; and
- unforeseen costs and expenses associated with creating an independent sales and marketing organization.

If we are unable to establish our own sales, marketing and distribution capabilities and we enter into arrangements with third parties to perform these services, our product revenues and our profitability, if any, are likely to be lower than if we were to market, sell and distribute any products that we develop ourselves. In addition, we may not be successful in entering into arrangements with third parties to sell, market and distribute any product candidates we may develop or may be unable to do so on terms that are acceptable to us. We likely will have little control over such third parties, and any of them may fail to devote the necessary resources and attention to sell and market our products effectively. If we do not establish sales, marketing and distribution capabilities successfully, either on our own or in collaboration with third parties, we will not be successful in commercializing any product candidates we may develop.

Our relationships with healthcare providers, physicians, patients and third-party payors may be subject to various anti-kickback, fraud and abuse, other healthcare laws and regulations, which could increase compliance costs, and our failure to comply with these laws and regulations could harm our reputation, subject us to significant fines and liability, or otherwise adversely affect our business.

Our business operations and current and future arrangements with investigators, healthcare professionals, consultants, third-party payors, patient organizations, and customers may expose us to broadly applicable fraud

and abuse and other healthcare laws and regulations. These laws may constrain the business or financial arrangements and relationships through which we conduct our operations, including how we research, market, sell, and distribute any products for which we obtain regulatory approval. Such laws include:

- the federal Anti-Kickback Statute, or AKS, which prohibits, among other things, persons or entities from knowingly and willfully soliciting, offering, receiving, or providing any remuneration (including any kickback, bribe, or certain rebates), directly or indirectly, overtly or covertly, in cash or in kind, in return for, either the referral of an individual or the purchase, lease, or order, or arranging for or recommending the purchase, lease, or order of any good, facility, item, or service, for which payment may be made, in whole or in part, under a federal healthcare program such as Medicare and Medicaid. A person or entity does not need to have actual knowledge of the federal AKS or specific intent to violate it in order to have committed a violation;
- the federal false claims laws, including the civil False Claims Act, or FCA, and civil monetary penalties laws, which prohibit, among other things, individuals or entities from knowingly presenting, or causing to be presented, to the federal government, claims for payment or approval that are false or fraudulent, knowingly making, using or causing to be made or used, a false record or statement material to a false or fraudulent claim, or from knowingly making or causing to be made a false statement to avoid, decrease or conceal an obligation to pay money to the federal government. In addition, the government may assert that a claim including items or services resulting from a violation of the federal AKS constitutes a false or fraudulent claim for purposes of the civil FCA. Manufacturers can be held liable under the FCA even when they do not submit claims directly to government payors if they are deemed to "cause" the submission of false or fraudulent claims; the FCA also permits a private individual acting as whistleblower to bring actions on behalf of the federal government alleging violations of the FCA and to share in any monetary recovery;
- the Civil Monetary Penalties Law, which covers a variety of conduct, often violations under other laws, and includes penalties for violating the AKS violations, causing the submission of false claims, and offering or transfer of remuneration to a Medicare or state healthcare program beneficiary if the person knows or should know it is likely to influence the beneficiary's selection of a particular provider, practitioner, or supplier of services reimbursable by Medicare or a state healthcare program;
- the federal Health Insurance Portability and Accountability Act of 1996, or HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act of 2009 and their implementing regulations, which imposes criminal and civil liability for, among other things, knowingly and willfully executing, or attempting to execute, a scheme to defraud any healthcare benefit program, or knowingly and willfully falsifying, concealing, or covering up a material fact or making any materially false statement, in connection with the delivery of, or payment for, healthcare benefits, items, or services. Similar to the federal AKS, a person or entity does not need to have actual knowledge of the statute or specific intent to violate it in order to have committed a violation. HIPAA also imposes obligations related to the privacy, security, and transmission of individually identifiable health information that apply to many healthcare providers, physicians, and third-party payors with whom we interact;
- federal consumer protection and unfair competition laws broadly regulate marketplace activities and activities that potentially harm consumers;
- federal government price reporting laws, which require pharmaceutical manufacturers to report certain calculated product prices to the government or provide certain discounts or rebates to government authorities or private entities, often as a condition of reimbursement under governmental healthcare programs;
- the federal Physician Payments Sunshine Act, which requires certain manufacturers of drugs, devices, biologics, and medical supplies for which payment is available under Medicare, Medicaid, or the Children's Health Insurance Program (with certain exceptions) to report annually to CMS, information related to payments and other "transfers of value" made to "physicians" (which has the same meaning as under Section 1861(r) of the Social Security Act, which generally includes doctors of medicine, osteopathy, dentists, podiatrists, optometrists and chiropractors who are legally authorized to practice by a state),

certain non-physician practitioners (physician assistants, nurse practitioners, clinical nurse specialists, certified nurse anesthetists, anesthesiology assistants, and certified nurse-midwives), and teaching hospitals and other healthcare providers, as well as ownership and investment interests held by such healthcare professionals and their immediate family members; and

- analogous state and foreign laws and regulations, such as state anti-kickback and false claims laws, which may apply to sales or marketing arrangements and claims involving healthcare items or services reimbursed by non-governmental third-party payors, including private insurers; some state laws require biopharmaceutical companies to comply with the biopharmaceutical industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by the federal government and may require drug manufacturers to report information related to payments and other transfers of value to physicians and other healthcare providers or marketing expenditures; some state laws that require biopharmaceutical companies to report information on the pricing of certain drug products; and some state and local laws that require the registration of pharmaceutical sales representatives.

Healthcare providers, physicians and third-party payors in the United States and elsewhere play a primary role in the recommendation and prescription of pharmaceutical products. Arrangements with third-party payors and customers can expose pharmaceutical manufacturers to broadly applicable fraud and abuse and other healthcare laws and regulations, which may constrain the business or financial arrangements and relationships through which such companies sell, market and distribute pharmaceutical products. In particular, the promotion, sales and marketing of healthcare items and services, as well as a wide range of pricing, discounting, marketing and promotion, structuring and commissions, certain customer incentive programs and other business arrangements, are subject to extensive laws designed to prevent fraud, kickbacks, self-dealing and other abusive practices. These laws and regulations may restrict or prohibit a wide range of pricing, discounting, marketing and promotion, structuring and commissions, support programs and other business arrangements generally. Activities subject to these laws also involve the improper use of information obtained in the course of patient recruitment for clinical trials.

Efforts to ensure that our current and future business arrangements with third parties will comply with applicable healthcare laws and regulations will involve substantial ongoing costs. It is possible that governmental authorities will conclude that our business practices, including certain consulting agreements and advisor agreements we have entered into with physicians who are paid, in part, in the form of stock or stock options, may not comply with current or future statutes, regulations or case law involving applicable fraud and abuse or other healthcare laws and regulations. Due to the breadth of these laws, the narrowness of statutory exceptions and regulatory safe harbors available, and the range of interpretations to which they are subject, it is possible that some of our current or future practices might be challenged under one or more of these laws. If our operations are found to be in violation of any of these laws or any other governmental regulations that may apply to us, we may be subject to significant penalties, including civil, criminal, and administrative penalties, damages, fines, disgorgement, imprisonment, exclusion from participation in government-funded healthcare programs, such as Medicare and Medicaid, integrity oversight and reporting obligations, contractual damages, reputational harm, diminished profits and future earnings, and the curtailment or restructuring of our operations. Defending against any such actions can be costly and time-consuming and may require significant financial and personnel resources. Therefore, even if we are successful in defending against any such actions that may be brought against us, our business may be impaired. Further, if any of the physicians or other healthcare providers or entities with whom we expect to do business are found be non-compliant with applicable laws or regulations, they may be subject to significant criminal, civil, or administrative sanctions, including exclusions from government-funded healthcare programs.

Current and future healthcare reform legislation or regulation may increase the difficulty and cost for us to obtain coverage for and commercialize our product candidates and may adversely affect the prices we may set.

In the United States and some foreign jurisdictions, there have been, and we expect there will continue to be, a number of legislative and regulatory changes to the healthcare system, including cost-containment measures that may reduce or limit coverage and reimbursement for newly approved drugs and biologics and affect our ability to profitably sell any product candidates for which we obtain regulatory approval. In particular, there have been

and continue to be a number of initiatives at the U.S. federal and state levels that seek to reduce healthcare costs and improve the quality of healthcare.

For example, the Patient Protection and Affordable Care Act and Health Care and Education Reconciliation Act, or the Affordable Care Act, which became law in United States in 2010, contains provisions will become more salient to our business if any of our product candidates are approved. The Affordable Care Act established an annual, nondeductible fee on any entity that manufactures or imports specified branded prescription drugs and biologic agents; established provisions that subject biological products to potential competition by lower-cost biosimilars; extended manufacturers' Medicaid rebate liability to covered drugs dispensed to individuals who are enrolled in Medicaid managed care organizations; expanded eligibility criteria for Medicaid programs; expanded the entities eligible for discounts under the 340B drug pricing program; increased the statutory minimum rebates a manufacturer must pay under the Medicaid Drug Rebate Program; established a new Patient-Centered Outcomes Research Institute to oversee, identify priorities in, and conduct comparative clinical effectiveness research, along with funding for such research; expanded federal healthcare fraud and abuse laws, including the False Claims Act and the Anti-Kickback Statue, new government investigative powers and enhanced penalties for noncompliance; and established a Center for Medicare & Medicaid Innovation at CMS to test innovative payment and service delivery models to lower Medicare and Medicaid spending. We may face uncertainties because of efforts to repeal, substantially modify or invalidate some or all of the provisions of the Affordable Care Act. There is no assurance that the Affordable Care Act, as currently enacted or as amended in the future, will not adversely affect our business and financial results, and we cannot predict how future federal or state legislative or administrative changes relating to healthcare reform will affect our business.

In addition, other legislative changes have been proposed and adopted since the Affordable Care Act was enacted. For example, beginning April 1, 2013, Medicare payments to providers were reduced under the sequestration required by the Budget Control Act of 2011, which will remain in effect through 2032, unless additional Congressional action is taken. Additionally, on January 2, 2013, the American Taxpayer Relief Act of 2012 was signed into law, which, among other things, further reduced Medicare payments to several providers, including hospitals, imaging centers, and cancer treatment centers, and increased the statute of limitations period for the government to recover overpayments to providers from three to five years. On March 11, 2021, the American Rescue Plan Act of 2021 was signed into law, which eliminates the statutory cap on Medicaid drug rebates beginning January 1, 2024. The rebate was previously capped at 100% of a drug's average manufacturer price. Additionally, the Inflation Reduction Act of 2022 includes several provisions such as drug pricing controls and Medicare redesign that are likely to impact our business to varying degrees, but its ultimate effect on our business and the healthcare industry in general is not yet known. See "Healthcare laws and regulation in the United States—Healthcare reform" section.

Further, there has been heightened governmental scrutiny in the United States of pharmaceutical pricing practices in light of the rising cost of prescription drugs. Such scrutiny has resulted in several recent congressional inquiries and proposed and enacted federal and state legislation designed to, among other things, bring more transparency to product pricing, review the relationship between pricing and manufacturer patient assistance programs, and reform government program reimbursement methodologies for products. There is also significant economic pressure on state budgets that may result in states increasingly seeking to achieve budget savings through mechanisms that limit coverage or payment for drugs or that would allow for importation of pharmaceutical products from lower cost jurisdictions outside the United States. State Medicaid programs are increasingly requesting manufacturers to pay supplemental rebates and requiring prior authorization by the state program for use of any drug for which supplemental rebates are not being paid. Government efforts to reduce Medicaid expenses may lead to increased use of managed care organizations by Medicaid programs. This may result in managed care organizations influencing prescription decisions for a larger segment of the population and a corresponding limitation on prices and reimbursement for our products, if approved.

These healthcare reforms, as well as other healthcare reform measures that may be adopted in the future, may result in additional reductions in Medicare and other healthcare funding, more rigorous coverage criteria, new payment methodologies and additional downward pressure on the price that we receive for any approved

product and/or the level of reimbursement physicians receive for administering any approved product we might bring to market. Reductions in reimbursement levels may negatively impact the prices we receive or the frequency with which our potential products are prescribed or administered. Any reduction in reimbursement from Medicare or other government programs may result in a similar reduction in payments from private payors.

We are subject to stringent privacy laws, information security laws, regulations, policies and contractual obligations related to data privacy and security and changes in such laws, regulations, policies, and contractual obligations and failure to comply with such requirements could subject us to significant fines and penalties, which may have a material adverse effect on our business, financial condition, results of operations or prospects.

We are subject to data privacy and protection laws, regulations, policies and contractual obligations that apply to the collection, transmission, storage and use of personally-identifying information, which among other things, impose certain requirements relating to the privacy, security and transmission of personal information, including comprehensive regulatory systems in the United States, European Union and United Kingdom. The legislative and regulatory landscape for privacy and data protection continues to evolve in jurisdictions worldwide, and there has been an increasing focus on privacy and data protection issues with the potential to affect our business. More than a dozen states in the United States have passed comprehensive data protection legislation, and the global regulatory environment pertaining to information security and privacy is increasingly demanding, with new and changing requirements, such as the European Union's General Data Protection Regulation, The Personal Information Protection Law of the People's Republic of China and Brazil's Lei Geral de Protecao de Dados. Complying with these laws and regulations may be more costly or take longer than we anticipate, and any failure to comply with any of these laws and regulations could result in enforcement action against us, including fines, claims for damages by affected individuals, damage to our reputation and loss of goodwill, any of which could have a material adverse effect on our business, financial condition, results of operations or prospects.

There are numerous U.S. federal and state laws and regulations related to the privacy and security of personal information. In particular, regulations promulgated pursuant to HIPAA establish privacy and security standards that limit the use and disclosure of individually identifiable health information, or protected health information, and impose requirements regarding the privacy and security of individually identifiable health information, including mandatory contractual terms, for covered entities, or certain healthcare providers, health plans, and healthcare clearinghouses, and their business associates that provide services to the covered entity that involve individually identifiable health information and their subcontractors that use, disclose, or otherwise process individually identifiable health information. While pharmaceutical and biotechnology companies are typically not directly regulated by HIPAA, our business may be indirectly impacted by HIPAA in our interactions with providers, payors, and others that have HIPAA compliance obligations. If we are unable to properly protect the privacy and security of protected health information, we could be found to have violated these privacy and security laws and/or breached certain contracts. Further, if we fail to comply with applicable privacy laws, including applicable HIPAA privacy and security standards, we could face significant civil and criminal penalties. HHS enforcement activity can result in financial liability and reputational harm, and responses to such enforcement activity can consume significant internal resources. In addition, state attorneys general are authorized to bring civil actions seeking either injunctions or damages in response to violations that threaten the privacy of state residents. We cannot be sure how these regulations will be interpreted, enforced or applied to our operations. In addition to the risks associated with enforcement activities and potential contractual liabilities, our ongoing efforts to comply with evolving laws and regulations at the federal and state level may be costly and require ongoing modifications to our policies, procedures and systems.

In addition to potential enforcement by HHS, we are also potentially subject to privacy enforcement from the Federal Trade Commission, or the FTC. The FTC has been particularly focused on the unpermitted processing of health and genetic data through its recent enforcement actions and is expanding the types of privacy violations that it interprets to be "unfair" under Section 5 of the Federal Trade Commission Act, as well as the types of activities it views to trigger the Health Breach Notification Rule, which the FTC also has the authority to enforce. The FTC is also in the process of developing rules related to commercial surveillance and data security that may impact our business. We will need to account for the FTC's evolving rules and guidance for proper privacy and data security practices in order to mitigate our risk for a potential enforcement action, which may be costly. If we are subject to

a potential FTC enforcement action, we may be subject to a settlement order that requires us to adhere to very specific privacy and data security practices, which may impact our business. We may also be required to pay fines as part of a settlement, depending on the nature of the alleged violations. If we violate any consent order that we reach with the FTC, we may be subject to additional fines and compliance requirements.

As we conduct clinical trials in Australia and may in the future conduct clinical trials or seek to commercialize our products outside of the United States, we will also be subject to a variety of foreign data protection laws and regulations. For our clinical trials in Australia, to the extent that the sites for our trials include certain university, company or government agencies, we may be subject to restrictions and data protection obligations under the Privacy Act 1988 (Cth). We may, otherwise, be subject to additional data protection laws in Australia in the states and territories in which we conduct our trials, which have similar restrictions on our ability to collect, analyze and transfer medical records and other patient data. These laws may impact our business. Our failure to comply with these privacy laws and regulations or significant changes in the laws and regulations restricting our ability to obtain required patient information could significantly impact our business and our future business plans.

Laws and regulations governing any international operations we may have in the future may preclude us from developing, manufacturing and selling certain product candidates outside of the United States and require us to develop and implement costly compliance programs.

We are subject to numerous laws and regulations in each jurisdiction outside the United States in which we operate. The creation, implementation and maintenance of international business practices compliance programs is costly and such programs are difficult to enforce, particularly where reliance on third parties is required.

The Foreign Corrupt Practices Act, or the FCPA, prohibits any U.S. individual or business from paying, offering, or authorizing the provision of money or anything of value, directly or indirectly through parties, to any foreign official, official of a public international organization, or political party official or candidate for the purpose of influencing any act or decision of the foreign entity in order to assist the individual or business in obtaining or retaining business. The FCPA also obligates companies whose securities are listed in the United States to comply with certain accounting provisions requiring the company to maintain books and records that accurately and fairly reflect all transactions of the corporation, including international subsidiaries, and to devise and maintain an adequate system of internal accounting controls for international operations. The anti-bribery provisions of the FCPA are enforced primarily by the Department of Justice. The SEC is involved with enforcement of the books and records provisions of the FCPA.

Compliance with the FCPA and other anti-corruption laws potentially applicable to our business is expensive and difficult, particularly in countries in which corruption is a recognized problem. In addition, compliance with the FCPA and other anti-corruption laws presents particular challenges in the pharmaceutical industry, because, in many countries, hospitals are operated by the government, and doctors and other hospital employees are considered foreign officials.

Various U.S. export and sanctions laws, regulations and executive orders also restrict the use and dissemination outside of the United States, or the sharing with certain non-U.S. nationals, of certain products and technical data relating to those products. Furthermore, such export and sanctions laws include restrictions or prohibitions on the sale or supply of certain products and services to U.S. embargoed countries or sanctioned countries, governments, persons and entities. Our expansion outside of the United States has required, and will continue to require, us to dedicate additional resources to comply with these laws, and these laws may preclude us from developing, manufacturing or selling certain drugs and drug candidates outside of the United States, which could limit our growth potential and increase our development costs. The failure to comply with laws governing international business practices may result in substantial penalties, including suspension or debarment from government contracting. Violation of the FCPA and export and sanctions laws can result in significant civil and criminal penalties, imprisonment, the loss of export or import privileges, debarment, breach of contract and fraud litigation, reputational harm, and other consequences. Indictment alone under the FCPA can lead to suspension of the right to do business with the U.S. government until the pending claims are resolved. Conviction of a violation of the FCPA can result in long-term disqualification as a government contractor. The termination of a government

contract or relationship as a result of our failure to satisfy any of our obligations under laws governing international business practices would have a negative impact on our operations and harm our reputation and ability to procure government contracts. The SEC also may suspend or bar issuers from trading securities on U.S. exchanges for violations of the FCPA's accounting provisions.

Inadequate funding for the FDA, the Securities and Exchange Commission, or the SEC, and other government agencies, including from government shut downs, or other disruptions to these agencies' operations, could hinder their ability to hire and retain key leadership and other personnel, prevent new products and services from being developed or commercialized in a timely manner or otherwise prevent those agencies from performing normal business functions on which the operation of our business may rely, which could negatively impact our business.

The ability of the FDA and comparable foreign regulatory authorities to review and approve or certify new products can be affected by a variety of factors, including government budget and funding levels, ability to hire and retain key personnel and accept the payment of user fees and statutory, regulatory and policy changes. Average review times at the agency have fluctuated in recent years as a result. Disruptions at the FDA, other agencies and authorities may also slow the time necessary for new product candidates to be reviewed and/or approved, which would adversely affect our business. In addition, government funding of the SEC and other government agencies on which our operations may rely, including those that fund research and development activities, is subject to the political process, which is inherently fluid and unpredictable.

Disruptions at the FDA, other agencies, and authorities may also slow the time necessary for new product candidates to be reviewed and/or approved by necessary government agencies, foreign regulatory authorities, which would adversely affect our business. For example, over the last several years the U.S. government has shut down several times and certain regulatory agencies, such as the FDA and the SEC, have had to furlough critical employees and stop critical activities. If a prolonged government shutdown occurs, it could significantly impact the ability of the FDA to timely review and process our regulatory submissions, which could have a material adverse effect on our business. Further, future government shutdowns could impact our ability to access the public markets and obtain necessary capital in order to properly capitalize and continue our operations.

Risks related to reliance on third parties

We rely, and intend to continue to rely, on third parties to perform some of our preclinical studies and conduct our clinical trials. If these third parties do not successfully carry out their contractual duties, fail to comply with applicable regulatory requirements, or do not meet expected deadlines, our development programs may be delayed or subject to increased costs or we may be unable to obtain regulatory approval for or commercialize our product candidates.

We are dependent on third parties to perform some of our preclinical studies and to conduct our ongoing and planned clinical trials. Specifically, we rely on, and intend to continue to rely on, medical institutions, clinical investigators, CROs, consultants and other third parties to perform some of our preclinical studies and conduct our clinical trials and the subsequent collection and analysis of data. These third parties play a significant role in the conduct and timing of our research, preclinical studies and clinical trials. While we have and will have agreements governing the committed activities of these third parties, we have limited influence over their actual performance. Nevertheless, we are responsible for ensuring that each of our clinical trials is conducted in accordance with the applicable protocol and legal, regulatory, and scientific standards and requirements, and our reliance on third parties does not relieve us of our regulatory responsibilities. In addition, we and these third parties are required to comply with GLP requirements for certain preclinical studies, as well as GCP requirements, which are regulations and guidelines enforced by the FDA and comparable foreign regulatory authorities for clinical trials of all of our product candidates. Regulatory authorities enforce these GCP requirements through periodic inspections of trial sponsors, principal investigators, and trial sites. If we or any of these third parties fail to comply with applicable GLP or GCP or other requirements, the clinical data generated in our preclinical studies or clinical trials may be deemed unreliable, and the FDA, TGA or comparable foreign regulatory authorities may require us to perform additional preclinical studies or clinical trials before approving our marketing applications, if ever. Further, our

clinical trials must be conducted with product produced in accordance with current GMP regulations. Failure to comply with these regulations may require us to repeat clinical trials, which would delay the regulatory approval process.

There is no guarantee that any such CROs, clinical investigators or other third parties on which we rely will devote adequate time and resources to our development activities or perform as contractually required. If any of these third parties fail to meet expected deadlines, adhere to our clinical protocols, or meet regulatory requirements, or otherwise perform in a substandard manner or terminate their engagements with us, the timelines for our development programs may be extended, delayed or subject to increased costs, or our clinical trials may be extended, delayed, or terminated. In addition, many of these third parties may also have relationships with other entities, including our competitors, for whom they may also be conducting clinical trials or other development activities that could harm our competitive position. If these third parties do not successfully carry out their contractual duties or obligations or meet expected deadlines, if they need to be replaced or if the quality or accuracy of the preclinical or clinical data they obtain is compromised due to the failure to adhere to our protocols or regulatory requirements or for other reasons, our development timelines, including clinical development timelines, may be extended, delayed or terminated and we may not be able to complete development of, obtain regulatory approval of or successfully commercialize our product candidates. As a result, our financial results and the commercial prospects for our product candidates would be harmed, our costs could increase and our ability to generate revenue could be delayed or precluded entirely.

If any of our relationships with these third parties terminate, we may not be able to enter into arrangements with alternative third parties on commercially reasonable terms, in a timely manner or at all. Switching or adding additional CROs, clinical investigators, and other third parties involves additional cost and requires our management's time and focus. In addition, there is a natural transition period when a new CRO or other third party commences work. As a result, delays occur, which can materially impact our ability to meet our desired clinical development timelines. Though we endeavor to carefully manage our relationships with our CROs, clinical investigators and other third parties, there can be no assurance that we will not encounter challenges or delays in the future or that these delays or challenges will not have a material adverse impact on our business, financial condition, and prospects.

We currently depend on third-party suppliers for the manufacture of our product candidates. The loss of these or future third-party suppliers, or their inability to provide us with sufficient supply, could harm our business.

We do not own or operate manufacturing facilities and have no current plans to develop our own clinical or commercial-scale manufacturing capabilities. We rely on third-party suppliers for the manufacture of our product candidates. We expect to continue to depend on third-party suppliers for the manufacture of any product candidates that we evaluate in preclinical studies and clinical trials, as well as for commercial manufacture if those product candidates receive marketing approval. The facilities used by third-party manufacturers to manufacture our product candidates must be approved by the FDA and any comparable foreign regulatory authority pursuant to inspections that will be conducted after we submit an NDA to the FDA or any comparable filing to a foreign regulatory authority. We have limited control over the manufacturing process of, and are completely dependent on, third-party manufacturers or Contract Manufacturing Organizations, or CMOs, for compliance with GMP requirements for manufacture of products. If these third-party manufacturers cannot successfully manufacture material that conforms to our specifications and the strict regulatory requirements of the FDA or any comparable foreign regulatory authority, they will not be able to secure and/or maintain regulatory approval for their manufacturing facilities.

In addition, we have limited control over the ability of third-party manufacturers to maintain adequate quality control, quality assurance and qualified personnel. If the FDA or any comparable foreign regulatory authority does not approve these facilities for the manufacture of any product candidates we may develop or if it withdraws any such approval in the future, we may need to find alternative manufacturing facilities, which would significantly impact our ability to develop, obtain regulatory approval for or market any product candidates we may develop, if approved. Our failure or the failure of our third-party manufacturers to comply with applicable regulations could result in sanctions being imposed on us, including clinical holds, fines, injunctions, civil penalties, delays in

approval or other delays, suspension or withdrawal of approvals, seizures or recalls of product candidates or products, operating restrictions and criminal prosecutions, any of which could significantly and adversely affect supplies of our product candidates.

In addition, certain of the raw materials for our product candidates are currently provided by two Chinese companies, Hongene Biotech and WuXi TIDES, a subsidiary of WuXi AppTec, and we expect to rely on these suppliers for the foreseeable future on an as-needed basis. Certain Chinese biotechnology companies and CMOs, including these suppliers, may become subject to trade restrictions, sanctions, and other regulatory requirements by the U.S. government, which could restrict or even prohibit our ability to work with such entities, thereby potentially disrupting the supply of material to us. In January 2024, the U.S. House of Representatives introduced the BIOSECURE Act (H.R. 7085), which was subsequently amended on May 15, 2024, and the Senate advanced a substantially similar bill (S.3558), both of which would prohibit U.S. federal executive agencies from contracting with any entity where the biotechnology equipment or services of a "biotechnology company of concern" would be used in the performance of that contract. Generally speaking, a "biotechnology company of concern" is a biotechnology company that is headquartered in or subject to the jurisdiction of a foreign adversary's government and poses a threat to national security. Both the House and Senate's version of the bills name WuXi Apptec, MGI, BGI, and Complete Genomics as biotechnology companies of concern, and authorize the U.S. government to include additional Chinese biotechnology companies of concern. The new House bill also names WuXi Biologics. The current House version of the BIOSECURE Act provides a grandfathering provision with respect to a contract or agreement entered into with a designated "biotechnology company of concern" before the effective date until January 1, 2032. The pathway and timing for the BIOSECURE Act or its provisions to become law are uncertain. However, should the BIOSECURE Act or its provisions become law with the currently proposed grandfathering provisions, we expect such grandfathering provisions will allow adequate time for us to identify alternative manufacturers, if necessary. To the extent any of our counterparties, or any of their subsidiaries or affiliates, is identified as a "biotechnology company of concern," our ability to purchase services or products from, or otherwise work with, such counterparty could be restricted or even prohibited. In addition to the BIOSECURE Act, any additional executive action, legislative action or potential sanctions applicable to our current and any future suppliers could materially impact our relationship with such suppliers. U.S. executive agencies have the ability to designate entities and individuals on various governmental prohibited and restricted parties lists. Depending on the designation, potential consequences can range from a comprehensive prohibition on all transactions or dealings with designated parties, or a limited prohibition on certain types of activities, such as exports and financing activities, with designated parties. If any current or future supplier is designated on any U.S. government prohibited party lists, such designation could impact and potentially restrict our engagement with such suppliers. Such disruption could have adverse effects on the development of our product candidates and our business operations.

Any failure by a third-party manufacturer to execute on our manufacturing requirements on commercially reasonable terms and in compliance with GMP could adversely affect our business in a number of ways, including:

- an inability to initiate preclinical studies or clinical trials of product candidates;
- delays in submitting regulatory applications, or receiving marketing approvals, for product candidates;
- subjecting third-party manufacturing facilities or our manufacturing facilities to additional inspections by regulatory authorities;
- requirements to cease development or to recall batches of product candidates; and
- in the event of approval to market and commercialize any product, an inability to meet commercial demands for the product.

We are party to manufacturing agreements with a number of third-party manufacturers. We may be unable to maintain these agreements or establish any additional agreements with third-party manufacturers or to do so on acceptable terms. Even if we are able to maintain or establish agreements with third-party manufacturers, reliance on third-party manufacturers entails additional risks, including:

- failure of third-party manufacturers to comply with regulatory requirements and maintain quality assurance;
- breach of the manufacturing agreement by the third party;
- failure to manufacture according to our specifications;
- failure to manufacture according to our schedule or at all;
- misappropriation of our proprietary information, including our trade secrets and know-how; and
- termination or nonrenewal of the agreement by the third party at a time that is costly or inconvenient for us.

We may compete with third parties for access to manufacturing facilities. There are a limited number of manufacturers that operate under GMP regulations and that might be capable of manufacturing for us.

We do not currently have arrangements in place for redundant supply or a second source for all required raw materials. If our existing or future third-party manufacturers cannot perform as agreed, we may be required to replace such manufacturers and we may be unable to replace them on a timely basis or at all. Additionally, if supply from one approved manufacturer is interrupted, there could be a significant disruption in supply. The regulatory agencies may also require additional studies or trials if a new manufacturer is relied upon for commercial production. Switching manufacturers may involve substantial costs and is likely to result in a delay in our desired clinical and commercial timelines.

These factors could cause the delay of clinical trials, regulatory submissions, required approvals or commercialization of our product candidates, cause us to incur higher costs and prevent us from commercializing our products successfully. Furthermore, if our suppliers fail to meet contractual requirements, and we are unable to secure one or more replacement suppliers capable of production at a substantially equivalent cost, our clinical trials may be delayed or we could lose potential revenue.

Our current and anticipated future dependence upon third parties for the manufacture of any product candidates we develop may adversely affect our development programs and our ability to commercialize any products that receive marketing approval on a timely and competitive basis.

Our reliance on third parties requires us to share our trade secrets, which increases the possibility that a competitor or other third party will discover our trade secrets or that our trade secrets will be misappropriated or disclosed.

Because we currently rely on certain third parties to manufacture all or part of our preclinical and clinical drug supply and to perform quality testing, and because we collaborate with various third parties for the advancement of our platform and pipeline, we must, at times, share our proprietary technology and confidential information, including trade secrets, with them. We seek to protect our proprietary technology, in part, by entering into confidentiality agreements and, if applicable, material transfer agreements, collaborative research agreements, consulting agreements and other similar agreements with our collaborators, advisors, employees, consultants, and other third parties prior to beginning research or disclosing any proprietary information. These agreements typically limit the rights of the third parties to use or disclose our confidential information. Despite the contractual provisions employed when working with third parties, the need to share trade secrets and other confidential information increases the risk that such trade secrets become known by our competitors or other third parties, are inadvertently incorporated into the technology of others or are disclosed or used in violation of these agreements. Despite our efforts to protect our trade secrets, our competitors may discover our trade secrets, either through breach of these agreements, independent development or publication of information including our trade secrets by third parties. Given that our proprietary position is based, in part, on our know-how and trade secrets, a competitor's or other third party's discovery of our proprietary technology and confidential information or other unauthorized use or disclosure would impair our competitive position and may harm our business, financial condition, results of operations and prospects.

We may enter into collaborations with third parties for the research, development and commercialization of certain of the product candidates we may develop. If any such collaborations are not successful, we may not be able to capitalize on the market potential of those product candidates.

We may seek third-party collaborators for the research, development and commercialization of certain of the product candidates we may develop. If we enter into any such arrangements with any third parties, we will likely have limited control over the amount and timing of resources that our collaborators dedicate to the development or commercialization of any product candidates we may seek to develop with them. Our ability to generate revenues from these arrangements will depend on our collaborators' abilities to successfully perform the functions assigned to them in these arrangements. We cannot predict the success of any collaboration that we enter into.

Collaborations involving our research programs or any product candidates we may develop pose numerous risks to us, including the following:

- collaborators would have significant discretion in determining the efforts and resources that they will apply to these collaborations;
- collaborators may not pursue development and commercialization of any product candidates we may develop or may elect not to continue or renew development or commercialization programs based on clinical trial results, changes in the collaborator's strategic focus or available funding or external factors such as an acquisition that diverts resources or creates competing priorities;
- collaborators may delay programs, preclinical studies or clinical trials, provide insufficient funding for programs, preclinical studies or clinical trials, stop a preclinical study or clinical trial or abandon a product candidate, repeat or conduct new clinical trials or require a new formulation of a product candidate for clinical testing;
- collaborators could independently develop, or develop with third parties, products that compete directly or indirectly with any product candidates we may develop if the collaborators believe that competitive products are more likely to be successfully developed or can be commercialized under terms that are more economically attractive than ours;
- collaborators may be acquired by a third party having competitive products or different priorities, causing the emphasis on our product development or commercialization program under such collaboration to be delayed, diminished or terminated;
- collaborators with marketing and distribution rights to one or more products may not commit sufficient resources to the marketing and distribution of such product or products;
- collaborators may not properly obtain, maintain, enforce or defend our intellectual property or proprietary rights or may use our proprietary information in such a way as to invite litigation that could jeopardize or invalidate our proprietary information or expose us to potential litigation;
- disputes may arise between the collaborators and us that result in the delay or termination of the research, development, or commercialization of any product candidates we may develop or that result in costly litigation or arbitration that diverts management attention and resources;
- we may lose certain valuable rights under certain circumstances, including if we undergo a change of control;
- collaborations may be terminated and, if terminated, may result in a need for additional capital to pursue further development or commercialization of the applicable product candidates we may develop; and
- collaboration agreements may not lead to development or commercialization of product candidates in the most efficient manner or at all.

If any collaborations into which we may enter do not result in the successful development and commercialization of product candidates, or if any future collaborator terminates its agreement with us, we may not receive any future research funding or milestone or royalty payments under the collaboration. If we do not receive the funding

we expect under these agreements, our development of product candidates could be delayed, and we may need additional resources to develop product candidates. In addition, if a future collaborator terminates its agreement with us, we may find it more difficult to find a suitable replacement collaborator or attract new collaborators, and our development programs may be delayed or the perception of us in the business and financial communities could be adversely affected. All of the risks relating to product development described in this “Risk factors” section apply to the activities of our collaborators.

These relationships, or those like them, may require us to incur non-recurring and other charges, increase our near- and long-term expenditures, issue securities that dilute our existing stockholders, or disrupt our management and business. If we license rights to any product candidates we or our collaborators may develop, we may not be able to realize the benefit of such transactions if we are unable to successfully integrate them with our existing operations and company culture.

If we are not able to establish collaborations on commercially reasonable terms, we may have to alter our development and commercialization plans.

Our research programs and product candidates and the potential commercialization of any product candidates we may develop will require substantial additional cash to fund expenses. For some of the product candidates we may develop, we plan to seek collaborations with pharmaceutical and biotechnology companies for the development and potential commercialization of those product candidates.

We face significant competition in seeking high-quality collaborators, and the negotiation process is time-consuming and complex. Whether we reach a definitive agreement for a collaboration will depend, among other things, upon our assessment of the collaborator’s resources and expertise, the terms and conditions of the proposed collaboration, and the proposed collaborator’s evaluation of a number of factors. Those factors may include the design or results of preclinical studies and clinical trials, the likelihood of approval by the FDA, TGA, EC, NCAs or similar regulatory authorities outside the United States, the potential market for the subject product candidate, the costs and complexities of manufacturing and delivering such product candidate to patients, the potential of competing products, the existence of uncertainty with respect to our ownership of technology, which can exist if there is a challenge to such ownership without regard to the merits of the challenge, and industry and market conditions generally. The collaborator may also consider alternative product candidates or technologies for similar indications that may be available to collaborate on and whether such a collaboration could be more attractive than one with our company.

Collaborations are complex and time-consuming to negotiate and document. We may not be able to negotiate collaborations on a timely basis, on acceptable terms, or at all. If we are unable to do so, we may have to curtail the development of the product candidate for which we are seeking to collaborate, reduce or delay its development program or one or more of our other development programs, delay its potential commercialization, reduce the scope of any sales or marketing activities, or increase our own expenditures on the development of the product candidate.

We are dependent on third-party vendors to provide certain licenses, products and services and our business and operations, including clinical trials, could be disrupted by any problems with our significant third-party vendors.

We engage a number of third-party suppliers and service providers to supply critical goods and services, such as contract research services, contract manufacturing services and IT services. Disruptions to the business, financial stability or operations of these suppliers and service providers, including due to strikes, labor disputes or other disruptions to the workforce or to their willingness and ability to produce or deliver such goods or provide such services in a manner that satisfies the requirements put forth by the authorities, or in a manner that satisfies our own requirements, could affect our ability to develop and market our product candidates on a timely basis. If these suppliers and service providers were unable or unwilling to continue to provide their goods or services in the manner expected, or at all, we could encounter difficulty finding alternative suppliers. Even if we are able to secure appropriate alternative suppliers in a timely manner, costs for such goods or services could increase significantly. Any of these events could adversely affect our results of operations and our business.

If we or third parties, including our CROs or contract manufacturers, use hazardous and biological materials in a manner that causes injury or violates applicable law, we may be liable for damages.

Our research and development activities may involve the controlled use of potentially hazardous substances, including chemical and biological materials, by us or third parties such as our CROs and contract manufacturers. We and such third parties are subject to federal, state and local laws and regulations in the United States governing the use, manufacture, storage, handling and disposal of medical and hazardous materials. Although we believe that our and such third parties' procedures for using, handling, storing and disposing of these materials comply with legally prescribed standards, we cannot completely eliminate the risk of contamination or injury resulting from medical or hazardous materials. As a result of any such contamination or injury, we may incur liability or local, city, state or federal authorities may curtail the use of these materials and interrupt our business operations. In the event of an accident, we could be held liable for damages or penalized with fines, and the liability could exceed our resources. Compliance with applicable environmental laws and regulations is expensive, and current or future environmental regulations may impair our research, development and production efforts, which could harm our business, prospects, financial condition or results of operations.

Risks related to our intellectual property

Our rights to develop and commercialize our product candidates are subject, in part, to the terms and conditions of licenses granted to us by third parties. If we fail to comply with our obligations under these arrangements or otherwise experience disruptions to our business relationships with our current or any future licensors, we could lose such intellectual property rights that are important to our business.

We are and expect to continue to be reliant upon third-party licensors for certain patent and other intellectual property rights that are important or necessary to the development of our technology and product candidates. For example, we rely on a license from the Whitehead Institute for Biomedical Research. Our current agreement with the Whitehead Institute for Biomedical Research imposes, and we expect that any future license agreements will also impose, specified diligence, milestone payment, royalty, commercialization, development and other obligations on us and require us to meet development timelines, or to exercise diligent or commercially reasonable efforts to develop and commercialize licensed products, in order to maintain the licenses. See "Business collaboration and license agreements—Whitehead Institute patent license agreement."

Furthermore, our licensors have, or may in the future have, the right to terminate a license if we materially breach the agreement and fail to cure such breach within a specified period or in the event we undergo certain bankruptcy events. In spite of our best efforts, our current or any future licensors might conclude that we have materially breached our license agreements and might therefore terminate the license agreements. If our license agreements are terminated, we may lose our rights to develop and commercialize our product candidates and technology, lose patent protection, experience significant delays in the development and commercialization of our product candidates and technology, and incur liability for damages. If these in-licenses are terminated, or if the underlying intellectual property fails to provide the intended exclusivity, our competitors or other third parties could have the freedom to seek regulatory approval of, and to market, products and technologies identical or competitive to ours and we may be required to cease our development and commercialization of certain of our product candidates and technology. In addition, we may seek to obtain additional licenses from our licensors and, in connection with obtaining such licenses, we may agree to amend our existing licenses in a manner that may be more favorable to the licensors, including by agreeing to terms that could enable third parties, including our competitors, to receive licenses to a portion of the intellectual property that is subject to our existing licenses and to compete with any product candidates we may develop and our technology. Any of the foregoing could have a material adverse effect on our competitive position, business, financial condition, results of operations and prospects. Disputes may arise regarding intellectual property subject to a licensing agreement, including:

- the scope of rights granted under the license agreement and other interpretation-related issues;
- our or our licensors' ability to obtain, maintain and defend intellectual property and to enforce intellectual property rights against third parties;

- the extent to which our technology, product candidates and processes infringe, misappropriate or otherwise violate the intellectual property of the licensor that is not subject to the license agreement;
- the sublicensing of patent and other intellectual property rights under our license agreements;
- our diligence, development, regulatory, commercialization, financial or other obligations under the license agreement and what activities satisfy those diligence obligations;
- the inventorship and ownership of inventions and know-how resulting from the joint creation or use of intellectual property by our current or future licensors and us and our partners; and
- the priority of invention of patented technology.

In addition, future license agreements are likely to be, complex, and certain provisions in such agreements may be susceptible to multiple interpretations. The resolution of any contract interpretation disagreement that may arise could narrow what we believe to be the scope of our rights to the relevant intellectual property or technology, or increase what we believe to be our diligence, development, regulatory, commercialization, financial or other obligations under the relevant agreement. In addition, if disputes over intellectual property that we have licensed or any other dispute related to our license agreements prevent or impair our ability to maintain our current license agreements on commercially acceptable terms, we may be unable to successfully develop and commercialize the affected product candidates and technology. Any of the foregoing could have a material adverse effect on our business, financial condition, results of operations and prospects.

License agreements we may enter into in the future may be non-exclusive. Accordingly, third parties may also obtain non-exclusive licenses from such licensors with respect to the intellectual property licensed to us under such license agreements. For example, our license agreement with the Whitehead Institute for Biomedical Research grants certain co-exclusive rights to a third-party to certain patent rights generally relating to, among other things, methods of modulating gene expression by targeting certain genomic sequences. Accordingly, these license agreements may not provide us with exclusive rights to use such licensed patent and other intellectual property rights, or may not provide us with exclusive rights to use such patent and other intellectual property rights in all relevant fields of use and in all territories in which we may wish to develop or commercialize our technology and any product candidates we may develop in the future.

Moreover, some of our in-licensed patent and other intellectual property rights may in the future be subject to third party interests such as co-ownership. If we are unable to obtain an exclusive license to such third-party co-owners' interest, in such patent and other intellectual property rights, such third-party co-owners may be able to license their rights to other third parties, including our competitors, and our competitors could market competing products and technology. We or our licensors may need the cooperation of any such co-owners of our licensed patent and other intellectual property rights in order to enforce them against third parties, and such cooperation may not be provided to us or our licensors.

Additionally, we may not have complete control over the preparation, filing, prosecution, maintenance, enforcement and defense of patents and patent applications that we license from third parties. It is possible that our licensors' filing, prosecution and maintenance of the licensed patents and patent applications, enforcement of patents against infringers or defense of such patents against challenges of validity or claims of enforceability may be less vigorous than if we had conducted them ourselves, and accordingly, we cannot be certain that these patents and patent applications will be prepared, filed, prosecuted, maintained, enforced and defended in a manner consistent with the best interests of our business. If our licensors fail to file, prosecute, maintain, enforce and defend such patents and patent applications, or lose rights to those patents or patent applications, the rights we have licensed may be reduced or eliminated, our right to develop and commercialize any of our technology and any product candidates we may develop that are the subject of such licensed rights could be adversely affected and we may not be able to prevent competitors or other third parties from making, using and selling competing products.

Furthermore, our owned and in-licensed patent rights may be subject to a reservation of rights by one or more third parties. When new technologies are developed with government funding, in order to secure ownership of

patent rights related to the technologies, the recipient of such funding is required to comply with certain government regulations, including timely disclosing the inventions claimed in such patent rights to the U.S. government and timely electing title to such inventions. A failure to meet these obligations may lead to a loss of rights or the unenforceability of relevant patents or patent applications. In addition, the U.S. government may have certain rights in such patent rights, including a non-exclusive license authorizing the U.S. government to use the invention or to have others use the invention on its behalf. If the U.S. government decides to exercise these rights, it is not required to engage us as its contractor in connection with doing so. The U.S. government's rights may also permit it to disclose the funded inventions and technology, which may include our confidential information, to third parties and to exercise march-in rights to use or allow third parties to use the technology that was developed using U.S. government funding. The U.S. government may exercise its march-in rights if it determines that action is necessary because we or our licensors failed to achieve practical application of the U.S. government-funded technology, because action is necessary to alleviate health or safety needs, to meet requirements of federal regulations, or to give preference to U.S. industry. In addition, our rights in such U.S. government-funded inventions may be subject to certain requirements to manufacture any product candidates we may develop embodying such inventions in the United States. Any of the foregoing could harm our business, financial condition, results of operations and prospects significantly.

If we or our licensors are unable to obtain, maintain, enforce and adequately protect our intellectual property rights with respect to our product candidates and technology, or if the scope of any patent or other intellectual property protection obtained is not sufficiently broad, our competitors could develop and commercialize products and technology similar or identical to ours, and our ability to successfully develop and commercialize our product candidates and technology may be adversely affected.

Our success depends in large part on our and our licensors' ability to obtain, maintain, enforce and adequately protect our intellectual property rights through patents, trade secrets, and trademarks in the United States and other jurisdictions with respect to our product candidates and our technology, as well as our ability to operate our business without infringing, misappropriating or otherwise violating the intellectual property rights of others.

Given the early stage of development of our product candidates and technology, our patent portfolio with respect to certain aspects of our product candidates and technology is similarly at a very early stage. For example, we do not currently own or in-license any issued patents directed to the composition of matter, or methods of use, of any of the product candidates that we have thus far developed using our RAP Platform. We have filed and intend to continue filing patent applications directed to the compositions of matter, and methods of use, of our current and future product candidates. Composition of matter patents for pharmaceutical product candidates often provide a strong form of intellectual property protection for those types of products, as such patents provide protection without regard to their method of use. However, we cannot be certain that any claims in our patent applications directed to the composition of matter of our product candidates will be considered patentable by the United States Patent and Trademark Office, or the USPTO, or by patent offices in foreign countries, or that, if issued, the claims in any such patents, if challenged, will be adjudicated to be not invalid and enforceable by courts and administrative bodies in the United States or foreign countries. Further, if issued, any composition of matter patents covering our product candidates may expire at such a date that competitors may not be prevented from developing, making and marketing a product identical to our product candidates after expiration of any applicable regulatory exclusivities. Method of use patents protect the use of a product for the specified method or indication. This type of patent does not prevent a competitor from making and marketing a product identical to our product candidate for an indication that is outside the scope of the patented methods of use. Moreover, even if competitors do not actively promote their product for indications covered by our patents, clinicians may prescribe these competitor products "off-label" for uses that are covered by our method of use patents. Although off-label prescriptions may infringe or contribute to the infringement of method of use patents, the practice is common and such infringement is difficult to prevent or prosecute. To establish our proprietary position, we own and have in-licensed certain intellectual property rights, and we and our licensors have filed and may file provisional and non-provisional patent applications in the United States or abroad relating to our product candidates and certain technologies that are important to our business. We may in the future also license or purchase intellectual property rights from others. Our ability to stop third parties from making, using, selling, marketing, offering to

sell, importing and commercializing our product candidates and technology is dependent upon the extent to which we have rights under valid and enforceable patents and other intellectual property rights that cover our product candidates and technology. We cannot predict whether or when our owned or licensed pending and future patent applications will result in the issuance of patents that provide us with any competitive advantage. If we or our licensors are unable to obtain, maintain, defend and enforce patents and other intellectual property rights with respect to our product candidates and technology, our business, financial condition, results of operations and prospects could be materially harmed.

The patent prosecution process is expensive, time-consuming and complex, and we and our licensors may not be able to file, prosecute, maintain, defend, enforce or license all necessary or desirable patent applications and patents at a reasonable cost or in a timely manner. In addition, we may not pursue or obtain patent protection in all relevant markets. U.S. provisional patent applications are not eligible to become issued patents until, among other things, we or our licensors file a non-provisional patent application within 12 months of the filing of one or more of our related provisional patent applications. Any failure to file a non-provisional patent application within this timeline could cause us or our licensors to lose our priority date with respect to the provisional patent application and any patent protection on the inventions disclosed in the provisional patent applications. We and our licensors may not be able to obtain, maintain or defend patents and patent applications due to the subject matter claimed in such patents and patent applications being in the public domain. It is also possible that we will fail to identify patentable aspects of our research and development output before it is too late to obtain patent protection. Although we enter into non-disclosure and confidentiality agreements with parties who have access to confidential or patentable aspects of our research and development output, such as our employees, corporate collaborators, external scientific collaborators, contract research organizations, contract manufacturers, consultants, advisors and other third parties, any of these parties may breach these agreements and disclose such output before a patent application is filed, thereby potentially jeopardizing our ability to seek patent protection.

The patent position of biotechnology and pharmaceutical companies generally is highly uncertain, involves complex legal and factual questions and has, in recent years, been the subject of much litigation. As a result, the issuance, scope, validity, enforceability and commercial value of patent rights are highly uncertain. Our owned and licensed pending and future patent applications may not result in patents being issued which protect our technology, our product candidates, or which effectively prevent others from commercializing competitive technologies and products or otherwise provide any competitive advantage. In fact, our owned or licensed patent applications may not issue as patents at all, and even if such patent applications do issue as patents, they may not issue in a form, or with a scope of claims, that will provide us with any meaningful protection, prevent others from competing with us, or otherwise provide us with any competitive advantage. In addition, the scope of the invention claimed in a patent application can be significantly reduced before a patent is issued, and the scope of claims of an issued patent can be reinterpreted after issuance. Any patents that eventually issue may be challenged, narrowed or invalidated by third parties. Moreover, changes in either the patent laws or interpretation of the patent laws in the United States and other jurisdictions may diminish the value of our patent rights or narrow the scope of our patent protection. Consequently, we do not know whether any of our product candidates will be protectable or remain protected by valid and enforceable patent rights. Furthermore, our competitors or other third parties may be able to circumvent our owned or licensed patents by developing similar or alternative technologies or products in a non-infringing manner.

Third parties have developed technologies that may be related or competitive to our own technologies and product candidates and may have filed or may file patent applications, or may have obtained issued patents, claiming inventions that may overlap or conflict with those claimed in our owned or licensed patent applications or issued patents. We may not be aware of all third-party intellectual property rights potentially relating to our current and future product candidates and technology. Publications of discoveries in the scientific literature often lag the actual discoveries, and patent applications in the United States and other jurisdictions are typically not published until 18 months after filing or, in some cases, not at all. Therefore, we cannot know for certain whether the inventors of our owned or licensed patents and patent applications were the first to make the inventions claimed in any owned or licensed patents or pending patent applications, or that we were the first to

file for patent protection of such inventions. If a third party can establish that we or our licensors were not the first to make or the first to file for patent protection of such inventions, our owned or licensed patent applications may not issue as patents and even if issued, may be challenged and invalidated or ruled unenforceable.

Furthermore, patents have a limited lifespan. In the United States, the expiration of a patent is generally 20 years from the earliest date of filing of the first non-provisional patent application to which the patent claims priority. Patent term adjustments and extensions may be available; however, the overall term of a patent, and the protection it affords, is limited. Given the amount of time required for the development, testing and regulatory review of new product candidates, patents protecting such product candidates might expire before or shortly after such product candidates are commercialized. As a result, our owned and licensed patent portfolio and other intellectual property rights may not provide us with sufficient rights to exclude others from commercializing products similar or identical to ours. Any of the foregoing could have a material adverse effect on our competitive position, business, financial condition, results of operations and prospects.

Third parties may initiate legal proceedings alleging that we are infringing, misappropriating or otherwise violating their intellectual property rights, the outcome of which would be uncertain and could harm our business.

Our commercial success depends upon our ability and the ability of our collaborators to develop, manufacture, commercialize, market and sell our product candidates and use our proprietary technology without infringing, misappropriating or otherwise violating the intellectual property rights of third parties. The biotechnology and pharmaceutical industries are characterized by extensive and complex litigation regarding patents and other intellectual property rights. We may become party to, or be threatened with, adversarial proceedings or litigation in which third parties may assert infringement, misappropriation or other violation claims against us, alleging that our product candidates, compositions, technology, or methods are covered by their patents. Given the vast number of patents and other intellectual property in our field of technology, we cannot be certain or guarantee that we do not infringe, misappropriate or otherwise violate patents or other intellectual property. Other companies and institutions have filed, and continue to file, patent applications that may be related to our product candidates, compositions, technology and methods. Some of these patent applications have already been allowed or issued and others may issue in the future. Since this area is competitive and of strong interest to pharmaceutical and biotechnology companies, there will likely be additional patent applications filed and additional patents granted in the future, as well as additional research and development programs expected in the future. Numerous U.S. and foreign issued patents and pending patent applications, which are owned by third parties, exist in the fields in which we are developing our product candidates and technology. As the biotechnology and pharmaceutical industries expand and more patents are issued, the risk increases that we may be subject to claims of infringement of the patent rights of third parties. If a patent holder believes the manufacture, use, sale or importation of any product candidates we may develop or our technology infringes its patent, the patent holder may sue us even if we have licensed other patent rights for our product candidates or technology.

We are aware of certain U.S. and foreign issued patents and pending patent applications that claim subject matter that relates to certain of our product candidates and technology. Although we believe that their claims are invalid and/or not infringed, such third parties may assert these patents against us in litigation. The outcome of any such litigation is uncertain and, even if we prevail, the costs of such litigation could have a material adverse effect on our financial position, distract key personnel from the continued development of our business, and adversely affect our ability to enter or maintain commercial relationships with collaborators, clients, customers or other third parties. If we are unsuccessful in such litigation, we could be prevented from commercializing products or could be required to take licenses from such third parties, which may not be available on commercially reasonable terms, if at all.

It is also possible that we have failed to identify relevant third-party issued patents or patent applications. Because patent applications can take many years to issue, may be confidential for 18 months or more after filing and can be revised before issuance, there may be applications now pending which may later result in issued patents that may be infringed by the manufacture, use, sale or importation of our product candidates, compositions, or our technology and we may not be aware of such patents. Furthermore, applications filed before November 29, 2000 and certain applications filed after that date that will not be filed outside the United States may remain

confidential until a patent issues. Moreover, it is difficult for industry participants, including us, to identify all third-party patent rights that may be relevant to our product candidates and our technology because patent searching is imperfect due to differences in terminology among patents, incomplete databases and the difficulty in assessing the meaning of patent claims. We may fail to identify relevant patents or patent applications or may identify pending patent applications of potential interest but incorrectly predict the likelihood that such patent applications may issue with claims of relevance to our technology. In addition, we may incorrectly conclude that a third-party patent is invalid, unenforceable or not infringed by our activities. Additionally, pending patent applications that have been published can, subject to certain limitations, be later amended in a manner that could cover our technology product candidates, compositions, or methods.

Third parties may assert infringement claims against us based on existing patents or patents that may be granted in the future, regardless of the merit of the claim. There is a risk that third parties may choose to engage in litigation with us to enforce or to otherwise assert their patent rights against us. Even if we believe such claims are without merit, a court of competent jurisdiction could hold that these third-party patents are not invalid, enforceable and infringed, which could adversely affect our ability to commercialize our product candidates or technology covered by the asserted third-party patents. In order to successfully challenge the validity of any such U.S. patent in federal court, we would need to overcome a presumption of validity. As this burden is a high one requiring us to present clear and convincing evidence as to the invalidity of any such U.S. patent claim, there is no assurance that a court of competent jurisdiction would invalidate the claims of any such U.S. patent. If we are found to infringe a third party's valid and enforceable intellectual property rights, we could be required to obtain a license from such third party to continue developing, manufacturing and marketing our product candidates and technology. However, we may not be able to obtain any required license on commercially reasonable terms or at all. Even if we were able to obtain a license, it could be non-exclusive, thereby giving our competitors and other third parties access to the same technologies licensed to us, and it could require us to make substantial licensing and royalty payments. We could be forced, including by court order, to cease developing, manufacturing and commercializing the infringing technology or product candidates. In addition, we could be found liable for monetary damages, including treble damages and attorneys' fees, if we are found to have willfully infringed a patent or other intellectual property right. A finding of infringement could prevent us from manufacturing and commercializing our product candidates or force us to cease some of our business operations, which could harm our business. In addition, we may be forced to redesign our product candidates or technology, seek new regulatory approvals, and indemnify third parties pursuant to contractual agreements. Claims that we have misappropriated the confidential information or trade secrets of third parties could have a similar negative impact on our business, financial condition, results of operations and prospects.

We may be involved in lawsuits to protect or enforce our patents or other intellectual property or the intellectual property of our licensors, which could be expensive, time-consuming, and unsuccessful.

Competitors may infringe our patents or other intellectual property rights, or the intellectual property rights of our licensors. To counter infringement or unauthorized use, we may be required to file infringement claims, which can be expensive and time-consuming and divert the time and attention of our management and scientific personnel. Our and our licensors' pending patent applications cannot be enforced against third parties practicing the technology claimed in such applications unless and until a patent issues from such applications. In addition, in an infringement proceeding or a declaratory judgment action, a court may decide that one or more of our or our licensors' patents is not valid or is unenforceable, or may refuse to stop the other party from using the technology at issue on the grounds that our patents do not cover the technology in question. An adverse result in any litigation or defense proceeding could put one or more of our or our licensors' patents at risk of being invalidated, held unenforceable, or interpreted narrowly and could put our or our licensors' patent applications at risk of not issuing. Defense of these claims, regardless of their merit, would involve substantial litigation expense and would be a substantial diversion of employee resources from our business.

Even if we establish infringement, the court may decide not to grant an injunction against further infringing activity and instead award only monetary damages, which may or may not be an adequate remedy. Furthermore, because of the substantial amount of discovery required in connection with intellectual property litigation,

there is a risk that some of our confidential information could be compromised by disclosure during litigation. In addition, there could be public announcements of the results of hearings, motions or other interim proceedings or developments. If securities analysts or investors perceive these results to be negative, it could have a substantial adverse effect on the price of our common stock. Moreover, there can be no assurance that we will have sufficient financial or other resources to file and pursue such infringement claims, which typically last for years before they are concluded. Even if we ultimately prevail in such claims, the monetary cost of such litigation and the diversion of the attention of our management and scientific personnel could outweigh any benefit we receive as a result of any proceedings. Any of the foregoing may have a material adverse effect on our business, financial condition, results of operations and prospects.

We may not be able to protect our intellectual property rights throughout the world.

Filing, prosecuting, maintaining, enforcing and defending patents and other intellectual property rights relating to our technology and product candidates in all jurisdictions throughout the world would be prohibitively expensive, and accordingly, our intellectual property rights in some jurisdictions outside the United States could be less extensive than those in the United States. In some cases, we or our licensors may not be able to obtain patent or other intellectual property protection for certain technology and product candidates outside the United States. In addition, the laws of some foreign jurisdictions do not protect intellectual property rights to the same extent as federal and state laws in the United States. Consequently, we and our licensors may not be able to obtain issued patents or other intellectual property rights covering our product candidates and our technology in all jurisdictions outside the United States and, as a result, we may not be able to prevent third parties from practicing our and our licensors' inventions in all countries outside the United States, or from selling or importing products made using our inventions in and into the United States or other jurisdictions. Third parties may use our technologies in jurisdictions where we and our licensors have not pursued and obtained patent or other intellectual property protection to develop their own products and, further, may export otherwise infringing, misappropriating or violating products to territories where we have patent or other intellectual property protection, but enforcement is not as strong as that in the United States. These products may compete with our product candidates and our technology and our or our licensors' patents or other intellectual property rights may not be effective or sufficient to prevent them from competing.

Additionally, many companies have encountered significant problems in protecting and defending intellectual property rights in foreign jurisdictions. The legal systems of certain jurisdictions, particularly certain developing countries, do not favor the enforcement of patents, trade secrets and other intellectual property protection, particularly those relating to biotechnology and pharmaceutical products, which could make it difficult for us to stop the infringement, misappropriation or other violation of our or our licensor's patent and other intellectual property rights or marketing of competing products in violation of our intellectual property rights generally. Proceedings to enforce our or our licensors' patent and other intellectual property rights in foreign jurisdictions could result in substantial costs and divert our efforts and attention from other aspects of our business, could put our or our licensor's patent and other intellectual property rights at risk of being invalidated or interpreted narrowly and our patent applications at risk of not issuing and could provoke third parties to assert claims against us. We or our licensors may not prevail in any lawsuits that we or our licensors initiate and the damages or other remedies awarded, if any, may not be commercially meaningful. Accordingly, our efforts to enforce our intellectual property rights around the world may be inadequate to obtain a significant commercial advantage from the intellectual property that we develop or license.

As another example, the complexity and uncertainty of European patent laws have also increased in recent years. In Europe, a new unitary patent system went into effect on June 1, 2023, which significantly impacts European patents, including those granted before the introduction of such system. Under the unitary patent system, European applications have the option, upon grant of a patent, of becoming a Unitary Patent which will be subject to the jurisdiction of the Unitary Patent Court, or the UPC. Existing European patents and published applications may be opted out of the jurisdiction of the UPC at any time before the end of a transitional period (at least seven years from the UPC Agreement which went into effect on June 1, 2023), unless an action has already been brought before the UPC in which case an opt-out request cannot be filed. As the UPC is a new court system, there is no precedent

for the court, increasing the uncertainty of any litigation. Patents granted before the implementation of the UPC will have the option of opting out of the jurisdiction of the UPC and remaining as national patents in the UPC countries. Patents that remain under the jurisdiction of the UPC will be potentially vulnerable to a single UPC-based revocation challenge that, if successful, could invalidate the patent in all countries who are signatories to the UPC. We cannot predict with certainty the long-term effects of any potential changes.

Many jurisdictions have compulsory licensing laws under which a patent owner may be compelled to grant licenses to third parties. In addition, many jurisdictions limit the enforceability of patents against government agencies or government contractors. In these jurisdictions, the patent owner may have limited remedies, which could materially diminish the value of such patents. If we or any of our licensors is forced to grant a license to third parties with respect to any patents relevant to our business, our competitive position may be impaired, and our business, financial condition, results of operations and prospects may be adversely affected.

Obtaining and maintaining our patent protection depends on compliance with various procedural, document submission, fee payment and other requirements imposed by governmental patent agencies, and our patent protection could be reduced or eliminated for non-compliance with these requirements.

Periodic maintenance fees, renewal fees, annuity fees and various other government fees on patents and/or patent applications will be due to be paid to the USPTO and foreign government patent agencies over the lifetime of our owned or licensed patent rights. We rely on our outside counsel and other professionals or our licensing partners to pay these fees due to the USPTO and foreign government patent agencies. The USPTO and foreign government patent agencies also require compliance with several procedural, documentary and other similar provisions during the patent application process. We rely on our outside counsel and other professionals to help us comply and we are also dependent on our licensing partners to take the necessary action to comply with these requirements with respect to our licensed intellectual property. In many cases, an inadvertent lapse can be cured by payment of a late fee or by other means in accordance with the applicable rules. There are situations, however, in which non-compliance can result in abandonment, loss of priority or lapse of the patent or patent application, resulting in partial or complete loss of patent rights in the relevant jurisdiction. In such an event, potential competitors might be able to enter the market and this circumstance could have a material adverse effect on our competitive position, business, financial condition, results of operations and prospects.

Changes in patent law in the United States and in foreign jurisdictions could diminish the value of patents in general, thereby impairing our ability to protect our product candidates and our technology.

As is the case with other biotechnology and pharmaceutical companies, our success is heavily dependent on intellectual property, particularly patents. Obtaining, defending and enforcing patents in the biotechnology and pharmaceutical industry involves both technological and legal complexity, and is therefore costly, time-consuming and inherently uncertain. Changes in either the patent laws or their interpretation in the United States and in foreign jurisdictions, including patent reform legislation such as the Leahy-Smith America Invents Act, or the" Leahy-Smith Act", signed into law on September 16, 2011, could increase these uncertainties and costs. The Leahy-Smith Act includes a number of significant changes to U.S. patent law. These changes include provisions that affect the way patent applications are prosecuted, redefine prior art, provide more efficient and cost-effective avenues for competitors to challenge the validity of patents, and enable third-party submission of prior art to the USPTO during patent prosecution and additional procedures to attack the validity of a patent at USPTO-administered post-grant proceedings, including post-grant review, *inter partes* review, and derivation proceedings. In addition, the Leahy-Smith Act transformed the U.S. patent system into a "first-to-file" system. The first-to-file provisions, however, became effective on March 16, 2013. As such, the Leahy-Smith Act and its implementation could increase the uncertainties and costs surrounding the prosecution of our owned and in-licensed patent applications and the enforcement or defense of our owned and in-licensed issued patents and issued patents we may own or in-license in the future, all of which could have a material adverse effect on our business, financial condition, results of operations and prospects.

Moreover, recent U.S. Supreme Court rulings have narrowed the scope of patent protection available in certain circumstances and weakened the rights of patent owners in certain situations. In addition to increasing uncertainty

with regard to our or our licensors' ability to obtain patents in the future, this combination of events has created uncertainty with respect to the validity and enforceability of patents once obtained. Depending on future actions by the U.S. Congress, the federal courts and the USPTO, the laws and regulations governing patents could change in unpredictable ways that could have a material adverse effect on our patent rights and our ability to protect, defend and enforce our patent rights in the future.

If we obtain any patents covering our product candidates or our technology, they could nonetheless be found invalid or unenforceable if challenged in court or before administrative bodies in the United States or abroad.

The issuance of a patent is not conclusive as to its inventorship, scope, validity or enforceability. Our owned and licensed patent rights, including any patent of our owned or in-licensed patent applications that may issue in the future, may be subject to priority, validity, inventorship and enforceability disputes. If we or our licensors are unsuccessful in any of these proceedings, such patent rights may be narrowed, invalidated or held unenforceable, we may be required to obtain licenses from third parties, which may not be available on commercially reasonable terms or at all, or we may be required to cease the development, manufacture and commercialization of one or more of our product candidates. Any of the foregoing could have a material adverse effect on our business, financial condition, results of operations and prospects.

If we or one of our licensors initiate legal proceedings against a third party to enforce a patent covering our product candidates or our technology, the defendant could counterclaim that the patent is invalid and/or unenforceable. In patent litigation in the United States, defendant counterclaims alleging invalidity and/or unenforceability are commonplace, and there are numerous grounds upon which a third party can assert invalidity or enforceability of a patent. Grounds for a validity challenge could be an alleged failure to meet any of several statutory requirements, including lack of novelty, obviousness, lack of written description or non-enablement. Grounds for an unenforceability assertion could be an allegation that someone connected with prosecution of the patent withheld information material to patentability from the USPTO, or made a misleading statement, during prosecution. Third parties also may raise similar claims before administrative bodies in the United States or abroad, even outside the context of litigation. Such mechanisms include re-examination, interference proceedings, derivation proceedings, post grant review, *inter partes* review and equivalent proceedings in foreign jurisdictions, such as opposition, invalidation and revocation proceedings. Such proceedings could result in the revocation or cancellation of or amendment to our or our licensors' patents in such a way that they no longer cover our product candidates or our technology or prevent third parties from competing with our product candidates or our technology. The outcome following legal assertions of invalidity and unenforceability is unpredictable. With respect to the validity question, for example, we cannot be certain that there is no invalidating prior art, of which we, our licensing partners, or the patent examiners were unaware during prosecution. If a third party were to prevail on a legal assertion of invalidity or unenforceability, we could lose at least part, and perhaps all, of any patent protection we may eventually obtain relating to our f our product candidates or technology. Such a loss of patent protection could have a material adverse effect on our business, financial condition, results of operations and prospects.

If we do not obtain patent term extension and data exclusivity for our product candidates, our business may be materially harmed.

Depending upon the timing, duration and specifics of any FDA marketing approval of our current or future product candidates that we may receive, one or more of our owned or in-licensed U.S. patents that we may obtain in the future may be eligible for limited patent term extension under the Drug Price Competition and Patent Restoration Act, or the "Hatch-Waxman Amendments". The Hatch-Waxman Amendments permit a patent extension term of up to five years as compensation for patent term lost during the FDA regulatory review process. A patent term extension cannot extend the remaining term of a patent beyond a total of 14 years from the date of product approval, only one patent may be extended and only those claims covering the approved product, a method for using it, or a method for manufacturing it may be extended. The application for the extension must be submitted prior to the expiration of the patent for which extension is sought. A patent that covers multiple products for which approval is sought can only be extended in connection with one of the approvals. However, we may not be granted an extension because of, for example, failing to exercise due diligence during the testing phase or

regulatory review process, failing to apply within applicable deadlines, failing to apply prior to expiration of relevant patents or otherwise failing to satisfy applicable requirements. Moreover, the applicable time period or the scope of patent protection afforded could be less than we request. In addition, to the extent we wish to pursue patent term extension based on a patent that we in-license from a third party, we would need the cooperation of that third party. If we are unable to obtain patent term extension or the term of any such extension is less than we request, our competitors may obtain approval of competing products following our patent expiration, and our revenue could be reduced. Any of the foregoing could have a material adverse effect on our business, financial condition, results of operations and prospects.

We may be subject to claims challenging the inventorship or ownership of our patent and other intellectual property rights.

We or our licensors may be subject to claims that former employees, collaborators or other third parties have an interest in our owned or in-licensed patent rights, trade secrets or other intellectual property as an inventor or co-inventor. For example, we or our licensors may have inventorship disputes arise from conflicting obligations of employees, consultants or others who are involved in developing our product candidates or our technology. Litigation may be necessary to defend against these and other claims challenging inventorship or our or our licensors' ownership of our owned or in-licensed patent rights, trade secrets or other intellectual property. If we or our licensors fail in defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights, such as exclusive ownership of or right to use intellectual property that is important to our product candidates or our technology. Even if we are successful in defending against such claims, litigation could result in substantial costs and be a distraction to management and other employees. Any of the foregoing could have a material adverse effect on our business, financial condition, results of operations and prospects.

If we are unable to protect the confidentiality of our trade secrets, our business and competitive position would be harmed.

In addition to seeking patents for some of our product candidates, we also rely on trade secrets, including unpatented know-how, technology and other proprietary information, to maintain our competitive position. We seek to protect these trade secrets, in part, by entering into non-disclosure and confidentiality agreements with parties who have access to them, such as our employees, corporate collaborators, external scientific collaborators, contract manufacturers, consultants, advisors, and other third parties. We also enter into confidentiality and invention or patent assignment agreements with our employees and consultants. Despite these efforts, any of these parties may breach the agreements and disclose our proprietary information, including our trade secrets, and we may not be able to obtain adequate remedies for such breaches. Enforcing a claim that a party illegally disclosed or misappropriated a trade secret is difficult, expensive, and time consuming, and the outcome is unpredictable. If we are unable to prevent unauthorized material disclosure of our intellectual property to third parties, we may not be able to establish or maintain a competitive advantage, which could materially adversely affect our business, operating results and financial condition. If we choose to go to court to stop a third party from using any of our trade secrets, we may incur substantial costs. These lawsuits may consume our time and other resources even if we are successful. In addition, some courts inside and outside the United States are less willing or unwilling to protect trade secrets. As a result, we may encounter significant problems in protecting and defending our intellectual property both in the United States and abroad. If any of our trade secrets were to be lawfully obtained or independently developed by a competitor, we would have no right to prevent them from using that technology or information to compete with us. If any of our trade secrets were to be disclosed to or independently developed by a competitor, our competitive position would be harmed.

We may not be successful in obtaining necessary rights to product candidates we may develop through acquisitions and in-licenses.

We currently have rights to certain intellectual property through licenses from third parties. Because our product candidates and technology may require the use of additional intellectual property rights held by third parties, the growth of our business likely will depend, in part, on our ability to acquire, in-license or use these intellectual property rights. We may be unable to acquire or in-license any intellectual property rights related to

compositions, methods of use, processes or other technology from third parties that we identify as necessary to our business operations on commercially reasonable terms, if at all. We may need to cease use of the compositions, methods of use, processes or other technology covered by such intellectual property rights, and may need to seek to develop alternative approaches that do not infringe on such intellectual property rights which may entail significant costs and development delays, even if we are able to develop such alternatives, which may not be feasible. Even if we are able to acquire or in-license any such necessary intellectual property, it could be on non-exclusive terms, thereby giving our competitors and other third parties access to the same intellectual property licensed to us, and the applicable licensors could require us to make substantial licensing and royalty payments. The licensing or acquisition of third-party intellectual property rights is a competitive area, and several more established companies may pursue strategies to license or acquire third-party intellectual property rights that we may consider attractive or necessary. These established companies may have a competitive advantage over us due to their size, capital resources and greater clinical development and commercialization capabilities. In addition, companies that perceive us to be a competitor may be unwilling to assign or license rights to us. We also may be unable to license or acquire third-party intellectual property rights on terms that would allow us to make an appropriate return on our investment.

We sometimes collaborate with non-profit and academic institutions to accelerate our preclinical research or development under written agreements with these institutions. In certain cases, these institutions provide us with an option to negotiate a license to any of the institution's rights in technology resulting from the collaboration. Regardless of such option, we may be unable to negotiate a license within the specified timeframe or under terms that are acceptable to us. If we are unable to do so, the institution may offer the intellectual property rights to third parties, potentially blocking our ability to pursue our research programs and develop and commercialize our product candidates.

If we are unable to successfully obtain rights to required third-party intellectual property rights or maintain the existing intellectual property rights we have licensed, we may be required to expend significant time and resources to redesign our product candidates, the methods for manufacturing them, or to develop or license replacement technology, all of which may not be feasible on a technical or commercial basis. If we are unable to do so, we may be unable to develop or commercialize the affected product candidates or technology, which could have a material adverse effect on our business, financial condition, results of operations and prospects.

We may be subject to claims asserting that our employees, consultants, independent contractors or advisors have wrongfully used or disclosed alleged trade secrets of their former employers or other third parties or claims asserting ownership of what we regard as our own intellectual property.

We have received, and will continue to receive, confidential and proprietary information from third parties. In addition, many of our employees, consultants, independent contractors or advisors are currently, or were previously, employed at universities or other biotechnology or pharmaceutical companies, including our competitors or potential competitors. Although we try to ensure that our employees, consultants, independent contractors and advisors do not use the proprietary information or know-how of others in their work for us, we may be subject to claims that these individuals or we have deliberately, inadvertently or otherwise used or disclosed intellectual property, including trade secrets or other proprietary information, of any such individual's current or former employers, competitors or other third parties, or to claims that we have improperly used or obtained such trade secrets or other proprietary information. We may be subject to claims that we or our employees, consultants, independent contractors or advisors have inadvertently or otherwise used or disclosed confidential information of third parties. Litigation may be necessary to defend against these claims. If we fail in defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights or be required to obtain licenses to such intellectual property rights, which may not be available on commercially reasonable terms or at all. An inability to incorporate such intellectual property rights would harm our business and may prevent us from successfully commercializing any product candidates or technology we may develop or at all. In addition, we may lose personnel as a result of such claims and any such litigation or the threat thereof may adversely affect our ability to hire employees or contract with consultants, independent contractors or advisors. A loss of key personnel or their work product could hamper or prevent our ability to commercialize any

product candidates and our technology, which would have a material adverse effect on our business, results of operations, financial condition and prospects. Even if we are successful in defending against such claims, litigation could result in substantial costs and be a distraction to our scientific and management personnel.

In addition, while it is our policy to require our employees and consultants who may be involved in the conception or development of intellectual property to execute agreements assigning such intellectual property to us, we may be unsuccessful in executing such an agreement with each party who, in fact, conceives or develops intellectual property that we regard as our own. Moreover, even when we obtain agreements assigning intellectual property to us, the assignment of intellectual property rights may not be self-executing or the assignment agreements may be breached, and we may be forced to bring claims against third parties, or defend claims that they may bring against us, to determine the ownership of what we regard as our intellectual property. Furthermore, individuals executing agreements with us may have pre-existing or competing obligations to a third party, such as an academic institution, and thus an agreement with us may be ineffective in perfecting ownership of inventions developed by that individual. Disputes about the ownership of intellectual property that we own may have a material adverse effect on our business, financial condition, results of operations and prospects.

If our trademarks and trade names are not adequately protected, then we may not be able to build name recognition in our markets of interest and our business may be adversely affected.

Our trademarks or trade names may be challenged, infringed, circumvented or declared generic or determined to be infringing on other marks. We rely on both registration and common law protection for our trademarks. During trademark registration proceedings, we may receive rejections. Although we would be given an opportunity to respond to those rejections, we may be unable to overcome such rejections. In addition, in the USPTO and in comparable agencies in many foreign jurisdictions, third parties are given an opportunity to oppose pending trademark applications and to seek to cancel registered trademarks. Opposition or cancellation proceedings may be filed against our trademarks, and our trademarks may not survive such proceedings. As a means to enforce our trademark rights and prevent infringement, we may be required to file trademark claims against third parties or initiate trademark opposition proceedings. This can be expensive and time-consuming, particularly for a company of our size. We may not be able to protect our rights to these trademarks and trade names, which we need to build name recognition among potential partners, clients or customers in our markets of interest. At times, third parties may adopt trade names or trademarks similar to ours, thereby impeding our ability to build brand identity and possibly leading to market confusion. In addition, there could be potential trade name or trademark infringement claims brought by owners of other registered trademarks or trademarks that incorporate variations of our registered or unregistered trademarks or trade names. Over the long term, if we are unable to establish name recognition based on our trademarks and trade names, then we may not be able to compete effectively and our business may be adversely affected. Our efforts to enforce or protect our proprietary rights related to trademarks, trade secrets, domain names, copyrights or other intellectual property may be ineffective and could result in substantial costs and diversion of resources. Any of the foregoing could have a material adverse effect on our business, financial condition, results of operations or prospects.

Intellectual property rights do not necessarily address all potential threats.

The degree of future protection afforded by our intellectual property rights is uncertain because intellectual property rights have limitations and may not adequately protect our business or permit us to maintain our competitive advantage. For example:

- others may be able to make products that are similar to our product candidates or that utilize similar technology but that are not covered by the intellectual property rights, including the claims of the patents, that we own or license currently or in the future;
- we, or our current or future license partners or collaborators, might not have been the first to make the inventions covered by the issued patent or pending patent application that we own or license currently or in the future;
- we, or our current or future license partners or collaborators, might not have been the first to file patent applications covering certain of our or their inventions;

- others may independently develop similar or alternative technologies or duplicate any of our technologies without infringing, misappropriating or otherwise violating our owned or licensed intellectual property rights;
- it is possible that our current or future owned or licensed pending patent applications will not lead to issued patents;
- issued patents that we hold rights to may be held invalid or unenforceable, including as a result of legal challenges by third parties;
- third parties might conduct research and development activities in jurisdictions where we do not have patent or other intellectual property rights and then use the information learned from such activities to develop competitive products for sale in our major commercial markets;
- we may not develop additional proprietary technologies that are patentable;
- the patents or other intellectual property rights of others may have an adverse effect on our business; and
- we may choose not to file a patent for certain trade secrets or know-how, and a third party may subsequently file a patent covering such intellectual property.

Should any of these events occur, they could significantly harm our business, financial condition, results of operations and prospects.

Intellectual property discovered through government funded programs may be subject to federal regulations such as "march-in" rights, certain reporting requirements and a preference for United States-based companies. Compliance with such regulations may limit our exclusive rights and limit our ability to contract with non-United States manufacturers.

We have in-licensed certain patents and patent applications that were generated through the use of United States government funding or grants, and we may acquire or license in the future intellectual property rights that have been generated through the use of United States government funding or grants. Pursuant to the Bayh-Dole Act of 1980, the United States government has certain rights in inventions developed with government funding. These United States government rights include a non-exclusive, non-transferable, irrevocable worldwide license to use inventions for any governmental purpose. In addition, the United States government has the right, under certain limited circumstances, to require us to grant exclusive, partially exclusive, or non-exclusive licenses to any of these inventions to a third party if it determines that: (1) adequate steps have not been taken to commercialize the invention; (2) government action is necessary to meet public health or safety needs; or (3) government action is necessary to meet requirements for public use under federal regulations (also referred to as "march-in" rights). If the United States government exercised its march-in rights in our current or future intellectual property rights generated through the use of United States government funding or grants, we could be forced to license or sublicense intellectual property developed by us or that we license on terms unfavorable to us, and there can be no assurance that we would receive compensation from the United States government for the exercise of such rights. The United States government also has the right to take title to these inventions if the grant recipient fails to disclose the invention to the government or fails to file an application to register the intellectual property within specified time limits. Intellectual property generated under a government funded program is also subject to certain reporting requirements, compliance with which may require us to expend substantial resources. In addition, the United States government requires that any products embodying any of these inventions or produced through the use of any of these inventions be manufactured substantially in the United States. This preference for United States industry may be waived by the federal agency that provided the funding if the owner or assignee of the intellectual property can show that reasonable but unsuccessful efforts have been made to grant licenses on similar terms to potential licensees that would be likely to manufacture substantially in the United States or that under the circumstances domestic manufacture is not commercially feasible. This preference for United States industry may limit our ability to contract with non-United States product manufacturers for products covered by such intellectual property. Any failure by us to comply with federal

regulations regarding intellectual property rights that were developed through the use of United States government funding could have a material adverse effect on our business, financial condition, results of operations and prospects.

Risks related to our business operations and industry

Our operating results may fluctuate significantly, which makes our future operating results difficult to predict and could cause our operating results to fall below expectations or any guidance we may provide.

Our quarterly and annual operating results may fluctuate significantly, which makes it difficult for us to predict our future operating results. These fluctuations may occur due to a variety of factors, many of which are outside of our control, including, but not limited to:

- the timing and cost of, and level of investment in, research, development, regulatory approval, and commercialization activities relating to our product candidates, which may change from time to time, including the need to conduct unanticipated clinical trials or trials that are larger or more complex than anticipated;
- our ability to enroll patients in clinical trials and the timing of enrollment;
- the timing and success or failure of preclinical studies or clinical trials for our product candidates or competing products, or any other change in the competitive landscape of our industry, including consolidation among our competitors or partners;
- coverage and reimbursement policies with respect to our product candidates, if approved, and potential future drugs that compete with our product candidates;
- the cost of manufacturing our product candidates, which may vary depending on the quantity of production and the terms of our agreements with third-party manufacturers;
- expenditures that we may incur to acquire, develop, or commercialize additional product candidates and technologies;
- the level of demand for any approved product candidates, which may vary significantly and be difficult to predict;
- our ability to establish and maintain collaborations, licensing, or other arrangements;
- potential unforeseen business disruptions that increase our costs or expenses;
- future accounting pronouncements or changes in our accounting policies; and
- the timing and amount of any milestone, royalty, or other payments payable by us or due to us under any collaboration, licensing, or other similar agreement.

The cumulative effects of these factors could result in large fluctuations and unpredictability in our quarterly and annual operating results. As a result, comparing our operating results on a period-to-period basis may not be meaningful. Investors should not rely on our past results as an indication of our future performance.

This variability and unpredictability could also result in our failing to meet the expectations of industry or financial analysts or investors for any period. If our revenue or operating results fall below the expectations of analysts or investors or below any forecasts we may provide to the market, or if the forecasts we provide to the market are below the expectations of analysts or investors, the price of our common stock could decline substantially. Such a stock price decline could occur even when we have met any previously publicly stated revenue or earnings guidance we may provide.

We face substantial competition, which may result in others discovering, developing or commercializing products before us or more successfully than we do.

The development and commercialization of new drug products is highly competitive. We may face competition with respect to any product candidates that we may develop from major pharmaceutical companies, specialty

pharmaceutical companies and biotechnology companies worldwide. There are a number of large pharmaceutical and biotechnology companies that currently market and sell products or are pursuing the development of products for the treatment of some of the disorders for which we are conducting research and development programs. Potential competitors also include academic institutions, government agencies and other public and private research organizations that conduct research, seek patent protection and establish collaborative arrangements for research, development, manufacturing and commercialization.

Our commercial opportunity could be reduced or eliminated if our competitors develop and commercialize products that are safer, more effective, have fewer or less severe side effects, are more convenient or are less expensive than our product candidates or that would render any product candidates that we may develop obsolete or non-competitive. Our competitors also may obtain FDA or other regulatory approval for their products more rapidly than we may obtain approval for ours, which could result in our competitors establishing a strong market position before we are able to enter the market. Additionally, technologies developed by our competitors may render our product candidates uneconomical or obsolete, and we may not be successful in marketing any product candidates we may develop against competitors.

We expect to face competition from existing products and product candidates in development for each of our programs and product candidates. In addition to the current standard-of-care treatments to address the diseases we are targeting in therapeutic development programs, numerous commercial and academic preclinical studies and clinical trials are being undertaken by a large number of parties to assess novel technologies and product candidates.

For the broad treatment of patients with UCDs, we will compete with Amgen Inc., who has commercialized Ravicti, a nitrogen scavenger. Other therapeutics in development are focused on patients with OTC deficiency only, where we will potentially compete with Ultragenyx Pharmaceutical Inc., Arcturus Therapeutics Holdings Inc., and iECure, among others, assuming they are successful in clinical development. Large pharmaceutical companies that have commercialized or are developing treatments for hypercholesterolemia include Amgen Inc., Regeneron Pharmaceuticals, Inc. and Novartis AG. Companies that compete with us directly on the level of the development of product candidates targeting SYNGAP1-related disorders include Stoke Therapeutics, Inc. and Praxis Precision Medicines, Inc. Companies engaged in the commercialization and development of antisense oligonucleotides as therapeutics include Alnylam Pharmaceuticals, Inc. and Ionis Pharmaceuticals Inc.

Many of the companies against which we compete or against which we may compete in the future have significantly greater financial resources and expertise in research and development, manufacturing, preclinical testing, conducting clinical trials, obtaining regulatory approvals and marketing approved products than we do. Accordingly, our competitors may be more successful than us in research and development, manufacturing, preclinical testing, conducting clinical trials, obtaining regulatory and marketing approvals, and achieving widespread market acceptance, rendering our product candidates obsolete or non-competitive.

Additionally, mergers and acquisitions in the pharmaceutical and biotechnology industries may result in even more resources being concentrated among a smaller number of our competitors. Smaller and other early-stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies. These third parties compete with us in recruiting and retaining qualified scientific and management personnel, establishing clinical trial sites and patient registration for clinical trials, as well as in acquiring technologies complementary to, or necessary for, our programs.

If we successfully obtain approval for any product candidate, we will face competition based on many different factors, including the safety and effectiveness of our products and the ease with which our products can be administered, the timing and scope of regulatory approvals for these products, the availability and cost of manufacturing, marketing and sales capabilities, price, reimbursement coverage and patent position. Competing products could present superior treatment alternatives, including by being more effective, safer, more convenient, less expensive or marketed and sold more effectively than any products we may develop. Competitive products or technological approaches may make any products we develop, or our RAP Platform, obsolete or noncompetitive before we recover the expense of developing and commercializing our product candidates. If we

are unable to compete effectively, our opportunity to generate revenue from the sale of our products we may develop, if approved, could be adversely affected.

Our international activities subject us to various risks, and our failure to manage these risks could adversely affect our results of operations.

We face significant operational risks as a result of doing business internationally, such as:

- fluctuations in foreign currency exchange rates;
- differing payor reimbursement regimes, governmental payors or patient self-pay systems and price controls;
- potentially adverse and/or unexpected tax consequences, including penalties due to the challenge by tax authorities on our tax position;
- potential changes to the accounting standards, which may influence our financial situation and results;
- compliance with tax, employment, immigration and labor laws should we have any employees living or traveling abroad;
- becoming subject to the different, complex and changing laws, regulations and court systems of multiple jurisdictions and compliance with a wide variety of foreign laws, treaties;
- reduced protection of, or significant difficulties in enforcing, intellectual property rights, or increased risk of intellectual property disputes, in certain countries;
- difficulties in attracting and retaining qualified consultants, contractors, and personnel;
- restrictions imposed by any applicable local labor practices and laws on our business and operations, including unilateral cancellation or modification of contracts;
- rapid changes in global government, economic and political policies and conditions, political or civil unrest or instability, terrorism or epidemics and other similar outbreaks or events, and potential failure in confidence of our suppliers or customers due to such changes or events;
- geopolitical tensions that affect our activities, operations and/or operations of our contractors, consultants, collaborators, vendors or partners; and
- tariffs, trade protection measures, import or export licensing requirements, trade embargoes and other trade barriers.

We conduct certain research and development operations through our wholly-owned Australian subsidiary. If we lose our ability to operate in Australia, or if our subsidiary is unable to receive the research and development incentive payment allowed by Australian regulations, our business and results of operations could suffer.

In September 2023, we formed a wholly-owned Australian subsidiary, CAMP4 Therapeutics Pty Ltd, to conduct various clinical activities for our product candidates in Australia. Due to the geographical distance and lack of employees currently in Australia, as well as our lack of experience operating in Australia, we may not be able to efficiently or successfully monitor our clinical activities in Australia, including conducting clinical trials. Furthermore, we have no assurance that the results of any clinical trials that we conduct for our product candidate in Australia will be accepted by the FDA or comparable foreign regulatory authorities for development and commercialization approvals.

In addition, current Australian tax regulations provide for a refundable research and development incentive plan of up to 18.5% of qualified expenditures. If our subsidiary loses its ability to operate in Australia, or if we are ineligible or unable to receive the research and development incentive payment, or the Australian government significantly reduces or eliminates the incentive program, our business and results of operation may be adversely affected.

Our future success depends on our ability to retain key executives and to attract, retain and motivate qualified personnel.

We are highly dependent on the research and development, clinical, financial, operational and other business expertise of our executive officers, as well as the other principal members of our management, scientific and clinical teams. Although we expect to enter into employment offer letters with each of our executive officers in connection with this offering, our executive officers may terminate their employment with us at any time. We do not maintain "key person" insurance for any of our executives or other employees. Recruiting and retaining qualified scientific, clinical, manufacturing, accounting, legal and sales and marketing personnel will also be critical to our success.

The loss of the services of our executive officers or other key employees could impede the achievement of our research, development and commercialization objectives and seriously harm our ability to successfully implement our business strategy. Furthermore, replacing executive officers and key employees may be difficult and may take an extended period of time because of the limited number of individuals in our industry with the breadth of skills and experience required to successfully develop, gain regulatory approval of and commercialize products. Competition to hire from this limited pool is intense, and we may be unable to hire, train, retain or motivate these key personnel on acceptable terms given the competition among numerous pharmaceutical and biotechnology companies for similar personnel. We also experience competition for the hiring of scientific and clinical personnel from universities and research institutions. In addition, we rely on consultants and advisors, including scientific and clinical advisors, to assist us in formulating our research and development and commercialization strategy. Our consultants and advisors may be employed by employers other than us and may have commitments under consulting or advisory contracts with other entities that may limit their availability to us. Our success as a public company also depends on implementing and maintaining internal controls and the accuracy and timeliness of our financial reporting. If we are unable to continue to attract and retain high quality personnel, our ability to pursue our growth strategy will be limited.

We may encounter difficulties in managing our growth and expanding our operations successfully.

As of March 31, 2024, we had 64 full-time employees. As we continue development and pursue the potential commercialization of our product candidates, as well as transition to functioning as a public company, we will need to expand our financial, development, regulatory, manufacturing, information technology, marketing, and sales capabilities or contract with third parties to provide these capabilities for us. As our operations expand, we expect that we will need to manage additional relationships with various strategic partners, suppliers, and other third parties, and we may not be successful in doing so. Our future financial performance and our ability to develop and commercialize our product candidates and to compete effectively will depend, in part, on our ability to manage any future growth effectively.

We may engage in strategic transactions that could impact our liquidity, increase our expenses, and present significant distractions to our management.

From time to time, we may consider strategic transactions, such as acquisitions of companies, asset purchases, and out-licensing or in-licensing of intellectual property, products, or technologies. Additional potential transactions that we may consider in the future include a variety of business arrangements, including spin-offs, strategic partnerships, joint ventures, restructurings, divestitures, business combinations, and investments. Any future transactions could increase our near and long-term expenditures, result in potentially dilutive issuances of our equity securities, including our common stock, or the incurrence of debt, contingent liabilities, amortization expenses or acquired in-process research and development expenses, any of which could affect our financial condition, liquidity, and results of operations. Future acquisitions may also require us to obtain additional financing, which may not be available on favorable terms or at all. These transactions may never be successful and may require significant time and attention of our management. In addition, the integration of any business that we may acquire in the future may disrupt our existing business and may be a complex, risky, and costly endeavor for which we may never realize the full benefits. Furthermore, we may experience losses related to investments in other companies, including as a result of failure to realize expected benefits or the materialization of unexpected

liabilities or risks, which could have a material negative effect on our results of operations and financial condition. Accordingly, although there can be no assurance that we will undertake or successfully complete any additional transactions of the nature described above, any additional transactions that we do complete could have a material adverse effect on our business, results of operations, financial condition, and prospects.

Clinical trial and product liability lawsuits against us could divert our resources, could cause us to incur substantial liabilities and could limit commercialization of our product candidates.

We will face an inherent risk of clinical trial and product liability exposure related to the testing of our product candidates in clinical trials, and we will face an even greater risk if we commercially sell any products that we may develop. While we currently have no product candidates that have been approved for commercial sale, the use of product candidates by us in clinical trials, and the sale of any approved products in the future, may expose us to liability claims. These claims might be made by patients that use the product, healthcare providers, pharmaceutical companies or others selling such products. If we cannot successfully defend ourselves against claims that our product candidates or products caused injuries, we will incur substantial liabilities. Regardless of merit or eventual outcome, liability claims may result in:

- decreased demand for any product candidates we may develop;
- injury to our reputation and significant negative media attention;
- withdrawal of clinical trial participants and inability to continue clinical trials;
- initiation of investigations by regulators;
- significant costs to defend any related litigation;
- product recalls, withdrawals or labeling, marketing or promotional restrictions;
- substantial monetary awards to trial participants or patients;
- loss of revenue;
- exhaustion of any available insurance and our capital resources;
- declined in our stock price;
- reduced resources of our management to pursue our business strategy; and
- the inability to commercialize any product candidates we may develop.

Although we maintain clinical trial liability insurance coverage, such insurance may not be adequate to cover all liabilities that we may incur. The cost of any product liability litigation or other proceeding, even if resolved in our favor, could be substantial. We may need to increase our insurance coverage as we expand our clinical trials or if we commence commercialization of any product candidates that receive marketing approval. Insurance coverage is increasingly expensive. We may not be able to maintain insurance coverage at a reasonable cost or in an amount adequate to satisfy any liability that may arise. If a successful clinical trial or product liability claim or series of claims is brought against us for uninsured liabilities or in excess of insured liabilities, our assets may not be sufficient to cover such claims and our business operations could be impaired.

Our insurance policies are expensive and protect us from only some business risks, which will leave us exposed to significant uninsured liabilities.

We do not carry insurance for all categories of risk that our business may encounter. Some of the policies we currently maintain or will maintain upon completion of this offering include property, general liability, employee benefits liability, workers' compensation, clinical trial liability, cyber liability, and directors' and officers' insurance. We do not know, however, if we will be able to maintain insurance with adequate levels of coverage. No assurance can be given that an insurance carrier will not seek to cancel or deny coverage after a claim has occurred. Any significant uninsured liability may require us to pay substantial amounts, which would adversely affect our financial position and results of operations.

Our internal network and information technology systems, or those of our vendors, collaborators, consultants, service providers and other contractors may suffer failure, security breach, loss or leakage of data, or other disruptions or compromise, which could result in a material disruption of our product development programs, compromise sensitive information, prevent us from accessing critical information, trigger contractual and legal obligations, or otherwise disrupt our business and materially impact our operations, potentially exposing us to liability, reputational harm, or other adverse effects on our business and financial results.

We are increasingly dependent upon information technology systems, infrastructure and data, some of which is managed by third parties, to operate our business. In the ordinary course of business, we collect, store and transmit large amounts of confidential information (including, but not limited to, intellectual property, proprietary business information and personal information). The secure processing, maintenance, and transmission of this information—including maintaining the availability, security, confidentiality, privacy and integrity of such confidential information—is critical to our operations and business. We have also outsourced elements of our operations to third parties, and as a result a number of third-party vendors, collaborators, consultants, service providers and other contractors (including our contract research organizations, CMOs and CROs) may or could have access to our confidential information, including our research and development efforts.

Despite the implementation of security measures, given the size and complexity of our internal information technology systems and those of any current or future vendors, collaborators, consultants, service providers and other contractors, and the increasing amounts of confidential information we maintain, such information technology systems are vulnerable to breakdown or other damage or interruption due to service interruptions, system malfunctions, natural disasters, terrorism, war and telecommunication and electrical failures, as well as security breaches from inadvertent or intentional actions by our employees, vendors, collaborators, consultants, service providers, other contractors and/or other third parties, or from cyber-attacks by malicious third parties (including the deployment of harmful malware, ransomware, computer viruses, denial-of-service attacks, social engineering, “phishing” scams, network security breaches and other means to affect the service reliability and threaten the confidentiality, integrity and availability of information), which may compromise our system infrastructure, or that of our vendors, collaborators, consultants, service providers and other contractors, or lead to data leakage. In addition to such risks, the adoption of new technologies may also increase our exposure to cybersecurity breaches or failures. The risk of a security breach or disruption, particularly through cyber-attacks or cyber intrusion, including by computer hackers, foreign governments and cyber terrorists, has generally increased as the number, intensity, and sophistication of attempted attacks and intrusions from around the world have increased. We may not be able to anticipate all types of security threats, and we may not be able to implement preventive measures that are effective against all such security threats. The techniques used by cyber criminals change frequently, may not be recognized until launched, and can originate from a wide variety of sources, including outside groups such as external service providers, organized crime affiliates, terrorist organizations or hostile foreign governments or agencies.

Although we seek to protect our information technology systems our efforts may not be successful. If such an event were to occur, it could result in a delay or disruption of our development programs and our business operations, whether due to a loss of our data, trade secrets or other proprietary or confidential information or other disruptions, and we could incur liability and reputational damage. For example, the loss of clinical trial data could result in delays in our regulatory approval efforts and significantly increase our costs to recover or reproduce the data. If we were to experience disruptions or security breaches of our information technology systems, the costs associated with the investigation, remediation and potential notification of the breach to counterparties, data subjects, regulators or others could be material. In addition, our remediation efforts may not be successful. Moreover, if the information technology systems of our vendors, collaborators, consultants, service providers and other contractors become subject to disruptions or security breaches, we may have insufficient recourse against such third parties and we may have to expend significant resources to mitigate the impact of such an event, and to develop and implement protections to prevent future events of this nature from occurring. If we do not allocate and effectively manage the resources necessary to build and sustain the proper technology and cybersecurity infrastructure, we could suffer significant business disruption, including transaction errors, supply chain or manufacturing interruptions, processing inefficiencies, data loss or the loss of or damage to intellectual property or other proprietary information. With the evolving nature of cybersecurity threats, the

scope and impact of any information security incident cannot be predicted. In addition, more than a dozen states in the United States have also passed comprehensive data protection legislation, and the global regulatory environment pertaining to information security and privacy is increasingly demanding, with new and changing requirements, such as the European Union's General Data Protection Regulation, The Personal Information Protection Law of the People's Republic of China, and Brazil's Lei Geral de Protecao de Dados. Complying with these laws and regulations may be more costly or take longer than we anticipate, and any failure to comply could result in fines or penalties.

To the extent that any disruption or security breach were to result in a loss of, or damage to, our or our vendors', collaborators', consultants', service providers' or other contractors' data or applications, or inappropriate disclosure of confidential or proprietary information, we could incur liability including litigation exposure, penalties and fines, we could become the subject of regulatory action or investigation, our competitive position and reputation could be harmed and the further development and commercialization of our product candidates could be delayed. As a result of such an event, we may be in breach of our contractual obligations. Furthermore, any such event that leads to unauthorized access, use, or disclosure of personal information, including personal information regarding our employees or current or future clinical trial participants, could harm our reputation, compel us to comply with federal and/or state breach notification laws and foreign law equivalents, subject us to mandatory corrective action, and otherwise subject us to liability under laws and regulations that protect the privacy and security of personal information, which could result in significant legal and financial exposure and reputational damage. Any of the above could have a material adverse effect on our business, financial condition, results of operations or prospects.

The financial exposure from the events referenced above could either not be insured against or not be fully covered through any insurance that we maintain and could have a material adverse effect on our business, financial condition, results of operations or prospects. In addition, we cannot be sure that our existing insurance coverage will continue to be available on acceptable terms or that our insurers will not deny coverage as to any future claim. There can be no assurance that the limitations of liability in our contracts would be enforceable or adequate or would otherwise protect us from liabilities or damages as a result of the events referenced above.

Our operations or those of the third parties upon whom we depend might be affected by the occurrence of a natural disaster, pandemic or other catastrophic event.

We depend on our employees, consultants, vendors, service providers, and other contractors (including CMOs and CROs), as well as regulatory agencies and other third parties, for the continued operation of our business. Despite any precautions we take for natural disasters or other catastrophic events, these events, including terrorist attack, pandemics, hurricanes, fire, floods and ice and snowstorms, could result in significant disruptions to our research and development, preclinical studies, clinical trials, and, ultimately, commercialization of our products. Long-term disruptions in infrastructure caused by events, such as natural disasters, the outbreak of war, the escalation of hostilities and acts of terrorism or other "acts of God," particularly involving those places in which we maintain office space or at our manufacturing or clinical trial sites, could adversely affect our businesses. Although we carry business interruption insurance policies and typically have provisions in our contracts that protect us in certain events, our coverage might not respond or be adequate to compensate us for all losses that may occur. Any natural disaster or catastrophic event affecting us, our consultants, vendors, service providers, and other contractors, regulatory agencies or other parties with which we are engaged could have a significant negative impact on our operations and financial performance.

Our business could be affected by litigation, government investigations, and enforcement actions.

We currently operate in a number of jurisdictions in a highly regulated industry, and we could be subject to litigation, government investigation, and enforcement actions on a variety of matters in the United States or foreign jurisdictions, including, without limitation, intellectual property, regulatory, product liability, environmental, whistleblower, false claims, privacy, anti-kickback, anti-bribery, securities, commercial, employment, and other claims and legal proceedings that may arise from conducting our business. Any determination that our operations or activities are not in compliance with existing laws or regulations could result in the imposition of fines, civil

and criminal penalties, equitable remedies, including disgorgement, injunctive relief, and/or other sanctions against us, and remediation of any such findings could have an adverse effect on our business operations.

Legal proceedings, government investigations, and enforcement actions can be expensive and time-consuming. An adverse outcome resulting from any such proceedings, investigations or enforcement actions could result in significant damages awards, fines, penalties, exclusion from the federal healthcare programs, healthcare debarment, injunctive relief, product recalls, reputational damage, and modifications of our business practices, which could have a material adverse effect on our business and results of operations. Even if such a proceeding, investigation, or enforcement action is ultimately decided in our favor, the investigation and defense thereof could require substantial financial and management resources.

Our employees, consultants, collaborators, vendors, service providers and other contractors may engage in misconduct or other improper activities, including non-compliance with regulatory standards and requirements and insider trading.

We are exposed to the risk of employee and third-party fraud or other misconduct or failure to comply with applicable regulatory requirements. Any past, current or future misconduct or non-compliance by our prior, existing or future employees, consultants, vendors, service providers and other contractors with any industry or regulatory standards or requirements may result in a material adverse effect on our operations or harm our reputation. Misconduct by these parties could include intentional failures to comply with FDA regulations and/or those of comparable applicable regulatory authorities, provide accurate information to such regulatory authorities, comply with manufacturing standards, comply with healthcare fraud and abuse laws and regulations in the United States and abroad, report financial information or data accurately, or disclose unauthorized activities to us. In particular, sales, marketing and other business arrangements in the healthcare industry are subject to extensive laws and regulations intended to prevent fraud, misconduct, kickbacks, self-dealing and other abusive practices. These laws and regulations may restrict or prohibit a wide range of business activities, including, but not limited to, research, manufacturing, distribution, pricing, discounting, marketing and promotion, sales commission, support programs and other business arrangements. Misconduct by these parties could also involve the improper use of individually identifiable information or information obtained in the course of clinical trials or interactions with the FDA, TGA or other regulatory authorities, which could result in regulatory sanctions and cause serious harm to our reputation. We have adopted a code of conduct applicable to all of our employees, but it is not always possible to identify and deter employee or third-party misconduct, and the precautions we take to detect and prevent this activity may not be effective in controlling unknown or unmanaged risks or losses or in protecting us from government investigations or other actions or lawsuits stemming from a failure to comply with these laws or regulations. If any such actions are instituted against us, those actions could have a significant impact on our business, including the imposition of significant civil, criminal and administrative penalties, damages, monetary fines, disgorgement of profits, imprisonment, possible exclusion from participation in Medicare, Medicaid and other federal healthcare programs, or other government-supported healthcare in other jurisdictions, contractual damages, reputational harm, diminished profits and future earnings, additional reporting or oversight obligations if we become subject to a corporate integrity agreement or other agreement to resolve allegations of non-compliance with the law and curtailment or restructuring of our operations, any of which could adversely affect our ability to operate. and we are not successful in defending ourselves or asserting our rights, those actions could have a significant impact on our business, financial condition, results of operations and prospects, including the imposition of significant civil, criminal and administrative penalties, damages, monetary fines, disgorgement of profits, imprisonment, possible exclusion from participation in Medicare, Medicaid and other federal healthcare programs, or other government-supported healthcare in other jurisdictions, contractual damages, reputational harm, diminished profits and future earnings, additional reporting or oversight obligations if we become subject to a corporate integrity agreement or other agreement to resolve allegations of non-compliance with the law and curtailment or restructuring of our operations, or other sanctions, any of which could adversely affect our ability to operate.

Our ability to utilize our net operating loss carryforwards and certain other tax attributes may be subject to limitations.

We have a history of cumulative losses and anticipate that we will continue to incur significant losses in the foreseeable future; thus, we do not know whether or when we will generate taxable income necessary to utilize

our net operating losses, or NOLs, or research and development tax credit carryforwards. As of December 31, 2023, we had federal NOL carryforwards of $69.8 million and state NOL carryforwards of $66.6 million.

In general, under Sections 382 and 383 of the U.S. Internal Revenue Code of 1986, as amended, or the Code, and corresponding provisions of state law, a corporation that undergoes an "ownership change," generally defined as a greater than 50 percentage point change (by value) in its equity ownership by certain stockholders over a three-year period, is subject to limitations on its ability to utilize its pre-change NOLs and pre-change research and development tax credit carryforwards to offset post-change taxable income. We have not yet conducted a study to determine if any such changes have occurred that could limit our ability to use the NOL and tax credit carryforwards. As a result, if, and to the extent that, we earn net taxable income, our ability to use our NOL carryforwards and research and development tax credit carryforwards to offset such taxable income may be subject to limitations.

There is also a risk that due to regulatory changes, such as suspensions on the use of NOLs, or other unforeseen reasons, our existing NOLs could expire or otherwise become unavailable to offset future income tax liabilities. Tax legislation enacted in 2017, informally titled the Tax Cuts and Jobs Act, or the Tax Act, as amended by the Coronavirus Aid, Relief, and Economic Security Act, or the CARES Act, includes changes to U.S. federal tax rates and the rules governing NOL carryforwards that may significantly impact our ability to utilize our NOLs to offset taxable income in the future. In addition, state NOLs generated in one state cannot be used to offset income generated in another state. For these reasons, even if we attain profitability, we may be unable to use a material portion of our NOLs and other tax attributes.

Exchange rate fluctuations may affect our results of operations and financial conditions.

Fluctuations in exchange rates, particularly between the U.S. dollar and the Australian dollar, may adversely affect us. Although we are incorporated in Delaware in the United States, we currently conduct clinical development in Australia. As a result, our business and the price of our common stock may be affected by fluctuations in foreign exchange rates, which may have a significant impact on our results of operations and cash flows from period to period. Currently, we do not have any exchange rate hedging arrangements in place.

Risks related to this offering and ownership of our common stock

There has been no public market for our common stock. An active, liquid, and orderly market for our common stock may not develop, or we may in the future fail to satisfy the continued listing requirements of Nasdaq, and investors may not be able to resell their common stock at or above the initial public offering price or at all.

Prior to this offering, there has been no public market for our common stock and the completion of this offering is contingent on receiving approval for listing on the Nasdaq Global Market, or Nasdaq. Although we have applied to list our common stock on Nasdaq, an active trading market for our common stock may never develop or may not be sustained following this offering. We and the representatives of the underwriters will determine the initial public offering price of our common stock through negotiation. This price will not necessarily reflect the price at which investors in the market will be willing to buy and sell our shares following this offering. In addition, an active trading market may not develop following the consummation of this offering or, if it does develop, may not be sustained. The lack of an active market may impair investors' ability to sell their shares at the time they wish to sell them or at a price they consider reasonable. An inactive market may also impair our ability to raise capital by selling shares and may impair our ability to acquire other businesses or technologies using our shares as consideration, which, in turn, could materially adversely affect our business.

If, after listing, we fail to satisfy the continued listing requirements of Nasdaq, such as the corporate governance requirements or the minimum closing bid price requirement, Nasdaq may take steps to delist our common stock. Such a delisting would likely have a negative effect on the price of our common stock and would impair investors' ability to sell or purchase our common stock when they wish to do so. In the event of a delisting, we can provide no assurance that any action taken by us to restore compliance with listing requirements would allow our common stock to become listed again, stabilize the market price or improve the liquidity of our common stock, prevent our

common stock from dropping below the Nasdaq minimum bid price requirement, or prevent future non-compliance with the listing requirements of Nasdaq.

The trading price of the shares of our common stock could be highly volatile, and purchasers of our common stock could incur substantial losses.

Our stock price is likely to be volatile. The stock market in general and the market for stock of biopharmaceutical companies in particular have experienced extreme volatility that has often been unrelated to the operating performance of individual companies. As a result of this volatility, investors may not be able to sell their common stock at or above the initial public offering price. The market price for our common stock may be influenced by those factors discussed in this "Risk factors" section and many others, including:

- results of our clinical trials and preclinical studies, and the results of trials of our competitors or those of other companies in our market sector;
- our ability to enroll patients in our current and any future clinical trials;
- our ability to obtain and maintain regulatory approval of our product candidates or additional indications thereof, or limitations to specific label indications or patient populations for its use, or changes or delays in the regulatory review process;
- regulatory or legal developments in the United States and foreign countries;
- changes in the structure of healthcare payment systems;
- the success or failure of our efforts to develop, acquire, or license additional product candidates;
- innovations, clinical trial results, product approvals and other developments regarding our competitors;
- announcements by us or our competitors of significant acquisitions, strategic partnerships, joint ventures, or capital commitments;
- manufacturing, supply, or distribution delays or shortages;
- any changes to our relationship with any manufacturers, suppliers, collaborators, or other strategic partners;
- achievement of expected product sales and profitability;
- variations in our financial results or development timelines or those of companies that are perceived to be similar to us, including variations from expectations of securities analysts or investors;
- market conditions in the biopharmaceutical sector and issuance of securities analysts' reports or recommendations;
- trading volume of our common stock;
- an inability to obtain additional funding;
- sales of our stock by us, our insiders, or our stockholders, as well as the anticipation of lock-up releases or expiration of market stand-off or lock-up agreements;
- general economic, industry, geopolitical, and market conditions, such as military conflict or war, inflation and financial institution instability, or pandemic or epidemic disease outbreaks, many of which are beyond our control;
- additions or departures of senior management, directors, or key personnel;
- intellectual property, product liability, or other litigation against us or our inability to enforce our intellectual property;
- changes in our capital structure, such as future issuances of securities and the incurrence of additional debt; and

- changes in accounting standards, policies, guidelines, interpretations, or principles.

In addition, in the past, stockholders have initiated class action lawsuits against biopharmaceutical companies following periods of volatility in the market prices of these companies' stock. Such litigation, if instituted against us, could cause us to incur substantial costs, divert our management's attention and resources and damage our reputation, which could have a material adverse effect on our business, financial condition and results of operations and prospects.

We may allocate the net proceeds from this offering in ways that stockholders may not approve.

Our management will have broad discretion in the application of the net proceeds from this offering, including for any of the purposes described in the section titled "Use of proceeds." Because of the number and variability of factors that will determine our use of the net proceeds from this offering, their ultimate use may vary substantially from their currently intended use. Our management might not apply our net proceeds in ways that ultimately increase the value of your investment, and the failure by our management to apply these funds effectively could harm our business. Pending their use, we may invest the net proceeds from this offering in short-and intermediate-term, interest-bearing obligations, investment-grade instruments, certificates of deposit, or direct or guaranteed obligations of the U.S. government. These investments may not yield a favorable return to our stockholders. If we do not invest or apply the net proceeds from this offering in ways that enhance stockholder value, we may fail to achieve expected results, which could cause our stock price to decline.

You will suffer immediate and substantial dilution in the net tangible book value of the common stock you purchase in this offering.

The initial public offering price of our common stock is substantially higher than the pro forma as adjusted net tangible book value per share of our outstanding common stock immediately after the closing of this offering. Purchasers of common stock in this offering will experience immediate dilution of approximately $ per share, assuming an initial public offering price of $ per share, which is the midpoint of the price range set forth on the cover page of this prospectus. In the past, we issued options to acquire common stock at prices significantly below the initial public offering price. To the extent these outstanding options are ultimately exercised, investors purchasing common stock in this offering will sustain further dilution. For a further description of the dilution that you will experience immediately after this offering, see the section titled "Dilution."

A significant portion of our total outstanding shares is restricted from immediate resale but may be sold into the market in the near future, which could cause the market price of our common stock to decline significantly, even if our business is doing well.

Sales of a substantial number of shares of our common stock in the public market could occur at any time. These sales upon the expiration of the lock-up agreements (described in the "Underwriting" section of this prospectus), the early release of the lock-ups, or the perception in the market that the holders of a large number of shares of common stock intend to sell shares, could reduce the market price of our common stock. After the completion of this offering, we will have shares of common stock outstanding, or shares if the underwriters exercise their over-allotment option in full, in each case based on the shares of our common stock outstanding as of , 2024. Of these shares, the shares (or shares if the underwriters exercise in full their option to purchase additional shares) we are selling in this offering may be resold in the public market immediately, unless purchased by our affiliates. The remaining shares are currently restricted under securities laws or as a result of lock-up or other agreements, but will be able to be sold after the completion of this offering as described in the "Shares eligible for future sale" section of this prospectus. J.P. Morgan and Leerink Partners may release some or all of the shares of common stock subject to lock-up agreements at any time in their sole discretion and without notice, except for directors and officers, which would allow for earlier sales of shares in the public market.

Moreover, after the completion of this offering, holders of an aggregate of shares of our common stock will have rights, subject to conditions, to require us to file registration statements covering their shares or to include their shares in registration statements that we may file for ourselves or other stockholders. We also plan to

register all shares of common stock that we may issue under our equity compensation plans or that are issuable upon exercise of outstanding options. Once we register these shares, they can be freely sold in the public market upon issuance and once vested, subject to volume limitations applicable to affiliates and the lock-up agreements described in the "Underwriting" section of this prospectus. If any of these additional shares are sold, or if it is perceived that they will be sold, in the public market, the market price of our common stock could decline.

After this offering, our executive officers, directors, and principal stockholders, if they choose to act together, will continue to have the ability to significantly influence all matters submitted to stockholders for approval.

Following the completion of this offering, our executive officers, directors and greater than 5% stockholders, in the aggregate, will own approximately % of our outstanding common stock (assuming no exercise of the underwriters' option to purchase additional shares and no exercise of outstanding options and without giving effect to any potential purchases by such persons in this offering). As a result, such persons, acting together, will have the ability to significantly influence all matters submitted to our board of directors or stockholders for approval, including the appointment of our management, the election and removal of directors and approval of any significant transaction, as well as our management and business affairs. This concentration of ownership may have the effect of delaying, deferring, or preventing a change in control, impeding a merger, consolidation, takeover or other business combination involving us, or discouraging a potential acquiror from making a tender offer or otherwise attempting to obtain control of our business, even if such a transaction would benefit other stockholders.

We do not currently intend to pay dividends on our common stock, so any returns on your investment will be limited to the value of our common stock.

We have never declared or paid any cash dividend on our common stock. We currently anticipate that we will retain future earnings for the development, operation, and expansion of our business and do not anticipate declaring or paying any cash dividends for the foreseeable future. In addition, any future debt agreements may preclude us from paying dividends. Any return to stockholders will therefore be limited to the appreciation of their stock. There is no guarantee that shares of our common stock will appreciate in value or even maintain the price at which stockholders have purchased their shares.

Sales of a substantial number of shares of our common stock by our existing stockholders in the public market could cause our stock price to fall.

Sales of a substantial number of shares of our common stock in the public market or the perception that these sales might occur could significantly reduce the market price of our common stock and impair our ability to raise adequate capital through the sale of additional equity or equity-linked securities.

Based on shares of common stock outstanding as of , 2024, upon the closing of this offering, we will have a total of shares of common stock outstanding, assuming no exercise of the underwriters' option to purchase additional shares and no exercise of outstanding options. Of these shares, only the shares of common stock sold in this offering by us, plus any shares sold upon exercise of the underwriters' option to purchase additional shares, will be freely tradable, without restriction, in the public market immediately following this offering, unless they are purchased by one of our affiliates.

Our directors and executive officers and the holders of substantially all of our common stock have entered into lock-up agreements with the representatives pursuant to which they may not, with limited exceptions and among other things, for a period of 180 days from the date of this prospectus, offer, sell or otherwise transfer or dispose of any of our securities, without the prior written consent of J.P. Morgan and Leerink Partners. The underwriters may permit our officers, directors, and other securityholders who are subject to the lock-up agreements to sell shares prior to the expiration of the lock-up agreements at any time in their sole discretion. See the section titled "Underwriting." Sales of these shares, or perceptions that they will be sold, could cause the trading price of our common stock to decline. After the lock-up agreements expire, up to an additional shares of common stock will be eligible for sale in the public market, of which shares will be held by

directors, executive officers and other affiliates and will be subject to volume limitations under Rule 144 under the Securities Act of 1933, as amended, or the Securities Act, in each without giving effect to any potential purchases by such persons in this offering.

In addition, as of , 2024, shares of common stock that are subject to outstanding options under our employee benefit plans will become eligible for sale in the public market to the extent permitted by the provisions of various vesting schedules, the lock-up agreements, and Rule 144 and Rule 701 under the Securities Act. If these additional shares of common stock are sold, or if it is perceived that they will be sold, in the public market, the trading price of our common stock could decline.

After this offering, the holders of shares of our outstanding common stock, or approximately % of our total outstanding common stock based on shares outstanding as of December 31, 2023, will be entitled to rights with respect to the registration of their shares under the Securities Act, subject to the 180-day lock-up agreements described above. See the section titled "Description of capital stock—Registration rights." Registration of these shares under the Securities Act would result in the shares becoming freely tradable without restriction under the Securities Act, except for shares held by affiliates, as defined in Rule 144 under the Securities Act. Any sales of securities by these stockholders could have a material adverse effect on the trading price of our common stock.

We are an "emerging growth company" and a "smaller reporting company," and the reduced disclosure requirements applicable to emerging growth companies and smaller reporting companies may make our common stock less attractive to investors.

We are an "emerging growth company," or an EGC, as defined in the Jumpstart Our Business Startups Act of 2012, or the JOBS Act. We are also a "smaller reporting company," as defined in Rule 12b-2 under the Securities Exchange Act of 1934, or the Exchange Act. We may remain an EGC until December 31, 2029, although if the market value of our common stock that is held by non-affiliates exceeds $700.0 million as of any June 30 before that time or if we have annual gross revenues of $1.235 billion or more in any fiscal year, we would cease to be an EGC as of December 31 of the applicable year. We would also cease to be an EGC if we issue more than $1.0 billion of non-convertible debt over a three-year period. For so long as we remain an EGC, we are permitted and intend to rely on exemptions from certain disclosure requirements that are applicable to other public companies that are not EGCs. These exemptions include:

- not being required to comply with the auditor attestation requirements in the assessment of our internal control over financial reporting;
- not being required to comply with any requirement that may be adopted by the Public Company Accounting Oversight Board regarding mandatory audit firm rotation or a supplement to the auditor's report providing additional information about the audit and the financial statements;
- reduced disclosure obligations regarding executive compensation; and
- exemptions from the requirements of holding a nonbinding advisory vote on executive compensation and stockholder approval of any golden parachute payments not previously approved.

Even after we no longer qualify as an EGC, we may continue to qualify as a smaller reporting company, which would allow us to take advantage of many of the same exemptions from disclosure requirements, including reduced disclosure obligations regarding executive compensation in our periodic reports and proxy statements. In addition, if we are a smaller reporting company with less than $100.0 million in annual revenue, we would not be required to comply with the auditor attestation requirements of Section 404 of the Sarbanes-Oxley Act of 2002, or Section 404. We would cease to be a smaller reporting company if the market value of our common stock that is held by non-affiliates exceeds $250.0 million and we had annual revenues in excess of $100.0 million or if the market value of our common stock that is held by non-affiliates exceeds $700.0 million, each as determined on an annual basis.

We cannot predict whether investors will find our common stock less attractive if we rely on these exemptions. If some investors find our common stock less attractive as a result, there may be a less active trading market for our common stock and our stock price may be more volatile.

In addition, the JOBS Act permits an EGC to take advantage of an extended transition period to comply with new or revised accounting standards applicable to public companies until those standards would otherwise apply to private companies. We have elected to take advantage of such extended transition period, which means that when a standard is issued or revised and it has different application dates for public or private companies, we will adopt the new or revised standard at the time private companies adopt the new or revised standard and will do so until such time that we either irrevocably elect to "opt out" of such extended transition period or no longer qualify as an EGC. We may choose to early adopt any new or revised accounting standards whenever such early adoption is permitted for private companies.

Provisions in our corporate charter documents and under Delaware law may have anti-takeover effects that could discourage an acquisition of our company by others, even if an acquisition would be beneficial to our stockholders, and may prevent attempts by our stockholders to replace or remove our current management.

Our amended and restated certificate of incorporation and our amended and restated bylaws, which will become effective prior to the completion of this offering, and Delaware law contain provisions that may have the effect of discouraging, delaying or preventing a change in control of our company or changes in our management that stockholders may consider favorable, including transactions in which you might otherwise receive a premium for your shares. These include provisions that:

- authorize "blank check" preferred stock, which could be issued by our board of directors without stockholder approval and may contain voting, liquidation, dividend and other rights superior to our common stock;
- create a classified board of directors whose members serve staggered three-year terms;
- specify that special meetings of our stockholders can be called only by our board of directors;
- prohibit stockholder action by written consent;
- establish an advance notice procedure for stockholder approvals to be brought before an annual meeting of our stockholders, including proposed nominations of persons for election to our board of directors;
- provide that vacancies on our board of directors may be filled only by a majority of directors then in office, even though less than a quorum;
- provide that our directors may be removed only for cause;
- specify that no stockholder is permitted to cumulate votes at any election of directors;
- expressly authorize our board of directors to modify, alter or repeal our amended and restated bylaws; and
- require supermajority votes of the holders of our common stock to amend specified provisions of our amended and restated certificate of incorporation and amended and restated bylaws.

These provisions, alone or together, could delay or prevent hostile takeovers and changes in control or changes in our management. These provisions could also limit the price that investors might be willing to pay in the future for shares of our common stock, thereby depressing the market price of our common stock.

In addition, because we are incorporated in the State of Delaware, we are governed by the provisions of Section 203 of the General Corporation Law of the State of Delaware, or the DGCL, which prohibits a person who owns in excess of 15% of our outstanding voting stock from merging or combining with us for a period of three years after the date of the transaction in which the person acquired in excess of 15% of our outstanding voting stock, unless the merger or combination is approved in a prescribed manner.

Any provision of our amended and restated certificate of incorporation, amended and restated bylaws or Delaware law that has the effect of delaying or deterring a change in control could limit the opportunity for our

stockholders to receive a premium for their shares of our common stock, and could also affect the price that some investors are willing to pay for our common stock.

Our amended and restated certificate of incorporation will designate specific courts as the sole and exclusive forum for certain claims or causes of action that may be brought by our stockholders, which could discourage lawsuits against us and our directors and officers.

Our amended and restated certificate of incorporation will provide that, subject to limited exceptions, the Court of Chancery of the State of Delaware (or, if, and only if, the Court of Chancery of the State of Delaware dismisses a Covered Claim (as defined below) for lack of subject matter jurisdiction, any other state or federal court in the State of Delaware that does have subject matter jurisdiction) will, to the fullest extent permitted by applicable law, be the sole and exclusive forum for the following types of claims: (i) any derivative claim brought in our right, (ii) any claim asserting a breach of a fiduciary duty to us or the our stockholders owed by any of our current or former directors, officers or other employees or stockholders, (iii) any claim against us arising pursuant to any provision of the DGCL, our amended and restated certificate of incorporation or amended and restated bylaws, (iv) any claim to interpret, apply, enforce or determine the validity of our amended and restated certificate of incorporation or amended and restated bylaws, (v) any claim against us governed by the internal affairs doctrine, and (vi) any other claim, not subject to exclusive federal jurisdiction and not asserting a cause of action arising under the Securities Act, brought in any action asserting one or more of the claims specified in clauses (a)(i) through (v) herein above, each, a Covered Claim. This provision would not apply to claims brought to enforce a duty or liability created by the Exchange Act.

Our amended and restated certificate of incorporation will further provide that the federal district courts of the United States of America will be the exclusive forum for resolving any complaint asserting a cause of action arising under the Securities Act. In addition, our amended and restated certificate of incorporation will provide that any person or entity purchasing or otherwise acquiring any interest in the shares of capital stock of the company will be deemed to have notice of and consented to these choice-of-forum provisions and waived any argument relating to the inconvenience of the forums in connection with any Covered Claim.

The choice of forum provisions to be contained in our amended and restated certificate of incorporation may limit a stockholder's ability to bring a claim in a judicial forum that it finds favorable for disputes with us or any of our directors, officers, other employees or stockholders, which may discourage lawsuits with respect to such claims, although our stockholders will not be deemed to have waived our compliance with federal securities laws and the rules and regulations thereunder. While the Delaware courts have determined that such choice of forum provisions are facially valid, it is possible that a court of law in another jurisdiction could rule that the choice of forum provisions to be contained in our amended and restated certificate of incorporation are inapplicable or unenforceable if they are challenged in a proceeding or otherwise, which could cause us to incur additional costs associated with resolving such action in other jurisdictions. The choice of forum provisions may also impose additional litigation costs on stockholders who assert that the provisions are not enforceable or invalid.

Participation in this offering by our existing stockholders and/or their affiliated entities may reduce the public float for our common stock.

To the extent certain of our existing stockholders and their affiliated entities participate in this offering, such purchases would reduce the non-affiliate public float of our shares, meaning the number of shares of our common stock that are not held by officers, directors, and controlling stockholders. A reduction in the public float could reduce the number of shares that are available to be traded at any given time, thereby adversely impacting the liquidity of our common stock and depressing the price at which you may be able to sell shares of common stock purchased in this offering.

General risk factors

We will incur significant increased costs as a result of operating as a public company, and our management will be required to devote substantial time to new compliance initiatives.

As a public company, we will incur significant legal, accounting, and other expenses that we did not incur as a private company. We will be subject to the reporting requirements of the Exchange Act, which will require, among

other things, that we file with the SEC annual, quarterly, and current reports with respect to our business and financial condition. In addition, Sarbanes-Oxley, as well as rules subsequently adopted by the SEC and Nasdaq to implement provisions of Sarbanes-Oxley, impose significant requirements on public companies, including requiring establishment and maintenance of effective disclosure and financial controls and certain corporate governance practices. Further, pursuant to the Dodd-Frank Wall Street Reform and Consumer Protection Act of 2010, the SEC has adopted additional rules and regulations in these areas, such as mandatory "say on pay" voting requirements that will apply to us when we cease to be an emerging growth company. Stockholder activism, the current political environment and the current high level of government intervention and regulatory reform may lead to substantial new regulations and disclosure obligations, which may lead to additional compliance costs and impact the manner in which we operate our business in ways we cannot currently anticipate.

We expect the rules and regulations applicable to public companies to substantially increase our legal and financial compliance costs and to make some activities more time consuming and costly. The increased costs will decrease our net income or increase our net loss, and may require us to reduce expenditures in other areas of our business. For example, we expect these rules and regulations to make it more difficult and more expensive for us to obtain director and officer liability insurance, and we may be required to incur substantial costs to maintain the same or similar coverage. We cannot predict or estimate the amount or timing of additional costs we may incur to comply with these requirements. The impact of these requirements could also make it more difficult for us to attract and retain qualified persons to serve on our board of directors, our board committees, or as executive officers. If these requirements divert the attention of our management and personnel from other business concerns, they could have a material adverse effect on our business, financial condition, and results of operations.

We are subject to U.S. and certain foreign export and import controls, sanctions, embargoes, anti-corruption laws, and anti-money laundering laws and regulations. We could face criminal liability and other serious consequences for violations, which could harm our business.

We are subject to export control and import laws and regulations, including the U.S. Export Administration Regulations, U.S. Customs regulations, and various economic and trade sanctions regulations administered by the U.S. Treasury Department's Office of Foreign Assets Controls and anti-corruption and anti-money laundering laws and regulations, including the U.S. Foreign Corrupt Practices Act of 1977, as amended, the U.S. domestic bribery statute contained in 18 U.S.C. § 201, the U.S. Travel Act, the USA PATRIOT Act and other state and national anti-bribery and anti-money laundering laws in the countries in which we conduct activities. Anti-corruption laws are interpreted broadly and prohibit companies and their employees, agents, CROs, contractors and other collaborators and partners from authorizing, promising, offering, providing, soliciting, or receiving, directly or indirectly, improper payments or anything else of value to recipients in the public or private sector. We may engage third parties for clinical trials outside of the United States, to sell our products abroad if we enter a commercialization phase, and/or to obtain necessary permits, licenses, patent registrations and other regulatory approvals. We have direct or indirect interactions with officials and employees of government agencies or government-affiliated hospitals, universities, and other organizations. We can be held liable for the corrupt or other illegal activities of our employees, agents, CROs, contractors, and other collaborators and partners, even if we do not explicitly authorize or have actual knowledge of such activities, and any training or compliance programs or other initiatives we undertake to prevent such activities may not be effective.

Any violations of the laws and regulations described above may result in substantial civil and criminal fines and penalties, imprisonment, the loss of export or import privileges, debarment, tax reassessments, breach of contract and fraud litigation, reputational harm, and other consequences.

Furthermore, U.S. export control laws and economic sanctions prohibit the provision of certain products and services to countries, governments, and persons targeted by U.S. sanctions. U.S. sanctions that have been or may be imposed as a result of military conflicts in other countries may impact our ability to continue activities at future clinical trial sites within regions covered by such sanctions. If we fail to comply with export and import regulations and such economic sanctions, penalties could be imposed, including fines and/or denial of certain export privileges. These export and import controls and economic sanctions could also adversely affect our supply chain.

Unstable market and economic conditions and adverse developments with respect to financial institutions and associated liquidity risk may have serious adverse consequences on our business, financial condition, and stock price.

From time to time, the global credit and financial markets have experienced extreme volatility and disruptions, including severely diminished liquidity and credit availability, declines in consumer confidence, declines in economic growth, increases in unemployment rates, and uncertainty about economic stability. The financial markets and the global economy may also be adversely affected by the current or anticipated impact of military conflict, including the conflict between Russia and Ukraine and Israel and Hamas, terrorism, or other geopolitical events. Sanctions imposed by the United States and other countries in response to such conflicts, including the one in Ukraine, may also adversely impact the financial markets and the global economy, and any economic countermeasures by the affected countries or others could exacerbate market and economic instability. In addition, in 2023 the closures of financial institutions and their placement into receivership with the FDIC created bank-specific and broader financial institution liquidity risk and concerns. Future adverse developments with respect to specific financial institutions or the broader financial services industry may lead to market-wide liquidity shortages, impair the ability of companies to access near-term working capital needs, and create additional market and economic uncertainty. There can be no assurance that future credit and financial market instability and a deterioration in confidence in economic conditions will not occur. Our general business strategy may be adversely affected by any such economic downturn, liquidity shortages, volatile business environment or continued unpredictable and unstable market conditions. If the equity and credit markets deteriorate, or if adverse developments are experienced by financial institutions, it may cause short-term liquidity risk and may make any necessary debt or equity financing more difficult, more costly, more onerous with respect to financial and operating covenants and more dilutive. Failure to secure any necessary financing in a timely manner and on favorable terms could have a material adverse effect on our growth strategy, financial performance and stock price and could require us to delay or abandon clinical development plans. In addition, there is a risk that one or more of our current service providers, financial institutions, manufacturers, and other partners may be adversely affected by the foregoing risks, which could directly affect our ability to attain our operating goals on schedule and on budget.

Inflation could adversely affect our business and results of operations.

From 2021 to 2023, the U.S. economy experienced a material level of inflation. The impact of geopolitical developments, such as the conflicts in Ukraine and the Middle East may continue to increase uncertainty in the outlook of near-term and long-term economic activity, including whether inflation will continue and how long, and at what rate. Increases in inflation raise our costs for labor, materials and services and other costs required to grow and operate our business, and failure to secure these on reasonable terms may adversely impact our financial condition. Additionally, increases in inflation, along with the uncertainties surrounding geopolitical developments and global supply chain disruptions, have caused, and may in the future cause, global economic uncertainty and uncertainty about the interest rate environment, which may make it more difficult, costly or dilutive for us to secure additional financing. A failure to adequately respond to these risks could have a material adverse impact on our financial condition, results of operations or cash flows.

Changes in tax law may materially adversely affect our financial condition, results of operations and cash flows, or adversely impact the value of an investment in our common stock.

New income, sales, use or other tax laws, statutes, rules, regulations, or ordinances could be enacted at any time, or interpreted, changed, modified, or applied adversely to us, any of which could adversely affect our business operations and financial performance.

Our disclosure controls and procedures may not prevent or detect all errors or acts of fraud.

We are subject to certain reporting requirements of the Exchange Act. Our disclosure controls and procedures are designed to reasonably assure that information required to be disclosed by us in reports we file or submit under the Exchange Act is accumulated and communicated to management, recorded, processed, summarized and reported within the time periods specified in the rules and forms of the SEC. We believe that any disclosure controls

and procedures or internal controls and procedures, no matter how well conceived and operated, can provide only reasonable, not absolute, assurance that the objectives of the control system are met. These inherent limitations include the realities that judgments in decision-making can be faulty, and that breakdowns can occur because of simple error or mistake. Additionally, controls can be circumvented by the individual acts of some persons, by collusion of two or more people or by an unauthorized override of the controls. Accordingly, because of the inherent limitations in our control system, misstatements or insufficient disclosures due to error or fraud may occur and not be detected.

If securities or industry analysts do not publish research or reports or publish unfavorable research or reports about our business, our stock price and trading volume could decline.

The trading market for our common stock will depend in part on the research and reports that securities or industry analysts publish about us, our business, our market, or our competitors. We do not currently have and may never obtain research coverage by securities and industry analysts. If no securities or industry analysts commence coverage of our company, the trading price for our stock would be negatively impacted. In the event we obtain securities or industry analyst coverage, if one or more of the analysts who covers us downgrades our stock, or if we fail to meet the expectations of one or more of these analysts, our stock price would likely decline. If one or more of these analysts ceases to cover us or fails to regularly publish reports on us, interest in our stock could decrease, which could cause our stock price or trading volume to decline.

If we fail to maintain proper and effective internal control over financial reporting, our ability to produce accurate and timely financial statements could be impaired, investors may lose confidence in our financial reporting, and the trading price of our common stock may decline.

Pursuant to Section 404 of Sarbanes-Oxley, our management will be required to report upon the effectiveness of our internal control over financial reporting beginning with the second annual report following this offering. When we lose our status as an EGC and do not otherwise qualify as a “smaller reporting company,” our independent registered public accounting firm will be required to attest to the effectiveness of our internal control over financial reporting. The rules governing the standards that must be met for our management to assess our internal control over financial reporting are complex and require significant documentation, testing and possible remediation. To comply with the requirements of being a reporting company under the Exchange Act, we may need to upgrade our information technology systems; implement additional financial and management controls, reporting systems and procedures; and hire additional accounting and finance staff. If we or, if required, our auditors are unable to conclude that our internal control over financial reporting is effective, investors may lose confidence in our financial reporting and the trading price of our common stock may decline.

We cannot assure investors that there will not be material weaknesses or significant deficiencies in our internal control over financial reporting in the future. Any failure to maintain internal control over financial reporting could severely inhibit our ability to accurately report our financial condition, results of operations or cash flows. If we are unable to conclude that our internal control over financial reporting is effective, or if our independent registered public accounting firm determines we have a material weakness or significant deficiency in our internal control over financial reporting once that firm begins its Section 404 reviews, investors may lose confidence in the accuracy and completeness of our financial reports, the market price of our common stock could decline, and we could be subject to sanctions or investigations by Nasdaq, the SEC, or other regulatory authorities. Failure to remedy any material weakness in our internal control over financial reporting, or to implement or maintain other effective control systems required of public companies, could also restrict our future access to the capital markets.

As a public company, we may be at an increased risk of securities class action litigation.

Historically, securities class action litigation has often been brought against a company following a decline in the market price of its securities. This risk is especially relevant for us because biotechnology and pharmaceutical companies have experienced significant stock price volatility in recent years. If we were to be sued, it could result in substantial costs and a diversion of management’s attention and resources, which could harm our business.

Special note regarding forward-looking statements

This prospectus, including the sections titled “Prospectus summary,” “Risk factors,” “Management’s discussion and analysis of financial condition and results of operations” and “Business,” contains forward-looking statements that involve substantial risks and uncertainties because they relate to events and depend on circumstances that may or may not occur in the future. All statements other than statements of historical fact contained in this prospectus, including statements regarding our strategy, future operations, future financial position, prospects, plans, objectives of management and expected growth, are forward-looking statements. These statements are based on our current beliefs, expectations and assumptions regarding our intentions, beliefs or current expectations concerning, among other things, the future of our business, future plans and strategies, our operational results and other future conditions. Forward-looking statements involve known and unknown risks, uncertainties and other important factors that may cause our actual results, performance or achievements to be materially different from any future results, performance or achievements expressed or implied by the forward-looking statements.

In some cases, you can identify forward-looking statements by terms such as “may,” “will,” “should,” “expect,” “plan,” “anticipate,” “could,” “intend,” “target,” “project,” “estimate,” “believe,” “predict,” “potential” or “continue” or the negative of these terms or other similar expressions intended to identify statements about the future, although not all forward-looking statements contain these identifying words. These forward-looking statements include, without limitation, statements about the following:

- the initiation, timing, progress, results and costs of our research and development programs and of our current and future preclinical studies and clinical trials of our product candidates, including statements regarding the timing of initiation and completion of studies or trials and related preparatory work, as well as the period during which the results of the trials are expected become available;
- the timing of our planned good laboratory practices toxicology studies and regulatory submissions, initiation of planned clinical trials and timing of expected clinical results for CMP-CPS-001 and CMP-FH and CMP-SYNGAP programs, if applicable, and our other future product candidates;
- the timing of any submissions of filings for regulatory approval of, and our ability to obtain and maintain regulatory approvals for, CMP-CPS-001 and any other product candidates;
- our ability to identify patients with the diseases treated by our product candidates, and to enroll patients in trials;
- our expectations regarding the size of the patient populations, market acceptance and opportunity for and clinical utility of our product candidates, if approved for commercial use;
- our reliance on third party manufacturing partners to comply with significant regulations with respect to manufacturing our products;
- our expectations regarding the scope of any approved indication for CMP-CPS-001 or any other product candidate;
- our ability to successfully commercialize our product candidates, if approved;
- our ability to leverage our RAP Platform to identify and develop future product candidates;
- our estimates of our expenses, ongoing losses, future revenue, capital requirements and our need for or ability to obtain additional funding before we can expect to generate any revenue from product sales;
- our ability to establish or maintain strategic collaborations or arrangements, including potential business development opportunities and potential licensing partnerships, and our ability to attract collaborators with development, regulatory and commercialization expertise;
- our ability to identify, recruit and retain key personnel;
- our reliance upon intellectual property licensed from third parties and our ability to obtain such licenses on commercially reasonable terms or at all;

- our ability to protect and enforce our intellectual property position for our product candidates, and the scope of such protection;
- our financial performance;
- our anticipated use of the net proceeds to us from this offering and the sufficiency of our existing cash and cash equivalents and the proceeds from this offering to fund our future operating expenses and capital expenditure requirements;
- our competitive position and the development of and projections relating to our competitors or our industry;
- our estimates regarding future expenses and needs for additional financing;
- the impact of laws and regulations;
- general economic, industry, geopolitical and market conditions, such as military conflict or war, inflation and financial institution instability, or pandemic or epidemic disease outbreaks, many of which are beyond our control; and
- our expectations regarding the time during which we will be an emerging growth company and smaller reporting company under the JOBS Act.

Although we base these forward-looking statements on assumptions that we believe are reasonable when made, we caution you that forward-looking statements are not guarantees of future performance and that our actual results of operations, financial condition and liquidity, and the development of the industry in which we operate may differ materially from those made in or suggested by the forward-looking statements contained in this prospectus. In addition, even if our results of operations, financial condition and liquidity, and the development of the industry in which we operate are consistent with the forward-looking statements contained in this prospectus, those results or developments may not be indicative of results or developments in subsequent periods.

Given these risks and uncertainties, you are cautioned not to place undue reliance on these forward-looking statements. Any forward-looking statement that we make in this prospectus speaks only as of the date of such statement. Except as required by law, we assume no obligation to update these forward-looking statements publicly, or to update the reasons actual results could differ materially from those anticipated in any forward-looking statements, whether as a result of new information, future events or otherwise. Comparisons of results for current and any prior periods are not intended to express any future trends or indications of future performance, unless specifically expressed as such, and should only be viewed as historical data. You should, therefore, not rely on these forward-looking statements as representing our views as of any date subsequent to the date of this prospectus.

In addition, statements that “we believe” and similar statements reflect our beliefs and opinions on the relevant subject. These statements are based upon information available to us as of the date of this prospectus, and while we believe such information forms a reasonable basis for such statements, such information may be limited or incomplete, and our statements should not be read to indicate that we have conducted an exhaustive inquiry into, or review of, all potentially available relevant information. These statements are inherently uncertain and investors are cautioned not to unduly rely upon these statements.

You should carefully read this prospectus and the documents that we have filed as exhibits to the registration statement, of which this prospectus is a part, completely and with the understanding that our actual future results may be materially different from what we expect. We qualify all of the forward-looking statements in this prospectus by these cautionary statements.

Market and industry data

Unless otherwise indicated, market and industry data contained in this prospectus, including potential market opportunities, is based on our management's estimates and research, as well as industry and general publications and research and studies conducted by third parties. Although we believe that the information from these third-party publications, research and studies included in this prospectus is reliable, and we are responsible for the accuracy of such information, neither we nor the underwriters have independently verified the accuracy or completeness of this information. Management's estimates are derived from publicly available information, their knowledge of our industry and their assumptions based on such information and knowledge, which we believe to be reasonable. This data involves a number of assumptions and limitations and the industry in which we operate is subject to a high degree of uncertainty and risk due to a variety of factors, including those described in the sections titled "Risk factors" and "Special note regarding forward-looking statements." These and other factors could cause our future performance to differ materially from our assumptions and estimates.

Use of proceeds

We estimate that the net proceeds to us from the issuance and sale of shares of our common stock in this offering will be approximately $ million (or approximately $ million if the underwriters exercise in full their option to purchase additional shares), assuming an initial public offering price of $ per share, which is the midpoint of the price range set forth on the cover page of this prospectus, and after deducting the estimated underwriting discounts and commissions and estimated offering expenses payable by us.

Each $1.00 increase or decrease in the assumed initial public offering price of $ per share, which is the midpoint of the price range set forth on the cover page of this prospectus, would increase or decrease, as applicable, the net proceeds to us from this offering by approximately $ million, assuming the number of shares offered by us, as set forth on the cover page of this prospectus, remains the same and after deducting the estimated underwriting discounts and commissions and estimated offering expenses payable by us. Similarly, each increase or decrease of 1,000,000 in the number of shares we are offering would increase or decrease the net proceeds to us from this offering by $ million, respectively, after deducting the estimated underwriting discounts and commissions and estimated offering expenses payable by us, assuming the assumed initial public offering price stays the same. We do not expect that a change in the offering price or the number of shares by these amounts would have a material effect on our intended uses of the net proceeds from this offering, although it may impact the amount of time prior to which we may need to seek additional capital.

As of , 2024, we had cash and cash equivalents of $ million. We currently intend to use the net proceeds from this offering, together with our existing cash and cash equivalents, as follows:

- approximately $ million to ;
- approximately $ million to ; and
- the remainder for working capital and other general corporate purposes, including the additional costs associated with being a public company.

We may also use a portion of the net proceeds from this offering to in-license, acquire or invest in products, technologies, or businesses, although we have no current agreements, commitments or understandings to do so. The amounts and timing of our actual expenditures will depend on numerous factors, including the progress of our preclinical development efforts, our operating costs and other factors described under “Risk factors” in this prospectus.

Based on our current operating plan, we believe that the anticipated net proceeds from this offering, together with our existing cash and cash equivalents as of , 2024, will be sufficient to fund our operating expenses and capital expenditure requirements for from the date of this prospectus. This estimate and our expectation regarding the sufficiency of the net proceeds from this offering are based on assumptions that may prove to be wrong, and we could exhaust our available capital resources sooner than we expect. We do not expect that the anticipated net proceeds from this offering, together with our existing cash and cash equivalents, will be sufficient for us to fund any of our product candidates through regulatory approval, and we will need to raise substantial additional capital to complete the development and commercialization of our product candidates. We may satisfy our future cash needs through the sale of equity securities, debt financings, working capital lines of credit, corporate collaborations or license agreements, grant funding, interest income earned on invested cash balances or a combination of one or more of these sources.

This anticipated use of net proceeds from this offering and our existing cash and cash equivalents represents our current intentions based upon our present plans and business conditions, which could change in the future as our plans and business conditions evolve. The amounts and timing of our actual expenditures may vary significantly depending on numerous factors, including the progress of our development, the status of and results from clinical and preclinical trials, the timing and outcome of any regulatory submissions, as well as any collaborations that we may enter into with third parties for the development of product candidates developed using our RAP Platform and any other product candidates we develop, and any unforeseen cash needs.

Our management will have broad discretion in the application of the net proceeds from this offering, and investors will be relying on the judgment of our management regarding the application of those net proceeds. The timing and amount of our actual expenditures will be based on many factors, including cash flows from operations and the anticipated growth of our business, and we may find it necessary or advisable to use the net proceeds from this offering for other purposes. Pending their use, we plan to invest the net proceeds from this offering in short-term, interest-bearing obligations, investment-grade instruments, certificates of deposit or direct or guaranteed obligations of the United States government.

Dividend policy

We have never declared or paid, and do not anticipate declaring or paying, in the foreseeable future, any cash dividends on our capital stock. We currently intend to retain all available funds and any future earnings, if any, to support our operations and finance the growth and development of our business. Any future determination related to our dividend policy will be made at the discretion of our board of directors and will depend upon, among other factors, our results of operations, financial condition, capital requirements, contractual restrictions, business prospects and other factors our board of directors may deem relevant. Our ability to pay cash dividends on our capital stock in the future may also be limited by the terms of any preferred securities we may issue or agreements governing any indebtedness we may incur.

Capitalization

The following table sets forth our cash and cash equivalents and our capitalization as of , 2024:

- on an actual basis;
- on a pro forma basis to give effect to (i) the automatic conversion of all of the outstanding shares of our convertible preferred stock and convertible preferred stock warrants as of , 2024 into an aggregate of shares of our common stock, based on an assumed initial public offering price of $ per share, which is the midpoint of the price range set forth on the cover page of this prospectus, immediately prior to the closing of this offering, and (ii) the filing and effectiveness of our Restated Charter, which will be effective immediately prior to the completion of this offering; and
- on a pro forma as adjusted basis to give further effect our issuance and sale of shares of common stock in this offering at an assumed initial public offering price of $ per share, which is the midpoint of the price range set forth on the cover page of this prospectus, after deducting the estimated underwriting fees and commissions and estimated offering expenses payable by us.

The pro forma as adjusted information is illustrative only and our capitalization following the completion of this offering will depend on the actual initial public offering price and other terms of this offering determined at pricing. You should read the information in this table in conjunction with our financial statements and the related notes appearing at the end of this prospectus, the section of this prospectus titled “Management’s discussion and analysis of financial condition and results of operations” and other financial information contained in this prospectus.

	As of , 2024		
(in thousands, except share and per share data)	**Actual**	**Pro forma**	**Pro forma as adjusted(1)**
Cash and cash equivalents	$	$	$
Preferred stock warrant liability	$		
Series A Prime convertible preferred stock, $0.0001 par value; shares authorized, shares issued and outstanding, actual; no shares authorized, issued or outstanding pro forma and pro forma as adjusted			
Series B convertible preferred stock, $0.0001 par value; shares authorized, shares issued and outstanding, no shares authorized, issued or outstanding pro forma and pro forma as adjusted			
Stockholders’ (deficit) equity:			
Preferred stock, $0.0001 par value; no shares authorized, issued or outstanding, actual; shares authorized and no shares issued or outstanding, pro forma and pro forma as adjusted			
Common stock, $0.0001 par value; shares authorized, shares issued and outstanding, actual; shares authorized, shares issued and outstanding, pro forma; shares authorized, shares issued and outstanding, pro forma as adjusted			
Additional paid-in capital			
Accumulated (deficit) equity			
Total stockholders’ (deficit) equity			
Total capitalization	$	$	$

(1) Each $1.00 increase or decrease in the assumed initial public offering price of $ per share, which is the midpoint of the price range set forth on the cover page of this prospectus, would increase or decrease, as applicable, the pro forma as adjusted amount of each of cash and cash equivalents, additional paid-in capital, total stockholders’ (deficit) equity and total capitalization by approximately $ million, assuming that the number of shares offered by us, as set forth on the cover page of this prospectus, remains the same and after deducting the estimated underwriting discounts and commissions and estimated offering expenses payable by us. Similarly, each increase or decrease of 1,000,000 shares in the number of shares

offered by us at the assumed initial public offering price per share of $ per share, which is the midpoint of the price range set forth on the cover page of this prospectus, would increase or decrease the pro forma as adjusted amount of each of additional paid-in capital, total stockholders' (deficit) equity and total capitalization by approximately $ million, assuming no change in the assumed initial public offering price per share and after deducting the estimated underwriting discounts and commissions and estimated offering expenses payable by us.

If the underwriters exercise in full their option to purchase additional shares, our pro forma as adjusted cash and cash equivalents, additional paid-in capital, total stockholders' (deficit) equity and total capitalization as of , 2024 would be $ million, $ million, $ million and $ million, respectively.

The information above is illustrative only and our capitalization following the completion of this offering will be adjusted based on the actual initial public offering price and other terms of this offering determined at pricing. The number of shares of our common stock to be outstanding after this offering on a pro forma and pro forma as adjusted basis is based on shares of our common stock outstanding as of , 2024 after giving effect to the automatic conversion of all outstanding shares of our convertible preferred stock and convertible preferred stock warrants, into an aggregate of shares of our common stock, based on an assumed initial public offering price of $ per share, which is the midpoint of the price range set forth on the cover page of this prospectus, immediately prior to the completion of this offering, and excludes:

- shares of our common stock issuable upon the exercise of stock options outstanding as of , 2024 pursuant to our 2016 Plan, with a weighted-average exercise price of $ per share;
- shares of our common stock reserved for future issuance under the 2016 Plan as of , 2024, which shares will cease to be available for issuance at the time our 2024 Plan, becomes effective in connection with this offering;
- shares of our common stock issuable upon the exercise of warrants outstanding at , 2024 at a weighted-average exercise price of $ per share;
- shares of our common stock reserved for future issuance under the 2024 Plan, which will become effective in connection with this offering, as well as any automatic increases in the number of shares of common stock reserved for future issuance under the 2024 Plan; and
- shares of our common stock reserved for future issuance under our ESPP, which will become effective in connection with this offering, as well as any automatic increases in the number of shares of common stock reserved for future issuance under the ESPP.

Dilution

If you invest in our common stock in this offering, your ownership interest will be immediately diluted to the extent of the difference between the initial public offering price per share of our common stock and the pro forma as adjusted net tangible book value per share of our common stock after this offering.

As of , 2024, we had a historical net tangible book value (deficit) of $ million, or $ per share of common stock. Our historical net tangible book value (deficit) per share represents total tangible assets less total liabilities and convertible preferred stock, divided by the number of shares of our common stock outstanding as of , 2024.

Our pro forma net tangible book value as of , 2024 was $ million, or $ per share of our common stock. Pro forma net tangible book value represents the amount of our total tangible assets less our total liabilities, after giving effect to the automatic conversion of all of the outstanding shares of our convertible preferred stock and convertible preferred stock warrants into an aggregate of shares of our common stock immediately prior to the closing of this offering (based on assumed initial public offering price of $ per share, which is the midpoint of the price range set forth on the cover page of this prospectus), as if such conversions had occurred on , 2024. Pro forma net tangible book value per share represents pro forma net tangible book value divided by the total number of shares outstanding as of , 2024, after giving effect to the pro forma adjustments described above.

After giving further effect to our issuance and sale of shares of common stock in this offering at an assumed initial public offering price of $ per share, which is the midpoint of the price range set forth on the cover page of this prospectus, and after deducting the estimated underwriting discounts and commissions and estimated offering expenses payable by us, our pro forma as adjusted net tangible book value as of , 2024 would have been $ million, or $ per share. This amount represents an immediate increase in pro forma net tangible book value of $ per share to our existing stockholders and immediate dilution of $ per share to new investors participating in this offering. We determine dilution by subtracting the pro forma as adjusted net tangible book value per share after this offering from the assumed initial public offering price per share paid by new investors.

The following table illustrates this dilution on a per share basis (without giving effect to any exercise by the underwriters of their option to purchase additional shares):

Assumed initial public offering price per share		$
Historical net tangible book value (deficit) per share as of , 2024	$	
Pro forma increase in net tangible book value (deficit) per share attributable to the pro forma transactions described above		
Pro forma net tangible book value per share as of , 2024		
Increase in pro forma as adjusted net tangible book value per share attributable to new investors participating in this offering		
Pro forma as adjusted net tangible book value per share after this offering		
Dilution per share to new investors participating in this offering		$

The dilution information discussed above is illustrative only and will change based on the actual initial public offering price and other terms of this offering determined at pricing. Each $1.00 increase or decrease in the assumed initial public offering price of $ per share, which is the midpoint of the price range set forth on the cover page of this prospectus, would increase or decrease, as applicable, the pro forma as adjusted net tangible book value by approximately $ million, or approximately $ per share, and increase or decrease, as applicable, the dilution per share to investors participating in this offering by approximately $, assuming that the number of shares offered by us, as set forth on the cover page of this prospectus, remains the same and after deducting the estimated underwriting discounts and commissions and estimated offering expenses payable by us. An increase of 1,000,000 shares in the number of shares we are offering would increase the

pro forma as adjusted net tangible book value per share after this offering by $ and decrease the dilution per share to new investors participating in this offering by $, assuming no change in the assumed initial public offering price per share and after deducting the estimated underwriting discounts and commissions and estimated offering expenses payable by us. A decrease of 1,000,000 shares in the number of shares we are offering would decrease the pro forma as adjusted net tangible book value per share after this offering by $ and increase the dilution per share to new investors participating in this offering by $, assuming no change in the assumed initial public offering price per share and after deducting the estimated underwriting discounts and commissions and estimated offering expenses payable by us.

If the underwriters exercise their option to purchase additional shares of our common stock in full, the pro forma as adjusted net tangible book value after this offering would be $ per share, the increase in pro forma net tangible book value would be $ per share and the dilution to new investors would be $ per share, in each case assuming an initial public offering price of $ per share, which is the midpoint of the price range set forth on the cover page of this prospectus, and after deducting the estimated underwriting discounts and commissions and estimated offering expenses payable by us.

The following table sets forth, on a pro forma as adjusted basis as of , 2024, the number of shares of common stock purchased from us, the total consideration paid to us and the average price per share paid by existing stockholders and to be paid by new investors purchasing shares of common stock in this offering at an assumed initial public offering price of $ per share, which is the midpoint of the price range set forth on the cover page of this prospectus, before deducting the estimated underwriting discounts and commissions and estimated offering expenses payable by us.

	Total shares		Total consideration		Weighted average price per share
	Number	Percent	Number	Percent	
Existing stockholders	$	%	$	%	
New Investors		%	$	%	
Total		100%	$	100%	

The table above assumes no exercise by the underwriters of their option to purchase additional shares of our common stock in this offering. If the underwriters were to exercise in full their option to purchase additional shares from us, the number of shares of common stock held by existing stockholders would be reduced to % of the total number of shares of common stock to be outstanding upon completion of this offering, and the number of shares of common stock held by new investors participating in this offering will be increased to % of the total number of shares of our common stock to be outstanding upon completion of the offering.

Each $1.00 increase or decrease in the assumed initial public offering price of $ per share, which is the midpoint of the price range set forth on the cover page of this prospectus, would increase or decrease, as applicable, the total consideration paid by new investors by approximately $ million, assuming that the number of shares offered by us, as set forth on the cover page of this prospectus, remains the same. We may also increase or decrease the number of shares we are offering. Similarly, each increase or decrease of 1,000,000 in the number of shares offered by us would increase or decrease, as applicable, total consideration paid by new investors by approximately $ million, assuming no change in the assumed initial public offering price.

The foregoing tables and calculations (other than historical net tangible book value) are based on the number of shares of our common stock outstanding as of , 2024, after giving effect to the automatic conversion of all of the outstanding preferred shares of our convertible preferred stock and convertible preferred stock warrants into an aggregate of shares of our common stock upon the closing of this offering (based on assumed initial public offering price of $ per share, which is the midpoint of the price range set forth on the cover page of this prospectus), as if such conversions had occurred on , 2024, and excludes:

- shares of our common stock issuable upon the exercise of stock options outstanding as of , 2024 pursuant to our 2016 Plan, with a weighted-average exercise price of $ per share;

- shares of our common stock reserved for future issuance under the 2016 Plan as of , 2024, which shares will cease to be available for issuance at the time our 2024 Plan, becomes effective in connection with this offering;
- shares of our common stock issuable upon the exercise of warrants outstanding at , 2024 at a weighted-average exercise price of $ per share;
- shares of our common stock reserved for future issuance under the 2024 Plan, which will become effective in connection with this offering, as well as any automatic increases in the number of shares of common stock reserved for future issuance under the 2024 Plan; and
- shares of our common stock reserved for future issuance under our ESPP, which will become effective in connection with this offering, as well as any automatic increases in the number of shares of common stock reserved for future issuance under the ESPP.

To the extent that stock options or warrants are exercised, new stock options or other equity awards are issued under our equity incentive plan or we issue additional shares of common stock in the future, there will be further dilution to investors participating in this offering. In addition, we may choose to raise additional capital because of market conditions or strategic considerations, even if we believe that we have sufficient funds for our current or future operating plans. If we raise additional capital through the sale of equity or convertible debt securities, the issuance of these securities could result in further dilution to our stockholders.

Management's discussion and analysis of financial condition and results of operations

You should read the following discussion and analysis of our financial condition and results of operations together with our consolidated financial statements and related notes appearing elsewhere in this prospectus. Some of the information contained in this discussion and analysis or set forth elsewhere in this prospectus, including information with respect to our plans and strategy for our business and related financing, and includes forward-looking statements that involve risks and uncertainties. As a result of many factors, including those factors set forth in the section titled "Risk factors," our actual results could differ materially from the results described in or implied by the forward-looking statements contained in the following discussion and analysis. See also the section titled "Special note regarding forward-looking statements." Our historical results are not necessarily indicative of the results that may be expected for any period in the future.

We are a clinical-stage biopharmaceutical company pioneering the discovery and development of regulatory RNA-based therapeutics with the goal of upregulating gene expression and restoring healthy protein levels to treat a broad range of genetic diseases. Regulatory RNAs, or regRNAs, play a central role in the regulation of every protein-coding gene by contributing to gene activation and suppression. Our approach is designed to amplify messenger RNA, or mRNA, expression by harnessing the power of regRNAs that form localized complexes with transcription factors and regulate gene expression. Our proprietary RNA Actuating Platform, or RAP Platform, allows us to rapidly and systematically identify and characterize the active regulatory elements controlling every expressed gene and tens of thousands of druggable enhancer and promoter regRNA sequences that control protein-coding genes. Once a disease-associated target gene is identified, we apply our RAP Platform to identify the controlling regRNA and rapidly generate novel antisense oligonucleotide, or ASO, candidates, which we also refer to as RNA Actuators. These ASOs are designed to bind to the identified regRNA and amplify the expression of the target gene in a specific and controllable way. We are initially focused on metabolic and central nervous system diseases with validated disease biology, and we believe our RAP Platform allows us to address a broad range of genetic diseases in which a modest increase in protein expression has the potential to be clinically meaningful.

Since our inception in 2015, we have focused substantially all of our resources primarily on developing our RAP Platform, identifying, developing and progressing our product candidates through preclinical and clinical development, organizing and staffing our company, research and development activities, establishing and protecting our intellectual property portfolio, and raising capital. To date, we have primarily funded our operations with proceeds from the sale of convertible preferred stock and revenues from our license and collaboration agreements. Through December 31, 2023, we have received gross proceeds of $188.3 million from the sale of our convertible preferred stock. In addition, through December 31, 2023, we have recognized $17.4 million in research collaboration and license revenue through our development and license agreements. Our ability to generate any product revenue and, in particular, our ability to generate product revenue sufficient to achieve profitability, will depend on the successful development and eventual commercialization of product candidates.

We have incurred significant operating losses and negative cash flows from operations since our inception. Our net losses were $49.3 million and $44.2 million for the years ended December 31, 2023 and 2022, respectively. As of December 31, 2023, we had an accumulated deficit of $160.0 million. Substantially all of our net losses have resulted from costs incurred in connection with our research and development programs and, to a lesser extent, from general and administrative costs associated with our operations. Our net losses may fluctuate significantly from quarter-to-quarter and year-to-year, depending on the timing of our clinical trials and preclinical studies, our other research and development activities and capital expenditures, and the timing and amount of any milestone or royalty payments due under our existing or future license or collaboration agreements. In addition, following the closing of this offering, we expect to incur additional costs associated with operating as a public company, including significant legal, audit, accounting, regulatory and tax-related services associated with maintaining compliance with exchange listing and SEC requirements, director and officer liability insurance costs, investor and public relations costs, and other expenses that we did not incur as a private company. If we obtain regulatory approval for our product candidates, we expect to incur significant expenses related to developing our commercialization capability to support product sales, marketing and distribution. We anticipate that our expenses will increase substantially if and as we:

- advance our lead product candidate, CMP-CPS-001, through clinical trials;
- finalize preclinical development for our programs for familial hypercholesterolemia and SYNGAP1-related disorders;
- conduct preclinical studies and clinical trials of any future product candidates;
- expand the capabilities of our RAP Platform and seek to identify and develop additional product candidates;
- seek marketing approvals for any product candidates that successfully complete clinical trials;
- obtain, expand, maintain, defend and enforce our intellectual property portfolio;
- hire additional clinical, regulatory and scientific personnel;
- contract with manufacturing sources for preclinical and clinical development of any future product candidates we may develop and commercial supply with respect to any such product candidates that receive regulatory approval;
- ultimately establish a sales, marketing and distribution infrastructure to commercialize any products for which we may obtain marketing approval; and
- add operational, legal, compliance, financial and management information systems and personnel to support our research, product development and future commercialization efforts, as well as to support our operations as a public company.

Because of the numerous risks and uncertainties associated with the development of therapeutics, we are unable to accurately predict the timing or amount of increased expenses and when, or if, we will be able to achieve or maintain profitability. Even if we are able to generate product sales, we may not become profitable. If we fail to become profitable or are unable to sustain profitability on a continuing basis, then we may be unable to continue our operations at planned levels and may be forced to reduce or terminate our operations.

We do not have any products approved for sale and have not generated any revenue from product sales. We will not generate revenue from product sales unless and until we successfully complete clinical development and obtain regulatory approval for our current and any future product candidates, which we expect will take a number of years or may never occur. As a result, we will need substantial additional funding in addition to the net proceeds from this offering to support our continuing operations and pursue our growth strategy. Until such time as we can generate significant revenue from product sales, if ever, we expect to finance our operations through equity offerings, debt financings, or other capital sources, including current or potential future collaborations, licenses, and other similar arrangements. However, we may be unable to raise additional funds or enter into such other agreements or arrangements when needed on favorable terms, or at all. If we fail to raise capital or enter into such agreements or arrangements as, and when needed, we may delay, limit, reduce or terminate our product development or future commercialization efforts or grant rights to develop and market product candidates that we would otherwise prefer to develop and market ourselves, or even cease operations.

As of December 31, 2023, we had cash and cash equivalents of $38.4 million. Based on our current operating plan, we estimate that our existing cash and cash equivalents as of the date of this prospectus, together with the net proceeds from this offering, will be sufficient to fund our projected operating expenses and capital expenditure requirements through at least . However, we have based this estimate on assumptions that may prove to be wrong, and our operating plan may change as a result of many factors currently unknown to us. In addition, we could utilize our available capital resources sooner than we expect. See the sections titled "—Liquidity and capital resources" and "Risk factors—Risks related to our financial position and need for additional capital" included elsewhere in this prospectus.

We do not own or operate, and currently have no plans to establish, any manufacturing facilities. We rely, and expect to continue to rely, on third parties for clinical supplies as well as commercial supplies if we obtain marketing approval. In addition, we rely on third parties to package, label, store, and distribute our clinical supply and we intend to rely on third parties to conduct the same activities for our commercial products if we obtain

regulatory approval. We believe that this strategy allows us to maintain a more efficient infrastructure by eliminating the need for us to invest in our own manufacturing facilities, equipment, and personnel while also enabling us to focus our expertise and resources on the development of product candidates and continued enhancement of our RAP Platform.

Collaboration and license agreements

Below is a summary of the key terms and financial statement impact of certain of our license and collaboration agreements. For a more detailed description of these agreements, see the section titled “Business—License and collaboration agreements.”

In-license agreements

Whitehead Institute for Biomedical Research

In October 2019, we entered into a patent license agreement with the Whitehead Institute for Biomedical Research, or the Whitehead Institute, which was subsequently amended on December 14, 2021, or the Whitehead First Amendment, and on November 7, 2023, or the Whitehead Second Amendment. Under the agreement, we were granted a worldwide, royalty-bearing, sublicensable license under certain patent rights owned or controlled by the Whitehead Institute. As part of the agreement, we paid an initial $0.1 million license issuance fee, and de minimis additional fees in connection with each of the Whitehead First Amendment and Whitehead Second Amendment that were recorded as research and development expense in our consolidated statement of operations and comprehensive loss. We are also obligated to pay annual license maintenance fees for the term of the agreement, pursuant to which we have paid an aggregate of $0.16 million through December 31, 2023. In addition, we are obligated to pay certain filing, prosecution and maintenance fees with respect to certain patent rights licensed to us under the agreement, pursuant to which we have paid an aggregate of $0.22 million through December 31, 2023. We are obligated to pay potential development milestone payments of up to an aggregate of low single-digit millions of dollars under the terms of the agreement upon the achievement of certain specified contingent events. In addition, if we successfully commercialize a product under the agreement, we are also obligated to pay tiered royalties at percentage rates ranging from less than one percent to the mid-single digits of net sales or of running royalties of net sales, subject to specified reductions, until either the last-to-expire valid claim of a Whitehead Institute patent covering the product or a duration in the late single digit years after the first commercial sale, in each case on a product-by-product and country-by-country basis. We incurred $0.08 million and $0.04 million of license maintenance and amendment issuance fees owed to the Whitehead Institute in 2023 and 2022, respectively, under the amended agreement and recorded the amounts in our research and development expense in our consolidated statement of operations and comprehensive loss.

Children’s Medical Center Corporation

In April 2018, we entered into a development and license agreement, or the CMCC Agreement, with Children’s Medical Center Corporation, or the CMCC. The agreement allows us to use the proprietary intellectual property of the CMCC to conduct research, development and commercialization of products utilizing CMCC’s proprietary intellectual property in return for specified payments. The proprietary intellectual property licensed pursuant to this agreement is related to certain legacy programs we are not pursuing and was subsequently sublicensed to Fulcrum Therapeutics, Inc., or Fulcrum, as described below. As part of the CMCC Agreement, we issued a total of 169,624 shares of common stock to CMCC and certain of its affiliates based on the fair value of the common stock on the date of issuance.

We are obligated to pay potential development milestone payments under the terms of the CMCC Agreement of up to $7.7 million for the first licensed target, $3.9 million for the second licensed target and $1.9 million for the third licensed target upon the achievement of certain specified contingent events. If commercial sales of a licensed product commence, we will pay CMCC royalties at percentage rates ranging in the low- to mid-single digits on net sales of licensed products in countries where such product is protected by patent rights. We incurred $0.03 million of royalties owed to CMCC in both 2023 and 2022 under the CMCC Agreement and recorded the

amounts in research and development expense in the consolidated statement of operations and comprehensive loss. Further, under the terms of the CMCC Agreement, we are required to pay 10% of any upfront payment received under a sublicensing agreement entered into prior to the initiation of the first investigational new drug study. As such, we recorded $0.04 million of sublicense costs for the year ended December 31, 2023, which is presented in our research and development expenses on our consolidated statements of operations and comprehensive loss.

Out-license agreements

Fulcrum Therapeutics, Inc.

In July 2023, we entered into a license agreement with Fulcrum. Under this license agreement, we granted an exclusive license related to our related intellectual property and granted a non-exclusive sublicense for the intellectual property obtained through the CMCC Agreement. In exchange for the license rights, Fulcrum paid us a $0.35 million upfront payment. In the event that Fulcrum achieves development and commercial milestones, Fulcrum will be obligated to pay us one-time milestone payments ranging from $1.0 million to $20.0 million (with respect to a Tier 1 Product, as defined in the agreement) or $0.6 million to $12.0 million (with respect to a Tier 2 Product, as defined in the agreement), depending on the milestone achieved. In addition, this license agreement includes both potential nominal minimum annual royalty payments as well as sales-based royalties upon commercialization of up to the low-double digits.

During the year ended December 31, 2023, we recorded $0.35 million in research and collaboration revenue pursuant to this out-license agreement with Fulcrum.

Collaborative arrangement

Eli Lilly and Company

In July 2023, we executed a Material Transfer Agreement, or MTA, with Eli Lilly and Company, or Eli Lilly. As part of the MTA, we and Eli Lilly agreed to perform research and development activities to generate up to three ASOs in accordance with a prescribed workplan. For the year ended December 31, 2023, we received $0.4 million from Eli Lilly related to the MTA. We and Eli Lilly are jointly overseeing the research and development activities under the MTA. During the year ended December 31, 2023, we recorded $0.5 million as a reduction in research and development expense in the consolidated statement of operations and comprehensive loss. Additionally, we had an unbilled receivable of $0.1 million recorded within prepaid expenses and other current assets on our consolidated balance sheet as of December 31, 2023.

Components of our results of operations

Revenue

For the year ended December 31, 2023, we have recognized $0.35 million in research collaboration and license revenue through our collaboration and license agreements. We did not recognize any research collaboration and license revenue during the year ended December 31, 2022. We have not generated any revenue from the sale of products, however, and do not expect to generate any revenue from the sale of products in the foreseeable future, if at all. If our or our collaborators' development efforts for product candidates and any future product candidates are successful and result in regulatory approval, we may generate revenue in the future from product sales, payments from existing or potential future collaboration or license agreements with third parties, or any combination thereof.

Operating expenses

Our operating expenses consist of (i) research and development, or R&D, expenses and (ii) general and administrative expenses.

Research and development expenses

Research and development expenses consist primarily of external and internal costs incurred in performing clinical and preclinical development activities.

Our R&D expenses consist of:

- external costs incurred under agreements with contract research organizations, or CROs, contract manufacturing organizations, or CMOs, consultants and other third parties to conduct and support our clinical trials and preclinical studies;
- internal costs, including R&D personnel-related expenses such as salaries and stock-based compensation and benefits, as well as allocated facilities costs and depreciation; and
- costs associated with our licensing activities.

We expense R&D costs as incurred. Certain third-party costs for R&D activities are recognized based on an evaluation of the progress to completion of specific tasks using information and data provided to us by our management and scientific personnel, vendors and third-party service providers. Non-refundable advance payments for goods and services that will be used over time for R&D are deferred and capitalized as R&D prepaid expenses on our consolidated balance sheets. The capitalized amounts are recognized as an expense as the goods are delivered or as the related services are performed. Since our inception, substantially all of our external costs were related to the development of product candidates. We use internal resources for platform development, early pipeline discovery, preclinical development, management of clinical development activities, technical operations and oversight of manufacturing partners. We do not track our research and development expenses on a program-by-program basis. Our third-party research and development expenses consist primarily of fees paid to outside consultants, CROs, CMOs and research laboratories in connection with our preclinical development, process development, manufacturing and clinical development activities. Our other R&D costs are internal costs primarily associated with our discovery efforts, laboratory supplies, and facilities, including depreciation that are deployed across multiple programs.

Although R&D activities are central to our business model, the successful development of any future product candidates is highly uncertain. There are numerous factors associated with the successful development of any product, including future trial design and various regulatory requirements, many of which cannot be determined with accuracy at this time based on our stage of development. In addition, future regulatory factors beyond our control may impact our clinical development programs. Product candidates in later stages of clinical development generally have higher development costs than those in earlier stages of clinical development, primarily due to the increased size and longer duration of later-stage clinical trials. As a result, we expect our R&D expenses will increase substantially in connection with our ongoing and planned clinical and preclinical development activities in the near term and in the future. At this time, we cannot accurately estimate or know the nature, timing and costs of the efforts that will be necessary to complete the preclinical and clinical development of our current product candidates and any future product candidates. Our future R&D expenses may vary significantly based on a wide variety of factors such as:

- the number and scope, rate of progress, expense and results of our clinical trials and preclinical studies and any future product candidates we may choose to pursue, including any modifications to clinical development plans based on feedback that we may receive from regulatory authorities;
- per patient trial costs;
- the number of trials required for approval;
- the number of sites included in the trials;
- the countries in which the trials are conducted;
- the length of time required to enroll eligible patients;
- the number of patients that participate in the trials;

- the number of doses that patients receive;
- the drop-out or discontinuation rates of patients;
- the potential additional safety monitoring requested by regulatory agencies;
- the duration of patient participation in the trials and follow-up;
- the cost and timing of manufacturing clinical supply;
- the extent of changes in government regulation and regulatory guidance;
- the timing, receipt, and terms of any approvals from applicable regulatory authorities; and
- the extent to which we establish additional collaboration, license, or other arrangements.

A change in the outcome of any of these variables with respect to the development of our product candidates or any future product candidates could significantly change the costs and timing associated with the development of that product candidate.

General and administrative expenses

General and administrative expenses consist primarily of personnel-related expenses such as salaries and stock-based compensation and benefits for our personnel in executive, legal, finance and accounting, human resources and other administrative functions. General and administrative expenses also include legal fees relating to patent and corporate matters and professional fees paid for accounting, auditing, consulting and tax services, as well as facilities-related costs not otherwise included in R&D expenses and other costs such as insurance costs and travel expenses.

We anticipate our general and administrative expenses will increase substantially in the future as we expand our operations, including increasing our headcount to support our continued R&D activities and continue to advance the development of our product candidates. We also anticipate we will incur increased accounting, audit, legal, regulatory, compliance, director and officer insurance, and investor and public relations expenses associated with operating as a public company.

Other income (expense), net

Interest income

Interest income relates to interest earned on our invested cash and cash equivalent balances. We expect our interest income will increase as we invest the cash received from the net proceeds from this offering.

Other (expense)

Other (expense) consists of miscellaneous items, such as foreign exchange gains and losses and other insignificant amounts.

Comparison of the years ended December 31, 2023 and 2022

The following table summarizes our results of operations for the years ended December 31, 2023 and 2022 (in thousands):

	Year ended December 31,		
	2023	2022	Change ($)
Revenue			
Research and collaboration revenue	$ 350	$ —	$ 350
Operating expenses			
Research and development	$ 40,616	$ 34,771	$ 5,845
General and administrative	11,613	10,230	1,383
Total operating expenses	52,229	45,001	7,228
Loss from operations	(51,879)	(45,001)	(6,878)
Other income (expense), net			
Interest income	2,808	904	1,904
Other (expense)	(220)	(95)	(125)
Total other income (expense), net	2,588	809	1,779
Net loss and comprehensive loss	$ (49,291)	$ (44,192)	$ (5,099)

Research and collaboration revenue

Research and collaboration revenue was $0.35 million for the year ended December 31, 2023 compared to $0 for the year ended December 31, 2022. The increase of $0.35 million was due to revenue generated from the sublicense agreement with Fulcrum.

Research and development expenses

The following table summarizes our R&D expenses for the years ended December 31, 2023 and 2022 (in thousands):

	Year ended December 31,		
	2023	2022	Change ($)
Clinical and preclinical expenses	$ 19,841	$ 19,750	$ 91
Personnel-related expenses	14,715	11,050	3,665
Professional fees	1,324	509	815
Facility-related and other expenses	4,736	3,462	1,274
Total research and development expenses	$ 40,616	$ 34,771	$ 5,845

Research and development expenses were $40.6 million for the year ended December 31, 2023 compared to $34.8 million for the year ended December 31, 2022. The increase of $5.8 million in R&D expenses for the year ended December 31, 2023 was primarily due an increase of $3.7 million in personnel-related expenses due to increased average headcount and increased stock option grant activity, an increase of $0.7 million in depreciation associated to new property and equipment purchases, an increase of $0.8 million in professional and consulting fees associated with preclinical, regulatory and clinical affairs and continued development of our lead product candidate, and an increase of $0.6 million in lab operations and information technology expenses.

General and administrative expenses

The following table summarizes our general and administrative expenses for the years ended December 31, 2023 and 2022 (in thousands):

	Year ended December 31,		
	2023	2022	Change ($)
Personnel-related expenses	$ 6,909	$ 5,378	$ 1,531
Professional and consultant fees	2,670	2,926	(256)
Facilities, fees and other related costs	2,034	1,926	108
Total general and administrative expenses	$ 11,613	$ 10,230	$ 1,383

General and administrative expenses were $11.6 million for the year ended December 31, 2023 compared to $10.2 million for the year ended December 31, 2022. The increase of $1.4 million in general and administrative expenses for the year ended December 31, 2023 was primarily due to an increase in personnel-related expenses of $1.5 million from wages, stock-based compensation and bonus expense due to increased average headcount, higher facilities fees and other related costs of $0.1 million due to the commencement of the Boulder, CO operating lease, offset by decreased legal fees, accounting and consulting fees of $0.3 million due to increased average headcount.

Other income (expense), net

Other income (expense), net was $2.6 million for the year ended December 31, 2023 compared to $0.8 million for the year ended December 31, 2022. The increase of $1.8 million was primarily due to an increase in interest income due to higher average invested cash equivalent balances as well as higher interest rates in 2023. Other expense for the year ended December 31, 2023 was $0.2 million compared to other expense of $0.1 million for the year ended December 31, 2022, primarily due to an increase in foreign exchange losses.

Liquidity and capital resources

Sources of liquidity

Since our inception, we have not generated any revenue from product sales and have incurred significant operating losses and negative cash flows from operations. We expect to incur significant expenses and operating losses in the foreseeable future as we advance the development of product candidates. To date, we have primarily funded our operations with proceeds from the sale of shares of our convertible preferred stock and revenues from our license and collaboration agreements. Through December 31, 2023, we have received aggregate gross proceeds of $188.3 million from the sale of shares of our convertible preferred stock. In addition, through December 31, 2023, we have recognized $17.4 million in research and collaboration revenue through our collaboration and license agreements. As of December 31, 2023, we had cash and cash equivalents of $38.4 million.

Our current capital resources, which consist of cash and cash equivalents, will not be sufficient to fund operations through at least the next twelve months from the date the accompanying consolidated financial statements are issued based on our expected cash needs, which raises substantial doubt about our ability to continue as a going concern. In their report accompanying our audited financial statements for the years ended December 31, 2023 and 2022, our independent registered public accounting firm included an explanatory paragraph stating that our recurring losses from operations raise substantial doubt about our ability to continue as a going concern. Our future viability is dependent on our ability to generate cash from our operating activities or to raise additional capital to finance our operations. There is no assurance that we will succeed in obtaining sufficient funding on terms acceptable to us to fund continuing operations, if at all.

Future funding requirements

We expect our expenses to increase substantially in connection with our ongoing activities, particularly as we continue our development of, seek regulatory approval for, and potentially commercialize our product candidates

and seek to discover and develop additional product candidates, conduct our ongoing and planned clinical trials and preclinical studies, continue our research and development activities, hire additional personnel, expand and protect our intellectual property, and incur additional costs associated with being a public company.

The timing and amount of our funding requirements will depend on many factors, including:

- the scope, timing and progress of our ongoing CMP-CPS-001 clinical trial;
- the initiation, type, number, scope, progress, expansions, results, costs and timing of preclinical studies and clinical trials of our product candidates and any future product candidates we may choose to pursue, including the costs of modification to clinical development plans based on feedback that we may receive from regulatory authorities and any third-party products used as combination agents in our clinical trials;
- the costs and timing of manufacturing for our product candidates, including commercial manufacturing at sufficient scale, if any product candidate is approved;
- the costs, timing and outcome of regulatory meetings and reviews of product candidates or any future product candidates, including requirements of regulatory authorities in any additional jurisdictions in which we may seek approval and any future product candidates;
- the costs of obtaining, maintaining, enforcing and protecting our patents and other intellectual property and proprietary rights;
- our efforts to enhance operational systems and hire additional personnel to satisfy our obligations as a public company, including enhanced internal control over financial reporting;
- the costs associated with hiring additional personnel and consultants as our clinical and preclinical activities increase and as we operate as a public company;
- the timing and payment of milestone, royalty or other payments we must make pursuant to our existing and potential future license or collaboration agreements with third parties;
- the costs and timing of establishing or securing sales and marketing capabilities if our product candidates or any future product candidate is approved;
- our ability to achieve sufficient market acceptance, coverage, and adequate reimbursement from third-party payors and adequate market share and revenue for any approved products;
- patients' ability and willingness to pay out-of-pocket for any approved products in the absence of coverage and/or adequate reimbursement from third-party payors;
- the terms and timing of establishing and maintaining collaborations, licenses and other similar arrangements; and
- costs associated with any products or technologies that we may in-license or acquire.

Based upon our current operating plan, we estimate that our existing cash and cash equivalents as of the date of this prospectus, together with the net proceeds from this offering, will be sufficient to fund our projected operating expenses and capital expenditure requirements through at least . However, we have based this estimate on assumptions that may prove to be wrong, and we could deplete our capital resources sooner than we currently expect. Our operating plans and other demands on our cash resources may change as a result of many factors currently unknown to us, and we may need to seek additional funds sooner than planned.

We have no other committed sources of capital. Until such time, if ever, we can generate substantial product revenues, we expect to finance our operations through the sale of equity securities, debt financings, working capital lines of credit, strategic alliances and/or license arrangements, grant funding, interest income earned on invested cash balances or a combination of one or more of these sources. However, we may be unable to raise additional funds or enter into such other arrangements when needed, on favorable terms or at all. To the extent we raise additional capital through the sale of equity or convertible debt securities, investors' ownership interest

will be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect the rights of a common stockholder. Debt financing and preferred equity financing, if available, may involve agreements that include covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, making acquisitions, engaging in acquisition, merger or collaboration transactions, selling or licensing our assets, making capital expenditures, redeeming our stock, making certain investments or declaring dividends. If we raise additional funds through collaborations or license agreements with third parties, we may have to relinquish valuable rights to our technologies, future revenue streams, research programs or product candidates, or grant licenses on terms that may not be favorable to us. If we are unable to raise additional funds through equity or debt financings when needed, we may be required to delay, limit, reduce or terminate our product development or future commercialization efforts or grant rights to develop and market product candidates that we would otherwise prefer to develop and market ourselves, or even cease operations.

Material cash requirements for known contractual and other obligations

Leases

We have entered into two non-cancellable operating leases for our office and lab space in Cambridge, Massachusetts and Boulder, Colorado. The Cambridge, Massachusetts operating lease expires on June 30, 2027 and the Boulder, Colorado operating lease expires on September 30, 2028. See Note 7 to our consolidated financial statements for additional details related to our noncancellable operating leases.

Finance leases

We have entered into various finance leases for lab equipment. See Note 7 to our consolidated financial statements for additional details related to our finance leases.

Restricted cash

In connection with its operating leases, we are required to maintain security deposits, which were issued in the form of letters of credit with a bank. See Note 2 to our consolidated financial statements for additional details related to our restricted cash.

Research and development costs

We are continuing to invest in the clinical development of CMP-CPS-001 and have entered into contractual obligations with CROs relating to the performance of clinical trial services. Each contract shall continue until the completion of the trial. Our clinical trial costs are dependent on, among other things, the size and length of our clinical trial. We also incur research and development costs related to the enhancement of our existing product candidates.

Other capital requirements and additional royalty obligations

We enter into agreements in the normal course of business with various vendors, which are generally cancellable with a contractually defined notice period. Payments due upon cancellation typically consist of payments for services provided or expenses incurred, as well as non-cancellable obligations of service providers, up to the date of cancellation.

The timing of when we will pay or receive royalty payments is uncertain as the payments are contingent upon future activities, including the successful discovery, development, regulatory approval and commercialization of product candidates.

Cash flows

The following table provides information regarding our cash flows for the years ended December 31, 2023 and 2022 (in thousands):

	Year ended December 31,	
	2023	2022
Net cash used in operating activities	$ (44,155)	$ (38,543)
Net cash used in investing activities	(678)	(4,025)
Net cash provided by financing activities	301	100,157
Net (decrease) increase in cash and cash equivalents	$ (44,532)	$ 57,589

Operating activities

During the year ended December 31, 2023, operating activities used $44.2 million of cash, primarily resulting from our net loss of $49.3 million and net cash used in changes in our operating assets and liabilities of $3.9 million, partially offset by non-cash charges of $9.1 million, including depreciation and amortization, stock-based compensation expense and non-cash operating lease expense.

During the year ended December 31, 2022, operating activities used $38.5 million of cash, primarily resulting from our net loss of $44.2 million, partially offset by non-cash charges of $3.9 million, including stock-based compensation expense and non-cash operating lease expense, and net cash used in changes in our operating assets and liabilities of $1.7 million.

Investing activities

During the year ended December 31, 2023, net cash used in investing activities was $0.7 million, due to purchases of property and equipment.

During the year ended December 31, 2022, net cash used in investing activities was $4.0 million, due to purchases of property and equipment.

Financing activities

During the year ended December 31, 2023, net cash provided by financing activities was $0.3 million, consisting primarily of net proceeds of $0.7 million from a financing obligation and proceeds of $0.2 million from the exercise of common stock options, offset by $0.3 million of finance lease principal payments and $0.3 million of repayments for our financing liability related to such financing obligation.

During the year ended December 31, 2022, net cash provided by financing activities was $100.2 million, consisting of net proceeds from the issuance of Series B convertible preferred stock and proceeds from the exercise of common stock options.

Critical accounting policies and significant judgments and estimates

Our consolidated financial statements are prepared in accordance with generally accepted accounting principles in the United States. The preparation of our consolidated financial statements and related disclosures requires us to make estimates and judgments that affect the reported amounts of assets, liabilities, costs and expenses, and the disclosure of contingent assets and liabilities in our consolidated financial statements. We base our estimates on historical experience, known trends and events, and various other factors we believe are reasonable under the circumstances, the results of which form the basis for making judgments about the carrying values of assets and liabilities that are not readily apparent from other sources. We evaluate our estimates and assumptions on an ongoing basis. Our actual results may differ from these estimates under different assumptions or conditions.

While our significant accounting policies are described in more detail in Note 2 to our consolidated financial statements included elsewhere in this prospectus, we believe the following accounting policies are those most critical to the judgments and estimates used in the preparation of our consolidated financial statements.

Research and development expenses and related prepaid and accrued expenses

As part of the process of preparing our consolidated financial statements, we are required to estimate our R&D expenses as of each balance sheet date. This process involves reviewing open contracts and purchase orders, communicating with our personnel to identify services that have been performed on our behalf, and estimating the level of service performed and the associated cost incurred for the service when we have not yet been invoiced or otherwise notified of the actual cost. We make estimates of our R&D expenses as of each balance sheet date based on facts and circumstances known to us at that time. The significant estimates in our R&D expenses include the costs incurred for services performed by our vendors in connection with services for which we have not yet been invoiced. We base our expenses related to R&D activities on our estimates of the services received and efforts expended pursuant to quotes and contracts with vendors that conduct R&D on our behalf. The financial terms of these agreements are subject to negotiation, vary from contract to contract, and may result in uneven payment flows.

There may be instances in which payments made to our vendors will exceed the level of services provided and result in a prepayment of the R&D expense. In accruing service fees, we estimate the time period over which services will be performed and the level of effort to be expended in each period. If the actual timing of the performance of services or the level of effort varies from our estimate, we adjust the accrual or prepaid expense accordingly. Advance payments for goods and services that will be used in future R&D activities are expensed when the activity has been performed or when the goods have been received rather than when the payment is made.

Although we do not expect our estimates to be materially different from amounts actually incurred, if our estimates of the status and timing of services performed differ from the actual status and timing of services performed, it could result in us reporting amounts that are too high or too low in any particular period. To date, there have been no material differences between our estimates of such expenses and the amounts actually incurred.

Stock-based compensation

We periodically grant equity-based payment awards in the form of stock options to employees, directors and non-employees and record stock-based compensation expenses for awards of stock-based payments based on their estimated fair value at the grant date. We recognize stock-based compensation expense for all equity-based payments, including stock options. Stock-based compensation costs are calculated based on the estimated fair value of the underlying option using the Black-Scholes option-pricing model on the date of grant for stock options and recognized as expense in the accompanying consolidated statement of operations and comprehensive loss on a straight-line basis over the requisite service period, which is typically the vesting period. Determining the appropriate fair value model and related input assumptions requires judgment, including estimating the fair value of our common stock, and stock price volatility. Estimating the fair value of equity awards at the grant date using valuation models, such as the Black-Scholes option -pricing model, is affected by assumptions regarding a number of variables, including:

- the risk-free interest rate used is based on the published U.S. Department of Treasury interest rates in effect at the time of stock option grant for zero coupon U.S. Treasury notes with maturities approximating each grant's expected term;
- the dividend yield is zero as we have not paid dividends and do not anticipate paying a cash dividend in the foreseeable future;
- the expected term for options granted is calculated using the simplified method and represents the average time that options are expected to be outstanding based on the mid-point between the vesting date and the end of the contractual term of the award;
- expected volatility is derived from the historical volatilities of a select group of representative public companies, for a look-back period commensurate with the expected term of the stock options, as we have no trading history of common stock; and

- fair value of common stock is derived from the third-party valuations discussed further below.

See Note 10 to our consolidated financial statements included elsewhere in this prospectus for information concerning certain of the specific assumptions we used in applying the Black-Scholes option pricing model to determine the estimated fair value of our stock options granted in the periods presented.

The intrinsic value of all outstanding options as of , 2024 was $ million based on the assumed initial public offering price of $ per share, which is the midpoint of the price range set forth on the cover of this prospectus, of which approximately $ was related to vested options and approximately $ was related to unvested options.

Determination of fair value of our common stock

Given the absence of a public trading market to date, the fair value of our common stock has been determined by our board of directors at the time of each option grant, with input from management, considering contemporaneous independent third-party valuations of common stock, and our board of directors' assessment of additional objective and subjective factors that it believed were relevant and which may have changed from the date of the most recent valuation through the date of the grant, including: the prices at which we sold shares of our convertible preferred stock to outside investors in arms-length transactions, and the superior rights, preferences, and privileges of the convertible preferred stock relative to the common stock at the time of each grant; the progress of our company's R&D programs, including their stages of development, and our company's business strategy; operating and financial performance; the lack of liquidity of the common stock and trends in the broader economy and biotechnology industry also impact the determination of the fair value of the common stock; the likelihood of achieving a liquidity event for our company's securityholders, such as an initial public offering or a sale of the company, taking into consideration prevailing market conditions; and the hiring of key personnel and the experience of management.

These independent third-party valuations were performed in accordance with the guidance outlined in the American Institute of Certified Public Accountants' Auditing and Valuation Guide, *Valuation of Privately-Held-Company Equity Securities Issued as Compensation*, or the Guide. The methodology to determine the fair value of our common stock included estimating the fair value of the enterprise using a market approach, which estimates the fair value of a company by including an estimation of the value of the business based on guideline public companies under a number of different scenarios. The Guide identifies various available methods for allocating enterprise value across classes and series of capital stock to determine the estimated fair value of common stock at each valuation date.

In accordance with the Guide, we considered the following methods:

- *Option Pricing Method, or OPM*. Under the OPM, shares are valued by creating a series of call options with exercise prices based on the liquidation preferences and conversion terms of each equity class. The estimated fair values of the convertible preferred stock and common stock are inferred by analyzing these options. This method is appropriate to use when the range of possible future outcomes is so difficult to predict that estimates would be highly speculative, and dissolution or liquidation is not imminent.
- *Probability-Weighted Expected Return Method, or PWERM*. The PWERM is a scenario-based analysis that estimates value per share based on the probability-weighted present value of expected future investment returns, considering each of the possible outcomes available to us, as well as the economic and control rights of each share class.
- *Hybrid Method*. The Hybrid Method is a hybrid between PWERM and OPM, where the equity value is estimated based on probability-weighted value across multiple scenarios where the OPM is used to estimate the allocation of value within one or more of those scenarios.

Based on our early stage of development, the difficulty in predicting the range of specific outcomes (and their likelihood), and other relevant factors, we determined the OPM scenario was most appropriate for valuations through April 2024.

These third-party valuations were performed at various dates, which resulted in valuation of our common stock of $ per share as of , 2024. Our board of directors considered various objective and subjective factors to determine the fair value of our common stock as of each grant date, including:

- the prices at which we sold shares of convertible preferred stock and the superior rights and preferences of the convertible preferred stock relative to our common stock at the time of each grant;
- the progress of our research and development programs, including the status and results of clinical and preclinical studies for our product candidates;
- our stage of development and our business strategy;
- external market conditions affecting the biopharmaceutical industry and trends within the biopharmaceutical industry;
- our financial position, including cash on hand, and our historical and forecasted performance and operating results;
- the lack of an active public market for our common stock and our convertible preferred stock;
- the likelihood of achieving a liquidity event, such as an initial public offering, an IPO, or sale of our company in light of prevailing market conditions; and
- the analysis of IPOs and the market performance of similar companies in the biopharmaceutical industry.

The assumptions underlying these valuations were highly complex and subjective and represented management's best estimates, which involved inherent uncertainties and the application of management's judgment. As a result, if we had used significantly different assumptions or estimates, the fair value of our common stock and our stock-based compensation expense could be materially different.

Once a public trading market for our common stock has been established in connection with the completion of this offering, it will no longer be necessary for our board of directors to estimate the fair value of our common stock in connection with our accounting for granted stock options or for any other such awards we may grant, as the fair value of our common stock will be determined based on the closing price of our common stock as reported on the date of grant on the primary stock exchange on which our common stock is traded.

Off-balance sheet arrangements

We did not have, during the periods presented, and we do not currently have, any off-balance sheet arrangements, as defined in the rules and regulations of the SEC.

Recently issued accounting standards

A description of recently issued accounting standards that may potentially impact our financial position, cash flows, and results of operations is included in Note 2 to our consolidated financial statements.

Emerging growth company and smaller reporting company status

We are an emerging growth company, as defined in the JOBS Act, and we may remain an emerging growth company for up to five years following the completion of this offering. For so long as we remain an emerging growth company, we are permitted and intend to rely on certain exemptions from various public company reporting requirements, including not being required to have our internal control over financial reporting audited by our independent registered public accounting firm pursuant to Section 404 of the Sarbanes-Oxley Act, reduced disclosure obligations regarding executive compensation in our periodic reports and proxy statements, exemptions from the requirements of holding a nonbinding advisory vote on executive compensation and any golden parachute payments not previously approved and an exemption from compliance with the requirements regarding the communication of critical audit matters in the auditor's report on financial statements. In particular, in this prospectus, we have provided only two years of audited financial statements and have not included all of

the executive compensation-related information that would be required if we were not an emerging growth company. Accordingly, the information contained herein may be different than the information you receive from other public companies in which you hold equity interests.

In addition, the JOBS Act provides that an emerging growth company can take advantage of an extended transition period for complying with new or revised accounting standards. We have elected to avail ourselves of this extended transition period, which means that when a standard is issued or revised and it has different application dates for public or private companies, we will adopt the new or revised standard at the time private companies adopt the new or revised standard and will do so until such time that we either (i) irrevocably elect to "opt out" of such extended transition period or (ii) no longer qualify as an emerging growth company. We may choose to early adopt any new or revised accounting standards whenever such early adoption is permitted for private companies. As a result of this election, our financial statements may not be comparable to those of companies that are not emerging growth companies.

We will remain an emerging growth company until the earliest to occur of: (i) the last day of the fiscal year in which we have at least $1.235 billion in annual revenue; (ii) the last day of the fiscal year in which we are deemed to be a "large accelerated filer," as defined in Rule 12b-2 under the Exchange Act, which would occur if the market value of our common stock held by non-affiliates exceeded $700.0 million as of the last business day of the second fiscal quarter of such year; (iii) the date on which we have issued more than $1.0 billion in nonconvertible debt securities during the prior three-year period; and (iv) the last day of the fiscal year ending after the fifth anniversary of this offering.

We are also a "smaller reporting company," meaning that the market value of our stock held by non-affiliates plus the proposed aggregate amount of gross proceeds to us as a result of this offering is less than $700.0 million and our annual revenue is less than $100.0 million during the most recently completed fiscal year. We may continue to be a smaller reporting company after this offering if either (i) the market value of our stock held by non-affiliates is less than $250.0 million; or (ii) our annual revenue was less than $100.0 million during the most recently completed fiscal year and the market value of our stock held by non-affiliates is less than $700.0 million. If we are a smaller reporting company at the time we cease to be an emerging growth company, we may continue to rely on exemptions from certain disclosure requirements that are available to smaller reporting companies. Specifically, as a smaller reporting company, we may choose to present only the two most recent fiscal years of audited financial statements in our Annual Report on Form 10-K and, similar to emerging growth companies, smaller reporting companies have reduced disclosure obligations regarding executive compensation. We may continue to be a smaller reporting company until the fiscal year following the determination that we no longer meet the requirements necessary to be considered a smaller reporting company.

Quantitative and qualitative disclosures about market risks

Interest rate risk

Our cash and cash equivalents consist of cash held in readily available checking and money market accounts. Our primary exposure to market risk is interest rate sensitivity, which is affected by changes in the general level of U.S. interest rates. However, because of the short-term nature of the instruments in our portfolio, a sudden change in market interest rates would not be expected to have a material impact on our financial condition or results of operations.

Foreign currency exchange risk

We are not currently exposed to significant market risk related to changes in foreign currency exchange rates. As we continue to develop our business, our results of operations and cash flows will likely be more affected by fluctuations in foreign currency exchange rates, including the Euro and other currencies, which could adversely affect our results of operations. To date, we have not entered into any foreign currency hedging contracts to mitigate our exposure to foreign currency exchange risk. We do not believe that a hypothetical 10% increase

or decrease in exchange rates during any of the periods presented would have had a material impact on our consolidated financial statements included elsewhere in this prospectus.

Effects of inflation

Inflation could affect us by increasing our cost of labor and R&D costs. We do not believe inflation has had a material effect on our business, financial condition or results of operations, or on our consolidated financial statements included elsewhere in this prospectus.

Business

CAMP4 is the final camp before the summit of Mount Everest. It is also home to a climbing haven in Yosemite National Park where the world's greatest climbers gather to push the boundaries for what is thought to be possible. Like these elite climbers, we are pushing the boundaries of biology to discover and develop new and potentially life changing therapeutics.

Our company

We are a clinical-stage biopharmaceutical company pioneering the discovery and development of regulatory RNA-based therapeutics with the goal of upregulating gene expression and restoring healthy protein levels to treat a broad range of genetic diseases. Regulatory RNAs, or regRNAs, play a central role in the regulation of every protein-coding gene by contributing to gene activation and suppression. Our approach is designed to amplify messenger RNA, or mRNA, expression by harnessing the power of regRNAs that form localized complexes with transcription factors and regulate gene expression. Our proprietary RNA Actuating Platform, or RAP Platform, allows us to rapidly and systematically identify and characterize the active regulatory elements controlling every expressed gene and tens of thousands of druggable enhancer and promoter regRNA sequences that control protein-coding genes. Once a disease-associated target gene is identified, we apply our RAP Platform to identify the controlling regRNA and rapidly generate novel antisense oligonucleotide, or ASO, candidates, which we also refer to as RNA Actuators. These ASOs are designed to bind to the identified regRNA and amplify the expression of the target gene in a specific and controllable way. We are initially focused on metabolic and central nervous system, or CNS, diseases with validated disease biology, and we believe our RAP Platform allows us to address a broad range of genetic diseases in which a modest increase in protein expression has the potential to be clinically meaningful.

Based on our preclinical studies, we believe our lead product candidate, CMP-CPS-001, has the potential to be the first disease-modifying therapy for the treatment of the most prevalent urea cycle disorders, or UCDs. UCDs are a group of severe, inherited metabolic diseases caused by mutations in the genes that encode one or more of the eight enzymes and transporters necessary to convert ammonia into urea. The inability of the body to properly metabolize ammonia leads to the accumulation of toxic levels in circulation, ultimately resulting in severe health outcomes, such as neurologic disability, seizure and death. CMP-CPS-001 is designed to improve urea cycle activity by amplifying expression of carbamoyl phosphate synthetase 1, or CPS1, an enzyme that catalyzes the first step of the urea cycle, by binding to a CPS1-specific regRNA. Our preclinical studies have demonstrated that modulating the activity of the target regRNA increases expression of the *CPS1* gene, resulting in increased CPS1 enzyme levels, which allows for more ammonia to be converted into urea, thereby lowering ammonia levels to normal, healthy ranges. These preclinical studies also demonstrated that CMP-CPS-001 can increase the level of, or upregulate, the production of multiple enzymes responsible for converting ammonia into urea, potentially allowing us to address more than 85% of patients with UCDs, which we refer to as our pan-UCD approach. We are in the early stages of development and are evaluating CMP-CPS-001 in an ongoing Phase 1 clinical trial in healthy volunteers and expect to report data from the single ascending dose, or SAD, portion of the trial in and from the multiple ascending dose, or MAD, portion of the trial in . We are also leveraging our RAP Platform to advance two preclinical programs for the treatment of both heterozygous familial hypercholesterolemia, or FH, and synaptic Ras GTPase activating protein 1, or SYNGAP1,-related disorders. We expect to initiate final Good Laboratory Practice, or GLP, toxicology studies in our FH and SYNGAP1 programs in to enable the filing of clinical trial applications.

The transcription of DNA into mRNA, the molecular template that is then translated into protein, is a complex yet carefully coordinated cellular process involving numerous components. Only a small portion of the DNA in the human genome is transcribed into RNA that codes for proteins. The vast majority of the transcriptome originates from non-coding regions of DNA, a portion of which, referred to as enhancers and promoters, perform a crucial role in determining the specificity, timing and level at which a particular gene is expressed. RegRNAs are non-coding RNAs that are transcribed by these enhancer and promoter DNA regions that form localized complexes with transcription factors to control the expression of protein-coding genes, either increasing or decreasing their

expression within natural physiological ranges. The approximately 20,000 genes that code for mRNA in the human genome are controlled by hundreds of thousands of DNA enhancers and their associated regRNAs.

Deficient protein levels characterize over a thousand diseases. Haploinsufficient diseases are dominantly inherited conditions in which inadequate gene expression is driven by a mutation in a single allele, or gene copy, and results in reductions of protein levels by as much as 50%. Numerous other genetic conditions are caused by recessive mutations that result in diminished gene activity. Data from our preclinical studies and research reports published by third parties demonstrate that increasing expression of disease-associated genes by modest amounts can restore healthy protein levels and provide therapeutic benefit in these disorders. Therefore, modest increases in protein expression have the potential to be clinically meaningful in both haploinsufficient and recessive partial loss-of-function disorders, of which there are more than 1,200. Our RAP Platform has the potential to identify the regRNA associated with all of these diseases, which we believe enables us to design RNA Actuators to address the underlying biology of these diseases. We aim to leverage our RAP Platform to develop product candidates designed to regulate transcription in a gene-specific manner to restore healthy protein levels and remedy these diseases. However, our approach is unproven and may not lead to successful efforts to develop and commercialize our product candidates and to identify and discover additional potential product candidates.

Our RAP Platform

We believe our RAP Platform can unlock the potential of the human genome and have broad applications across a range of diseases caused by sub-optimal levels of protein expression. Our technology is based upon the pioneering work in transcription regulation conducted by our co-founders, Richard Young, PhD and Leonard Zon, MD. We have built our RAP Platform to identify and characterize every regRNA that controls protein-coding genes and to develop novel ASO-based therapeutics to modulate regRNA activity to increase the expression of protein-coding genes of interest and thereby address the underlying cause of genetic diseases. Based on our proprietary mapping of regRNAs and screening and optimizing of ASOs, we have established a leadership position in regRNA-targeting therapies. Our goal is to be the preeminent company focused on discovering, developing and delivering regRNA-targeting therapeutics to patients. We believe that the ability to upregulate genes selectively through targeting regRNA could provide a new way to treat a wide range of human diseases and has the potential to become a class of new medicines.

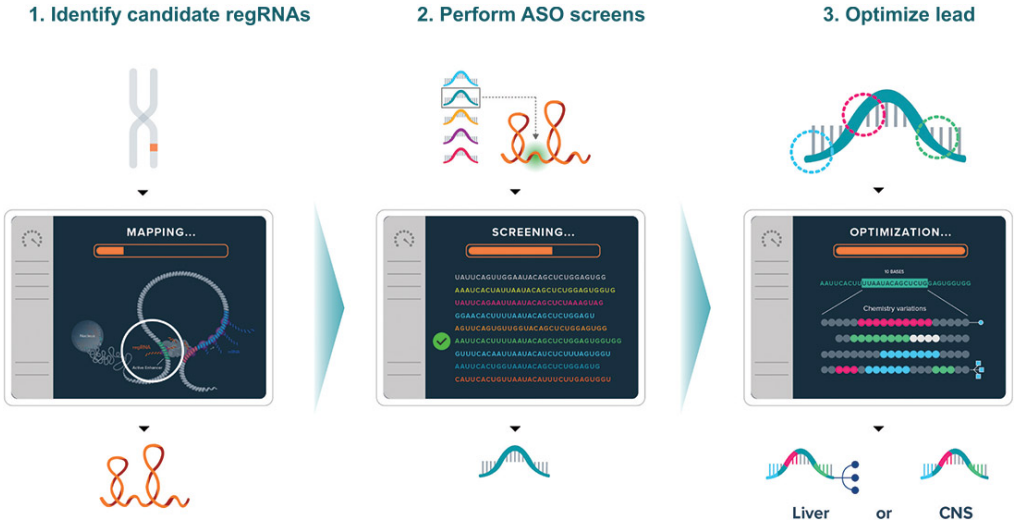
At present, very few regRNAs are described in public genomic databases, as they are often expressed at low levels and their importance was not fully understood. Our RAP Platform utilizes next-generation sequencing technologies and custom sequence analyses to map the active regulatory elements controlling every expressed gene. These data empower our proprietary machine learning algorithm, known as EPIC, to identify the specific control elements that regulate any gene of interest in the most specific manner, including elements that may restrict gene expression to a particular cell type. This enables us to identify the exact sites of regRNA synthesis and ultimately map the complete sequence of every candidate regRNA to target for therapeutic gene control. To date, we have mapped multiple cell types in as little as three months, comprising a number of potentially addressable diseases in the liver, CNS, heart, skeletal muscle and immune system. Our in-house development and application of this technology has enabled us to identify tens of thousands of enhancer and promoter regRNA sequences and their key biological properties, resulting in what we believe to be the most robust regRNA dataset available.

We combine our RAP Platform with ASO chemistry that has been utilized and validated in U.S. Food and Drug Administration, or FDA,-approved products to develop programmable RNA Actuators that are designed to precisely upregulate gene expression at the transcriptional level. Once a target gene is nominated, our RAP Platform rapidly identifies the controlling regRNA sequence, and we perform ASO screens to identify regions where ASO binding results in optimal upregulation of that target gene. Further rational design is applied to the ASOs identified in the screen. Our proprietary technology enables us to design RNA Actuators that optimize for specificity by avoiding binding to regRNAs that act on more than one gene and any other similar sequences found elsewhere in the transcriptome. As a result, our sequence-specific approach enables us to precisely target regRNA transcripts

to increase gene expression. Our approach is designed to enable the efficient and systematic creation of RNA Actuators to target regRNAs of interest. Building upon the power of this technology, our RNA Actuators can be programmed to engage regRNA targets, producing tunable increases in protein expression. While other ASOs have received regulatory approval, no regulatory authorities to date have approved ASOs that are directed towards regRNAs and, as a result, there is uncertainty as to the safety and efficacy profile of our product candidates compared to currently approved ASOs.

The key steps involved in our platform are illustrated below:

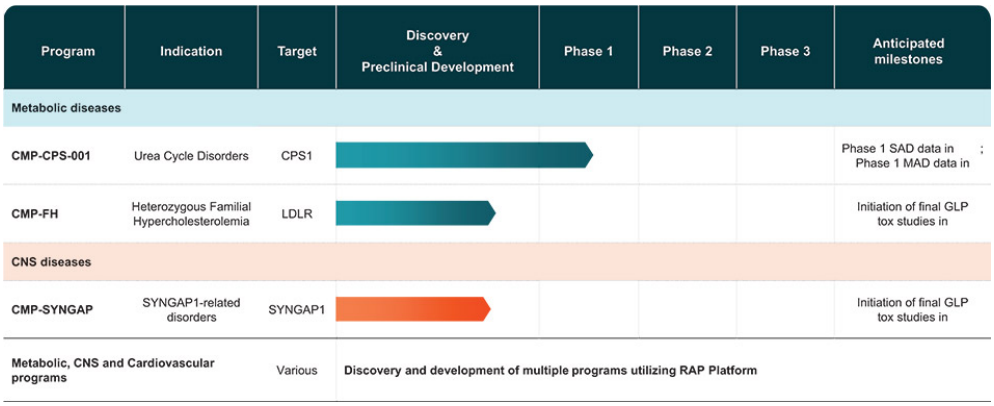
Our proprietary RAP Platform



We design RNA Actuators to leverage existing oligonucleotide delivery approaches to enable drug delivery to specific types of tissues throughout the body. We believe our RAP Platform can address any disease where a modest increase in protein expression can be clinically meaningful, including haploinsufficient diseases or recessive loss-of-function diseases. Furthermore, as we continue to map regRNAs and conduct ASO screens in more cell types, the data generated will improve the algorithms we use to identify the candidate regRNAs to specifically control gene expression. We believe the knowledge and learnings from our initial programs will significantly expedite selection of lead candidates and position us to rapidly expand our pipeline.

Our pipeline

We are leveraging our RAP Platform to advance a pipeline of programs initially focused on metabolic and CNS disorders with validated disease biology and attractive potential market opportunities due to the significant unmet need of affected patients. We retain exclusive, worldwide development and commercialization rights to all of our product candidates and preclinical programs.



CMP-CPS-001: Potential treatment for urea cycle disorders

Based on our preclinical studies, we believe our lead product candidate, CMP-CPS-001, has the potential to be the first disease-modifying therapy for the treatment of the most prevalent UCDs. UCDs are a group of severe, inherited metabolic diseases caused by mutations in the genes that encode one or more of the eight enzymes and transporters necessary to convert ammonia into urea, which is then excreted from the body. The inability of the body to properly metabolize ammonia leads to the accumulation of toxic systemic levels in circulation, ultimately resulting in severe health outcomes, such as neurologic disability, seizure and death. UCDs occur across all age groups, from infants to adults, and mild symptoms may go unnoticed until a stressor, such as illness, surgery, protein consumption or environmental stress, overwhelms compensatory functions, resulting in hyperammonemic crisis, or extremely high levels of ammonia. The prevalence of UCDs is estimated to be approximately 3,700 patients in the United States, of which we estimate 90% are late onset, defined as having severe symptom onset after one month of life, and 96% of these late onset patients have enzyme deficiencies we can address. The incidence of UCDs in the United States is estimated to be approximately 1 in 35,000 births, with similar prevalence and incidence estimated for Europe. The most common UCD, accounting for approximately 60% of UCD diagnoses, is ornithine transcarbamylase, or OTC, deficiency, caused by mutations in the *OTC* gene. The next two most common genetic subtypes are caused by mutations in the genes coding for the enzymes argininosuccinate lyase, or ASL, and argininosuccinate synthetase, or ASS1, deficiencies which affect approximately 16% and 14% of UCD patients, respectively.

There are no FDA-approved, disease-modifying therapies to treat the most prevalent UCDs. The standard of care is supportive in nature and intended to reduce the frequency of, but not eliminate, hyperammonemic crises. Current protocols for patients involve efforts to lower plasma ammonia levels. Reduction in plasma ammonia is achieved through nitrogen scavengers to remove excess nitrogen, along with the dosing of supplemental citrulline. These nitrogen scavenger agents carry an onerous pill regimen and significantly diminish the quality of life for patients. Longer-term maintenance regimens involve strict adherence to a low-protein diet along with the prophylactic use of nitrogen scavenger agents. When necessary, hemodialysis is used to reduce ammonia concentrations. The existing supportive measures are not sufficient, with many patients suffering neurological disability and premature death. Therapies currently in development are targeting only a select subgroup of patients with UCD, which includes those with OTC deficiency and patients 12 years and older. We have designed CMP-CPS-001 to be broadly applicable to UCD patients and to overcome the limitations of the current standard of care as well as programs in development for the treatment of late onset UCDs by using an established ASO modality and convenient once-monthly subcutaneous administration in order to provide UCD patients with the potential for a safe and efficacious treatment option. We are initially targeting our development of CMP-CPS-001 in the most prevalent late-onset patients (those with OTC, ASL and ASS1 deficiencies, which together constitute more than 80% of patients with UCDs) and we may expand into additional groups of patients with less common forms of UCD.

CMP-CPS-001 is designed to improve urea cycle activity by amplifying expression of CPS1, a key enzyme that catalyzes the first step of the urea cycle, by binding to a CPS1-specific regRNA. CMP-CPS-001 is a subcutaneously

injected ASO conjugated to N-acetylgalactosamine, or GalNAc, a ligand that enables targeted delivery to the liver, designed to be administered monthly. Increasing *CPS1* expression enhances the metabolism of ammonia and upregulates multiple urea cycle enzymes, including OTC, resulting in elevated urea cycle activity. Our RAP Platform enabled us to (i) identify the key enhancer modulating *CPS1* expression, (ii) screen ASOs directed to the regRNAs expressed by this enhancer, and (iii) generate a lead RNA Actuator designed to increase *CPS1* expression.

Our preclinical studies have demonstrated that modulating the activity of the target regRNA increases expression of the *CPS1* gene, resulting in increased CPS1 enzyme levels, which allows for more ammonia to be converted into urea, thereby lowering ammonia levels to normal, healthy ranges. This includes studies in a mouse model where we demonstrate that increasing *Cps1* expression can overcome a partial loss of function mutation in the urea cycle enzyme, *Otc*, and improve ammonia clearance. These preclinical studies also demonstrated that CMP-CPS-001 can upregulate the production of multiple enzymes responsible for converting ammonia into urea, which supports our pan-UCD approach. In non-human primate, or NHP, studies, the administration of CMP-CPS-001 increased the synthesis of urea, commonly referred to as ureagenesis. In these NHP studies, labeled sodium acetate was used as part of a ureagenesis rate test, or URT, to measure the metabolic output of the urea cycle. Carbaglu, approved for ultra-rare N-actylgluatamate synthesase, or NAGS-deficient patients, utilized the URT in healthy volunteers and showed that minimal increases in ureagenesis translated to substantial ammonia reductions in NAGS-deficient patients. Rates of ureagenesis were found to exceed those achieved by placebo in a statistically significant manner. This assay is also being used in our Phase 1 clinical trial. An increase in the metabolic output of the urea cycle, as indicated by an increase in the amount of labeled sodium acetate metabolized, is expected to correlate with an increase in the amount of ammonia metabolized. Although we believe that an increase in ureagenesis in our Phase 1 clinical trial may correspond with clinically meaningful improvements in ammonia metabolism in UCD patients, ureagenesis is not an established clinical endpoint and the URT results obtained in our Phase 1 clinical trial in healthy adult volunteers should not be interpreted as evidence of efficacy of CMP-CPS-001. For a further discussion of our use of this assay, please see “Risk factors—The outcome of preclinical studies and earlier-stage clinical trials may not be predictive of future results or the success of later preclinical studies and clinical trials.” We are evaluating CMP-CPS-001 in a randomized, double-blind and placebo-controlled Phase 1 clinical trial to evaluate safety, tolerability and pharmacokinetics in healthy volunteers in Australia. We expect to report Phase 1 clinical trial data from the SAD portion in and from the MAD portion in .

CMP-FH: Program for the treatment for heterozygous familial hypercholesterolemia

Our CMP-FH program is developing an RNA Actuator as a disease-modifying therapy to lower LDL cholesterol, or LDL-c, levels for the treatment of FH. FH is a group of genetic disorders that lead to reduced levels of low-density lipoprotein, or LDL, receptor, or LDLR, and/or impaired receptor function in the liver, thereby diminishing liver-mediated removal of LDL. The most common genetic cause of FH is due to mutations in the *LDLR* gene, accounting for an estimated 85% to 90% of all FH cases and is a significant contributor to early-onset cardiovascular disease. Heterozygous FH is caused by LDLR haploinsufficiency and is a common genetic disorder affecting approximately one in 200 to one in 300, or over 3 million patients in the United States and Europe, in the aggregate.

In addition to dietary and lifestyle modifications, patients with heterozygous FH are often treated first with statins. However, up to 60% of patients on statins alone still remain above recommended LDL-c levels and at increased risk for cardiovascular events. Furthermore, patient adherence to the required daily dosing schedule is often poor and statin intolerance is estimated to affect approximately 10% of patients. Monoclonal antibodies and short-interfering RNAs, or siRNAs, have emerged as a promising therapeutic approach, but despite the availability of these treatments, a significant unmet medical need remains in up to 40% of patients on combinations of statins and these therapies who may seek an alternative because they still remain above recommended LDL-c levels or their disease is unresponsive to the use of statins and existing therapies.

Our CMP-FH program utilizes a GalNAc-conjugated, subcutaneously delivered ASO designed to increase the expression of LDLR, a well-validated target that directly lowers LDL-c levels. Leveraging our RAP Platform, we

(i) identified the key regRNAs that modulate *LDLR* expression, (ii) screened ASOs targeting the regRNAs and (iii) generated multiple lead RNA Actuators that increase LDLR-encoding mRNA. Our preclinical studies have demonstrated that increased transcription of *LDLR* led to a meaningful increase in LDLR protein synthesis and cellular uptake of LDL-c, which provides evidence of our therapeutic approach. These preclinical studies have further demonstrated that increases in LDLR mRNA levels up to 100% were sufficient to reduce circulating LDL-c levels by approximately 25%. This effect represents a clinically meaningful decrease, which we believe has the potential to reduce the risk of serious coronary events. Moreover, we have shown that the increase of *LDLR* expression results in approximately 50% increase in plasma high-density lipoprotein, or HDL, levels. These data indicate that our LDLR regRNA-targeting ASOs can reduce LDL-c that contributes to atherosclerotic cardiovascular disease while increasing HDL, which promotes reduction of circulating cholesterol and is inversely correlated with disease. *In vitro* assessments have also suggested that use of one of our lead candidate RNA Actuators may work in a complementary, additive fashion with statins. We expect to initiate final GLP toxicology studies in to enable the filing of a clinical trial application.

CMP-SYNGAP: Program for the treatment for SYNGAP1-related disorders

Our initial CNS development program, CMP-SYNGAP, aims to address the underlying cause of SYNGAP1-related disorders. SYNGAP1-related disorders are a group of neurodevelopmental conditions caused by pathogenic variants in the *SYNGAP1* gene leading to a haploinsufficient state that reduces SYNGAP protein levels by as much as 50%. SYNGAP plays a critical role in the development of cognition and proper synaptic function. Epilepsy is a common characteristic of these disorders and nearly all patients present with some degree of developmental delay and cognitive impairment. Patient estimates for SYNGAP1-related disorders vary significantly. We estimate that 5,000 individuals have been diagnosed with these disorders in the United States, though we believe many more with mild symptoms remain undiagnosed and are not included in this estimate. Incidence estimates of SYNGAP1-related disorders range from 1 to 40 in 100,000 individuals and the disorder is reported to represent 0.5% to 1.0% of all intellectual disability cases.

There are no FDA-approved, disease-modifying therapies for SYNGAP1-related disorders. There is also no definitive treatment protocol, which is dependent on seizure type and severity and other neurological characteristics. Treatment is often limited to supportive physical, occupational and speech therapy. A combination of non-specific anti-seizure medications may be prescribed to treat seizures, though SYNGAP1-related disorders have proven difficult to control with available therapeutics. As many as 50% of patients do not adequately respond to medication, in which case implantable devices, such as those for vagus nerve stimulation, may offer incremental therapeutic benefit.

We are advancing our CMP-SYNGAP program to address the significant unmet need for these patients by targeting the direct cause of SYNGAP1-related disorders, haploinsufficiency, which we believe is amenable to targeting through regRNAs. Our CMP-SYNGAP program is a novel approach that targets the *SYNGAP1* gene at the transcriptional level to restore SYNGAP function and improve symptoms, by utilizing an intrathecally delivered ASO. We have identified specific regRNA sequences involved in *SYNGAP1* transcription and leverage our RAP Platform to generate ASOs that function to increase *SYNGAP1* transcription. Upregulation of *SYNGAP1* gene expression may increase SYNGAP protein levels in amounts sufficient to yield therapeutic benefit. Our preclinical studies demonstrated a dose-dependent increase in SYNGAP1 mRNA levels accompanied by a reduction in *SYNGAP1* expression. We expect to initiate final GLP toxicology studies in to enable the filing of a clinical trial application.

Our team

Our management team brings a depth of experience and knowledge in platform research, drug discovery and development and commercialization. Our team is led by our President and Chief Executive Officer Josh Mandel-Brehm, who brings over 18 years of leadership experience with life sciences companies, including business development and operational experience from his time at Biogen, Sanofi and Genzyme; David Bumcrot, PhD, our Chief Scientific Officer, an industry expert who was responsible for the initial therapeutic initiatives utilizing CRISPR technology at Editas Medicine and the start of RNAi therapeutic development at Alnylam Pharmaceuticals;

Yuri Maricich, MD, our Chief Medical Officer, who led clinical, regulatory, quality and medical affairs functions as a member of the executive team of several early-stage biopharmaceutical companies, including Pear Therapeutics; and Kelly Gold, our Chief Financial Officer, who was previously part of the corporate finance and business planning groups at Biogen and the healthcare investment banking group of Deutsche Bank.

Our technology is based on the pioneering work in transcription regulation conducted by our distinguished co-founders, Richard Young, PhD, of the Whitehead Institute for Biomedical Research and the Massachusetts Institute of Technology, and Leonard Zon, MD, who is affiliated with Boston Children's Hospital and the Harvard Medical School.

Since our inception, we have raised $188.3 million. Our investor group includes entities affiliated with 5AM Ventures; AH Bio Fund I, L.P.; Everest Aggregator, LP, an affiliate of Enavate Sciences; entities affiliated with the Kaiser Permanente Group Trust; entities affiliated with Northpond Ventures, LLC; entities affiliated with Polaris Partners; and SMRS-TOPE LLC. Prospective investors should not rely on the investment decisions of our existing investors, as these investors may have different risk tolerances and strategies and have purchased their shares in prior offerings at prices lower than the price offered to the public in this offering. In addition, some of these investors may not be subject to reporting requirements under Section 16 of the Securities Exchange Act of 1934, and, thus, prospective investors may not necessarily know the total amount of investment by each of the prior investors and if and when some of the prior investors decide to sell any of their shares. See the sections titled "Certain relationships and related person transactions" and "Principal stockholders" for more information on prior purchases by and current holdings of these stockholders.

Our strategy

Our mission has been to decode the rules of human gene expression to develop a new class of medicines that can transform the treatment paradigm for a wide range of genetic-based diseases. To accomplish this, we leverage our proprietary RAP Platform to map cells and discover regRNAs that regulate protein-coding genes in diseases characterized by sub-optimal levels of protein expression where modest increases in protein production can have a clinically meaningful therapeutic effect on patients. The key elements of our strategy include:

- ***Advance our lead candidate, CMP-CPS-001, through clinical trials and become the first approved disease-modifying therapy for UCDs.*** Based on our preclinical studies, we believe our lead product candidate, CMP-CPS-001, has the potential to be the first disease-modifying therapy for the treatment of the most prevalent UCDs and is designed to improve urea cycle activity by amplifying expression of CPS1. Our preclinical studies have demonstrated that modulating the activity of the target regRNA increases expression of the *CPS1* gene, resulting in increased CPS1 enzyme levels, which allows for more ammonia to be converted into urea, thereby lowering ammonia levels to normal, healthy ranges. These preclinical studies also demonstrated that CMP-CPS-001 can upregulate the production of multiple enzymes responsible for converting ammonia into urea, potentially allowing us to address more than 85% of patients with UCDs. We are investigating CMP-CPS-001 in an ongoing Phase 1 clinical trial in healthy volunteers. We expect to report Phase 1 clinical trial data from the SAD portion in and from the MAD portion in .
- ***Rapidly advance our disease-modifying candidates for heterozygous familial hypercholesterolemia and SYNGAP1-related disorders into clinical development.*** Our CMP-FH program is developing an RNA Actuator as a disease-modifying therapy to lower LDL cholesterol, or LDL-c, levels for the treatment of FH. In our preclinical studies, we have demonstrated that increased transcription of *LDLR* led to a meaningful increase in LDLR protein synthesis and cellular uptake of LDL-c, which provides evidence of our therapeutic approach. Our initial CNS development program, CMP-SYNGAP, aims to address the underlying cause of SYNGAP1-related disorders. CMP-SYNGAP utilizes a novel approach that targets the *SYNGAP1* gene at the transcriptional level designed to restore SYNGAP function. In preclinical studies, we have demonstrated that the administration of an ASO targeting a Syngap1 regRNA was able to increase Syngap1 mRNA levels in a dose-dependent manner in the brains of mice. For each of our CMP-FH and CMP-SYNGAP programs, we expect to initiate final GLP toxicology studies in to enable the filing of a clinical trial application.

- ***Leverage our RAP Platform to expand our pipeline in metabolic, CNS and other disease areas characterized by sub-optimal levels of protein expression.*** Our approach is designed to amplify gene expression in a specific and controllable way within a desired physiologic range, in diseases where modest increases in protein expression can be clinically meaningful in both haploinsufficient and recessive partial loss-of-function disorders, of which there are more than 1,200. Our RAP Platform has the potential to identify the regRNA associated with all of these diseases, which we believe enables us to design RNA Actuators to address the underlying biology of these diseases. We have advanced programs in liver-mediated and CNS diseases where we believe we can leverage validated disease biology and delivery mechanisms and established regulatory pathways followed by current FDA-approved, ASO-based therapies. We plan to expand the potential of our RAP Platform by developing a deep pipeline of product candidates addressing other haploinsufficient or loss-of function diseases including, but not limited to, diseases of the heart, muscle, and eye.
- ***Leverage validated modalities to efficiently advance programs through clinical development and regulatory approval.*** ASOs have substantial familiarity among regulatory agencies, including the FDA, as an established treatment modality whose use currently includes FDA-approved drugs, and protocols for manufacturing and production at scale are accessible. By utilizing chemistry in approved products, we can take advantage of regulatory familiarity, established manufacturing processes and existing delivery systems.
- ***Pursue strategic partnerships to maximize the value of our product candidates and RAP Platform.*** We intend to seek strategic collaborations where we believe the resources and expertise of third-party pharmaceutical or biotechnology companies could accelerate new programs into the clinic and towards approvals and help realize the therapeutic and market potential of our product candidates. The capabilities of our RAP Platform extend to numerous additional indications, and we intend to evaluate opportunities with third-party collaborators to capitalize on the broad potential of our RAP Platform.
- ***Build a leading regRNA-targeting therapeutic company.*** We are a pioneer in the field of regRNA-based therapeutics and our goal is to be the preeminent company focused on discovering, developing and delivering regRNA-targeting therapeutics to patients. Our proprietary RAP Platform, know-how and scientific expertise have enabled us to discover regRNAs and develop ASOs targeting these regRNAs. We aim to advance RNA Actuators as a new class of therapy for patients by demonstrating their potential across recessive loss-of-function and haploinsufficient disorders of the liver and CNS. We plan to continue our leadership position in regRNA by innovating and expanding upon our RAP Platform and technological capabilities.

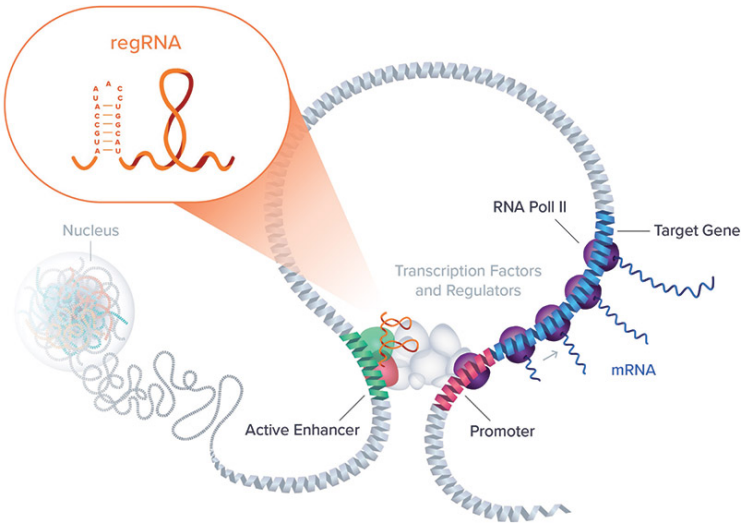
The role of regRNA in controlling transcription

The transcription of DNA into mRNA, the molecular template that is then translated into protein, is a complex yet carefully coordinated biological process involving numerous components. Only a small portion of the DNA encodes for proteins. Much of the remaining DNA comprises regions, known as promoters and enhancers, that control gene expression. The approximately 20,000 genes which code for mRNA in the human genome are controlled by hundreds of thousands of these elements and their associated regRNAs. The promoter region of the gene is located immediately before the DNA sequence encoding for an mRNA. Promoters bind transcription factors, coactivators and RNA polymerase leading to transcription initiation. Enhancers bind transcriptional regulatory proteins and interact with promoters to determine the specificity, timing and level at which a particular gene is expressed.

More than a decade ago, it was discovered that all active gene regulatory elements are also transcribed. These non-coding RNA transcripts generated by both enhancer and promoter DNA regions are defined as regRNAs. Recently, it was discovered that regRNAs play a central role in the formation of localized molecular complexes with transcriptional activators and suppressors, which function to control mRNA transcription. As shown in the illustration below, promoters and enhancers are brought into close proximity when a gene is being actively transcribed. RegRNAs generated from these regulatory elements remain closely associated with the complex that

forms at the enhancer-promoter interface. Thus, regRNAs act in a gene-specific manner, only influencing the expression of the gene near the sites where they arise.

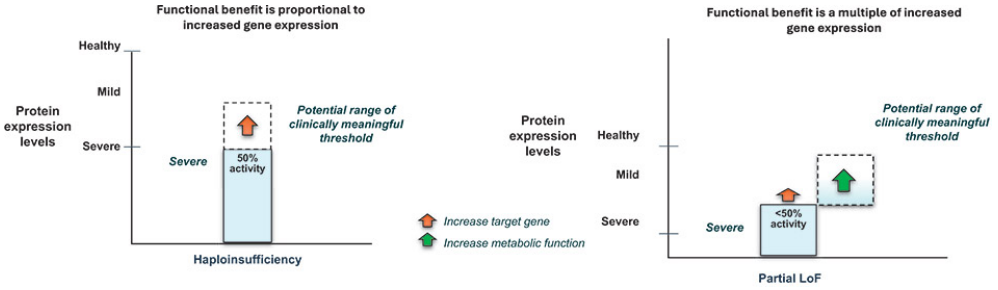
Interactions between enhancer and promoter DNA regions are critical regulators of gene expression



Deficient protein levels characterize over a thousand human diseases. Haploinsufficient diseases are dominantly inherited conditions in which inadequate gene expression, driven by a mutation in a single allele, or gene copy, results in reductions of protein levels by as much as 50%. Data from our preclinical studies and research reports published by third parties demonstrates that increasing expression of disease-associated genes by modest amounts can restore healthy protein levels and provide therapeutic benefit in these disorders. In addition to haploinsufficiencies, numerous other genetic conditions are characterized by a loss of protein function. These include recessive loss-of-function diseases caused by mutations in both alleles that reduce, but do not completely abolish, protein function. Similar to haploinsufficiencies, our preclinical studies and research conducted by third parties shows that even modest increases in expression of these partially active proteins can be therapeutically beneficial.

The figures below illustrate the concept that modest increases in protein expression can lead to clinically meaningful therapeutic benefits in both haploinsufficient and recessive partial loss-of-function disorders, of which there are more than 1,200. Our RAP Platform has the potential to identify the regRNA associated with all of these diseases, which we believe enables us to design RNA Actuators to address the underlying biology of these diseases. The image on the left illustrates that, with respect to haploinsufficient diseases, it is anticipated that an increase in functional protein levels, even below those expressed in the wild-type phenotype, would be clinically meaningful. The image on the right illustrates that, with respect to partial loss-of-function disorders, the increase of protein expression, even where the protein continues to be mutated, may be sufficient to achieve a clinically meaningful result for affected patients. We are leveraging proprietary insights into the regulatory activities of regRNAs generated internally using our proprietary RAP Platform to pioneer the development of novel therapeutics designed to achieve this objective.

Gene expression increases have the potential to reduce or eliminate disease related to haploinsufficiency and loss of function disorders



LoF denotes loss of function.

Our RAP Platform

The evolving view of gene regulation now recognizes RNA as a key regulator of transcription. We were founded on the pioneering work in transcription regulation conducted by our co-founders, Richard Young, PhD and Leonard Zon, MD. We believe our RAP Platform can unlock the potential of the human genome and have broad applications across a range of diseases caused by sub-optimal levels of protein expression. We have built our proprietary RAP Platform to discover the regRNAs that control protein-coding genes and develop novel ASO-based therapeutics to modulate regRNA activity to increase the expression of protein-coding genes of interest and thereby address the underlying cause of genetic diseases. Based on our proprietary mapping of regRNAs and screening and optimizing of ASOs, we have established a leadership position in regRNA-targeting therapies. Our goal is to be the preeminent company focused on discovering, developing and delivering regRNA-targeting therapeutics to patients. We believe that the ability to upregulate genes selectively through targeting regRNA could provide a new way to treat a wide range of human diseases and has the potential to become a new class of medicines.

Our proprietary RAP Platform is built to map every regRNA for the tunable amplification of gene expression. Only a few regRNAs are described in public genomic databases, as they are often expressed at low levels and their importance was not fully understood. Our RAP Platform utilizes next-generation sequencing technologies and custom sequence analyses to map the active regulatory elements controlling every expressed gene. Further distinguishing the robust capabilities of RAP Platform is our ability to use primary human cell lines, rather than immortalized cultured cells, to preserve the functional integrity of specific cell types. To date, we have mapped multiple cell types in as little as three months, comprising a number of potentially addressable diseases in the liver, CNS, heart, skeletal muscle and immune system. We have demonstrated that we can identify regRNA-targeting ASOs that increase specific gene expression in those tissues. These data are analyzed with our proprietary machine learning algorithms to select candidate regRNA targets that regulate transcription in a gene-specific manner to increase protein production within a physiological range. Our in-house development and application of this technology has enabled us to identify tens of thousands of regRNA sequences and their key physiological properties resulting in what we believe to be the most robust regRNA dataset available. Moreover, we believe the ability of our RAP Platform to select the most likely regRNAs controlling a given gene from the large number of candidates is a key advantage of our technology, and represents a significant barrier to others seeking to develop this approach.

Our approach is designed to enable the efficient and systematic creation of RNA Actuators to target regRNAs of interest. Building upon the power of this technology, our RNA Actuators can be programmed to engage regRNA targets and induce tunable increases in protein expression. As we continue to map regRNAs and conduct ASO screens in more cell types, the data generated will improve the algorithms we use to identify the candidate regRNAs to specifically control gene expression. Thus, we believe the knowledge and learnings from our initial programs will significantly expedite selection of lead candidates and position us to rapidly expand our pipeline.

We combine our proprietary RAP Platform with validated ASO chemistry to develop programmable RNA Actuators that are designed to precisely upregulate gene expression at the transcriptional level. An ASO construct is a single-stranded, chemically modified, nucleic-acid sequence that binds to a target regRNA sequence and modulates its activity. ASOs block or remove key interactions and lead to both increased mRNA and protein expression. Once a target gene is selected, our RAP Platform rapidly identifies the controlling regRNA, and we perform ASO screens to identify regions where ASO binding results in optimal upregulation of that target gene. Further rational design is applied to lead sequences utilizing established approaches to optimize ASOs. Our RAP Platform enables us to design RNA Actuators that potentially optimize for specificity by avoiding the potential of binding to similar sequences found elsewhere in the transcriptome which may result in deleterious side effects. As a result, our sequence-specific approach enables us to precisely target regRNA transcripts to increase gene expression.

Our use of validated ASO chemistry to generate potential therapeutic candidates provides us the flexibility to screen using a range of target sequences and to design and synthesize multiple ASO construct variations that integrate a range of chemical modifications and tissue-targeting delivery vehicles intended to maximize therapeutic potency and target specificity.

We design RNA Actuators to leverage existing oligonucleotide delivery approaches to enable drug delivery to specific types of tissues throughout the body. Our metabolic programs utilize subcutaneous administration of GalNAc-conjugated ASOs for efficient liver delivery. Our CNS program utilizes intrathecal delivery of an unconjugated ASO which provides sufficient distribution in the CNS. Our RAP Platform has the potential to address any disease where increasing protein expression can be clinically meaningful, including haploinsufficient diseases or recessive loss-of-function diseases, by returning protein levels to a normal physiological range. Furthermore, given the versatility of our platform, we believe the knowledge and learnings from our initial programs will expedite selection of lead candidates and position us to rapidly expand our pipeline.

Our programs

We are leveraging our RAP Platform to advance a pipeline of programs initially focused on metabolic and CNS disorders with validated disease biology and attractive potential market opportunities due to the significant unmet need of affected patients. We retain exclusive, worldwide development and commercialization rights to all of our product candidates and preclinical programs. Our lead product candidates and programs include a product candidate currently in a Phase 1 clinical trial, CMP-CPS-001, for the treatment of UCDs, and two preclinical programs, CMP-FH for the treatment of heterozygous FH and CMP-SYNGAP for the treatment of SYNGAP1-related disorders, for which we expect to initiate final GLP toxicology studies in to enable the filing of a clinical trial application.

CMP-CPS-001: Our lead product candidate for urea cycle disorders

Based on our preclinical studies, we believe our lead product candidate, CMP-CPS-001, has the potential to be the first disease-modifying therapy for the most prevalent UCDs, and is designed to improve urea cycle activity by amplifying expression of CPS1 by binding to a CPS1-specific regRNA. CMP-CPS-001 is a subcutaneously injected ASO conjugated to GalNAc and designed to be administered monthly. Our preclinical studies have demonstrated that modulating the activity of the target regRNA increases expression of the *CPS1* gene, resulting in increased CPS1 enzyme levels, which allows for more ammonia to be converted into urea, thereby lowering ammonia levels to normal, healthy ranges. Our preclinical studies have also demonstrated that CMP-CPS-001 can upregulate the production of multiple enzymes responsible for converting ammonia into urea, potentially allowing us to address more than 85% of patients with UCDs. We are evaluating CMP-CPS-001 in an ongoing Phase 1 clinical trial in healthy volunteers and expect to report data from the SAD portion of the trial in and from the MAD portion of the trial in .

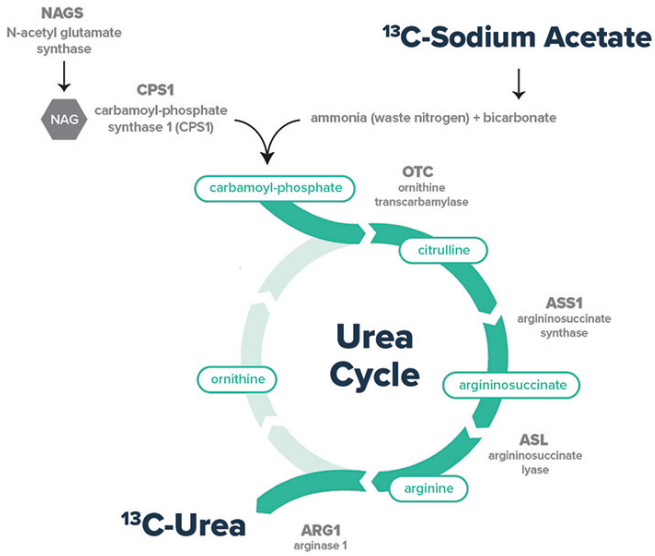
Urea cycle disorders

UCDs are a group of severe, inherited metabolic diseases caused by mutations in the genes that encode one or more of the eight enzymes and transporters necessary to convert ammonia into urea, which is then excreted from

the body. The urea cycle is the key metabolic pathway for removing excess ammonia, a waste by-product of protein metabolism, and toxic—particularly to the central nervous system—from the body. In the liver, nitrogen containing ammonia is converted to urea, which is nontoxic, water-soluble and easily excreted from the body through the kidneys as a component of urine. The inability of the body to properly metabolize ammonia leads to toxic systemic levels in circulation, ultimately resulting in severe health outcomes, such as neurologic disability, seizure and death.

Six enzymes are involved in conversion of ammonia to urea including CPS1, *N*-acetylglutamate synthase, or NAGS, OTC, ASS1, ASL, and arginase 1, or ARG1. In addition, two transporter proteins, ORNT1 and Citrin, play critical roles in the proper functioning of the urea cycle. A genetic aberration, which results in deficiency or reduced function, in any one of these enzymes or transporter proteins results in a UCD and a buildup of ammonia. A schematic representation of the urea cycle is presented below.

The urea cycle converts ammonia into urea

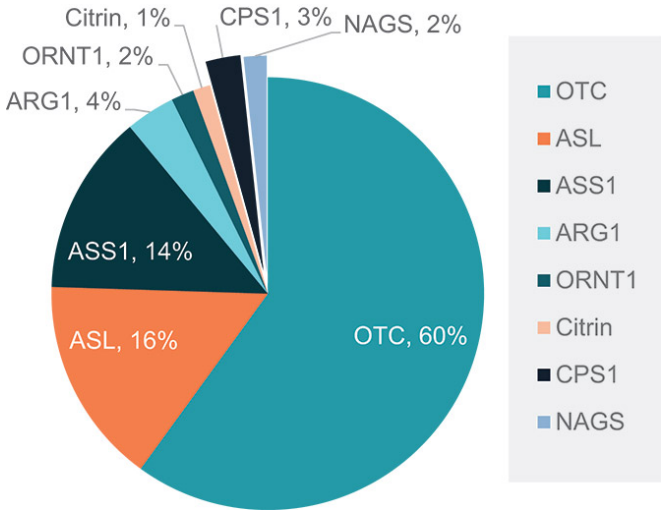


UCDs occur across all age groups, from infants to adults, and mild symptoms may go unnoticed until a stressor, such as illness, surgery, protein consumption or environmental stress, overwhelms compensatory functions, generally resulting in hyperammonemic crisis. We estimate that the prevalence of severe, symptomatic UCDs in the United States is approximately 3,700, of which we estimate 90% are late onset and 96% of these late onset patients have enzyme deficiencies we can address. The incidence of UCDs in the United States is estimated to be approximately 1 in 35,000 births, with similar prevalence and incidence rates estimated for Europe. The onset and severity of UCDs is highly variable, with severity correlating with the degree of impairment of the conversion of ammonia to urea and inversely with the amount of residual enzyme function. An estimated 10% of patients with UCDs present as neonatal onset, with severe symptom onset presenting before the first month of life, with enzyme levels less than 5% of normal. In neonatal onset UCD, ammonia concentrations in the blood rise rapidly, with the clinical consequences of the disease often presenting within a week of birth. A liver transplant is typically required by six months of age. Patients with late onset forms of the disease may present as severe with numerous neuropsychological complications including development delays, learning and intellectual disabilities, attention deficit hyperactivity disorder and executive function deficit. Moreover, recurrent hyperammonemic crises are common despite existing supportive management, and acute life-threatening episodes can occur at any age, regardless of disease severity at initial presentation.

The most common UCD, accounting for approximately 60% of UCD diagnoses, is OTC deficiency, caused by mutations in the *OTC* gene. Unlike other UCDs, which are autosomal recessive disorders, OTC enzyme deficiency is

an X-chromosome linked disorder. As such, particularly severe cases of the disorder are more prevalent in males, since males only have a single copy of the X chromosome. There are also female carriers for OTC deficiency who are mildly symptomatic and could benefit from our therapy, but these patients are not readily diagnosed nor included in our prevalence estimates. The incidence of OTC deficiency in the United States is estimated to be 1 in 56,500 births. The next two most common genetic subtypes are caused by mutations in the genes coding for the enzymes ASS1 and ASL, deficiencies which affect approximately 14% and 16% of UCD patients, respectively.

UCD patient population by deficiency



Current treatments for urea cycle disorders and their limitations

There are no FDA-approved, disease-modifying therapies to treat the most prevalent UCDs. The standard of care is supportive in nature and intended to reduce the frequency, but not eliminate hyperammonemic crises. Current protocols for patients involve efforts to lower plasma ammonia levels. Reduction in plasma ammonia is achieved through nitrogen scavengers to remove excess nitrogen, along with the dosing of supplemental citrulline. When necessary, hemodialysis is used to reduce ammonia concentrations.

Longer-term maintenance regimens involve strict adherence to a low-protein diet along with the prophylactic use of nitrogen scavenger agents that carry an onerous pill regimen and significantly diminish the quality of life for patients. The objective of maintenance therapy is to minimize nitrogen intake while facilitating its removal through alternate pathways. The existing supportive measures are not sufficient, with many patients suffering neurological disability and premature death. A liver transplant, which is usually limited to early onset patients, is intended to prevent further hyperammonemic crises and neuropsychological deterioration and is the only curative treatment, but is available to fewer than 10% of patients.

In addition to approved maintenance therapies, we are aware of several other product candidates in development for the treatment of only a portion of UCDs. These candidates are administered through infusion as they utilize lipid nanoparticle, or LNP, or adeno-associated virus-based, or AAV-based, approaches that target correcting the under-expression of OTC only. Despite the therapeutic potential of these technologies, there is little published clinical data to date on these programs and some have been hampered by delays. Furthermore, the AAV-based gene therapy approach has an irreversible mechanism of action and is designed to only address OTC deficiency in patients 12 years or older. This could limit initial clinical utility of these AAV-based therapeutics to a small patient pool. Like all AAV-based therapeutics, these product candidates are not able to be redosed due to immunogenicity, potentially limiting their long-term utility. Additionally, there is potential for efficacy waning as liver cells turn over, leaving additional need for other therapeutics to be used in patients where the effects of gene

therapy diminish. While LNPs can be redosed, they face the challenge of potential toxicity associated with repeated administration.

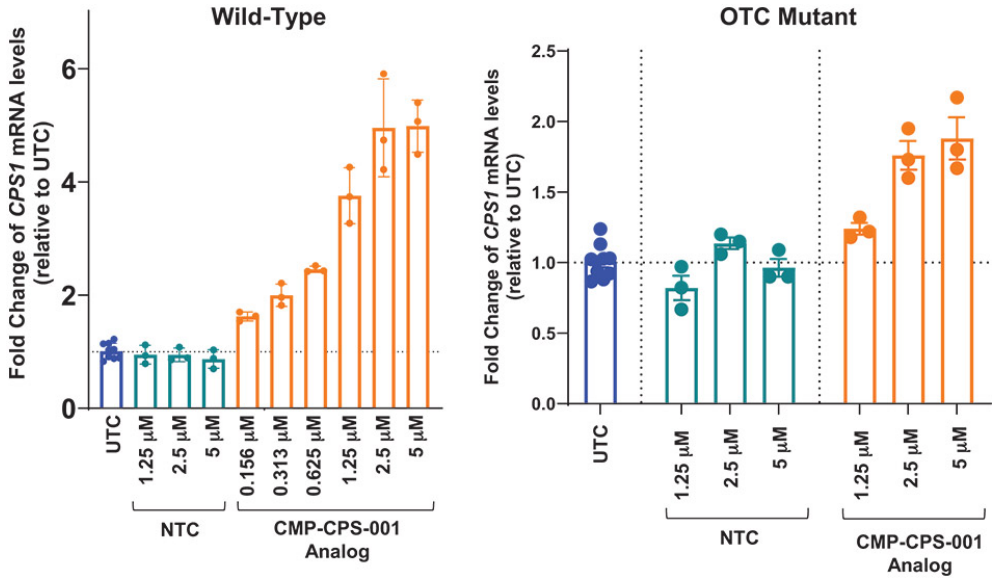
Our solution for UCDs: CMP-CPS-001

Our lead product candidate, CMP-CPS-001, is a potentially disease-modifying therapy designed to amplify expression of CPS1, an enzyme that catalyzes the first step of the urea cycle, by binding to a CPS1-specific regRNA. In our preclinical studies, we have demonstrated that modulating the activity of this regRNA increases expression of the *CPS1* gene, causing increased CPS1 enzyme levels, which allows for more ammonia to be converted into urea, thereby lowering ammonia levels to normal, healthy ranges. Our preclinical studies have also demonstrated that CMP-CPS-001 can upregulate the production of multiple enzymes responsible for converting ammonia into urea, potentially allowing us to address more than 85% of patients with UCDs. This includes the OTC deficient patient population as well as ASS1 and ASL, and others, with the exception of CPS1 and NAGS deficiencies.

Our RAP Platform enabled us to (i) identify the key enhancer modulating *CPS1* expression, (ii) screen ASOs directed to the regRNAs expressed by this enhancer, and (iii) generate a RNA Actuator designed to increase *CPS1* expression. We commenced work on the program, identified the CPS1 regulatory RNA target and identified the lead ASO sequence for CMP-CPS-001 in 2021.

We have demonstrated the controllability of our RNA Actuators in *in vitro* and *in vivo* studies. The figures below illustrate the concentration-dependent increase in *CPS1* mRNA achieved by the lead regRNA-targeting ASO in healthy human donor hepatocytes in an *in vitro* study. This ASO, designated “CMP-CPS-001 Analog,” has the same sequence and chemical modifications as CMP-CPS-001, but lacks a GalNAc conjugate, which is not necessary for *in vitro* delivery. In this study, both healthy human donor hepatocytes and OTC mutant hepatocytes were treated with a range of concentrations of the CMP-CPS-001 Analog and *CPS1* mRNA levels were measured and normalized to untreated cells. As depicted in the figures below, the results of this study show that the ASO elevated expression of *CPS1* in a concentration-dependent manner in both wild-type and *OTC* mutant human hepatocytes. The GalNAc-conjugated version of the CMP-CPS-001 Analog is our lead product candidate, CMP-CPS-001.

Concentration-dependent increase in *CPS1* mRNA



Error bars represent standard error of the mean; UTC denotes untreated control; NTC denotes non-targeting control.

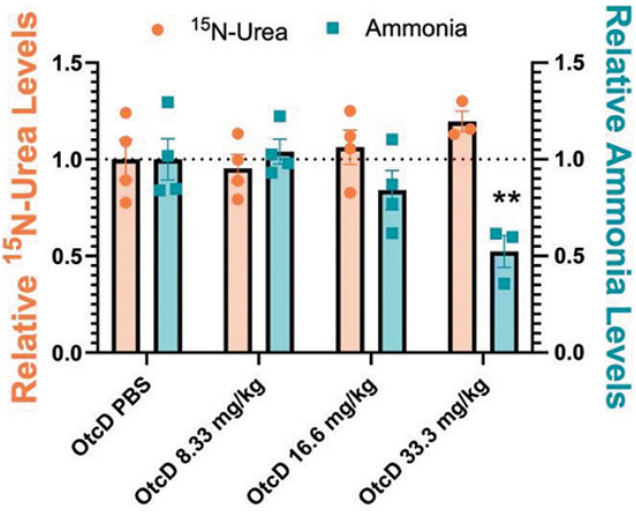
Our preclinical studies

Our preclinical evaluation of CPS1 upregulation in a mouse Otc deficiency model

The Otc$^{spf\text{-}ash}$ mouse is an established animal model of OTC deficiency which carries an *Otc* mutation that reduces Otc expression to less than 10% of wild-type levels. Following an acute ammonia challenge, these mice displayed elevated plasma ammonia levels as compared to wild-type mice. We used this model for proof of concept that elevating the expression of *CPS1* can overcome a deficiency in OTC, the enzyme most commonly mutated in UCDs. We applied our RAP Platform to identify a surrogate ASO targeting mouse *Cps1* regRNA in order to conduct studies in this model.

In the first study, adult Otc$^{spf\text{-}ash}$ mice were administered eight doses (on days 1, 3, 5, 9, 11, 13, 15 and 17) of the ASO specifically engineered to target mouse *Cps1* regRNA, at three different dose levels (8.33 (N=4), 16.6 (N=4) and 33.3 mg/kg (N=3)). A control group received placebo (phosphate-buffered saline, or PBS). Two days after the last dose, the mice were challenged with an injection of ^{15}N-labeled ammonium chloride. Thirty minutes later, blood was drawn to measure total ammonia and ^{15}N-urea levels. Statistical significance between groups was determined using appropriate statistical tests including two-way ANOVA and p-values reported. P-values (or probability values) are used to determine if the outcome of an experiment is statistically significant. A low p-value means that there is a very low likelihood that a given outcome was a result of a random occurrence. A high p-value means that assuming the null hypothesis is true, this outcome was very likely due to random occurence. Generally, a p-value of less than 0.05 (or 5% odds of the event being random) is regarded as statistically significant. In our preclinical studies, p-values of <0.05 were considered statistically significant. In some cases, we identified directional trends in effects that did not meet statistical significance due to limited group sizes, small effect size, and variability. As is depicted in the graph below, treatment of these Otc-deficient mice with the mouse surrogate tool ASO led to a dose-dependent decrease in ammonia, including a 48% decrease at the highest dose level (p<.01), along with a trend toward increased urea synthesis, including a 20% increase at the highest dose level) compared to mice treated with placebo, referred to as phosphate-buffered saline, or PBS. This demonstrates that upregulation of *Cps1* can improve ammonia metabolism in the context of a pathogenic mutation in the downstream *Otc* gene, and that the reduction in toxic ammonia is greater than the increase in urea.

Targeting *Cps1* regRNA with an ASO leads to decreased ammonia levels and increased urea production

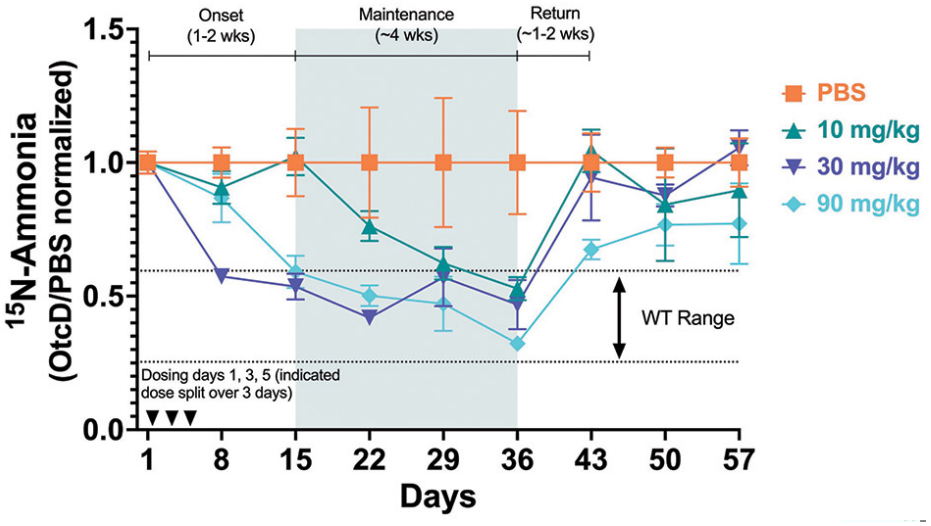


** denotes p<.01; OtcD denotes Otc deficiency; PBS denotes phosphate-buffered saline; Error bars represent standard error of the mean.

A second Otc$^{spf\text{-}ash}$ mouse study was conducted to investigate the onset and duration of the pharmacodynamic effect of elevating *Cps1* expression in the context of Otc deficiency. In this study, mice were administered three dose

levels of ASO totaling 10, 30 and 90 mg/kg split over three days (days 1, 3 and 5). Control animals received placebo. Ammonia challenges were administered to cohorts of animals prior to dosing and at weekly intervals beginning three days after the final dose for a total of eight weeks. Blood was drawn 30 minutes post-challenge and total ammonia levels were measured. As shown in the figure below, the onset of effect was dose-dependent with ammonia levels reduced to within the range of wild-type animals, or WT Range, by 3, 10 and 22 days post-dose for each of the high, mid and low dose groups, respectively. Ammonia levels approach baseline levels by 5 to 6 weeks post-dose, demonstrating a durable, greater than one month, pharmacodynamic effect. In addition, the impact of ASO treatment on *Cps1* mRNA was assessed, and demonstrated a peak increase of approximately 71% for the high-dose group at day 15 that returned to baseline by day 29. The mid-dose group exhibited an approximately 27% increase in *Cps1* mRNA along with prominent reductions in ammonia. The low-dose group exhibited minimal impact on *Cps1* mRNA at the timepoints tested, had slower onset of ammonia reduction but still exhibited an overall effect. These data demonstrate that significant reductions on ammonia result from modest effects on transcription and associated increased flux through the urea cycle.

Targeting *Cps1* regRNA with an ASO leads to sustained decrease in ammonia levels

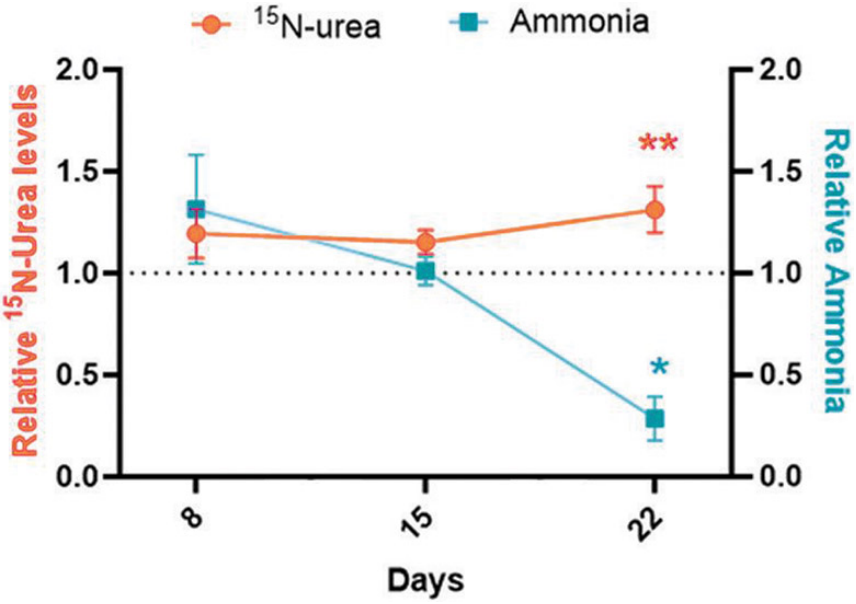


Error bars represent standard error of the mean; PBS denotes phosphate-buffered saline.

Our preclinical evaluation of CMP-CPS-001 in mice with humanized livers

The above studies were conducted using a mouse surrogate ASO that targets mouse *Cps1* regRNA. To assess the impact on ureagenesis of CMP-CPS-001 *in vivo* we utilized mice whose livers had been repopulated with hepatocytes from a healthy human donor. These humanized-liver mice were given a ^{15}N-ammonia challenge on day 1, then administered four doses of 25 mg/kg CMP-CPS-001 on days 8, 12, 15 and 19. In addition to day 1, the mice received ^{15}N-ammonia challenges before dosing on days 8 and 15, and then again on day 22. Total ammonia and ^{15}N-urea were measured at each of these time points. Consistent with the studies using Otc$^{spf\text{-}ash}$ mice involving the mouse surrogate ASO above, the results of this assessment, which are illustrated in the figure below, also demonstrated that CMP-CPS-001 targeting the *Cps1* regRNA produced a statistically significant decrease in ammonia levels (approximately 71% on day 22, $p<.05$) along with increased ureagenesis (approximately 31% on day 22, $p<0.01$). Similar to the Otc-deficient mouse study, this study in wild-type humanized liver mice demonstrated that large decreases in ammonia (approximately 71%) are associated with more modest increases in urea (approximately 31%).

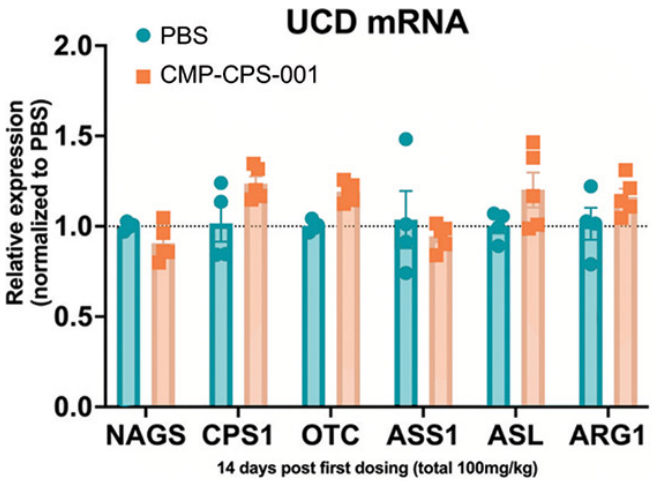
CMP-CPS-001 produced statistically significant changes in levels of ammonia and ureagenesis in wild-type humanized mice compared to placebo treated mice at day 22



Error bars represent standard error of the mean; * denotes p<.05; ** denotes p<.01.

On day 22, animals were sacrificed and livers collected to measure expression levels of CPS1 and other urea cycle enzymes. As shown in the figure below, treatment with CMP-CPS-001 resulted in a directional, though not statistically significant, increase in the expression of *CPS1* by approximately 20%, with a similar increase in *OTC*. In addition, similar elevations of two other urea cycle enzymes (*ASL* and *ARG1*) were observed. This demonstrates that an increase in the expression of *CPS1* can enhance enzymatic activity at multiple stages of the urea cycle, supporting development of CMP-CPS-001 as a potential therapeutic for urea cycle disorders in addition to OTC deficiency. The results of our studies of UCD-relevant mRNA transcription across multiple urea cycle enzymes is presented below.

CMP-CPS-001 increases transcription of mRNA of multiple urea cycle enzymes



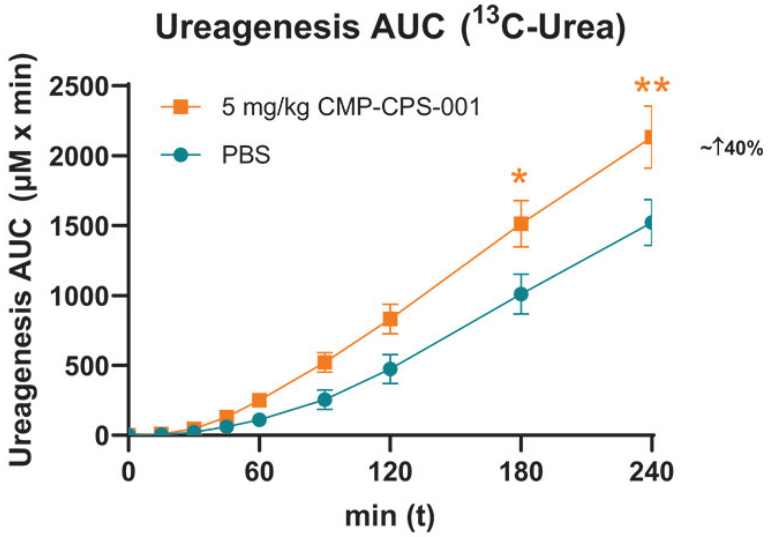
Error bars represent standard error of the mean; PBS denotes phosphate-buffered saline.

An ammonia challenge cannot be utilized in a clinical trial due to safety concerns, as ammonia is an extremely toxic molecule. Instead, the metabolic output of the urea cycle can be assessed by using a URT in which subjects are administered ^{13}C-sodium acetate. Sodium acetate is a salt that is commonly found in food sources and, like ammonia, the carbon is metabolized through the urea cycle and excreted in the urine. ^{13}C-sodium acetate, a labeled isotope of sodium acetate, is ingested by participants as part of the URT to measure the overall activity of the urea cycle, and blood is drawn at multiple time points to measure the amount of ^{13}C-urea that is generated. This measure of ureagenesis represents a clinically meaningful signal of the metabolic output of the urea cycle. An increase in the metabolic output of the urea cycle, as indicated by an increase in the amount of ^{13}C-sodium acetate metabolized, is expected to correlate with an increase in the amount of ammonia metabolized. The rate of ureagenesis is inversely related to the severity of UCD. Studies have shown that while baseline plasma urea levels of asymptomatic carriers of these disorders are indistinguishable from those of healthy volunteers, baseline plasma urea levels among symptomatic patients are significantly lower. Notably, the measurement of ureagenesis in prior clinical trials of therapeutics designed to treat patients with OTC deficiency has been demonstrated to translate well to clinical response. Although preclinical studies suggest increases in urea are less pronounced than decreases in ammonia, we believe ureagenesis is a reliable indicator of therapeutic efficacy. Thus, we have incorporated this assay in ongoing assessments of CMP-CPS-001 in our healthy volunteer Phase 1 clinical trial. Based on our preclinical studies we believe small increases (approximately 20%) in ureagenesis may ultimately translate to meaningful clinical activity in patients in subsequent trials. It is possible that we may observe smaller increases in healthy volunteers, as their ureagenesis rates are operating at full capacity, underrepresenting the potential efficacy when tested in patients with low ureagenesis rates. However, it is possible that an increase in ^{13}C-sodium acetate metabolism, as measured by the URT, will not correlate to an increase in ammonia metabolism and that variability in the results of the assay could render interpretation difficult. For a further discussion of our use of this assay, please see “Risk factors—The outcome of preclinical studies and earlier-stage clinical trials may not be predictive of future results or the success of later preclinical studies and clinical trials.”

Our preclinical evaluation of CMP-CPS-001 in non-human primates

The effect of increased CPS1 production on ureagenesis was also studied in wild-type cynomolgus monkeys. These NHPs were administered two doses of 5 mg/kg CMP-CPS-001, 30 days apart, with urea production measured one week after the second dose. To measure ureagenesis, animals were administered ^{13}C-sodium acetate, and blood was drawn at eight time points over a four-hour period. The concentration of ^{13}C-urea was measured utilizing a URT. As shown below, CMP-CPS-001 treatment increased ureagenesis by 40% compared to those animals administered the placebo ($p<0.05$ at 180 minutes; $p<0.01$ at 240 minutes). As in the humanized mouse study, this study shows that CMP-CPS-001 can increase activity of the urea cycle in wild-type animals. Moreover, the NHP study measured ureagenesis with the same assay being employed in the ongoing Phase 1 clinical trial in healthy volunteers, supporting this approach to measure a pharmacodynamic effect in humans.

CMP-CPS-001 increased ureagenesis compared to placebo in wild-type NHPs



Error bars represent standard error of the mean; PBS denotes phosphate-buffered saline; * denotes p<.05; ** denotes p<.01.

Preclinical safety evaluations

CMP-CPS-001 was evaluated in three-month GLP toxicity studies of both mice and NHPs. Animals were dosed subcutaneously, once monthly for three months. In both species, CMP-CPS-001 was generally well tolerated with all clinical observations considered non-adverse due to low severity and lack of clinical correlates. No observable adverse effects were noted at 12.5 mg/kg and 50 mg/kg dosing, the highest dose levels tested in mice and NHPs respectively. Drug metabolism and pharmacokinetic evaluations reflected findings consistent with the drug class.

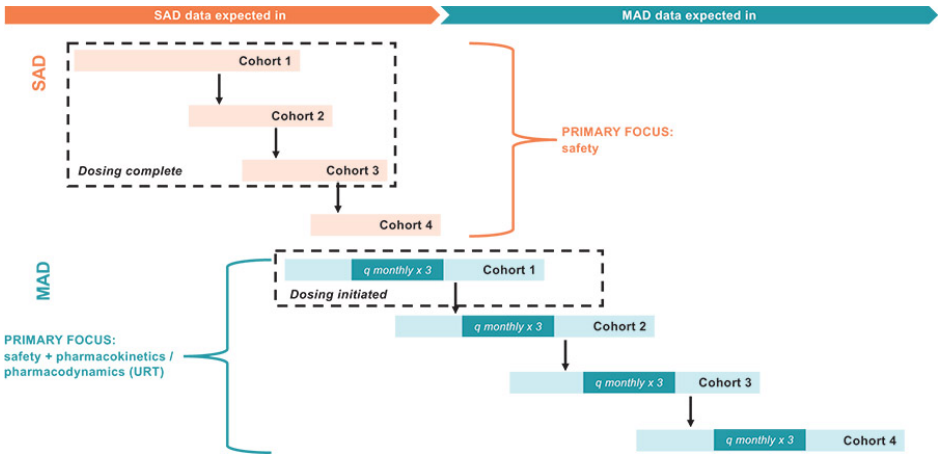
Our ongoing Phase 1 clinical trial

CMP-CPS-001 is currently being evaluated in an approximately 96-person randomized, double-blind, and placebo-controlled Phase 1 clinical trial to evaluate safety, tolerability as well as pharmacokinetics and pharmacodynamics in healthy volunteers in Australia, where we are able to benefit from certain cost-effective tax incentives provided by the Australian government. CMP-CPS-001 is being administered by subcutaneous injection on a monthly basis. Primary endpoints of this trial include safety and tolerability and a secondary endpoint is to assess change in ureagenesis using the same URT utilized in our NHP study. Inclusion of the URT assessment is designed to enable the establishment of URT methodology that can be used to optimize the design of future registrational studies in patients with UCDs as well as enable a reference range of normal ureagenesis rates as both a tool for studies in patients with UCDs and as support for our engagement with regulators.

The SAD portion of the trial is segregated into four cohorts of 10-12 subjects each. Nine subjects in each cohort are to receive CMP-CPS-001 with the additional three subjects to receive placebo. Dosing levels are to begin at 0.2 mg/kg and increase with each cohort unless a maximum tolerated dose is reached, with dose escalation between cohorts only after a two-week safety review committee confirmation of safety and tolerability. Assuming no drug-related adverse events are observed in the first two SAD cohorts, the trial is designed to then initiate an evaluation of CMP-CPS-001 in four MAD cohorts of 12 subjects each, staggered concurrently to the latter SAD cohorts. As is the design for the SAD portion of the Phase 1 clinical trial, the active therapeutic candidate to placebo participant ratio in each cohort of the MAD portion of the Phase 1 clinical trial will be 3:1. Initial dosing levels and dose ranges for the MAD portion of the Phase 1 clinical trial are to be determined based on observations from the ongoing SAD trial portion. Dose escalation will occur after a 59-day safety review committee

confirmation for the MAD portion of the Phase 1 clinical trial. We dosed the first participant in this Phase 1 SAD trial in March 2024. We expect to report data from the SAD portion of the trial in and from the MAD portion of the trial in .

Phase 1 clinical trial design



Planned clinical trials

Assuming the successful completion of our ongoing Phase 1 clinical trial in healthy adult volunteers and regulatory feedback from regulatory agencies, we plan to utilize a stepwise development approach in which we would initiate one or more 52-week Phase 2/3 clinical trials involving CMP-CPS-001, with the potential for an open-label extension. We anticipate the first of these two Phase 2/3 clinical trials to enroll patients, two years of age or older, who have been diagnosed with an OTC, ASL or ASS1 deficiency, to be randomized to either our active therapeutic candidate or to placebo. We currently expect that the Phase 2/3 clinical trial would initially start with adults, and step down by age segment into patients two years or older as required by regulators. Key endpoints are likely to include responder analysis defined as a reduction and/or maintenance in ammonia levels compared to baseline, diet liberalization, nitrogen scavenger reduction, and increase in ureagenesis, along with a maintenance of no or a decrease in clinical episodes during the treatment period. Assuming a positive assessment of the OTC trial results during the interim analysis, we envision initiating a second Phase 2/3 clinical trial expanding enrollment to include ASS1 and ASL deficient patient populations.

CMP-FH program for familial hypercholesterolemia

Our CMP-FH program is developing an RNA Actuator as a disease-modifying therapy to lower LDL cholesterol, or LDL-c, levels for the treatment of FH. Our CMP-FH development program utilizes a GalNAc-conjugated, subcutaneously delivered ASO designed to increase the expression of *LDLR*, a well-validated target that directly lowers LDL-c levels. FH is a group of genetic disorders that lead to reduced levels of LDLR and/or impaired receptor function in the liver, thereby diminishing liver-mediated removal of LDL-c. The most common genetic cause of FH is due to mutations in the *LDLR* gene, accounting for an estimated 85% to 90% of all FH cases and is a significant contributor to early-onset cardiovascular disease. We also believe it may have applications in other patients presenting with atherosclerotic cardiovascular disease. In our preclinical studies, we have demonstrated that increased transcription of *LDLR* led to a meaningful increase in LDLR protein synthesis and cellular uptake of LDL-c, which provides evidence for this therapeutic approach. We expect to initiate final GLP toxicology studies in to enable the filing of a clinical trial application.

Familial hypercholesterolemia

FH is a group of genetic disorders that lead to reduced levels of LDLR and/or impaired receptor function in the liver, thereby diminishing liver-mediated removal of LDL-c, which is commonly referred to as “bad cholesterol”. The

most common genetic cause of FH is due to mutations in the *LDLR* gene, accounting for an estimated 85% to 90% of all FH cases and is a significant contributor to early-onset cardiovascular disease. Mutations in the *LDLR* gene reduce the number of functioning LDLRs in the liver, which results in diminished liver-mediated removal of LDL-c and causes excessive LDL-c accumulation in the blood. The reduction in LDLRs also negatively impacts the efficacy of therapeutics, such as statins, that are frequently prescribed to treat high cholesterol in individuals. Although rare, gain-of-function mutations in the *PCSK9* gene and loss-of-function mutations in the *APOB* and *LDLRAP1* gene can cause FH.

FH is primarily considered an inherited, autosomal dominant disorder. As such, individuals who are heterozygous for FH, or have only one copy of the gene mutation causing a haploinsufficient state, may develop cardiovascular disease prematurely, often in their 30's. Heterozygous FH is a relatively common genetic disorder, with recent epidemiological studies indicating a disease incidence of approximately 1 in 200 to 1 in 300 people, or over 3 million people in the United States and Europe, in the aggregate.

Current treatments and their limitations

There are no FDA-approved, disease-modifying therapies to treat heterozygous FH. In addition to dietary and lifestyle modifications, heterozygous FH is often treated first with statins, which may be administered along with fibrates and bile duct sequestrants. However, up to 60% of patients on statins alone still remain above recommended LDL-c levels and at increased risk for cardiovascular events. Furthermore, patient adherence to the required daily dosing schedule is often poor and statin intolerance is estimated to affect approximately 10% of patients.

Monoclonal antibodies and siRNAs, which inhibit the PCSK9 protein from binding to LDLRs and acts to block the subsequent degradation of a more limited number of these existing receptors, have emerged as a promising therapeutic approach for treating FH. These medications, which are administered by infusion in the case of antibodies or subcutaneous injection in the case of siRNAs, have produced LDL reductions of as high as 70% in third-party clinical evaluations. Despite the availability of these treatments, a significant unmet medical need remains in a sizeable subset of patients who may seek an alternative to these therapies or whose disease is unresponsive to their use.

Our solution

We are currently evaluating the use of an RNA Actuator to treat heterozygous FH by increasing *LDLR* levels for disease intervention. Leveraging our RAP Platform, we (i) identified the key regRNAs that modulate *LDLR* expression, (ii) screened ASOs targeting the regRNAs and (iii) generated multiple lead RNA Actuators that increase LDLR-encoding mRNA. LDLR is a well-validated therapeutic target and is the most proximal target to lowering LDL-c. Increasing *LDLR* expression increases the LDL receptors on the surface of hepatocytes to increase removal of LDL-c from circulation.

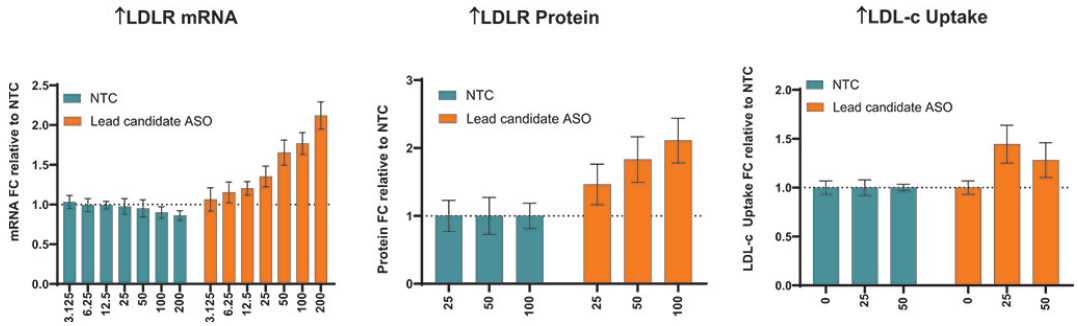
In addition to the proximal advantages of LDLR's spatial relationship with LDL-c, we believe that by increasing expression of the LDLR protein in patients' liver cells by as little as one and one-half baseline levels, we can achieve clinically meaningful decreases in plasma levels of LDL-c, which is anticipated to reduce serious coronary events. To this end, we have designed our program to target regRNA sequences involved in *LDLR* gene transcription, intended to bolster gene expression. We believe that an increase in the level of LDLR may translate into greater LDL-c uptake as compared to inhibition of the PCSK9 receptor alone, which acts to block degradation of a more limited number of existing receptors. Our ASO approach may introduce an alternative treatment paradigm, as the first add-on therapy to statins, in place of PCSK9 inhibitors. It may also serve as add-on to statin-PCSK9 combination therapy for patients with insufficient response to PCSK9 inhibition, as a study has shown that approximately 40% of patients may fail to reach target LDL-c levels on statin and PCSK9 regimens. Therapeutic use may extend from the genetic hypercholesterolemia population to the significantly larger patient population who have atherosclerotic cardiovascular disease. Clinical measurement of cholesterol levels and composition, which can be obtained through widely available, non-invasive tests, provide readily accessible biomarkers for rapid

determination of efficacy. We expect to initiate final GLP toxicology studies in to enable the filing of a clinical trial application.

Our preclinical studies

We identified lead candidate ASOs targeting a *LDLR* regRNA that upregulate *LDLR* gene expression in a human hepatocyte-derived cell line. Cells were treated with a lead candidate ASO for 48 hours and mRNA, protein, or LDL-c uptake assessed. As depicted in the figure below, increased transcription of *LDLR* (left) led to a meaningful increase in LDLR protein synthesis (middle) and cellular uptake of LDL-c (right), which provides evidence of our therapeutic approach in treating FH. *In vitro* assessments have also shown that a lead candidate ASO exhibits an additive effect on LDLR levels in combination with statins.

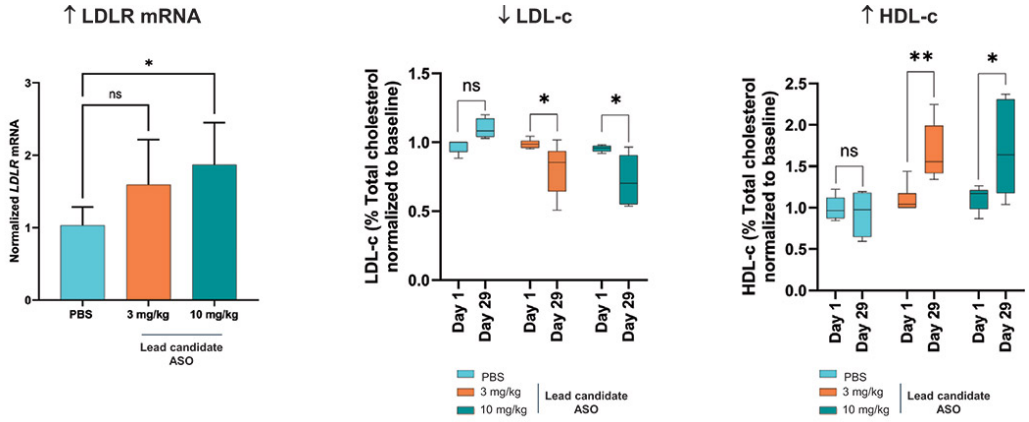
***In vitro* studies demonstrate that increased transcription of *LDLR* led to meaningful increase in LDLR protein synthesis and cellular uptake of LDL-c**



Values are in nanomolar (nM). Error bars in graph denote standard error of the mean; NTC denotes non-targeting control.

In vivo studies of a lead candidate ASO demonstrated a similar ability to increase *LDLR* expression as was observed in the *in vitro* studies. We evaluated a lead candidate ASO at both 3 mg/kg and 10 mg/kg dose levels along with a placebo in mice whose livers were repopulated with human hepatocytes by intrahepatic injections of human hepatocytes. These mice exhibit lipoprotein profiles similar to humans and provide a model system to evaluate human-specific drug candidates. Baseline measurements of LDL-c were recorded for each of the five to seven mice per group at both 21 and 1 day before administration of the first of two doses of ASO. A second dose was administered on day 15. LDL-c levels were measured on days 1, 8, 15, 22 and 28 after the first ASO dosing. As is reflected in the results presented in the figure below, increases in LDLR mRNA levels up to 87% ($p<0.05$) were sufficient to reduce circulating LDL-c levels by approximately 25% with a maximal effect of 50% by day 28 ($p<0.05$). In addition, there was a 50% increase in HDL ($p<0.01$ at 3 mg/kg and $p<0.05$ at 10 mg/kg). These data indicate that our LDLR regRNA-targeting ASO can reduce LDL-c that contributes to atherosclerotic cardiovascular disease while increasing HDL, which promotes reduction of circulating cholesterol and is inversely correlated with disease.

Modest increases in LDLR mRNA transcription correlated to significant reductions in LDL-c



Error bars represent standard deviation; * denotes p<0.05; ** denotes p<0.01; ns denotes not statistically significant; PBS denotes phosphate-buffered saline.

We expect to initiate final GLP toxicology studies in to enable the filing of a clinical trial application.

CMP-SYNGAP for SYNGAP1-related disorders

Our initial CNS development program, CMP-SYNGAP, aims to address the underlying cause of SYNGAP1-related disorders. CMP-SYNGAP utilizes a novel approach that targets the *SYNGAP1* gene at the transcriptional level designed to restore SYNGAP function. We are advancing our CMP-SYNGAP program to address the significant unmet need for these patients by targeting the direct cause of SYNGAP1-related disorders, haploinsufficiency, which we believe is amenable to treatment by targeting SYNGAP1 regRNAs. We expect to initiate final GLP toxicology studies in to enable the filing of a clinical trial application.

SYNGAP1-related disorders

Synaptic Ras GTPase activating protein, or SYNGAP, plays a critical role in the development of cognition and proper synaptic function, enabling synaptic plasticity and axon formation through signal attenuation. SYNGAP1-related disorders are a group of neurodevelopmental conditions caused by pathogenic variants in the *SYNGAP1* gene leading to a haploinsufficient state that reduces SYNGAP protein levels by as much as 50%. A majority of these SYNGAP1 pathogenic variants, or mutations, are mutations that result in truncation of the protein or destruction of the RNA by nonsense-mediated decay, ultimately resulting in lower protein levels and haploinsufficiency. These disorders can manifest with a variety of symptoms that can include developmental delays, movement disorders and features of autism spectrum disorder. Epilepsy is a common feature of SYNGAP1-related disorders, occurring in more than 95% of children with the condition, with seizures usually beginning in early childhood. Nearly all children have some degree of developmental delay and cognitive impairment, though disease symptoms and their severity vary widely. SYNGAP1-related disorders are autosomal dominant with clinical disease presentation if either of the two alleles have a mutation. In most cases the pathogenic variant occurs spontaneously and is not inherited. Patient estimates for SYNGAP1-related disorders vary significantly. We estimate that 5,000 individuals have been diagnosed with these disorders in the United States, though we believe many more with mild symptoms remain undiagnosed and are not included in this estimate. Incidence estimates of SYNGAP1-related disorders range from 1 to 40 in 100,000 individuals and the disorder is reported to represent 0.5% to 1.0% of all intellectual disability cases.

Current treatments and their limitations

There is currently no FDA-approved treatment, disease-modifying or otherwise, for SYNGAP1-related disorders. There is also no definitive treatment protocol, which is dependent on seizure type and severity and other

neurological characteristics of the disease. Treatment is often limited to supportive physical, occupational and speech therapy. A combination of anti-seizure medications may be prescribed to treat seizures, though SYNGAP1-related disorders have proven difficult to control with available therapeutics. As many as 50% of patients do not adequately respond to medication in which case implantable devices, such as those for vagus nerve stimulation, may offer incremental therapeutic benefit.

Our solution

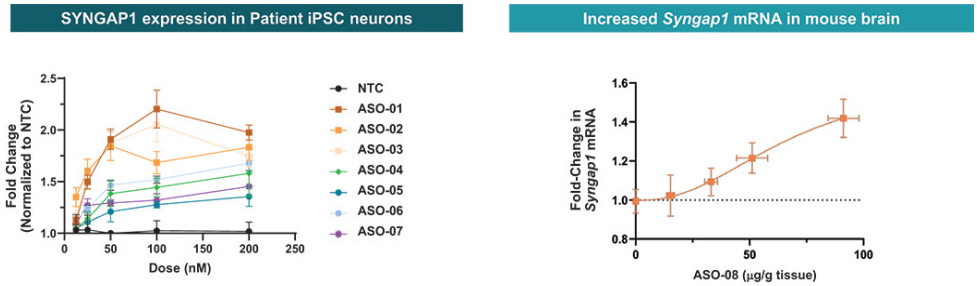
Our CMP-SYNGAP program is a novel approach that targets the *SYNGAP1* gene at the transcriptional level to restore SYNGAP function and improve symptoms. We are advancing our CMP-SYNGAP program to address the significant unmet need for these patients by targeting the direct cause of SYNGAP1-related disorders, haploinsufficiency, which we believe is amenable to targeting through regRNAs. As haploinsufficiency characterizes SYNGAP1-related disorders, upregulation of *SYNGAP1* gene expression may enable an increase in protein levels which may yield therapeutic benefit, including potential improvements to memory and incidence of seizures. Our CMP-SYNGAP program utilizes an intrathecally delivered ASO for the treatment of SYNGAP1-related disorders. We have identified specific regRNA sequences involved in *SYNGAP1* transcription and have leveraged our RAP Platform to generate ASOs that function to increase *SYNGAP1* transcription.

Our preclinical studies

We are currently pursuing parallel workstreams to identify both tool ASOs for mouse proof-of-concept studies, as well as human-specific ASOs as drug candidates to assess the clinical effect of ASOs on *SYNGAP1* levels in both patient-derived neurons and mice expressing human *SYNGAP1*.

Lead human-specific ASOs have been identified that increase *SYNGAP1* mRNA in human neurons *in vitro* as shown in the figure below (left). In addition, a mouse tool ASO was administered by intracerebroventricular injection to neonatal mice with the goal of confirming an ability to increase *Syngap1* expression. Assessment of brain tissue revealed a dose-dependent increase in *Syngap1* mRNA levels three weeks post-dose. These findings are presented in the graph below (right). Future studies will evaluate the impact of increased *Syngap1* expression on functional deficits caused by haploinsufficiency.

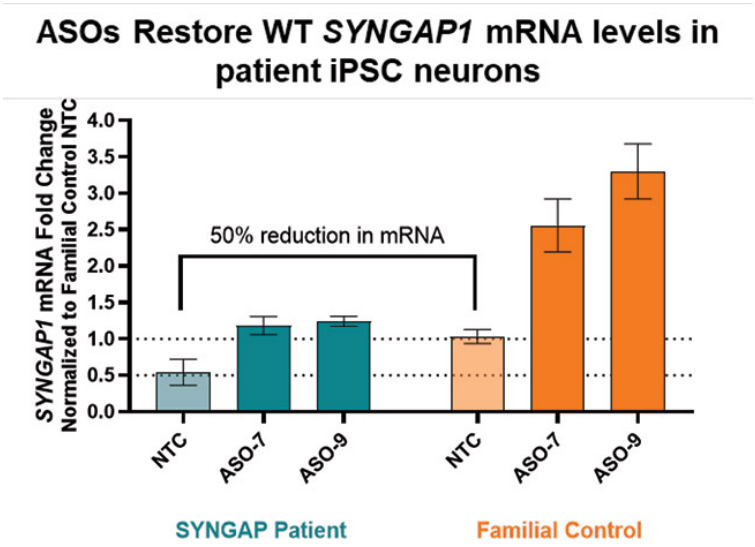
Dose-dependent increase in *Syngap1* mRNA in the brains of mice



Error bars represent standard error of the mean; NTC denotes non-targeting control.

Vertical error bars represent standard deviation of fold change increase in mRNA; Horizontal error bars represent standard deviation of measured ASO concentration.

As part of our assessment of lead human-specific ASOs in human neurons *in vitro*, we have confirmed that SYNGAP1 patient-derived induced pluripotent stem cell, or iPSC, neurons exhibit one-half as much *SYNGAP1* mRNA as those derived from a familial control iPSC neurons. Two representative lead ASOs demonstrated robust target engagement where they increased *SYNGAP1* mRNA at least two-fold in both control and mutant neurons, where *SYNGAP1* mRNA levels were fully restored to wild-type levels. We are continuing to explore electrophysiological and biochemical phenotypes in iPSC neurons *in vitro* to link increases in expression to benefits in disease-relevant phenotypes.



Error bars represent standard deviation of fold change increase in *SYNGAP1* mRNA levels; NTC denotes non-targeting control. ASO-7 and ASO-9 denote two lead candidate ASOs of our CMP-SYNGAP program.

We expect to initiate final GLP toxicology studies in to enable the filing of a clinical trial application.

Manufacturing strategy

We do not own or operate, and currently have no plans to establish, any manufacturing facilities. We rely on third- party contract manufacturers for the manufacture of our product candidate for our clinical trials, and, if we receive marketing approval, we will rely on such third parties for commercial manufacture. In addition, we rely on third parties to package, label, store and distribute our product candidate, and we intend to rely on third parties for our commercial products if marketing approval is obtained. We expect this strategy will enable us to maintain a more efficient infrastructure, avoiding dependence on our own manufacturing facility and equipment, while simultaneously enabling us to focus our expertise on the clinical development and future commercialization of our products. Chemistry, Manufacturing and Controls, or CMC, is a critical element of the drug development process and involves the various procedures used to assess the physical and chemical characteristics of drug products, to ensure their quality and consistency throughout manufacture. CMC increases in complexity as the development process matures.

License and collaboration agreements

Whitehead Institute patent license agreement

In October 2019, we and the Whitehead Institute for Biomedical Research (the "Whitehead Institute") entered into a patent license agreement (as amended in December 2021 and November 2023, the "Whitehead Agreement") pursuant to which we received a worldwide, royalty-bearing, sublicensable license under certain patent rights owned or controlled by the Whitehead Institute to develop, make, have made, use, sell, offer to sell, lease and import products, and to perform and have performed licensed processes, in each case, in the fields of human and animal therapeutics and diagnostics. The license granted under the Whitehead Agreement included an exclusive license to certain patent rights generally related to, among other things, methods of modulating gene expression using oligonucleotides, and a co-exclusive license to certain patent rights generally relating to, among other things, methods of modulating gene expression by targeting certain genomic sequences.

Under the Whitehead Agreement, the Whitehead Institute retains the right to practice the licensed patent rights for research, teaching, and other educational purposes, including use in third-party sponsored research, and to

grant non-exclusive licenses to other nonprofit and academic institutes solely for non-commercial research, teaching, and other educational purposes. The license granted to us under the Whitehead Agreement is also subject to certain rights held by the U.S. government under applicable law with respect to inventions that arose from federal research funding. In addition, the license is subject to a certain non-exclusive license for internal research purposes only that the Whitehead Institute granted to a certain third party, and to certain preexisting rights held by a certain third party who is a party to a certain sponsored research agreement, or SRA, with the Whitehead Institute. Under the SRA, the Whitehead Institute covenanted not to sue said third party if certain inventions arising under the SRA, or SRA inventions, are dominated by the licensed patent rights and we are thereby excluded from asserting certain patent rights licensed from the Whitehead Institute that cover the SRA inventions against said third party.

We are obligated to use certain efforts to develop one or more products or licensed processes and commercialize the products or licensed processes in a major market. Furthermore, beginning five years from the effective date and subject to certain terms and conditions, the Whitehead Agreement requires us to negotiate and potentially issue mandatory sublicenses to a third party under the exclusively licensed patent rights to make, have made, use, sell, offer to sell, or import a product or process that is not directly competitive with a licensed product or licensed process then offered for sale or in bona fide research or development by or on behalf of us.

Under the terms of the Whitehead Agreement, we paid to the Whitehead Institute an upfront license issuance fees of $0.1 million and de minimis additional fees in connection with each of the December 2021 and November 2023 amendments to the agreement that were recorded as research and development expense in our consolidated statement of operations and comprehensive loss. We are also obligated to make annual license maintenance fees under the agreement, pursuant to which we have paid an aggregate of $0.16 million through December 31, 2023. In addition, we are obligated to pay certain filing, prosecution and maintenance fees with respect to certain patent rights licensed to us under the agreement, pursuant to which we have paid an aggregate of $0.22 million through December 31, 2023. We are obligated to pay potential development milestone payments of up to an aggregate of low single-digit millions of dollars under the terms of the agreement upon the achievement of certain specified contingent events. In addition, if we successfully commercialize a product under the Whitehead Agreement, then we will be required to pay the Whitehead Institute tiered royalties at percentage rates ranging from less than one percent to the mid-single digits of net sales or of running royalties of net sales, subject to specified reductions, until either the last-to-expire valid claim of a Whitehead Institute patent covering the product or a duration in the late single digit years after the first commercial sale, in each case on a product-by-product and country-by-country basis.

The expected termination of the royalty obligations will depend on factors such as the availability and application of patent term extensions for the licensed patents in the licensed territory. The Whitehead Agreement will remain in effect until voluntarily terminated by the company and may be earlier terminated by the Whitehead Institute if the company fails to pay any amounts due under the agreement or materially breaches the agreement and fails to cure such breach. The last to expire patent, if issued, under the Whitehead Agreement, is expected to expire in 2043.

Competition

The biotechnology and pharmaceutical industries have made substantial investments in recent years into the rapid development of novel treatments for metabolic and CNS-related diseases and disorders.

We face substantial competition from multiple sources, including large and specialty pharmaceutical and biotechnology companies, academic research institutions and governmental agencies and public and private research institutions. Our competitors compete with us on the level of the technologies employed, or on the level of development of product candidates. In addition, many small biotechnology companies have formed collaborations with large, established companies to (i) obtain support for their research, development and commercialization of products or (ii) combine several treatment approaches to develop longer lasting or more efficacious treatments that may potentially directly compete with our current or future product candidates. We anticipate that we will continue to face increasing competition as new therapies and combinations thereof,

technologies, and data emerge within the field of antisense oligonucleotide therapeutics and, furthermore, within the treatment of metabolic and CNS-related diseases and disorders.

In addition to the current standard-of-care treatments to address the diseases we are targeting in therapeutic development programs, numerous commercial and academic preclinical studies and clinical trials are being undertaken by a large number of parties to assess novel technologies and product candidates.

For the broad treatment of patients with UCDs, we will compete with Amgen Inc., who has commercialized Ravicti, a nitrogen scavenger. Other therapeutics in development are focused on patients with OTC deficiency only, where we will potentially compete with Ultragenyx Pharmaceutical Inc., Arcturus Therapeutics Holdings Inc., and iECure, among others, assuming they are successful in clinical development. Ultragenyx Pharmaceutical Inc. is developing their potential therapy in OTC patients aged 12 and older; and iECure is initially targeting neonatal patients only. Large pharmaceutical companies that have commercialized or are developing treatments for hypercholesterolemia include Amgen Inc., Regeneron Pharmaceuticals, Inc. and Novartis AG. Companies that compete with us directly on the level of the development of product candidates targeting SYNGAP1-related disorders include Stoke Therapeutics, Inc. and Praxis Precision Medicines, Inc. Companies engaged in the commercialization and development of antisense oligonucleotides as therapeutics include Alnylam Pharmaceuticals, Inc. and Ionis Pharmaceuticals Inc.

Many of our competitors, either alone or in combination with their respective strategic partners, have significantly greater financial resources and expertise in research and development, manufacturing, the regulatory approval process, and marketing than we do. Mergers and acquisition activity in the pharmaceutical, biopharmaceutical and biotechnology sector is likely to result in greater resource concentration among a smaller number of our competitors. Smaller or early-stage companies may also prove to be significant competitors, particularly through sizeable collaborative arrangements with established companies. These competitors also compete with us in recruiting and retain qualified scientific and management personnel and establishing clinical trial sites and patient registration for clinical trials, as well as in acquiring technologies complementary to, or necessary for, our programs.

Our commercial opportunity could be reduced or eliminated if one or more of our competitors develop and commercialize products that are safer, more effective, better tolerated, or of greater convenience or economic benefit than our proposed product offering. Our competitors also may be in a position to obtain FDA or other regulatory approval for their products more rapidly, resulting in a stronger or dominant market position before we are able to enter the market. The key competitive factors affecting the success of all of our programs are likely to be product safety, efficacy, convenience and treatment cost.

Intellectual property

We believe that our intellectual property estate is a strategic asset that has the potential to provide us with a competitive advantage. We strive to protect and enhance the proprietary technology, inventions and improvements that we believe are important to our business, including pursuing, maintaining and defending patent rights, whether developed internally or licensed from third parties. We also rely on trade secrets and know-how relating to our proprietary technology and product candidates, continuing technological innovation and in-licensing opportunities to develop, strengthen and maintain our proprietary and intellectual property position. We additionally may rely on data exclusivity, market exclusivity and patent term extensions, when available, and plan to seek and rely on regulatory protection afforded through orphan drug designations. Our commercial success may depend in part on our ability to obtain and maintain patent and other proprietary protection for our technology, inventions, and improvements; to preserve the confidentiality of our trade secrets; to defend and enforce our proprietary rights, including our patents; and to operate without infringing on the valid and enforceable patents and other proprietary rights of third parties.

Our wholly owned and in-licensed patent portfolio includes patent rights covering various aspects of our RAP Platform and current product candidates, and certain legacy programs the company is no longer pursuing. As of

June 5, 2024, our patent portfolio consists of 36 patent families, including 2 owned U.S. issued patents, 34 in-licensed U.S. issued patents, 110 in-licensed foreign issued patents, 20 owned or in-licensed U.S. pending patent applications (including provisional patent applications), 31 owned or in-licensed foreign pending patent applications, and 5 owned or in-licensed pending Patent Cooperation Treaty applications, or PCT applications, that have not entered national phase. Our objective is to continue to expand our patent portfolio to protect our technology, inventions, improvements and current and future product candidates. Examples of the product candidates and technology areas covered by our intellectual property portfolio are described below.

Program-related intellectual property

The program-related patent rights in our patent portfolio provide coverage for product candidates designed to address certain diseases and disorders. The program-related patent applications for our lead programs include those described below. Each of the program-related patent applications described below is wholly owned by us.

CMP-CPS-001 program

Our lead product candidate, CMP-CPS-001, is designed to amplify CPS1 expression. As of June 5, 2024, we owned one pending PCT application filed in December 2022, and claiming priority to two separate U.S. provisional patent applications, the earliest of which was filed in December 2021, which relates to compositions of matter, including CMP-CPS-001, designed to amplify CPS1 expression, and methods of treating urea cycle disorders. We expect patents claiming priority to this patent application, if any, to expire in 2042, excluding any patent term adjustments or extensions.

CMP-FH program

Our CMP-FH program aims to amplify LDLR expression. As of June 5, 2024, we owned two pending provisional U.S. patent applications filed in May 2024 relating to compositions of matter, including ASOs designed to amplify LDLR expression, and methods of treating familial hypercholesterolemia. We expect patents claiming priority to these patent applications, if any, to expire between 2044 and 2045, excluding any patent term adjustments or extensions.

CMP-SYNGAP program

Our CMP-SYNGAP program aims to amplify SYNGAP1 expression. As of June 5, 2024, we owned one pending PCT application filed in December 2023, which claims priority to a U.S. provisional patent application filed in December 2022, as well as two pending U.S. provisional patent applications filed in June 2024, each relating to compositions of matter, including ASOs designed to amplify SYNGAP1 expression, and methods of treating SYNGAP1-related disorders. We expect patents claiming priority to these patent applications, if any, to expire between 2043 and 2045, excluding any patent term adjustments or extensions.

In addition to our programs listed above, we also have patent applications relating to ASO compositions directed to regRNAs involved in the transcription of additional gene targets and their use for treating additional diseases or disorders that may benefit from upregulation of gene expression. As of June 5, 2024, we owned one U.S. non-provisional patent application and eight foreign patent applications in Australia, Canada, China, Europe, Israel, India, Japan and Mexico relating to compositions and methods for treating urea cycle disorders. Each of these patent applications are national or regional phase applications based on a PCT application filed in September 2022, which claims priority to two separate provisional U.S. patent applications, the earliest of which was filed in September 2021. We expect patents issuing from or claiming priority from these pending patent applications, if any, to expire in 2042, excluding any patent term adjustments or extensions. As of June 5, 2024, we owned one pending PCT application filed in June 2023, claiming priority to three separate U.S. provisional patent applications, the earliest of which was filed in June 2022, which relates to compositions and methods for treating several diseases and disorders including frontotemporal dementia. We expect patents claiming priority from this pending application, if any, to expire in 2043, excluding any patent term adjustments or extensions. As of June 5, 2024, we owned one PCT application filed in November 2023, claiming priority to a U.S. provisional patent application filed in November 2022, which relates to compositions and methods for treating cholestatic liver

disease. We expect patents claiming priority from this pending application, if any, to expire in 2043, excluding any patent term adjustments or extensions.

Platform-related intellectual property

In addition to the program-related intellectual property, our intellectual property portfolio includes know-how and patent applications directed to our RAP Platform and other technologies developed internally or in-licensed from the Whitehead Institute for Biomedical Research, or the Whitehead Institute. Exemplary platform technologies that are subject to such patent applications include methods of modulating gene expression using oligonucleotides, methods for characterizing enhancer-promoter pairs, and methods for modulating condensate-dependent transcription. These platform technologies, and our intellectual property portfolio related thereto, relate broadly to our existing product candidates and those we may develop in the future.

We continually assess and refine our intellectual property strategy as we develop new product candidates and technologies. To that end, we expect to file additional patent applications in support of current and new product candidates as well as new technologies.

Our ability to stop third parties from making, using, selling, marketing, offering to sell, importing, and commercializing our product candidates and technology is dependent upon the extent to which we have rights under valid and enforceable patents and other intellectual property rights that cover our product candidates and technology. We cannot predict whether or when our owned or licensed pending and future patent applications will result in the issuance of patents, nor can we predict whether any patents that may be granted to us in the future will be commercially useful in protecting our product candidates and technology.

The terms of individual patents depend upon the legal term of the patents in the countries in which they are obtained. In most countries in which we file, including the United States, the patent term is 20 years from the earliest date of filing a non-provisional patent application. In the United States, a patent's term may be lengthened by patent term adjustment, which compensates a patentee for administrative delays by the U.S. Patent and Trademark Office, or USPTO, in granting a patent, or may be shortened if a patent is terminally disclaimed over an earlier-filed patent.

In the United States, the term of a patent that covers an FDA-approved drug may be eligible for a patent term extension under the Hatch-Waxman Act as compensation for the loss of patent term during the FDA regulatory review process. The period of extension may be up to five years beyond the expiration of the patent, but cannot extend the remaining term of a patent beyond a total of 14 years from the date of product approval. Only one patent among those eligible for an extension may be extended, and a given patent may only be extended once. Similar provisions are available in Europe and in certain other jurisdictions to extend the term of a patent that covers an approved drug. If our product candidates receive approval, we intend to apply for patent term extensions, if available, to extend the term of patents that cover the approved product candidates. We also intend to seek patent term extensions in any jurisdiction where they are available, however there is no guarantee that the applicable authorities, including the FDA in the United States, will agree with our assessment that such extensions should be granted, and if granted, the length of such extensions.

In addition to patent protection, we also rely on know-how and trade secret protection for our proprietary information to develop and maintain our proprietary and intellectual property position. However, trade secrets can be difficult to protect. Although we take steps to protect our proprietary information, including entering into agreements with our employees, corporate collaborators, external scientific collaborators, contract manufacturers, consultants, advisors, and other third parties, such individuals may breach such agreements and disclose our proprietary information, including our trade secrets, and we may not be able to obtain adequate remedies for such breaches. In addition, third parties may independently develop the same or similar proprietary information or may otherwise gain access to our proprietary information. As a result, we may be unable to meaningfully protect our know-how, trade secrets and proprietary information.

Government regulation in the United States

The FDA and other regulatory authorities at federal, state and local levels, as well as in foreign countries, extensively regulate, among other things, the research, development, testing, manufacture, pricing, reimbursement, sales, quality control, approval, packaging, storage, recordkeeping, labeling, advertising, promotion, distribution, marketing, post-approval monitoring and reporting and import and export of drugs. We, along with our contract manufacturers, or CMOs, contract research organizations, or CROs, and third-party vendors, will be required to satisfy these requirements in each of the countries in which we wish to conduct studies or seek approval of our product candidates. The process of obtaining marketing approvals and the subsequent compliance with appropriate federal, state, local, and foreign statutes and regulations require the expenditure of substantial time and financial resources.

In the United States, the FDA regulates drugs under the Federal Food, Drug, and Cosmetic Act, or FDCA, as amended, and its implementing regulations. Drugs are also subject to other federal, state and local statutes and regulations. FDA clearance of an Investigational New Drug, or IND, application must be obtained before commencing clinical testing of a new drug in the United States. FDA approval also must generally be obtained before a drug may be legally marketed in the United States.

Failure to comply with applicable regulatory requirements at any time during the product development, approval, or post-approval processes, could result in delays in the conduct of clinical trials or regulatory review and approval, as well as administrative or judicial sanctions or other legal consequences. These sanctions or consequences could include, among other things, the FDA's refusal to approve pending applications, issuance of clinical holds for planned or ongoing studies, suspension or revocation of existing product approvals, issuance of warning or untitled letters, adverse publicity, product withdrawals or recalls, marketing restrictions, product seizures, total or partial suspensions of manufacturing or distribution, import detentions or refusals, injunctions, fines, government investigations, civil penalties or criminal prosecution.

U.S. development process

The process for seeking approval to market and distribute a new drug in the United States generally involves the following:

- completion of nonclinical laboratory tests and animal studies according to GLP requirements and applicable requirements for the humane use of laboratory animals or other regulations;
- completion of the manufacture, under current Good Manufacturing Practices, or cGMPs, conditions of the drug substance and drug product that the sponsor intends to use in human clinical trials along with required analytical and stability testing;
- submission to the FDA of an IND application, which must become effective before human clinical trials may begin;
- approval by an institutional review board, or IRB, reviewing each clinical site before each clinical trial may be initiated;
- performance of adequate and well-controlled human clinical trials according to Good Clinical Practices, or GCPs, and any additional requirements for the protection of human research subjects and their health information, to establish the safety and efficacy of the drug for its intended use;
- preparation and submission to the FDA of a New Drug Application, or NDA, requesting marketing approval for one or more proposed indications, including submission of detailed information on the chemistry, manufacture and quality controls of the product in clinical development and proposed labeling;
- satisfactory completion of one or more FDA pre-approval inspections of the manufacturing facility or facilities where the drug will be produced, including those of third parties, to assess compliance with cGMP requirements;

- potential FDA audit of the nonclinical and clinical study sites that generated the data in support of the NDA to assess compliance with GLP and GCP and the integrity of clinical data in support of the NDA;
- payment of user fees under the Prescription Drug User Fee Act, or PDUFA, unless exempted;
- FDA review and approval of the NDA, including consideration of the views of any FDA advisory committee, prior to any commercial marketing or sale of the drug in the United States; and
- compliance with any post-approval requirements, including the potential requirement to implement a Risk Evaluation and Mitigation Strategy, or REMS, and the potential requirement to conduct post approval studies.

Before testing any drug in humans, the product candidate enters the preclinical testing stage. Nonclinical tests include laboratory evaluations of drug chemistry, formulation and stability, as well as in vitro and animal studies to assess safety and in some cases to establish the rationale for therapeutic use. The conduct of nonclinical studies is subject to federal and state regulation, including GLPs. The clinical study sponsor must submit the results of the nonclinical tests, together with manufacturing information, analytical data, any available clinical data or literature, and a proposed clinical protocol, to the FDA as part of the IND. Some nonclinical testing typically continues after the IND is submitted.

An IND is an exemption from the FDCA that allows an unapproved product to be shipped in interstate commerce for use in an investigational clinical trial and a request for FDA authorization to administer an investigational product to humans. The IND must become effective before clinical trials may begin. The IND automatically becomes effective 30 days after receipt by the FDA, unless before that time the FDA raises concerns or questions about the product or conduct of the proposed clinical trial, including concerns that human research subjects will be exposed to unreasonable health risks. If the FDA raises concerns or questions either during the initial 30-day period, or at any time during the IND review process, it may choose to impose a clinical hold. In such a case, the IND sponsor and the FDA must resolve any outstanding concerns before the clinical trial can begin. As a result, submission of an IND may or may not result in FDA authorization to begin a clinical trial, or to begin a clinical trial on the terms originally specified by the sponsor in the IND. A separate submission to an existing IND must also be made for each successive clinical trial conducted, and the FDA must grant permission, either explicitly or implicitly by not objecting, before each clinical trial can begin.

Clinical trials may involve the administration of the drug product candidate to healthy volunteers or subjects under the supervision of qualified investigators. Clinical trials involving some products for certain diseases, including some rare diseases may begin with testing in patients with the disease. Clinical trials are conducted under protocols detailing, among other things, the objectives of the clinical trial, dosing procedures, subject selection, and exclusion criteria, and the parameters to be used to monitor subject safety, including stopping rules that assure a clinical trial will be stopped if certain adverse events should occur. Each protocol and any amendments to the protocol must be submitted to the FDA as part of the IND. Clinical trials must be conducted and monitored in accordance with the FDA's regulations comprising the GCP requirements, including the requirement that all research subjects or his or her legal representative provide informed consent. Further, each clinical trial must be reviewed and approved by an independent IRB, at or servicing each institution at which the clinical trial will be conducted. An IRB is charged with protecting the welfare and rights of study participants and considers such items as whether the risks to individuals participating in the clinical trials are minimized and are reasonable in relation to anticipated benefits. The IRB also approves the form and content of the informed consent that must be signed by each clinical trial subject or his or her legal representative and must monitor the clinical trial until completed.

Some clinical trials also include oversight by an independent group of qualified experts organized by the clinical trial sponsor, known as a data safety monitoring board or data monitoring committee. This group may recommend continuation of the trial as planned, changes in trial conduct, or cessation of the trial at designated check points based on certain data from the trial to which only the group has access.

Human clinical trials are typically conducted in three sequential phases that may overlap or be combined:

- ***Phase 1***. The drug is initially introduced into healthy human subjects and tested for safety, including adverse effects, dose tolerance, absorption, metabolism, distribution, excretion and pharmacodynamics. In the case of some products for rare diseases, the initial human testing is often conducted in patients.
- ***Phase 2***. The drug is evaluated in a limited patient population to identify possible adverse effects and safety risks, preliminarily evaluate the efficacy of the product for specific targeted diseases, and determine dosage tolerance, optimal dosage, and dosing schedule. Multiple Phase 2 clinical trials may be conducted by the sponsor to obtain information prior to beginning larger and more costly Phase 3 clinical trials.
- ***Phase 3***. Phase 3 clinical trials typically proceed if the Phase 2 clinical trials demonstrate that a dose range of the product candidate is potentially effective and has an acceptable safety profile. Phase 3 clinical trials are generally undertaken within an expanded patient population to further evaluate dosage, provide substantial evidence of clinical efficacy and further test for safety, in a diverse patient population at multiple, geographically dispersed clinical trial sites. A well-controlled, statistically robust Phase 3 trial may be designed to deliver the data that regulatory authorities will use to decide whether or not to approve, and, if approved, how to appropriately label a product candidate.

In some cases, the FDA may approve an NDA for a product but require the sponsor to conduct additional clinical trials to further assess the product's safety and effectiveness after approval. Such post-approval trials are typically referred to as Phase 4 clinical trials. These trials are used to gain additional experience from the treatment of patients in the intended therapeutic indication and to document a clinical benefit for products approved under accelerated approval regulations. The failure to exercise due diligence with regard to conducting Phase 4 clinical trials could result in withdrawal of approval for products.

During all phases of clinical development, the FDA requires extensive monitoring and auditing of all clinical activities, clinical data, and clinical trial investigators. Annual progress reports detailing the results of the clinical trials must be submitted to the FDA. Written IND safety reports must be promptly submitted to the FDA and the investigators for serious and unexpected adverse events, any findings from other studies, tests in laboratory animals or in vitro testing that suggest a significant risk for human subjects, or any clinically important increase in the rate of serious suspected adverse reactions over those listed in the protocol or investigator brochure. The sponsor must submit an IND safety report within 15 calendar days after the sponsor determines that the information qualifies for such reporting. The sponsor also must notify the FDA of any unexpected fatal or life-threatening suspected adverse reaction within 7 calendar days after the sponsor's initial receipt of the information. Phase 1, Phase 2, and Phase 3 clinical trials may not be completed successfully within any specified period, if at all. At any time while clinical trials are ongoing under the IND, the FDA may impose a partial or complete clinical hold. Clinical holds may be imposed when there is concern for patient safety, and may be a result of new data, findings, or developments in clinical, nonclinical, and/or chemistry, manufacturing and controls or where there is non-compliance with regulatory requirements. If the FDA imposes a clinical hold, trials may not recommence without FDA authorization and then only under terms authorized by the FDA. Similarly, an IRB can suspend or terminate approval of a clinical trial at its institution if the clinical trial is not being conducted in accordance with the IRB's requirements and either the IRB or the data safety monitoring board may suspend a clinical trial at any time on various grounds, including a finding that the research subjects or patients are being exposed to an unacceptable health risk.

During the development of a new drug, sponsors are given opportunities to meet with the FDA at certain points. These points may be prior to submission of an IND, at the end of Phase 2, and before an NDA is submitted. Meetings at other times may be requested. These meetings can provide an opportunity for the sponsor to share information about the data gathered to date, for the FDA to provide advice, and for the sponsor and the FDA to reach agreement on the next phase of development.

Concurrent with clinical trials, companies must finalize a process for manufacturing the drug product in commercial quantities in accordance with cGMP requirements. The manufacturing process must be capable of consistently producing quality batches of the product candidate and manufacturers must develop, among other things, methods for testing the identity, strength, quality and purity of the final drug product. Additionally, appropriate packaging

must be selected and tested, and stability studies must be conducted to demonstrate that the product candidate does not undergo unacceptable deterioration over its shelf life.

There are also various laws and regulations regarding laboratory practices, the experimental use of animals, and the use and disposal of hazardous or potentially hazardous substances in connection with the research. In each of these areas, the FDA and other regulatory authorities have broad regulatory and enforcement powers, including the ability to levy fines and civil penalties, suspend or delay issuance of approvals, seize or recall products, and withdraw approvals.

Information about certain clinical trials must be submitted within specific timeframes for public dissemination on the clinicaltrials.gov website. Sponsors or distributors of investigational products for the diagnosis, monitoring, or treatment of one or more serious diseases or conditions must also have a publicly available policy on evaluating and responding to requests for expanded access requests.

A sponsor who wishes to conduct a clinical trial outside of the United States may, but need not, obtain FDA authorization to conduct the clinical trial under an IND. When a foreign clinical trial is conducted under an IND, all FDA IND requirements must be met unless waived. If a foreign clinical trial is not conducted under an IND, FDA will nevertheless accept the results of the study in support of an NDA if the study was conducted in accordance with GCP requirements, and the FDA is able to validate the data through an onsite inspection if deemed necessary. The FDA's regulations are intended to help ensure the protection of human subjects enrolled in non-IND foreign clinical trials, as well as the quality and integrity of the resulting data. They further help ensure that non-IND foreign trials are conducted in a manner comparable to that required for clinical trials in the United States.

U.S. review and approval processes

Assuming successful completion of the required clinical testing, the results of the preclinical studies and clinical trials, together with detailed information relating to the product's chemistry, manufacture, controls and proposed labeling, among other things, are submitted to the FDA as part of an NDA package requesting approval to market the product for one or more indications. An NDA is a request for approval to market a new drug for one or more specified indications and must contain proof of the drug's safety and efficacy for the requested indications. The marketing application is required to include both negative and ambiguous results of preclinical studies and clinical trials, as well as positive findings. Data may come from company-sponsored clinical trials intended to test the safety and efficacy of a product's use or from a number of alternative sources, including studies initiated by investigators. To support marketing approval, the data submitted must be sufficient in quality and quantity to establish the safety and efficacy of the investigational product to the satisfaction of the FDA. FDA must generally approve an NDA before a drug may be marketed in the United States.

The FDA reviews all submitted NDAs before it accepts them for filing and may request additional information rather than accepting the NDA for filing. The FDA must make a decision on accepting an NDA for filing within 60 days of receipt, and such decision could include a refusal to file by the FDA. Once the submission is accepted for filing, the FDA begins an in-depth substantive review of the NDA. The FDA reviews an NDA to determine, among other things, whether the drug is safe and effective for the indications sought and whether the facility in which it is manufactured, processed, packaged or held meets standards designed to assure the product's continued safety, quality and purity. The FDA likely will reanalyze the clinical trial data, which could result in extensive discussions between the FDA and the applicant during the review process. Under the goals and polices agreed to by the FDA under PDUFA, the FDA targets ten months, from the filing date, in which to complete its initial review of a NDA and respond to the applicant, and six months from the filing date of a NDA for priority review. The FDA does not always meet its PDUFA goal dates for standard or priority NDAs, and the review process is often extended by FDA requests for additional information or clarification. Each NDA must be accompanied by a substantial PDUFA user fee, which FDA adjusts on an annual basis. Fee waivers or reductions are available in certain circumstances, including a waiver of the application fee for the first application filed by a small business. Additionally, no user fees are assessed on NDAs for products designated as orphan drugs, unless the product also includes a non-orphan indication.

The FDA may refer an application for a novel drug or drug that presents difficult questions of safety and efficacy to an advisory committee. An advisory committee is a panel of independent experts, including clinicians and other scientific experts, which reviews, evaluates and provides a recommendation as to whether the application should be approved and under what conditions. The FDA is not bound by the recommendations of an advisory committee, but it considers such recommendations carefully when making decisions.

Before approving an NDA, the FDA typically will inspect the facility or facilities where the product is manufactured. The FDA will not approve an application unless it determines that the manufacturing processes and facilities are in compliance with cGMP requirements and are adequate to assure consistent production of the product within required specifications. Additionally, before approving an NDA, the FDA may inspect one or more clinical trial sites to assure compliance with GCP and other requirements and the integrity of the clinical data submitted to the FDA.

After evaluating the NDA and all related information, including the advisory committee recommendation, if any, and inspection reports regarding the manufacturing facilities and clinical trial sites, the FDA may issue an approval letter, or, in some cases, a complete response letter. A complete response letter generally contains a statement of specific conditions that must be met in order to secure final approval of the NDA and may require additional clinical or preclinical testing in order for the FDA to reconsider the application. The NDA sponsor will have one year to submit to the FDA information that represents a complete response to the deficiencies described in the letter. The FDA will then re-review the application, taking into consideration the response and determine whether the application meets the criteria for approval. If and when those conditions have been met to the FDA's satisfaction, the FDA will typically issue an approval letter. An approval letter authorizes commercial marketing of the drug with specific prescribing information for specific indications.

Even if the FDA approves a product, it may limit the approved indications for use of the product and require that contraindications, warnings or precautions be included in the product labeling. Additionally, the FDA may require post-approval studies, including Phase 4 clinical trials, to further assess a drug's efficacy or safety after approval. The agency may also require testing and surveillance programs to monitor the product after commercialization, or impose other conditions, including distribution restrictions or other risk management mechanisms under a REMS. A REMS can include medication guides, physician communication plans, assessment plans, and/or elements to assure safe use, such as restricted distribution methods, patient registries, or other risk-minimization tools, which can materially affect the potential market and profitability of the product. The FDA may prevent or limit further marketing of a product based on the results of post-marketing studies or surveillance programs. After approval, some types of changes to the approved product, such as adding new indications, manufacturing changes, and additional labeling claims, are subject to further testing requirements and FDA review and approval.

Post-approval requirements

Maintaining substantial compliance with applicable federal, state, and local statutes and regulations requires the expenditure of substantial time and financial resources. Rigorous and extensive FDA regulation of drugs continues after approval, particularly with respect to cGMP. If we obtain regulatory approval for any of our products, we will be required to comply with all post-approval regulatory requirements as well as any specific post-approval requirements that the FDA have imposed as part of the approval process. We will be required to report certain adverse reactions and production problems to the FDA, provide updated safety and efficacy information, and comply with requirements concerning advertising and promotional labeling requirements and record-keeping requirements. Further, if there are any modifications to the drug, including changes in indications, labeling or manufacturing processes or facilities, we may be required to submit and obtain FDA approval of a new NDA or NDA supplement, which may require the generation of additional data or the conduct of additional preclinical studies and clinical trials. Manufacturers and other parties involved in the drug supply chain for prescription drug products must also comply with product tracking and tracing requirements and for notifying the FDA of counterfeit, diverted, stolen and intentionally adulterated products or products that are otherwise unfit for distribution in the United States.

We will rely, and expect to continue to rely, on third parties for the production of clinical and commercial quantities of any products that we may commercialize. Manufacturers of our products are required to comply with applicable requirements in the cGMP regulations, including quality control and quality assurance and maintenance of records and documentation.

Manufacturing facilities are required to register their establishments with the FDA and certain state agencies and are subject to periodic inspections by the FDA and certain state agencies for compliance with ongoing regulatory requirements. Failure to comply with statutory and regulatory requirements may result in the issuance of an FDA Form 483 notice of inspectional observations, untitled letter, warning letter, or suspension of manufacturing or other legal or regulatory action, such as product seizures, injunctions, civil penalties or criminal prosecution. Additionally, defects in manufacturing of commercial products can result in product recalls.

Systems need to be put in place to record and evaluate adverse events reported by health care providers and patients and to assess product complaints. Later discovery of previously unknown problems with a product, including adverse events of unanticipated severity or frequency, or with manufacturing processes, or failure to comply with regulatory requirements, may result in revisions to the approved labeling to add new safety information, requirements for post-market studies or clinical trials to assess new safety risks, or imposition of distribution or other restrictions under a REMS. Other potential consequences include, among other things:

- restrictions on the marketing or manufacturing of the product, complete withdrawal of the product from the market or product recalls;
- the issuance of safety alerts, Dear Healthcare Provider letters, press releases or other communications containing warnings or other safety information about the product;
- fines, warning letters or holds on post-approval clinical trials;
- refusal of the FDA to approve applications or supplements to approved applications, or suspension or revocation of product approvals;
- product seizure or detention, or refusal to permit the import or export of products;
- injunctions or the imposition of civil or criminal penalties; and
- consent decrees, corporate integrity agreements, debarment or exclusion from federal healthcare programs; or mandated modification of promotional materials and labeling and issuance of corrective information.

The FDA strictly regulates the marketing, labeling, advertising and promotion of prescription drug products placed on the market. This regulation includes, among other things, standards and regulations for direct-to-consumer advertising, communications regarding unapproved uses, industry-sponsored scientific and educational activities and promotional activities involving the internet and social media. Promotional claims about a drug's safety or effectiveness are prohibited before the drug is approved. After approval, a drug product generally may not be promoted for uses or patient populations that are not approved by the FDA, as reflected in the product's prescribing information (known as "off-label" use). In the United States, healthcare professionals are generally permitted to prescribe drugs for such off-label uses because the FDA does not regulate the practice of medicine. However, FDA regulations impose rigorous restrictions on manufacturers' communications, prohibiting the promotion of off-label uses. Prescription drug promotional materials must be submitted to the FDA in conjunction with their first use.

If a company, including any agent of the company or anyone speaking on behalf of the company, is found to have promoted off-label uses, the company may become subject to adverse public relations and administrative and judicial enforcement by the FDA, the DOJ, or the Office of the Inspector General of the Department of Health and Human Services, as well as state authorities. This could subject a company to a range of penalties that could have a significant commercial impact, including civil and criminal fines and agreements that materially restrict the manner in which a company promotes or distributes drug products. The federal government has levied large civil

and criminal fines against companies for alleged improper promotion and has also requested that companies enter into consent decrees or permanent injunctions under which specified promotional conduct is changed or curtailed.

Orphan drug designation

Under the Orphan Drug Act, the FDA may grant Orphan Drug Designation, or ODD, to a drug intended to treat a rare disease or condition, which is generally a disease or condition that affects fewer than 200,000 individuals in the United States, or more than 200,000 individuals in the United States and for which there is no reasonable expectation that the cost of developing and making a drug available in the United States for this type of disease or condition will be recovered from sales of the product. ODD must be requested before submitting a marketing application. After the FDA grants ODD, the identity of the therapeutic agent and its potential orphan use are disclosed publicly by the FDA. ODD does not convey any advantage in or shorten the duration of the regulatory review and approval process.

If a product that has ODD receives the first FDA approval for the disease or condition for which it has such designation, the product is entitled to orphan product exclusivity, which means that the FDA may not approve any other applications to market the same drug for the same indication for seven years, except in limited circumstances, such as not being able to supply the product for patients or showing clinical superiority to the product with orphan exclusivity.

Competitors, however, may receive approval of different products for the indication for which the orphan product has exclusivity or obtain approval for the same product but for a different indication for which the orphan product has exclusivity. Orphan product exclusivity also could block the approval of one of our products for seven years if a competitor obtains approval of the same drug as defined by the FDA or if our product candidate is determined to be contained within the competitor's product for the same indication or disease. If a drug designated as an orphan product receives marketing approval for an indication broader than what is designated, it may not be entitled to orphan product exclusivity.

The FDA has historically taken the position that the scope of orphan exclusivity aligns with the approved indication or use of a product, rather than the disease or condition for which the product received orphan designation. However, in *Catalyst Pharms., Inc. v. Becerra*, 14 F.4th 1299 (11th Cir. 2021), the court disagreed with this position, holding that orphan-drug exclusivity blocked the FDA's approval of the same drug for all uses or indications within the same orphan-designated disease. On January 24, 2023, the FDA published a notice in the Federal Register to clarify that the FDA intends to continue to apply its longstanding interpretation of the regulations to all matters outside of the scope of the *Catalyst* order and will continue tying the scope of orphan-drug exclusivity to the uses or indications for which a drug is approved. It is unclear how future litigation, legislation, agency decisions, and administrative actions will impact the scope of the orphan drug exclusivity.

Expedited review and approval programs

The FDA has various programs, including Fast Track designation, priority review, accelerated approval, and breakthrough therapy designation, that are intended to expedite or simplify the process for the development and FDA review of drugs that are intended for the treatment of serious or life-threatening diseases or conditions and demonstrate the potential to address unmet medical needs. The purpose of these programs is to provide important new drugs to patients earlier than under standard FDA review procedures. To be eligible for a Fast Track designation, the FDA must determine, based on the request of a sponsor, that a drug is intended to treat a serious or life-threatening disease or condition and demonstrates the potential to address an unmet medical need. The FDA will determine that a product will fill an unmet medical need if it will provide a therapy where none exists or provide a therapy that may be potentially superior to existing therapy based on efficacy or safety factors. In addition to other benefits, such as the ability to have greater interactions with the FDA, the FDA may initiate review of sections of a Fast Track BLA before the application is complete, a process known as rolling review.

The FDA may give a priority review designation, such as a rare pediatric disease designation, to drugs that treat a serious condition and, if approved, would provide a significant improvement in safety or effectiveness. A priority

review means that the goal for the FDA to review an application is six months, rather than the standard review of ten months under current PDUFA guidelines. Most products that are eligible for Fast Track designation may also be considered appropriate to receive a priority review. In addition, drugs studied for their safety and effectiveness in treating serious or life-threatening illnesses and that provide meaningful therapeutic benefit over existing treatments may receive accelerated approval and may be approved on the basis of adequate and well-controlled clinical trials establishing that the drug has an effect on a surrogate endpoint that is reasonably likely to predict clinical benefit, or on a clinical endpoint that can be measured earlier than irreversible morbidity or mortality, that is reasonably likely to predict an effect on irreversible morbidity or mortality or other clinical benefit, taking into account the severity, rarity, or prevalence of the condition and the availability or lack of alternative treatments. As a condition of approval, the FDA may require a sponsor of a drug receiving accelerated approval to perform adequate and well-controlled post-marketing studies to verify and describe the predicted effect on irreversible morbidity or mortality or other clinical endpoint. Under the Food and Drug Omnibus Reform Act of 2022, or FDORA, the FDA may require, as appropriate, that such trials be underway prior to approval or within a specific time period after the date of approval for a product granted accelerated approval. Under FDORA, the FDA has increased authority for expedited procedures to withdraw approval of a drug or indication approved under accelerated approval if, for example, the confirmatory trial fails to verify the predicted clinical benefit of the product. In addition, for products being considered for accelerated approval, the FDA generally requires, unless otherwise informed by the agency, that all advertising and promotional materials intended for dissemination or publication within 120 days of marketing approval be submitted to the agency for review during the pre-approval review period.

Moreover, a sponsor can request designation of a product candidate as a “breakthrough therapy.” A breakthrough therapy is defined as a drug that is intended, alone or in combination with one or more other drugs, to treat a serious or life-threatening disease or condition, and preliminary clinical evidence indicates that the drug may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development. Drugs designated as breakthrough therapies are also eligible for accelerated approval. The FDA must take certain actions, such as holding timely meetings and providing advice, intended to expedite the development and review of an application for approval of a breakthrough therapy.

Even if a product qualifies for one or more of these programs, the FDA may later decide that the product no longer meets the conditions for qualification or decides that the time period for FDA review or approval will not be shortened. Furthermore, fast-track designation, priority review, accelerated approval, and breakthrough therapy designation do not change the standards for approval and may not ultimately expedite the development or approval process.

Pediatric information and pediatric exclusivity

The Pediatric Research Equity Act, or PREA, requires a sponsor to conduct pediatric clinical trials for most drugs, for a new active ingredient, new indication, new dosage form, new dosing regimen or new route of administration. Under PREA, as amended, certain NDAs and NDA supplements must contain data that can be used to assess the safety and efficacy of the drug for the claimed indications in all relevant pediatric subpopulations and to support dosing and administration for each pediatric subpopulation for which the product is safe and effective. The FDA may grant deferrals for submission of pediatric data or full or partial waivers. The FDCA requires that a sponsor who is planning to submit a marketing application for a drug that includes a new active ingredient, new indication, new dosage form, new dosing regimen or new route of administration submit an initial Pediatric Study Plan, or PSP, within 60 days of an end-of-Phase 2 meeting or, if there is no such meeting, as early as practicable before the initiation of the Phase 3 or Phase 2/3 study. The initial PSP must include an outline of the pediatric study or studies that the sponsor plans to conduct, including study objectives and design, age groups, relevant endpoints and statistical approach, or a justification for not including such detailed information, and any deferral or waiver requests. The FDA and the sponsor must reach an agreement on the PSP. A sponsor can submit amendments to an agreed-upon initial PSP at any time if changes to the pediatric plan need to be considered based on data collected from preclinical studies, early phase clinical trials and/or other clinical development programs.

The FDA may, on its own initiative or at the request of the applicant, grant deferrals for submission of some or all pediatric data until after approval of the product for use in adults, or full or partial waivers from the pediatric data requirements. Unless otherwise required by regulation, the pediatric data requirements do not apply to products with orphan designation.

A drug can also obtain pediatric market exclusivity in the United States. Pediatric exclusivity, if granted, adds six months to existing marketing exclusivity periods and patent terms. This six-month exclusivity, which runs from the end of other exclusivity protection or patent term, may be granted based on the voluntary completion of a pediatric study in accordance with an FDA-issued "Written Request" for such a study.

Marketing exclusivity

Market exclusivity provisions authorized under the FDCA can delay the submission or the approval of certain marketing applications. The FDCA provides a five-year period of non-patent marketing exclusivity within the United States to the first applicant to obtain approval of an NDA for a new chemical entity. A drug is a new chemical entity if the FDA has not previously approved any other new drug containing the same active moiety, which is the molecule or ion responsible for the action of the drug substance. During the exclusivity period, the FDA may not approve or even accept for review an abbreviated new drug application, or ANDA, or an NDA submitted under Section 505(b)(2), or 505(b)(2) NDA, submitted by another company for another drug based on the same active moiety, regardless of whether the drug is intended for the same indication as the original innovative drug or for another indication, where the applicant does not own or have a legal right of reference to all the data required for approval. However, an application may be submitted after four years if it contains a certification of patent invalidity or non-infringement to one of the patents listed with the FDA by the innovator NDA holder.

The FDCA alternatively provides three years of marketing exclusivity for an NDA, or supplement to an existing NDA if new clinical investigations, other than bioavailability studies, that were conducted or sponsored by the applicant are deemed by the FDA to be essential to the approval of the application, for example new indications, dosages or strengths of an existing drug. This three-year exclusivity covers only the modification for which the drug received approval on the basis of the new clinical investigations and does not prohibit the FDA from approving ANDAs or 505(b)(2) NDAs for drugs containing the active agent for the original indication or condition of use. Five-year and three-year exclusivity will not delay the submission or approval of a full NDA. However, an applicant submitting a full NDA would be required to conduct or obtain a right of reference to any preclinical studies and adequate and well-controlled clinical trials necessary to demonstrate safety and effectiveness.

In addition, pediatric exclusivity and orphan drug exclusivity, as described above, may offer a six-month or seven-year period of exclusivity, respectively, except in certain circumstances.

Patent term restoration and extension

Depending upon the timing, duration and specifics of FDA approval of our product candidates, some of a sponsor's U.S. patents may be eligible for limited patent term extension, or PTE, under the Hatch-Waxman Amendments. As compensation for patent term lost during product development and the FDA regulatory review process, the Hatch-Waxman Amendments permit a patent restoration term, which is limited to a maximum of five years, or less if the extended patent term would exceed 14 years after the date of the regulatory approval of the product. The patent term restoration period is generally one-half the time between the effective date of an IND and the submission date of an NDA plus the time between the submission date of an NDA, less any time the sponsor did not act with due diligence during the period and the approval of that application less any time the sponsor did not act with due diligence during the period. Only one patent applicable to an approved drug or drug product is eligible for the extension, only those claims covering the approved drug, a method for using it, or a method for manufacturing it may be extended, and the application for the extension must be submitted prior to the expiration of the patent. Moreover, a given patent may only extended once based on a single product. The USPTO, in consultation with the FDA, reviews and approves the application for any patent term extension or restoration. Similar provisions are available in Europe and other foreign jurisdictions to extend the term of a patent that covers an approved drug. In the future, we may intend to apply for restoration of a patent term for

one of our currently owned or licensed patents to add patent life beyond its current expiration date, depending on the expected length of the clinical trials and other factors involved in the filing of the relevant NDA. However, we may not receive an extension if we fail to exercise due diligence during the testing phase or regulatory review process, fail to apply within applicable deadlines, fail to apply prior to expiration of relevant patents or otherwise fail to satisfy applicable requirements. Moreover, the length of the extension could be less than we request. There can be no assurance that we will benefit from any PTE or favorable adjustment to the term of any of our patents.

Regulation outside of the United States

In addition to regulations in the United States, we are subject to a variety of regulations in other jurisdictions governing clinical studies, commercial sales, and distribution of our products. Most countries outside of the United States require that clinical trial applications be submitted to and approved by the local regulatory authority for each clinical study.

Our first clinical trial of CMP-CPS-001 is being conducted in Australia. The TGA and the National Health and Medical Research Council set the GCP requirements for clinical research in Australia, and compliance with these codes is mandatory. Australia has also adopted international codes, such as those promulgated by the International Council for Harmonization of Technical Requirements for Registration of Pharmaceuticals for Human Use, or the ICH. The ICH guidelines must be complied with across all fields of clinical research, including those related to pharmaceutical quality, nonclinical and clinical data requirements and trial designs. The basic requirements for preclinical data to support a first-in-human trial under ICH guidelines are applicable in Australia. Requirements related to adverse event reporting in Australia are similar to those required in other major jurisdictions.

Clinical trials conducted using “unapproved therapeutic goods” in Australia (those which have not yet been evaluated by the TGA for quality, safety and efficacy), must occur pursuant to either the Clinical Trial Notification Scheme, or the CTN Scheme, or the Clinical Trial Exemption Scheme, or the CTX Scheme. In each case, the trial is supervised by a Human Research Ethics Committee, or HREC, an independent review committee set up under guidelines of the Australian National Health and Medical Research Council that ensures the protection of rights, safety and well- being of human subjects involved in a clinical trial. A HREC does this by reviewing, approving and providing continuing examination of trial protocols and amendments, and of the methods and material to be used in obtaining and documenting informed consent of the trial subjects.

In the European Union an application must be submitted to the national competent authority and an independent ethics committee in each country in which we intend to conduct clinical trials, much like the FDA and IRB, respectively. Under the new Clinical Trials Regulation (EU) No 536/2014, which replaced the Clinical Trials Directive 2001/20/EC on January 31, 2022, a single application is now made through the Clinical Trials Information System, or CTIS, for clinical trial authorization in up to 30 EU/EEA countries at the same time and with a single set of documentation.

The assessment of applications for clinical trials is divided into two parts (Part I contains scientific and medicinal product documentation and Part II contains the national and patient-level documentation). Part I is assessed by a coordinated review by the competent authorities of all European Union Member States in which an application for authorization of a clinical trial has been submitted (Member States concerned) of a draft report prepared by a Reference Member State. Part II is assessed separately by each Member State concerned. The role of the relevant ethics committees in the assessment procedure will continue to be governed by the national law of the Member State concerned, however overall related timelines are defined by the Clinical Trials Regulation. The new Clinical Trials Regulation also provides for simplified reporting procedures for clinical trial sponsors.

In addition, whether or not we obtain FDA approval for a product, we must obtain approval of a product by the comparable regulatory authorities of countries outside the United States before we can commence marketing of the product in those countries. The approval process and requirements vary from country to country, so the number and type of nonclinical, clinical, and manufacturing studies needed may differ, and the time may be longer or shorter than that required for FDA approval.

To obtain regulatory approval of our medicinal products under the European Union regulatory system, we are required to submit a marketing authorization application, or MAA, to be assessed in the centralized procedure. The centralized procedure allows applicants to obtain a marketing authorization, or MA, that is valid throughout the European Union, and the additional Member States of the European Economic Area (Iceland, Liechtenstein and Norway), or EEA. It is compulsory for medicinal products manufactured using biotechnological processes, orphan medicinal products, advanced therapy medicinal products (gene-therapy, somatic cell-therapy or tissue-engineered medicines) and human products containing a new active substance which is not authorized in the European Union and which is intended for the treatment of HIV, AIDS, cancer, neurodegenerative disorders, auto-immune and other immune dysfunctions, viral diseases or diabetes. The centralized procedure is optional for any other products containing new active substances not authorized in the European Union or for products which constitute a significant therapeutic, scientific, or technical innovation or for which a centralized authorization is in the interests of patients at European Union level. When a company wishes to place on the market a medicinal product that is eligible for the centralized procedure, it sends an application directly to the EMA, to be assessed by the Committee for Medicinal Products for Human Use, or CHMP. The CHMP is responsible for conducting the assessment of whether a medicine meets the required quality, safety, and efficacy requirements, and whether the product has a positive risk/benefit profile. The procedure results in a European Commission decision, which is valid in all European Union Member States. The centralized procedure is as follows: full copies of the MAA are sent to a rapporteur and a co-rapporteur designated by the competent EMA scientific committee. They coordinate the EMA's scientific assessment of the medicinal product and prepare draft reports. Once the draft reports are prepared (other experts might be called upon for this purpose), they are sent to the CHMP, whose comments or objections are communicated to the applicant. The rapporteur is therefore the privileged interlocutor of the applicant and continues to play this role, even after the MA has been granted.

The rapporteur and co-rapporteur then assess the applicant's replies, submit them for discussion to the CHMP, and taking into account the conclusions of this debate, prepare a final assessment report. Once the evaluation is completed, the CHMP gives a favorable or unfavorable opinion as to whether to grant the authorization. When the opinion is favorable, it shall include the draft summary of product characteristics, or SmPC, the package leaflet, and the texts proposed for the various packaging materials. The time limit for the evaluation procedure is 210 days (excluding clock stops, when additional written or oral information is to be provided by the applicant in response to questions asked by the CHMP). The EMA then has fifteen days to forward its opinion to the European Commission, which will make a binding decision on the grant of an MA within 67 days of the receipt of the CHMP opinion.

There are two other procedures in the European Union for the grant of an MA in multiple European Union Member States. The decentralized procedure provides for approval by one or more other, or Concerned Member States, of an assessment of an application performed by one Member State, known as the Reference Member State. Under this procedure, an applicant submits an application, or dossier, and related materials including a draft SmPC, and draft labeling and package leaflet, to the Reference Member State and Concerned Member States. The Reference Member State prepares a draft assessment and drafts of the related materials within 120 days after receipt of a valid application. Within 90 days of receiving the Reference Member State's assessment report, each Concerned Member State must decide whether to approve the assessment report and related materials. If a Member State cannot approve the assessment report and related materials on the grounds of potential serious risk to the public health, the disputed points may eventually be referred to the European Commission, whose decision is binding on all Member States. Where a product has already been authorized for marketing in a European Union Member State, this national MA can be recognized in other Member States through the mutual recognition procedure.

The criteria for designating an "orphan medicinal product" in the European Union are similar in principle to those in the United States. Under Article 3 of Regulation (EC) 141/2000, a medicinal product may be designated as an orphan medicinal product if it is intended for the diagnosis, prevention, or treatment of a life-threatening or chronically debilitating condition that affects no more than five in 10,000 persons in the European Union when the application is made. In addition, orphan designation can be granted if the product is intended for a life threatening, seriously debilitating, or serious and chronic condition in the European Union and, without incentives,

it is unlikely that sales of the product in the European Union would be sufficient to justify the necessary investment in its development. Orphan designation is only available if there is no other satisfactory method approved in the European Union of diagnosing, preventing, or treating the applicable orphan condition, or if such a method exists, the proposed orphan medicinal product will be of significant benefit to patients affected by such condition, as defined in Regulation (EC) 847/2000.

Orphan designation provides opportunities for fee reductions, protocol assistance, and access to the centralized procedure. Fee reductions are limited to the first year after an MA, except for small and medium enterprises. In addition, if a product which has an orphan designation subsequently receives a centralized MA for the indication for which it has such designation, the product is entitled to orphan market exclusivity, which means the EMA may not approve any other application to market a similar medicinal product for the same indication for a period of ten years. A "similar medicinal product" is defined as a medicinal product containing a similar active substance or substances as contained in an authorized orphan medicinal product, and which is intended for the same therapeutic indication. The exclusivity period may be reduced to six years if, at the end of the fifth year, it is shown that the designation criteria are no longer met, including where it is shown that the product is sufficiently profitable not to justify maintenance of market exclusivity. Additionally, an MA may be granted to a similar medicinal product for the same indication at any time if:

- the second applicant can establish that its product, although similar to the authorized product, is safer, more effective or otherwise clinically superior;
- the MA holder of the authorized product consents to a second orphan medicinal product application; or
- the MA holder of the authorized product cannot supply enough orphan medicinal product.

A pediatric investigation plan, or PIP, in the European Union is aimed at ensuring that the necessary data are obtained to support the authorization of a medicine for children, through studies in children. All applications for MAs for new medicines have to include the results of studies as described in an agreed PIP, unless the medicine is exempt because of a deferral or waiver. This requirement also applies when an MA holder wants to add a new indication, pharmaceutical form, or route of administration for a medicine that is already authorized and covered by intellectual property rights. Several rewards and incentives for the development of pediatric medicines for children are available in the European Union. Medicines authorized across the European Union with the results of studies from a PIP included in the product information are eligible for an extension of their supplementary protection certificate, or SPC, by six months (provided an application for such extension is made at the same time as filing the SPC application for the product, or at any point up to two years before the SPC expires). This is the case even when the studies' results are negative. For orphan medicinal products, the incentive is an additional two years of market exclusivity. Scientific advice and protocol assistance at the EMA are free of charge for questions relating to the development of pediatric medicines. Medicines developed specifically for children that are already authorized but are not protected by a patent or supplementary protection certificate are eligible for a pediatric-use MA, or PUMA. If a PUMA is granted, the product will benefit from ten years of market protection as an incentive.

In March 2016, the EMA launched an initiative, the PRIority Medicines, or PRIME, scheme, to facilitate development of product candidates in indications, often rare, for which few or no therapies currently exist. The PRIME scheme is intended to encourage development of products in areas of unmet medical need and provides accelerated assessment of products representing substantial innovation reviewed under the centralized procedure. Products from small- and medium-sized enterprises may qualify for earlier entry into the PRIME scheme than larger companies on the basis of compelling non-clinical data and tolerability data from initial clinical trials. Many benefits accrue to sponsors of product candidates with PRIME designation, including but not limited to, early and proactive regulatory dialogue with the EMA, frequent discussions on clinical trial designs and other development program elements, and potentially accelerated MAA assessment once a dossier has been submitted. Importantly, once a candidate medicine has been selected for the PRIME scheme, a dedicated contact and rapporteur from the CHMP or from the Committee for Advanced Therapies, or CAT, are appointed early in the PRIME scheme facilitating increased understanding of the product at EMA's committee level. An initial meeting

with the CHMP/CAT rapporteur initiates these relationships and includes a team of multidisciplinary experts at the EMA to provide guidance on the overall development and regulatory strategies. PRIME eligibility does not change the standards for product approval, and there is no assurance that any such designation or eligibility will result in expedited review or approval.

The aforementioned European Union rules are generally applicable in the EEA. The United Kingdom left the European Union on January 31, 2020, and the United Kingdom and the European Union have concluded a trade and cooperation agreement, or TCA, which was provisionally applicable since January 1, 2021 and has been formally applicable since May 1, 2021.

The TCA includes specific provisions concerning pharmaceuticals, which include the mutual recognition of GMP, inspections of manufacturing facilities for medicinal products and GMP documents issued, but does not provide for wholesale mutual recognition of United Kingdom and European Union pharmaceutical regulations. At present, Great Britain has implemented European Union legislation on the marketing, promotion and sale of medicinal products through the Human Medicines Regulations 2012 (as amended). Except in respect of the new European Union Clinical Trials Regulation, the regulatory regime in Great Britain therefore largely aligns with current European Union medicines regulations, however it is possible that these regimes will diverge more significantly in future now that Great Britain's regulatory system is independent from the European Union and the TCA does not provide for mutual recognition of United Kingdom and European Union pharmaceutical legislation. However, notwithstanding that there is no wholesale recognition of European Union pharmaceutical legislation under the TCA, under a new framework mentioned below which will be put in place by the Medicines and Healthcare products Regulatory Agency, or MHRA, the United Kingdom's medicines regulator, from January 1, 2024, the MHRA has stated that it will take into account decisions on the approval of MAs from the EMA (and certain other regulators) when considering an application for a Great Britain MA.

On February 27, 2023, the United Kingdom government and the European Commission announced a political agreement in principle to replace the Northern Ireland Protocol with a new set of arrangements, known as the "Windsor Framework". This new framework fundamentally changes the existing system under the Northern Ireland Protocol, including with respect to the regulation of medicinal products in the United Kingdom. In particular, the MHRA will be responsible for approving all medicinal products destined for the United Kingdom market (i.e., Great Britain and Northern Ireland), and the EMA will no longer have any role in approving medicinal products destined for Northern Ireland. A single United Kingdom-wide MA will be granted by the MHRA for all medicinal products to be sold in the United Kingdom, enabling products to be sold in a single pack and under a single authorization throughout the United Kingdom. The Windsor Framework was approved by the European Union-United Kingdom Joint Committee on March 24, 2023, so the United Kingdom government and the European Union will enact legislative measures to bring it into law.

The MHRA has introduced changes to national licensing procedures, including procedures to prioritize access to new medicines that will benefit patients, an accelerated assessment procedure and new routes of evaluation for novel products and biotechnological products. All existing European Union MAs for centrally authorized products were automatically converted (grandfathered) into United Kingdom MAs free of charge on January 1, 2021. For a period of three years from January 1, 2021, the MHRA may rely on a decision taken by the European Commission on the approval of a new MA in the centralized procedure, in order to more quickly grant a new Great Britain MA. A separate application will, however, still be required. On January 24, 2023, the MHRA announced that a new international recognition framework will be put in place from January 1, 2024, which will have regard to decisions on the approval of MAs made by the EMA and certain other regulators when determining an application for a new Great Britain MA.

There is now no pre-MA orphan designation in Great Britain. Instead, the MHRA reviews applications for orphan designation in parallel to the corresponding MAA. The criteria are essentially the same, but have been tailored for the Great Britain market, i.e., the prevalence of the condition in Great Britain (rather than the European Union) must not be more than five in 10,000. Should an orphan designation be granted, the period or market exclusivity will be set from the date of first approval of the product in Great Britain or the European Union, wherever is earliest.

Government regulation in Australia

Our Phase 1 clinical trial for CMP-CPS-001 is being conducted in Australia. The Therapeutic Goods Administration (TGA) and the National Health and Medical Research Council (NHMRC) set the GCP requirements for clinical research in Australia.

Compliance with the regulations, standards and codes set by the TGA and NHMRC is mandatory. Under the *Therapeutic Goods Act 1989* (Cth) and the *Therapeutic Goods Regulations 1990* (Cth), it is a condition (amongst other conditions) of all clinical trials involving investigational medicinal products to comply with the National Statement on Ethical Conduct in Research Involving Humans, published by the NHMRC (the National Statement), and the Guideline for Good Clinical Practice published by the International Council for Harmonization of Technical Requirements for Registration of Pharmaceuticals for Human Use (ICH Guidelines). The ICH Guidelines have been adopted in Australia, and must be complied with across all fields of clinical research involving therapeutic goods, including those related to pharmaceutical quality, nonclinical and clinical data requirements and trial designs. The basic requirements for preclinical data to support a first-in-human trial under ICH Guidelines are applicable in Australia. Requirements related to adverse event reporting in Australia are generally similar to those required in other major jurisdictions, although reporting timeframes may differ to other jurisdictions.

Clinical trials conducted using “unapproved therapeutic goods” in Australia, being those which have not yet been evaluated by the TGA for quality, safety and efficacy (and including unapproved indications of therapeutic goods which have otherwise been approved for use in Australia) must occur pursuant to either the Clinical Trial Notification Scheme (CTN Scheme) or the Clinical Trial Approval Scheme (CTA Scheme). In each case, the trial is supervised by a Human Research Ethics Committee (HREC), an independent review committee constituted in accordance with the National Statement that ensures the protection of rights, safety and well-being of human subjects involved in a clinical trial. A HREC reviews, approves and provides continuing oversight of trial protocols (including any amendments), methods and materials intended to be used in obtaining and documenting informed consent of the clinical trial subjects.

The CTN Scheme broadly involves:

- submission to a HREC, of all material relating to the proposed clinical trial, including the trial protocol;
- the HREC reviews the scientific validity of the trial design, the balance of risk versus harm of the therapeutic good, the ethical acceptability of the trial process, and approves the trial protocol. The HREC is also responsible for monitoring the conduct of the trial;
- the institution or organization at which the trial will be conducted, referred to as the “Approving Authority”, giving final approval for the conduct of the trial at the site, in terms no less restrictive to those advised by the HREC; and
- the investigator submitting a ‘Notification of Intent to Conduct a Clinical Trial’ form (CTN Form) to the TGA. The CTN form must be signed by the sponsor, the principal investigator, the chairman of the HREC and a person responsible from the Approving Authority. The TGA does not review any data relating to the clinical trial however CTN trials cannot commence until the trial has been notified to the TGA.

Under the CTA Scheme:

- a sponsor submits an application to conduct a clinical trial to the TGA for evaluation and comment;
- a sponsor must forward any comments made by the TGA Delegate to the HREC(s) at the sites where the trial will be conducted;
- the HREC is responsible for considering the scientific and ethical issues of the proposed trial protocol.

A sponsor cannot commence a trial under the CTA Scheme until written advice has been received from the TGA regarding the application and approval for the conduct of the trial has been obtained from an ethics committee and the institution at which the trial will be conducted.

Approval for inclusion in the Australian Register of Therapeutic Goods (ARTG), is required before a therapeutic good (including pharmaceutical product) may be marketed (or supplied, imported, exported or manufactured) in Australia. Exceptions apply to therapeutic goods/pharmaceutical products that are supplied, imported, and exported to and from Australia for the purposes of a clinical trial, on the basis that certain conditions are met (e.g., the trial is conducted in accordance with the CTN or CTA scheme).

Once a sponsor decides to register a therapeutic good/pharmaceutical product in Australia, in order to obtain registration of the product on the ARTG, it is required that (amongst others):

- the sponsor submits appropriate documentation, including the outcomes of clinical trials and studies, to allow the TGA to assess the quality, safety and efficacy of the therapeutic product/pharmaceutical product; and
- the sponsor submits evidence which demonstrates that the manufacture of the therapeutic product/pharmaceutical product complies with the applicable GMP requirements.

The TGA has the ultimate discretion to decide whether to include the therapeutic product/pharmaceutical product in the ARTG.

Healthcare laws and regulations in the United States

Sales of our product candidate, if approved, or any other future product candidate, will be subject to healthcare regulation and enforcement by the federal government and the states and foreign governments in which we might conduct our business. The healthcare laws and regulations that may affect our ability to operate include the following:

- The federal Anti-Kickback Statute, or AKS, which makes it illegal for any person or entity to knowingly and willfully, directly or indirectly, solicit, receive, offer, or pay any remuneration that is in exchange for or to induce the referral of business, including the purchase, order, lease of any good, facility, item or service for which payment may be made, in whole or in part, under a federal healthcare program, such as Medicare or Medicaid. The term “remuneration” has been broadly interpreted to include anything of value. Liability may be established without proving actual knowledge of the statute or specific intent to violate it;
- Federal false claims, and false statement laws, including the federal civil False Claims Act, or FCA, which prohibits, among other things, any person or entity from knowingly presenting, or causing to be presented, for payment to, or approval by, federal programs, including Medicare and Medicaid, claims for items or services, including drugs and biologics, that are false or fraudulent. Manufacturers can be held liable under the FCA even when they do not submit claims directly to government payors if they are deemed to “cause” the submission of false or fraudulent claims; the FCA also permits a private individual acting as whistleblower to bring actions on behalf of the federal government alleging violations of the FCA and to share in any monetary recovery;
- The Civil Monetary Penalties Law, which covers a variety of conduct, often violations under other laws, and includes penalties for violating the AKS violations, causing the submission of false claims, and offering or transfer of remuneration to a Medicare or state healthcare program beneficiary if the person knows or should know it is likely to influence the beneficiary’s selection of a particular provider, practitioner, or supplier of services reimbursable by Medicare or a state healthcare program;
- The Health Insurance Portability and Accountability Act of 1996, or HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act of 2009 and their implementing regulations, imposes criminal and civil liability for knowingly and willfully executing, or attempting to execute, a scheme to defraud any healthcare benefit program, including private third-party payors or making any false, fictitious or fraudulent statement in connection with the delivery of or payment for healthcare benefits, items or services;
- HIPAA also imposes obligations related to the privacy, security, and transmission of individually identifiable health information that apply to many healthcare providers, physicians, and third-party payors with whom we interact;

- Federal consumer protection and unfair competition laws broadly regulate marketplace activities and activities that potentially harm consumers;
- Federal government price reporting laws, which require pharmaceutical manufacturers to report certain calculated product prices to the government or provide certain discounts or rebates to government authorities or private entities, often as a condition of reimbursement under governmental healthcare programs;
- The federal Physician Payments Sunshine Act which requires certain manufacturers of drugs, devices, biologics and medical supplies for which payment is available under Medicare, Medicaid or the Children's Health Insurance Program, with specific exceptions, to report annually to the Centers for Medicare & Medicaid Services, or CMS, information related to payments or other transfers of value made to "physicians" (which has the same meaning as under Section 1861(r) of the Social Security Act, which generally includes doctors of medicine, osteopathy, dentists, podiatrists, optometrists and chiropractors who are legally authorized to practice by a state) and teaching hospitals, as well as ownership and investment interests held by physicians and their immediate family members. Beginning in 2022, applicable manufacturers are also required to report such information regarding payments and transfers of value provided during the previous year to physician assistants, nurse practitioners, clinical nurse specialists, anesthesiologist assistants, certified nurse anesthetists and certified nurse-midwives; and
- The Foreign Corrupt Practices Act, or FCPA, which prohibits U.S. businesses and their representatives from offering to pay, paying, promising to pay or authorizing the payment of money or anything of value to a foreign official in order to influence any act or decision of the foreign official in his or her official capacity or to secure any other improper advantage in order to obtain or retain business. The scope of the FCPA includes interactions with certain healthcare professionals in many countries.

Many states have similar laws and regulations, such as anti-kickback and false claims laws, that may be broader in scope and may apply regardless of payor, in addition to items and services reimbursed under Medicaid and other state programs. Additionally, we may be subject to state laws that require pharmaceutical companies to comply with the federal government's and/or pharmaceutical industry's voluntary compliance guidelines, state laws that require drug and biologics manufacturers to report information related to payments and other transfers of value to physicians and other healthcare providers or marketing expenditures, as well as state and foreign laws governing the privacy and security of health information, many of which differ from each other in significant ways and often are not preempted by HIPAA. The scope and enforcement of each of these laws is uncertain and subject to rapid change in the current environment of healthcare reform. Additionally, to the extent that our product is sold in a foreign country, we may be subject to similar foreign laws.

Because of the breadth of these laws and the narrowness of the statutory exceptions and safe harbors available, it is possible that some of our business activities could be subject to challenge under one or more of such laws in the future. If our operations are found to be in violation of any of such laws or any other governmental regulations that apply to us, we may be subject to, on a corporate or individual basis, penalties, including civil and criminal penalties, damages, fines, the curtailment or restructuring of our operations, the exclusion from participation in federal and state healthcare programs and even imprisonment, any of which could materially adversely affect our ability to operate our business and our financial results. In addition, the cost of implementing sufficient systems, controls, and processes to ensure compliance with all of the aforementioned laws could be significant. Any action for violation of these laws, even if successfully defended, could cause us to incur significant legal expenses and divert management's attention from the operation of the company's business. If any of the physicians or other healthcare providers or entities with whom we expect to do business is found not to be in compliance with applicable laws, that person or entity may be subject to criminal, civil or administrative sanctions, including exclusions from government funded healthcare programs.

It is not always possible to identify and deter employee misconduct, and the precautions we take to detect and prevent inappropriate conduct may not be effective in controlling unknown or unmanaged risks or losses or in protecting us from governmental investigations or other actions or lawsuits stemming from a failure to be in

compliance with such laws or regulations. Efforts to ensure that our business arrangements will comply with applicable healthcare laws may involve substantial costs. It is possible that governmental and enforcement authorities will conclude that our business practices may not comply with current or future statutes, regulations or case law interpreting applicable fraud and abuse or other healthcare laws and regulations. If any such actions are instituted against us and we are not successful in defending ourselves or asserting our rights those actions, our business may be impaired.

Pharmaceutical coverage, pricing and reimbursement

Significant uncertainty exists as to the coverage and reimbursement status of any product candidates for which we obtain regulatory approval. In the United States and foreign markets, sales of any products for which we receive regulatory approval for commercial sale will depend, in part, on the extent to which third-party payors provide coverage, and establish adequate reimbursement levels for such drug products. In the United States, third-party payors include federal and state healthcare programs, private managed care organizations, private health insurers and other organizations.

In the United States, no uniform policy of coverage and reimbursement for products exists among third-party payors. One payor's determination to provide coverage for a drug product does not assure that other payors will also provide coverage for the drug product. Third-party payors are increasingly challenging the price, examining the medical necessity and reviewing the cost-effectiveness of medical drug products and medical services, in addition to questioning their safety and efficacy. As a result, obtaining coverage and reimbursement approval of a product from these payors can be a time-consuming and costly process that could require us to provide each payor supporting scientific, supporting scientific, clinical and cost-effectiveness data for the use of our products on a payor-by-payor basis, with no assurance that coverage and adequate reimbursement will be obtained. We may need to conduct expensive pharmaco-economic studies in order to demonstrate the medical necessity and cost-effectiveness of our products, in addition to the costs required to obtain the FDA approvals. Nonetheless, our product candidates may not be considered medically necessary or cost-effective. In the United States, the principal decisions about reimbursement for new medicines are typically made by CMS. CMS decides whether and to what extent a new medicine will be covered and reimbursed under Medicare and private payors tend to follow CMS to a substantial degree. Such payors may limit coverage to specific drug products on an approved list, also known as a formulary, which might not include all of the FDA-approved drugs for a particular indication.

Further, the process for determining whether a third-party payor will provide coverage for a drug product may be separate from the process for setting the price of a drug product or for establishing the reimbursement rate that such a payor will pay for the drug product. A payor's decision to provide coverage for a drug product does not imply that an adequate reimbursement rate will be approved. Even if we obtain coverage for a given product, the resulting reimbursement payment rates might not be adequate for us to achieve or sustain profitability or may require co-payments that patients find unacceptably high. There is significant uncertainty related to insurance coverage and reimbursement of newly approved products. It is difficult to predict at this time what third-party payors will decide with respect to the coverage and reimbursement for our product candidates.

Net prices for drugs may be reduced by mandatory discounts or rebates required by government healthcare programs or private payors and impacted by any future relaxation of laws that presently restrict imports of drugs from countries where they may be sold at lower prices than in the United States. In addition, many pharmaceutical manufacturers must calculate and report certain price reporting metrics to the government, such as average sales price and best price. Penalties may apply in some cases when such metrics are not submitted accurately and timely.

The marketability of any product candidates for which we receive regulatory approval for commercial sale may suffer if third-party payors fail to provide adequate coverage and reimbursement. In addition, emphasis on managed care in the United States has increased and could increase the pressure on pharmaceutical pricing. Coverage policies and third-party reimbursement rates may change at any time. Even if favorable coverage and reimbursement status is attained for one or more products for which we receive regulatory approval, less favorable coverage policies and reimbursement rates may be implemented in the future.

Healthcare reform

The United States and many foreign jurisdictions have enacted or proposed legislative and regulatory changes affecting the healthcare system. The United States government, state legislatures and foreign governments also have shown significant interest in implementing cost-containment programs to limit the growth of government-paid healthcare costs, including price controls, restrictions on reimbursement, and requirements for substitution of generic products for branded prescription drugs and biologics. In recent years, Congress has considered reductions in Medicare reimbursement levels for drugs and biologics administered by physicians. CMS, the agency that administers the Medicare and Medicaid programs, also has authority to revise reimbursement rates and to implement coverage restrictions for some drugs and biologics. Cost reduction initiatives and changes in coverage implemented through legislation or regulation could decrease utilization of and reimbursement for any approved products. While Medicare regulations apply only to drug benefits for Medicare beneficiaries, private payors often follow Medicare coverage policy and payment limitations in setting their own reimbursement rates. Therefore, any reduction in reimbursement that results from federal legislation or regulation may result in a similar reduction in payments from private payors.

By way of example, the United States and state governments continue to propose and pass legislation designed to reduce the cost of healthcare. In March 2010, the U.S. Congress enacted the Patient Protection and Affordable Care Act and the Health Care and Education Reconciliation Act, or the Affordable Care Act, which, among other things, includes changes to the coverage and payments for products under government healthcare programs. profitability of drug products through increased rebates for drugs reimbursed by Medicaid programs, extension of Medicaid rebates to Medicaid managed care plans, mandatory discounts for certain Medicare Part D beneficiaries and, annual fees based on pharmaceutical companies' share of sales to federal health care programs. Current laws, as well as other healthcare reform measures that may be adopted in the future, may result in more rigorous coverage criteria and in additional downward pressure on the price for any approved products. Since the enactment of the Affordable Care Act, there have been, and continue to be, numerous judicial, administrative, executive, and legislative challenges to certain aspects of the Affordable Care Act, and there could be additional amendments to the Affordable Care Act in the future. It is unclear whether the Affordable Care Act will be overturned, repealed, replaced, or further amended. We cannot predict what affect further changes to the Affordable Care Act would have on our business.

Additionally, there have been several U.S. congressional inquiries and proposed federal and proposed and enacted state legislation designed to, among other things, bring more transparency to drug pricing, review the relationship between pricing and manufacturer patient programs, reduce the costs of drugs under Medicare and reform government program reimbursement methodologies for drug products. For example, on August 16, 2022, the Inflation Reduction Act of 2022, or the IRA, was signed into law by President Biden. The IRA includes several provisions that may impact the pharmaceutical industry to varying degrees, including provisions that create a $2,000 out-of-pocket cap for Medicare Part D beneficiaries, impose new manufacturer financial liability on all drugs in Medicare Part D, allow the U.S. government to negotiate Medicare Part B and Part D pricing for certain high-cost drugs and biologics without generic or biosimilar competition, require companies to pay rebates to Medicare for drug prices that increase faster than inflation and delay the rebate rule that would require pass through of pharmacy benefit manager rebates to beneficiaries. The full impact of IRA on the pharmaceutical and healthcare industry in general is not yet known.

At the state level, legislatures have increasingly passed legislation and implemented regulations designed to control pharmaceutical product pricing, including price or patient reimbursement constraints, discounts, restrictions on certain product access, and marketing cost disclosure and transparency measures, and in some cases, designed to encourage importation from other countries and bulk purchasing.

We expect that additional federal, state, and foreign healthcare reform measures will be adopted in the future, any of which could limit the amounts that federal and state governments will pay for healthcare products and services, which could result in limited coverage and reimbursement and reduced demand for our products, once approved, or additional pricing pressures.

Employees and human capital resources

As of June 1, 2024, we had 58 employees, all of whom were full-time and 36 of whom were engaged in research and development activities. Twenty of our employees hold PhD or MD degrees. All laboratory personnel and our administrative team are based in and around Boston, Massachusetts. None of our employees are represented by a labor union or covered under a collective bargaining agreement. We consider our relationship with our employees to be good.

Our human capital resources objectives include, as applicable, identifying, recruiting, retaining, incentivizing and integrating our existing and new employees, advisors and consultants. The principal purposes of our equity and cash incentive plans are to attract, retain and reward personnel through the granting of stock-based and cash-based compensation awards, in order to increase stockholder value and the success of our company by motivating such individuals to perform to the best of their abilities and achieve our objectives.

Facilities

We currently lease approximately 30,000 square feet of office space and laboratory space in Cambridge, Massachusetts and approximately 5,300 square feet of office and laboratory space in Boulder, Colorado. We believe these facilities will be adequate for the foreseeable future and that suitable additional or substitute space will be available as and when needed.

Legal proceedings

From time to time, we may be subject to legal proceedings. We are not currently a party to or aware of any proceedings that we believe will have, individually or in the aggregate, a material adverse effect on our business, financial condition or results of operations. Regardless of outcome, litigation can have an adverse impact on us because of defense and settlement costs, diversion of management resources, and other factors.

Management

Executive officers and directors

The following table provides information regarding our current executive officers and directors, including their ages as of July 1, 2024:

Name	Age	Position(s)
Executive officers		
Josh Mandel-Brehm	41	President, Chief Executive Officer and Director
David Bumcrot, PhD	61	Chief Scientific Officer
Kelly Gold	47	Chief Financial Officer
Non-employee directors		
Steven Holtzman[(2)(3)]	70	Chair of the Board of Directors
James Boylan	57	Director
Jorge Conde[(2)(4)]	47	Director
Ingo Chakravarty	56	Director
Michael Higgins[(1)(2)]	62	Director
Amir Nashat, ScD[(3)]	51	Director
Paula Ragan, PhD[(1)]	54	Director
Andy Schwab[(3)]	53	Director
Ravi I. Thadhani, MD, MPH	58	Director
Richard Young, PhD	70	Director

(1) Member of the audit committee.

(2) Member of the compensation committee.

(3) Member of the nominating and corporate governance committee.

(4) Mr. Conde is expected to resign as a director on or prior to the closing of this offering.

Josh Mandel-Brehm has served as our President and Chief Executive Officer and as a member of our board of directors since 2017. Mr. Mandel-Brehm was previously an Entrepreneur Partner of Polaris Partners from 2017 to October 2021. Mr. Mandel-Brehm also previously served as part of the Business Development group at Biogen Inc. from 2013 to 2017, where he led multiple strategic activities and corresponding transactions. Prior to Biogen, Mr. Mandel-Brehm held several roles of increasing responsibility at Genzyme Corporation from 2009 to 2013, including as part of the business development group for the company's rare disease business unit. Mr. Mandel-Brehm has also served as a member of the board of directors of ProMIS Neurosciences, Inc., a clinical-stage biotechnology company focused on generating and developing antibody therapeutics for the treatment of neurodegenerative diseases, since September 2021. Mr. Mandel-Brehm earned a BA in Biology from Washington University in St. Louis and an MBA from the University of Michigan. We believe Mr. Mandel-Brehm's extensive knowledge of, and experience in, the biopharmaceutical industry paired with his business development and executive management expertise qualifies him to serve on our board of directors.

David Bumcrot, PhD has served as our Chief Scientific Officer and Senior Vice President of Research since March 2020. Dr. Bumcrot previously served as our Vice President, Head of Biology from 2017 to March 2019 and Senior Vice President of Biology from March 2019 to March 2020. Prior to CAMP4, Dr. Bumcrot served as Senior Director, Molecular & Cell Biology at Editas Medicine from 2014 to 2017, where his team established the company's initial therapeutic programs utilizing groundbreaking CRISPR technology. Dr. Bumcrot previously served as the Head Research Scientist for the Laboratory for RNA Therapeutics at the Koch Institute for Integrative Cancer Research at the Massachusetts Institute of Technology from 2012 to 2013, and from 2002 to 2012 served at Alnylam Pharmaceutics in positions of increasing responsibility, including most recently as Director of

Research. Dr. Bumcrot earned a BS in Biology from Cornell University, a PhD in Molecular Biology from the University of Pennsylvania and completed a post-doctoral fellowship in the department of Cell & Molecular Biology at Harvard University.

Kelly Gold has served as our Chief Financial Officer since April 2022. Ms. Gold previously served as our Chief Business Officer from April 2021 to March 2022, and held positions of increasing responsibility in Finance and Corporate Development at our company from 2017 to March 2021. Ms. Gold previously served as Associate Director, Strategic Corporate Finance at Biogen Inc. from 2014 to 2017, where she provided financial leadership for the company's late stage and marketed rare disease programs and developed long term strategic financial trajectories for the R&D organization. Ms. Gold also previously served as a Healthcare Investment Banking Associate at Deutsche Bank from 2009 to 2013, where she worked in the healthcare investment banking group. Ms. Gold earned Bachelor's degrees in Life Sciences and Mechanical Engineering from Queen's University in Ontario and an MBA from the MIT Sloan School of Management.

Steven Holtzman has served as Chair of our board of directors and as a Strategic Business Advisor to our company since October 2019. Mr. Holtzman was the first President and Chief Executive Officer and a member of the board of directors of Decibel Therapeutics, Inc., a biotechnology company, from 2016 to January 2020. Prior to Decibel, Mr. Holtzman served as Executive Vice President, Corporate Development of Biogen, Inc. from 2011 to 2016. Prior to Biogen, Mr. Holtzman served as the Chief Executive Officer of Infinity Pharmaceuticals, Inc. from 2001 to 2010. Mr. Holtzman has also served as a member of the board of directors of Molecular Partners AG, a clinical-stage biopharmaceutical company since May 2014. Mr. Holtzman earned a BA in Philosophy from Michigan State University and a BPhil in Philosophy from Corpus Christi College, Oxford University, which he attended as a Rhodes Scholar. We believe Mr. Holtzman's strategic development and industry experience qualifies him to serve on our board of directors.

James Boylan has served as a member of our board of directors since June 2022. Mr. Boylan has served as Chief Executive Officer of Enavate Sciences, a portfolio company of Patient Square Capital, since May 2022. Mr. Boylan previously served as President and Head of Investment Banking of SVB Leerink from 2009 to April 2021. Mr. Boylan has also served as a member of the board of directors of Immunome Inc., a biotechnology company developing targeted cancer therapies, since October 2023 and as a member of the board of Compass Therapeutics, Inc., a clinical stage biopharmaceutical company developing next generation antibodies into cancer therapies, since November 2022. Mr. Boylan earned a BS in Finance from Lehigh University and an MBA in finance from the Columbia Business School. We believe Mr. Boylan's extensive investment and business experience in the life sciences and biotechnology sectors qualifies him to serve on our board of directors.

Jorge Conde has served as a member of our board of directors since 2018 and is expected to resign as a director upon or prior to the closing of this offering. Mr. Conde has served as a General Partner at Andreessen Horowitz since June 2017, where he leads investments at the cross section of biology, computer science and engineering. Mr. Conde previously served as Chief Strategy Officer for Syros Pharmaceuticals, Inc. from 2016 to 2017, and as its Chief Product Officer from 2014 to 2016. Prior to joining Syros, from 2007 to 2014, Mr. Conde served in various roles at Knome, Inc., a genomics company, including as Founding Chief Executive Officer, Chief Financial Officer and Chief Product Officer. Earlier in his career, Mr. Conde served in marketing and operations at MedImmune, LLC, managed the business development function at Helicos Biosciences Corporation, a DNA sequencing company, and worked as a biotechnology investment banker at Morgan Stanley. Mr. Conde holds a B.A. in Biology from Johns Hopkins University, an M.S. from the Harvard-MIT Division of Health Sciences and Technology, and an M.B.A. from Harvard Business School. We believe Mr. Conde's extensive investment and life sciences industry experience qualifies him to serve on our board of directors.

Ingo Chakravarty has served as a member of our board of directors since April 2024. Mr. Chakravarty is an Operating Partner for Northpond Ventures, a venture capital firm. Previously, Mr. Chakravarty was President and Chief Executive Officer of Mesa Biotech, Inc., or Mesa Biotech, a point-of-care molecular diagnostic company, from April 2020 to February 2021, when it was acquired by Thermo Fisher Scientific Inc.; thereafter, Mr. Chakvaraty continued to serve as General Manager of Mesa Bioetch until March 2023. Prior to Mesa Biotech, Mr. Chakravarty was Chief Executive Officer of Navican Genomics, Inc., a precision care company, from 2016 to November 2019.

Mr. Chakravarty has a degree in Electrical Engineering from the Friedrich Heacker School in Germany. We believe Mr. Chakravarty's significant leadership experience in the life sciences and biotechnology industry qualifies him to serve on our board of directors.

Michael Higgins has served as a member of our board of directors since 2017. Mr. Higgins is a serial entrepreneur who has helped launch and build numerous companies during his career. He served as Entrepreneur-in-Residence at Polaris Partners, an investment company, from 2015 to 2020. From 2003 to 2014 he served as Senior Vice President, Chief Operating Officer at Ironwood Pharmaceuticals Inc, a biopharmaceutical company. Prior to 2003, Mr. Higgins held a variety of senior business positions at Genzyme Corporation, including Vice President of Corporate Finance and Vice President of Business Development. Mr. Higgins has served as a member of the board of directors of Voyager Therapeutics, Inc., or Voyager, since 2015. He was appointed Chair of the board of directors of Voyager in June 2019 and also served as the interim president and chief executive officer of Voyager from June 2021 to March 2022. Mr. Higgins has also served as a member of the board of directors of Cyclerion Therapeutics, Inc., a biopharmaceutical company, since November 2023 and as chair of the board of directors of Pulmatrix, Inc., a biopharmaceutical company, since April 2020. Mr. Higgins previously served as a member of the board of directors of Genocea Biosciences Inc., an immuno-oncology company, from 2015 to May 2022. Mr. Higgins earned a BS from Cornell University and an MBA from the Amos Tuck School of Business at Dartmouth College. We believe that Mr. Higgins' financial and business expertise, including his diversified background as an executive officer in public pharmaceuticals companies and service on the boards of directors of other life sciences companies, qualifies him to serve as a member of the board of directors.

Amir Nashat, ScD has served as a member of our board of directors since 2015. Mr. Nashat is an executive partner at Polaris Partners, a venture capital firm, where he has worked since 2002. Mr. Nashat currently represents Polaris on the board of directors of Morphic Holding, Inc., a biopharmaceutical company developing small molecule integrin therapeutics, where he has served since 2017. Mr. Nashat previously served as a member of the boards of directors of Scholar Rock Holding Corporation from 2012 to June 2024, of Fate Therapeutics, Inc., from 2007 to May 2020, of Selecta Biosciences, Inc., from 2008 to April 2020, and of Syros Pharmaceuticals, Inc., from 2016 to September 2022. Prior to joining Polaris, Mr. Nashat completed his ScD as a Hertz Fellow in Chemical Engineering at the Massachusetts Institute of Technology with a minor in Biology. Mr. Nashat also earned both his MS and BS in Materials Science and Mechanical Engineering at the University of California, Berkeley. We believe Dr. Nashat's extensive biotechnology investment experience qualifies him to serve on our board of directors.

Paula Ragan, PhD has served as a member of our board of directors since May 2019. Dr. Ragan has served as chief executive officer & president of X4 Pharmaceuticals, Inc., or X4, a commercial-stage clinical biopharmaceutical company, since 2014. Dr. Ragan previously served as consulting chief business officer of Lysosomal Therapeutics Inc. from to 2013 to 2014. Prior to LTI, Dr. Ragan served as senior director at Genzyme, where she led strategic partnering efforts for Genzyme's Rare Disease Business and headed the supply chain planning for Genzyme's flagship commercial products, from 2007 to 2012. Dr. Ragan has also served as a member of the board of directors of X4 since 2014. Dr. Ragan earned a BS in Mechanical Engineering from Tufts University, an MS in Biomedical Engineering from Boston University, and a PhD from the Massachusetts Institute of Technology. We believe Dr. Ragan's industry-specific business expertise and experience as a public company chief executive qualifies her to serve on our board of directors.

Andy Schwab has served as a member of our board of directors since March 2021. Mr. Schwab is a Founding Partner and Managing Partner of 5AM Venture Management, LLC, an investment firm focused on life sciences companies, where he has served since 2002. Mr. Schwab has served as a member of the board of directors of Skye Bioscience, Inc., a biopharmaceutical company focused on the development of cannabinoid derivatives, since August 2023. Mr. Schwab previously served as a member of the boards of directors of Enliven Therapeutics, Inc., a biopharmaceutical company focused on the development of small molecule kinase inhibitors, from January 2022 to June 2023, of Pear Therapeutics, Inc. from 2014 to June 2022 and of 5:01 Acquisition Corp. from September 2020 to October 2022. Mr. Schwab earned a BS with Honors in Genetics and Ethics from Davidson College. We believe Mr. Schwab's extensive experience in management positions and on the boards of several life sciences companies qualifies him to serve on our board of directors.

Ravi I. Thadhani, MD, MPH has served as a member of our board of directors since October 2021. Dr. Thadhani has served as Executive Vice President for Health Affairs of Emory University, Executive Director of Emory's Woodruff Health Sciences Center, and Vice Chair of the Emory Healthcare Board of Directors since January 2023. Dr. Thadhani previously served as Chief Academic Officer and Dean for Faculty Affairs of Mass General Brigham from November 2019 to December 2022, and as a Professor of Medicine at Harvard Medical School from 2012 to December 2022. Prior to this, Dr. Thadhani served as Vice Dean of Research and Graduate Research Education at Cedars-Sinai Medical Center from 2017 to October 2019. Dr. Thadhani earned a BA in Liberal Arts from the University of Notre Dame, an MPH from the Harvard T.H. Chan School of Public Health and an MD from the University of Pennsylvania School of Medicine. We believe Dr. Thadhani's research expertise and medical background and training qualifies him to serve on our board of directors.

Richard Young, PhD has served as a member of our board of directors since 2016. Dr. Young has served as a Professor of Biology at the Massachusetts Institute of Technology and a member of the Whitehead Institute since 1984. He was elected into the National Academy of Sciences in 2012 and the National Academy of Medicine in 2019. Dr. Young has served as an advisor to the National Institutes of Health and the World Health Organization. Dr. Young has also served as a member of the board of directors of Syros Pharmaceuticals, Inc., a biotechnology company, since 2011, and as a member of the board of directors of Omega Therapeutics, Inc., a biotechnology company, since 2017. Dr. Young earned a BS in Biological Science from Indiana University and a PhD in Molecular Biophysics and Biochemistry from Yale University. We believe Dr. Young's scientific expertise and his role as one of our scientific co-founders qualifies him to serve on our board of directors.

Board composition

Our business and affairs are managed under the direction of our board of directors, which currently consists of 11 members. The primary responsibilities of our board of directors are to provide oversight, strategic guidance, counseling and direction to our management. Our board of directors meets on a regular basis and additionally as required.

Upon the completion of this offering, our board of directors will consist of members, of whom were elected as directors pursuant to the board composition provisions of our Third Amended and Restated Voting Agreement, or the Voting Agreement, among us and our stockholders. The Voting Agreement will terminate upon the completion of this offering, at which point no stockholder will have any special rights regarding the election or designation of the members of our board of directors. Our current directors elected to our board of directors pursuant to the Voting Agreement will continue to serve as directors until a successor is duly elected and qualified, or until his or her earlier resignation or removal.

Our board of directors may establish the authorized number of directors from time to time by resolution. In accordance with our Restated Charter that will be in effect upon the closing of this offering, immediately after this offering, our board of directors will be divided into three classes with staggered three-year terms. At each annual meeting of stockholders, the successors to directors whose terms then expire will be elected to serve from the time of election and qualification until the third annual meeting following election. Our directors will be divided among the three classes as follows:

- Class I, which will consist of , and , and their terms will expire at our first annual meeting of stockholders to be held after the closing of this offering;
- Class II, which will consist of , and , and their terms will expire at our second annual meeting of stockholders to be held after the closing of this offering; and
- Class III, which will consist of , and , and their terms will expire at our third annual meeting of stockholders to be held after the closing of this offering.

Our Restated Bylaws, which will become effective upon the closing of this offering, will provide that the authorized number of directors may be changed only by resolution approved by a majority of our board of directors. Any additional directorships resulting from an increase in the number of directors will be distributed among the three

classes so that, as nearly as possible, each class will consist of one-third of the directors. The division of our board of directors into three classes with staggered three-year terms may delay or prevent a change of our management or a change of control.

Director independence

Under the rules of the Nasdaq Stock Market, or the Nasdaq Listing Rules, independent directors must comprise a majority of a listed company's board of directors within one year of the completion of its initial public offering. In addition, the Nasdaq Listing Rules require that, subject to specified exceptions, each member of a listed company's audit and compensation committees be independent and that director nominees be selected or recommended for the board's selection by independent directors constituting a majority of the independent directors or by a nominating and corporate governance committee comprised solely of independent directors. Under the Nasdaq Listing Rules, a director will only qualify as "independent" if, in the opinion of that company's board of directors, that person does not have a relationship that would interfere with the exercise of independent judgment in carrying out the responsibilities of a director and that such person is "independent" as defined under the Nasdaq Listing Rules and the rules under the Securities Exchange Act of 1934, as amended, or the Exchange Act.

Audit committee members must also satisfy the independence criteria set forth in Rule 10A-3 under the Exchange Act. In order to be considered independent for purposes of Rule 10A-3, a member of an audit committee of a listed company may not, other than in his or her capacity as a member of the audit committee, the board of directors or any other board committee: (1) accept, directly or indirectly, any consulting, advisory or other compensatory fee from the listed company or any of its subsidiaries or (2) be an affiliated person of the listed company or any of its subsidiaries.

Based upon information requested from and provided by each director concerning his or her background, employment and affiliations, including family relationships, our board of directors has determined that each of our directors, with the exception of , is an "independent director" as defined under the Nasdaq Listing Rules, including, in the case of all the members of our audit committee, the independence criteria set forth in Rule 10A-3 under the Exchange Act, and in the case of all the members of our compensation committee, the independence criteria set forth in Rule 10C-1 under the Exchange Act and are "non-employee directors" as defined in Section 16b-3 of the Exchange Act. In making these determinations, our board of directors considered the current and prior relationships that each non-employee director has with our company and all other facts and circumstances our board of directors deemed relevant in determining their independence, including the beneficial ownership of our shares by each non-employee director and the transactions described in the section titled "Certain relationships and related person transactions."

There are no family relationships among any of our directors or executive officers.

Role of the board in risk oversight

Our board of directors has, and, upon the completion of this offering, its committees will also have, an active role in overseeing the management of our risks. Our board of directors is responsible for general oversight of risks and regular review of information regarding our risks, including credit risks, liquidity risks and operational risks. The compensation committee will be responsible for overseeing the management of risks relating to our executive compensation plans and arrangements. The audit committee will be responsible for overseeing the management of risks relating to accounting matters and financial reporting, as well as risks relating to cybersecurity matters. The nominating and governance committee will be responsible for overseeing the management of risks associated with the independence of our board of directors and potential conflicts of interest. Although each committee will be responsible for evaluating certain risks and overseeing the management of such risks, the entire board of directors will be regularly informed through discussions from committee members about such risks.

Board committees

Our board of directors will establish an audit committee, a compensation committee and a nominating and corporate governance committee. The composition and responsibilities of each of the committees of our board of

directors are described below. Members serve on these committees until their resignation or until otherwise determined by our board of directors. Each committee intends to adopt a written charter that satisfies the application rules and regulation of the SEC and the Nasdaq Listing Rules, which we will post to our website at www.camp4tx.com upon the completion of this offering. Our board of directors may establish other committees as it deems necessary or appropriate from time to time. Information contained on, or accessible through, our website is not a part of this prospectus, and the inclusion of our website address in this prospectus is only an inactive textual reference.

Audit committee

Our audit committee will operate under a written charter, to be effective prior to the completion of this offering, that satisfies the applicable Nasdaq Listing Rules.

The audit committee's responsibilities upon completion of this offering will include:

- appointing, approving the compensation of, and evaluating the qualifications, performance, procedures and independence of, our independent registered public accounting firm;
- overseeing the work of our independent registered public accounting firm, including through the receipt and consideration of written periodic reports from such firm;
- pre-approving all audit and permitted non-audit services to be performed by our independent registered public accounting firm;
- reviewing and discussing with management and our independent registered public accounting firm our annual and quarterly financial statements and related disclosures, including earnings releases;
- overseeing and periodically reviewing with our independent registered public accounting firm our compliance with all applicable requirements of the Public Company Accounting Oversight Board;
- reviewing and discussing with management and our independent registered public accounting firm any material issues regarding accounting principles and financial statement presentations and the steps taken to deal with such issues;
- reviewing disclosures about any significant deficiencies or material weaknesses in our internal control structures and procedures, including disclosures in our annual and quarterly reports;
- coordinating our board of directors' oversight of our internal control over financial reporting, disclosure controls and procedures, code of business conduct and ethics, procedures for complaints and legal and regulatory matters;
- reviewing and discussing with management and our independent registered public accounting firm any material issues regarding cybersecurity risks and processes for assessing, identifying and managing material risks from cybersecurity threats;
- discussing our risk management policies with management;
- establishing policies regarding hiring employees from our independent registered public accounting firm and procedures for the receipt and retention of accounting related complaints and concerns;
- meeting independently with our independent registered public accounting firm and management;
- reviewing and approving any related person transactions;
- overseeing our guidelines and policies governing risk assessment and risk management;
- overseeing and periodically reviewing the integrity of our information technology systems, process and data;
- preparing the audit committee report required by SEC rules;
- reviewing and assessing, at least annually, the adequacy of the audit committee's charter; and

- performing, at least annually, an evaluation of the performance of the audit committee.

All audit services and all non-audit services, other than de minimis non-audit services, to be provided to us by our independent registered public accounting firm must be approved in advance by our audit committee.

The members of our audit committee are , and . chairs the audit committee. Our board of directors has determined that each member of our audit committee has sufficient knowledge in financial and auditing matters to serve on the audit committee. Our board of directors has also determined that is an "audit committee financial expert," as defined under Item 407 of Regulation S-K.

We expect to satisfy the member independence requirements for the audit committee prior to the end of the transition period provided under the Nasdaq Listing Rules and SEC rules and regulations for companies completing their initial public offering.

Compensation committee

Our compensation committee will operate under a written charter, to be effective prior to the completion of this offering, that satisfies the applicable Nasdaq Listing Rules.

Our compensation committee's responsibilities upon completion of this offering will include:

- reviewing and establishing our overall management compensation strategy and benefits philosophy and policies, including base salary, incentive compensation and equity-based grants;
- reviewing and approving performance goals and objectives relevant to compensation of our chief executive officer and other executive officers;
- evaluating the performance of the chief executive officer and executive officers in light of their performance goals and objectives, including during executive sessions of non-employee directors, and recommending to our board of directors the compensation of our chief executive officer and other executive officers;
- reviewing and making recommendations to the board of directors with respect to non-employee director compensation;
- reviewing, overseeing and administering our equity incentive plans, granting awards under such plan and making recommendations to the board of directors about the adoption of any new or modifying existing equity-based, cash-based, management incentive and deferred compensation plans;
- establishing and reviewing "clawback" policies that allow the recouping of incentive compensation;
- reviewing, considering and selecting, to the extent determined to be advisable, a peer group of appropriate companies for purposing of benchmarking and analysis of compensation for our executive officers and non-employee directors;
- recommending to our board of directors any stock ownership guidelines for our executive officers and non-employee directors, periodically assessing these guidelines and recommending revisions as appropriate, and monitoring individual compliance with these guidelines;
- retaining, appointing or obtaining advice of a compensation consultant, legal counsel or other advisor and determining the compensation and independence of such consultant or advisor;
- preparing, if required, the compensation committee report on executive compensation for inclusion in our annual report on Form 10-K and our proxy statement in accordance with SEC rules;
- monitoring our compliance with the requirements of Sarbanes-Oxley relating to loans to directors and officers;
- reviewing and approving all employment contract and other compensation, severance and change-in-control arrangements for our executive officers;
- establishing and periodically reviewing policies and procedures with respect to perquisites as they relate to our executive officers;

- reviewing the risks associated with our compensation policies and practices;
- overseeing the maintenance and presentation to our board of directors of management's plans for succession to senior management positions based on guidelines developed and recommended to the compensation committee to the full board of directors;
- reviewing our strategies, initiatives and programs with respect to our culture, talent recruitment, development, and retention, employee engagement and diversity and inclusion;
- maintaining minutes of the compensation committee and reporting its actions and any recommendations to the board of directors on a periodic basis;
- reviewing and assessing, at least annually, the adequacy of the compensation committee's charter; and
- performing, on an annual basis, an evaluation of the performance of the compensation committee.

The members of our compensation committee are , and . chairs the compensation committee.

We expect to satisfy the member independence requirements for the compensation committee prior to the end of the transition period provided under the Nasdaq Listing Rules and SEC rules and regulations for companies completing their initial public offering.

Nominating and corporate governance committee

Our nominating and corporate governance committee will operate under a written charter, to be effective prior to the completion of this offering, that satisfies the applicable Nasdaq Listing Rules.

Our nominating and corporate governance committee's responsibilities upon completion of this offering will include:

- actively seeking and identifying individuals qualified to become members of our board of directors consistent with criteria approved by the board and receiving nominations for such qualified individuals;
- recommending to our board of directors the persons to be nominated for election as directors and to each committee of the board;
- establishing a policy under which our stockholders may recommend a candidate to the nominating and corporate governance committee for consideration for nomination as a director;
- reviewing and recommending committee slates on an annual basis;
- recommending to our board of directors qualified candidates to fill vacancies on our board of directors;
- developing and recommending to our board of directors a set of corporate governance principles applicable to us and reviewing the principles on at least an annual basis;
- reviewing and making recommendations to our board with respect to our board size, composition, leadership structure and board committee structure;
- reviewing, in concert with our board of directors, our policies with respect to significant issues of corporate public responsibility, including but not limited to sustainability, diversity and inclusion and environmental, social and governance initiatives;
- making recommendations to our board of directors of processes for annual evaluations of the performance of our board of directors and committees of our board of directors;
- overseeing the process for annual evaluations of our board of directors and committees of our board of directors;
- considering and reporting to our board of directors any questions of possible conflicts of interest of members of our board of directors;

- reviewing with management the company's social corporate responsibility activities, policies, and program;
- providing new director orientation and continuing education for existing directors on a periodic basis;
- overseeing the maintenance and presentation to our board of directors of management's plans for succession to senior management positions in the company;
- reviewing and assessing, at least annually, the adequacy of the nominating and corporate governance committee's charter; and
- performing, on an annual basis, an evaluation of the performance of the nominating and corporate governance committee.

The members of our nominating and corporate governance committee are , and . chairs the nominating and corporate governance committee. Our board of directors has determined that each member of the nominating and corporate governance committee satisfies the independence standards of the applicable Nasdaq Listing Rules.

Our board of directors may establish other committees from time to time.

Code of business conduct and ethics

Effective upon the closing of this offering, we will adopt a Code of Business Conduct and Ethics, or the Code of Conduct, applicable to all of our employees, executive officers and directors. This includes our principal executive officer, principal financial officer and principal accounting officer or controller, or persons performing similar functions. Following the closing of this offering, the full text of the Code of Conduct will be available on our website at www.camp4tx.com. We intend to disclose on our website any future amendments of our Code of Business Conduct and Ethics or waivers that exempt any principal executive officer, principal financial officer, principal accounting officer or controller, persons performing similar functions or our directors from provisions in the Code of Business Conduct and Ethics. Information contained on, or that can be accessed through, our website is not incorporated by reference into this prospectus. We have included our website in this prospectus solely as an inactive textual reference.

Compensation committee interlocks and insider participation

None of the members of the compensation committee is currently, or has been at any time, one of our officers or employees. None of our executive officers currently serves, or has served during the last completed fiscal year, as a member of the board of directors or compensation committee of any entity that has one or more executive officers serving as a member of our board of directors or compensation committee.

Executive and director compensation

The following discussion and analysis of compensation arrangements should be read with the compensation tables and related disclosures set forth below. This discussion contains forward looking statements that are based on our current plans and expectations regarding future compensation programs. The actual compensation programs that we adopt may differ materially from the programs summarized in this discussion.

Introduction

This section describes the material elements of the compensation awarded to, earned by, or paid to our President and Chief Executive Officer, Joshua Mandel-Brehm, our CEO, and our next two most highly compensated executive officers, Kelly Gold, our Chief Financial Officer, and David Bumcrot, our Chief Scientific Officer, for our fiscal year ended December 31, 2023. These executives are collectively referred to as our named executive officers.

Prior to this offering, our board of directors, or the Board, was responsible for determining the compensation of our CEO and the compensation committee of our Board, or the Committee, made recommendations to our Board regarding such compensation and was responsible for determining the compensation of our other executive officers. Our CEO made recommendations to our Committee about the compensation of his direct reports, including Ms. Gold and Mr. Bumcrot.

Summary compensation table

The following table sets forth the compensation awarded to, earned by, or paid to our named executive officers in respect of their service to us for the fiscal year ended December 31, 2023:

Name and principal position	Year	Salary ($)	Nonequity incentive plan compensation ($)(1)	All other compensation ($)(2)	Total ($)
Joshua Mandel-Brehm *Chief Executive Officer*	2023	$546,000	$ 140,381	$ 9,900	$696,281
Kelly Gold *Chief Financial Officer*	2023	$425,000	$ 111,190	$ 92,644	$628,834
David Bumcrot *Chief Scientific Officer*	2023	$390,000	$ 102,033	$ 9,900	$501,933

(1) The amounts shown in this column represent annual bonuses earned with respect to fiscal year 2023 under our annual bonus program, which is described below under "Annual bonuses."

(2) The amounts shown in the "All other compensation" column reflect (i) in the case of Messrs. Mandel-Brehm and Bumcrot, the company's non-elective contribution to our 401(k) plan, described below under "Employee and retirement benefits" and (ii) in the case of Ms. Gold, the company's non-elective contribution to our 401(k) plan ($9,900), commuting and housing expenses ($73,094), and fees for professional memberships ($9,650).

Narrative disclosure to summary compensation table

Annual base salary

The initial base salary of Mr. Mandel-Brehm was set forth in his employment agreement, as subsequently amended and restated, and the initial base salaries for Ms. Gold and Mr. Bumcrot were set forth in their respective offer letter agreements. For 2023, Mr. Mandel-Brehm's annual base salary was $546,000, Ms. Gold's annual base salary was $425,000, and Mr. Bumcrot's annual base salary was $390,000.

Annual bonuses

During fiscal year 2023, each of our named executive officers was eligible to receive an annual performance bonus, with the target amount of such bonus, expressed as a percentage of base salary, equal to 30%. Annual

performance bonuses were based on the attainment of both a company and an individual performance factor. Our Committee makes a recommendation to our Board with respect to the company's achievement against its corporate goals, with our Board approving a final bonus pool. Our Board evaluates the individual performance of our CEO and our Committee, in consultation with our CEO, evaluates the individual performance of our other executives, including Ms. Gold and Mr. Bumcrot.

Agreements with our named executive officers

Each of our named executive officers is party to an employment agreement or offer letter with us that sets forth the terms and conditions of the executive officer's employment with us. We entered into an amended and restated employment agreement with Mr. Mandel-Brehm, dated December 12, 2019, and offer letter agreements with Ms. Gold and Mr. Bumcrot dated June 16, 2017 and July 1, 2017, respectively, that in each case provides for the executive's entitlement to an annual base salary, as described above, and participation in our employee benefit plans, as in effect from time to time. In addition, each executive is subject to a separate Employee Confidentiality and Assignment Agreement, which contains certain restrictive covenant obligations, including covenants relating to confidentiality and assignment of developments, as well as covenants not to compete or solicit certain of our service providers, customers and suppliers during employment and for a 12-month period following termination of employment.

Potential payments upon termination of employment

Each of our named executive officers is entitled to severance and other benefits upon a termination of his or her employment in certain circumstances, as described below. The terms "cause" and "good reason" referred to below are defined in the named executive officer's employment agreement or change in control severance agreement, as applicable, the term "sale event" referred to below is defined in our 2016 Plan and the term "change in control" is defined in the executive's change in control severance agreement.

Mr. Mandel-Brehm. Under his amended and restated employment agreement, if Mr. Mandel-Brehm's employment is terminated by us without cause or by him for good reason, each a "qualifying termination," other than in connection with a "sale event," he will be entitled to receive (i) continued payment of his annual base salary for a period of twelve months following termination of his employment and (ii) a monthly amount equal to the amount we contribute to Mr. Mandel-Brehm's group medical, dental, and/or vision insurance premiums, or the COBRA Continuation, until the end of his severance period or the expiration of his rights under COBRA. In the event of a qualifying termination within thirty days prior to, or twelve months following, a sale event, Mr. Mandel-Brehm will be entitled to receive (i) continued payment of his annual base salary for a period of 18 months following termination of his employment, (ii) COBRA Continuation until the end of such 18-month period, or, if earlier, the expiration of his COBRA rights, and (iii) full acceleration of his outstanding and unvested time-based equity awards.

Mr. Mandel-Brehm is not entitled to a tax gross-up payment for any "golden parachute" excise taxes, but his employment agreement provides for him to receive a cutback of any so-called "parachute payments" if such reduced amount would result in a greater economic benefit to him after accounting for the impact of the excise taxes on such unreduced parachute payments.

Pursuant to a change in control severance agreement with the company, each of Ms. Gold and Mr. Bumcrot is entitled to the following severance amounts in the event of a qualifying termination within thirty days prior to, or twelve months following, a change in control: (i) continued payment of her or his annual base salary for a period of six months following termination of her or his employment, (ii) a monthly COBRA amount equal to the company's portion of the premiums under its group health plan until the earlier of the end of the six-month severance period or the date on which the executive becomes covered under another employer's health plan and (iii) full acceleration of all of her or his outstanding and unvested time-based equity awards.

Our obligation to provide our named executive officers with severance payments and other benefits under their respective agreements is conditioned on the executive officer signing a separation agreement that includes a release of claims in favor of us.

Equity compensation

We did not grant any equity awards to our named executive officers during fiscal year 2023, but in prior years we granted equity awards to our named executive officers to help align their interests with those of our stockholders.

Employee and retirement benefits

We currently provide broad-based health and welfare benefits to our full-time employees, including our named executive officers, including health, life, disability, vision, and dental insurance. In addition, we maintain a safe-harbor 401(k) retirement plan under which we make a 3% non-elective contribution to eligible plan participants. Other than the 401(k) plan, we do not provide any qualified or non-qualified retirement or deferred compensation benefits to our employees, including our named executive officers.

Certain of our named executive officers also receive limited perquisites, which are described above in the footnotes to the “Summary compensation table.”

Outstanding equity awards at fiscal year-end

The following table sets forth information about the outstanding equity awards held by each of our named executive officers as of December 31, 2023:

	Option awards				Stock awards	
Name	Number of securities underlying unexercised options (#) exercisable	Number of securities underlying unexercised options (#) unexercisable	Option exercise price ($)	Option expiration date	Number of shares or units of stock that have not vested	Market value of shares or units of stock that have not yet vested(1)
Joshua Mandel-Brehm	280,940	—	$ 0.19	5/22/27		
	25,443	—	$ 0.19	12/4/27		
	344,549	—	$ 0.19	9/4/28		
	31,804	—	$ 0.19	3/12/29		
	15,570(2)	332	$ 0.19	2/19/30		
	328,125(3)	421,875	$ 0.49	3/23/32		
	1,197,593(4)	2,634,707	$ 0.75	12/7/32		
					332(5)	$ 269
					937,573(6)	$ 759,434
Kelly Gold	16,962	—	$ 0.19	9/26/27		
	4,240	—	$ 0.19	12/4/27		
	31,804	—	$ 0.19	9/4/28		
	6,360	—	$ 0.19	3/12/29		
	53,007	—	$ 0.19	6/18/29		
	8,304(2)	177	$ 0.19	2/19/30		
	72,187(3)	92,813	$ 0.49	3/23/32		
	324,156(4)	713,144	$ 0.75	12/7/32		
					177(5)	$ 143
					212,031(6)	$ 171,745

	Option awards				Stock awards	
Name	Number of securities underlying unexercised options (#) exercisable	Number of securities underlying unexercised options (#) unexercisable	Option exercise price ($)	Option expiration date	Number of shares or units of stock that have not vested	Market value of shares or units of stock that have not yet vested(1)
David Bumcrot	530	—	$ 0.19	5/22/27		
	56,188	—	$ 0.19	9/26/27		
	7,421	—	$ 0.19	12/4/27		
	6,360	—	$ 0.19	3/12/29		
	48,767	—	$ 0.19	6/18/29		
	46,089(2)	981	$ 0.19	2/19/30		
	76,562(3)	98,438	$ 0.49	3/23/32		
	307,062(4)	675,538	$ 0.75	12/7/32		
					160(5)	$ 130
					212,031(6)	$ 171,745

(1) Because we were not publicly traded during 2023, there was no readily ascertainable public market value for our shares. Stock awards reported in this table were valued based on the fair market value of a share of our common stock as of December 31, 2023 ($0.81), as determined by our Board based on a third-party valuation.

(2) Reflects stock options that fully vested on January 1, 2024.

(3) Reflects stock options that vest in equal monthly installments over the 48 monthly anniversaries of the vesting start date of March 1, 2022, subject to the named executive officer's continued employment.

(4) Reflects stock options that vest in equal monthly installments over the 48 monthly anniversaries of the vesting start date of September 1, 2022, subject to the named executive officer's continued employment.

(5) Reflects restricted stock awards that fully vested on January 1, 2024.

(6) Reflects restricted stock received upon the early exercise of a stock option award, which vests in equal monthly installments over the 48-month period beginning March 31, 2021, and on which restrictions will fully lapse on March 31, 2025, subject to the named executive officer's continued employment.

Director compensation

The following table sets forth the compensation paid to, received by, or earned during fiscal year 2023 by the non-employee directors of our Board. Directors, other than those individuals who were elected to the Board pursuant to the provisions of the Voting Agreement and who were not separately compensated for their Board service in 2023, receive an annual retainer of $35,000, which is payable either monthly or at the start of each calendar quarter. Mr. Holtzman receives an additional fee of $15,000 for serving as the Chairman of the Board; Dr. Young receives an additional fee of $7,500 for serving as the Chair of the Research & Development Committee; Mr. Higgins receives an additional fee of $7,500 for serving as the Chair of the Compensation Committee; and Dr. Ragan receives an additional fee of $7,500 for serving as the Chair of the Audit Committee.

Name	Fees earned or paid in cash ($)	All other compensation ($)	Total ($)
Richard Young, PhD(2)	$ 42,500	$ 100,000(1)	$142,500
Steve Holtzman(2)	$ 50,000	$ 75,000(1)	$125,000
Michael Higgins(2)	$ 42,500	—	$ 42,500
Paula Ragan, PhD(2)	$ 42,500	—	$ 42,500
Ravi Thadani, MD, MPH(2)	$ 35,000	—	$ 35,000
James Boylan	—	—	—
Andy Schwab	—	—	—
Amir Nashat, ScD	—	—	—

Name	Fees earned or paid in cash ($)	All other compensation ($)	Total ($)
Jorge Conde	—	—	—
Diana Bernstein	—	—	—
Ingo Chakravarty	—	—	—

(1) Dr. Young and Mr. Holtzman are each party to a consulting agreement with the company pursuant to which such individuals provide strategic and other business consulting services to the company in exchange for an annual retainer ($100,000 for Dr. Young and $75,000 for Mr. Holtzman).

(2) As of December 31, 2023, the following directors held unvested Company stock options and restricted stock awards, or RSAs, as follows: Dr. Young, 482,420 options and 246,486 RSAs; Mr. Holtzman, 675,263 options and 282,929 RSAs; Mr. Higgins 109,932 options and 59,634 RSAs; Ms. Ragan, 109,932 options and 59,634 RSAs; and Dr. Thadhani, 109,932 options and 75,536 RSAs.

Equity plans

2016 Plan

In 2016, our Board adopted, and our stockholders approved, our Amended and Restated 2016 Stock Option and Grant Plan, or the 2016 Plan. The 2016 Plan permits the grant of incentive stock options, nonqualified stock options, restricted stock awards, restricted stock units, or RSUs, and unrestricted stock awards to our officers, employees, directors, consultants, and other key individuals. As of , 2024, awards in respect of shares of our common stock were outstanding under the 2016 Plan and shares remained available for future issuance. This summary is not a complete description of all provisions of the 2016 Plan and is qualified in its entirety by reference to the 2016 Plan, which will be filed as an exhibit to the registration statement of which this prospectus is part.

Plan administration

Our Board administers the 2016 Plan. Our Board has the discretionary authority to interpret the 2016 Plan and any award issued thereunder, to determine eligibility for and grant awards, to determine and modify the terms and conditions of any award, to accelerate the exercisability or vesting of all or any portion of an award, and to prescribe rules, guidelines and practices for administration of the 2016 Plan and all awards, and otherwise make all determinations it deems advisable for administration of the 2016 Plan. Our Board may delegate any or all of its powers to a committee of the board comprised of not less than two directors. As used in this summary, the term “Administrator” refers to our Board and its authorized delegates, as applicable.

Eligibility

Our and our subsidiaries’ officers, employees, directors, consultants, and key persons are eligible to participate in the 2016 Plan. Eligibility for incentive stock options, or ISOs, is limited to our employees or employees of certain affiliates.

Transferability of awards

Except as determined by the Administrator, stock options are not transferable other than by will, or by the laws of descent and distribution. The transfer of shares subject to an award is subject to a number of requirements, including the provision of notice to the company and the company’s right to purchase any shares subject to a proposed transfer at the terms thereof.

Effect of a sale event

Upon the occurrence of a Sale Event, as defined below, the 2016 Plan and all outstanding stock options will terminate, and all unvested restricted stock and RSUs will be forfeited, unless there is a continuation, assumption or substitution of such awards by the surviving entity or its parent. The company also has the right, but not the obligation, to provide the holders of such cancelled or forfeited awards with a cash payment equal to the amount that could have been obtained upon the exercise or settlement of the vested portion of such awards. For purposes of the 2016 Plan, a “Sale Event” includes (i) the sale of all or substantially all of the assets of the

company, (ii) a merger, reorganization or consolidation pursuant to which the holders of the company's outstanding voting power immediately prior to such transaction do not own a majority of the outstanding voting power of the surviving entity, and (iii) the acquisition of all or a majority of the outstanding voting stock of the company in a single transaction or a series of related transactions by a person or group of persons, but which does not include this offering.

Adjustment provisions

In the event of certain changes to the company's capital stock resulting from, among other events, a reorganization, recapitalization, reclassification, stock dividend, stock split, or reverse stock split, the Administrator will make an appropriate and proportionate adjustment to the 2016 Plan and outstanding awards thereunder, including to the maximum number of shares reserved for issuance under the 2016 Plan and the number and kind of shares or other securities subject to any then outstanding awards.

Amendments and termination

Our Board may, at any time, amend or discontinue the 2016 Plan, and the Administrator may, at any time, amend or cancel any outstanding award for the purpose of satisfying changes in law or for any other lawful purpose provided, however, that no such action may adversely affect rights under any outstanding award without the consent of the holder of the award.

Emerging growth company status

We are an "emerging growth company," as defined in the JOBS Act. As an emerging growth company we will be exempt from certain requirements related to the disclosure of executive compensation, including the requirements to hold a nonbinding advisory vote on executive compensation and to provide information relating to the ratio of total compensation of our chief executive officer to the median of the annual total compensation of all of our employees, each as required by the Investor Protection and Securities Reform Act of 2010, which is part of the Dodd-Frank Wall Street Reform and Consumer Protection Act.

Other compensation and benefits

All of our current named executive officers are eligible to participate in our employee benefit plans, in each case on the same basis as all of our other employees. These employee benefit plans include medical, dental, vision, short- and long-term disability and life and accidental dismemberment insurance plans. We pay a portion of the premiums for the medical, dental, vision and life and accidental death and dismemberment insurance for all of our employees, including our named executive officers. In addition, we provide the opportunity to participate in a 401(k) plan to our employees, including each of our named executive officers, as discussed in the section titled "Employee and retirement benefits" below.

Clawback policy

In connection with this offering, we intend to adopt a compensation recovery policy that is compliant with the Nasdaq Listing Rules, as required by the Dodd Frank Act.

Limitations on liability and indemnification

Our Restated Charter, which will become effective immediately prior to the completion of this offering, will contain provisions that limit the liability of our current and former directors and officers for monetary damages to the fullest extent permitted by Delaware law. Delaware law provides that directors and officers of a corporation will not be personally liable for monetary damages for any breach of fiduciary duties as directors or officers, except liability for:

- any breach of the director's or officer's duty of loyalty to the corporation or its stockholders;
- any act or omission not in good faith or that involves intentional misconduct or a knowing violation of law;

- as a director, unlawful payments of dividends or unlawful stock repurchases or redemptions;
- as an officer, derivative claims brought on behalf of the corporation by a stockholder; or
- any transaction from which the director or officer derived an improper personal benefit.

Such limitation of liability does not apply to liabilities arising under federal securities laws and does not affect the availability of equitable remedies such as injunctive relief or rescission.

Our Restated Charter will authorize us to indemnify our directors, officers, employees and other agents to the fullest extent permitted by Delaware law. Our Restated Bylaws will provide that we are required to indemnify our directors and officers to the fullest extent permitted by Delaware law and may indemnify our other employees and agents. Our Restated Bylaws will also provide that, on satisfaction of certain conditions, we will advance expenses incurred by a director or officer in advance of the final disposition of any action or proceeding, and permit us to secure insurance on behalf of any officer, director, employee, or other agent for any liability arising out of his or her actions in that capacity regardless of whether we would otherwise be permitted to indemnify him or her under the provisions of Delaware law. We have entered and expect to continue to enter into agreements to indemnify our directors, executive officers and other employees as determined by the board of directors. With certain exceptions, these agreements provide for indemnification for related expenses including attorneys' fees, judgments, fines and settlement amounts incurred by any of these individuals in any action or proceeding.

We believe that these Restated Charter and Bylaw provisions and indemnification agreements are necessary to attract and retain qualified persons as directors and officers. We also maintain customary directors' and officers' liability insurance.

The limitation of liability and indemnification provisions in our Restated Charter and Restated Bylaws may discourage stockholders from bringing a lawsuit against our directors for breach of their fiduciary duty. They may also reduce the likelihood of derivative litigation against our directors and officers, even though an action, if successful, might benefit us and other stockholders. Further, a stockholder's investment may be adversely affected to the extent that we pay the costs of settlement and damage awards against directors and officers as required by these indemnification provisions.

Insofar as indemnification for liabilities arising under the Securities Act may be permitted for directors, executive officers, or persons controlling us, we have been informed that, in the opinion of the SEC, such indemnification is against public policy as expressed in the Securities Act and is therefore unenforceable.

Rule 10b5-1 plans

Our directors, officers and key employees may adopt written plans, known as Rule 10b5-1 plans, in which they will contract with a broker to buy or sell shares of our common stock on a periodic basis. Under a Rule 10b5-1 plan, a broker executes trades under parameters established by the director or officer when entering into the plan, without further direction from them. The director or officer may amend a Rule 10b5-1 plan in some circumstances and may terminate a plan at any time. Our directors and executive officers may also buy or sell additional shares outside of a Rule 10b5-1 plan when they do not possess of material nonpublic information, subject to compliance with the terms of our insider trading policy.

Certain relationships and related party transactions

The following is a description of transactions since January 1, 2021 to which we have been a participant in which the amount involved exceeded or will exceed the lesser of (i) $120,000 or (ii) one percent of the average of our total assets for the last two completed fiscal years, and in which any of our directors, executive officers or holders of five percent or more of any class of our capital stock, or any members of their immediately family or affiliated entities, had or will have a direct or indirect material interest, other than compensation arrangements that are described under “Management—Non-Employee director compensation” and “Executive compensation.”

Agreements and transactions with stockholders

Series A prime convertible preferred stock financing

In March 2021, we entered into a preferred stock purchase agreement with certain investors, including certain members of our board of directors, beneficial owners of greater than 5% of our capital stock and affiliates of members of our board of directors, pursuant to which we issued and sold an aggregate of 212,264,148 shares of our Series A Prime convertible preferred stock at a purchase price of $0.2120 per share for aggregate gross proceeds of $45.0 million. Each share of our Series A Prime convertible preferred stock will convert into one share of common stock upon the closing of this offering.

In March 2021, in connection with the sale of our Series A Prime convertible preferred stock, we effected a recapitalization, or the Recapitalization, pursuant to which (i) all outstanding Series Seed convertible preferred stock were converted into shares of Series A Prime convertible preferred stock at a rate of 1.51121 shares of Series A Prime convertible preferred stock for every one share of Series Seed convertible preferred stock, and (ii) all outstanding shares of Series A convertible preferred stock were converted into shares of Series A Prime convertible preferred stock at a rate of 1.51996 shares of Series A Prime convertible preferred stock for every one share of Series A convertible preferred stock. In May 2021, we effected a reverse stock split, or the 2021 Reverse Split, pursuant to which each 4.7163 shares of our outstanding common stock and Series A Prime convertible preferred stock became one share of common stock and Series A Prime convertible preferred stock, respectively.

The table below sets forth the aggregate number of shares of Series A Prime convertible preferred stock issued to our related parties in this financing, after giving effect to the 2021 Reverse Split:

Name	Series A prime convertible preferred stock (#)	Aggregate purchase price ($)
Entities affiliated with Polaris Partners(1)	8,840,737	$ 8,839,462.55
AH Bio Fund I, L.P., as nominee(2)	8,837,550	$ 8,836,274.22
Steven Holtzman(3)	110,014	$ 23,322.97
Richard Young, PhD(3)	110,014	$ 23,322.97
Leonard Zon(3)	110,014	$ 23,322.97

(1) Consists of (i) 8,262,703 shares of Series A Prime convertible preferred stock held by Polaris Partners VII, L.P., or Polaris Partners VII, and (ii) 578,034 shares of Series A Prime convertible preferred stock held by Polaris Entrepreneurs’ Fund VII, L.P., or Polaris Entrepreneurs’ VII and, together with Polaris Partners VII, the Polaris Funds. Amir Nashat, ScD, a member of our board of directors, is a managing member of Polaris Partners GP VIII, L.L.C., or Polaris GP VIII, the general partner of the Polaris Funds. Entities affiliated with Polaris Venture Partners collectively hold more than 5% of our voting securities.

(2) Jorge Conde, a member of our board of directors, is a General Partner on the Bio + Health team of Andreessen Horowitz, a venture capital firm. AH Bio Fund I, L.P., or AH Bio Fund, is an investment vehicle of Andreessen Horowitz that holds more than 5% of our voting securities.

(3) Each of Mr. Holtzman, Dr. Young and Dr. Zon served as a member of our board of directors at the time of the Series A Prime convertible preferred stock financing.

Series B convertible preferred stock financing

In June 2022, we entered into a preferred stock purchase agreement with certain investors, including beneficial owners of greater than 5% of our capital stock, affiliates of members of our board of directors and certain of our

executive officers, pursuant to which we issued and sold to such investors an aggregate of 68,258,635 shares of our Series B convertible preferred stock at a purchase price of $1.4724 per share for aggregate gross proceeds of $100.5 million. Each share of our Series B convertible preferred stock will convert into one share of common stock upon the closing of this offering.

The table below sets forth the aggregate number of shares of Series B convertible preferred stock issued to our related parties in this financing:

Name	Series B convertible preferred stock (#)	Aggregate purchase price ($)
Everest Aggregator, LP(1)	27,166,530	$ 39,999.998.78
Entities affiliated with 5AM Ventures(2)	7,640,586	$ 11,249,998.83
Northpond Ventures, LP(3)	5,287,303	$ 7,785.024.94
Entities affiliated with Polaris Partners(4)	4,244,770	$ 6,249,999.36
AH Bio Fund I, L.P., as nominee(5)	679,163	$ 999,999.61
Entities affiliated with Kaiser(6)	10,187,448	$ 14,999,998.44
State of Michigan Retirement Systems(7)	10,187,449	$ 14,999,999.91
Josh Mandel-Brehm	6,791	$ 9,999.07

(1) Everest Aggregator, LP, or Everest Aggregator, is limited partnership affiliated with Enavate Sciences. Enavate Sciences GP, LLC, or Enavate GP, is the general partner of Everest Aggregator. James Boylan, a member of our board of directors, is a manager of Enavate GP and disclaims beneficial ownership of the shares held by Everest Aggregator. Everest Aggregator holds more than 5% of our voting securities.

(2) Consists of (i) 4,244,770 shares of Series B convertible preferred stock held by 5AM Ventures VI, L.P., or 5AM Ventures VI, and (ii) 3,395,816 shares of Series B convertible preferred stock held by 5AM Opportunities II, L.P., or 5AM Opportunities, and, together with 5AM Ventures VI, 5AM Ventures. Andy Schwab, a member of our board of directors, is a Managing Member of 5AM Partners VI, LLC, the General Partner of 5AM Ventures VI and a Managing Member of 5AM Opportunities II (GP), LLC, the General Partner of 5AM Opportunities, and as a result, may be deemed to share voting and investment power with respect to the shares held by 5AM Ventures VI and 5AM Opportunities. Entities affiliated with 5AM Ventures collectively hold more than 5% of our voting securities.

(3) Ingo Chakravarty, a member of our board of directors, is an Operating Partner of Northpond Ventures LLC, an affiliate of Northpond Ventures, LP. Funds affiliated with Northpond Ventures, LLC beneficially own, in the aggregate, more than 5% of our voting securities.

(4) Consists of (i) 3,967,234 shares of Series B convertible preferred stock held by Polaris Partners VII and (ii) 277,536 shares of Series B convertible preferred stock held by Polaris Entrepreneurs' VII. Amir Nashat, ScD, a member of our board of directors, is a managing member of Polaris GP VIII, the general partner of each of the Polaris Funds. The Polaris Funds collectively hold more than 5% of our voting securities.

(5) Jorge Conde, a member of our board of directors, is a General Partner on the Bio + Health team of Andreessen Horowitz, a venture capital firm. AH Bio Fund is an investment vehicle of Andreessen Horowitz that holds more than 5% of our voting securities.

(6) Consists of (i) 6,791,632 shares of Series B convertible preferred stock held by Kaiser Permanente Group Trust and (ii) 3,395,816 shares of Series B convertible preferred stock held by Kaiser Foundation Hospitals. Kaiser Permanente Group Trust and Kaiser Foundation Hospitals together hold more than 5% of our voting securities.

(7) SMRS-TOPE LLC, on behalf of the State of Michigan Retirement Systems, holds more than 5% of our voting securities prior to this offering.

Investors' rights, voting and right of first refusal agreements

In connection with our preferred stock financings, we entered into an amended and restated investors' rights agreement, the Voting Agreement and an amended and restated right of first refusal and co-sale agreement, containing registration rights, information rights, rights of first offer, voting rights and rights of first refusal, among other things, with certain holders of our capital stock, including Everest Aggregator, entities affiliated with Kaiser, SMRS-TOPE LLC, entities affiliated with 5AM Ventures, AH Bio Fund, the Polaris Funds and Northpond Ventures. Josh Mandel-Brehm, our Chief Executive Officer, is a party to certain of these agreements in his capacity as a stockholder.

The foregoing stockholder agreements will terminate upon the closing of this offering, except for the registration rights granted under our amended and restated investors' rights agreement, as more fully described in the section titled "Description of capital stock—Registration rights."

Director affiliations

Certain of our directors are affiliated with and, prior to the completion of this offering, have served on our board of directors as representatives of entities which beneficially own or owned 5% or more of our voting securities, as indicated in the table below:

Director	Affiliated Stockholder
James Boylan	Entities affiliated with Everest Aggregator, LP
Jorge Conde	Entities affiliated with AH Bio Fund I, L.P.
Ingo Chakravarty	Entities affiliated with Northpond Ventures, LP
Amir Nashat, ScD	Entities affiliated with Polaris Partners
Andy Schwab	Entities affiliated with 5AM Ventures

Each of the directors identified above was elected pursuant to the board composition provisions of the Voting Agreement. The Voting Agreement, including the board composition provisions therein, will terminate upon the completion of this offering, after which there will be no further contractual obligations regarding the election of our directors.

Loans to related persons

In August 2021, we entered into a secured promissory note, or the Mandel-Brehm Promissory Note, with Josh Mandel-Brehm, our Chief Executive Officer, pursuant to which we loaned to Mr. Mandel-Brehm $565,999.96 to fund the payment associated with the early exercise of options held by Mr. Mandel-Brehm for 3,000,233 shares of Common Stock. The Mandel-Brehm Promissory Note was secured by a pledge to us of Mr. Mandel-Brehm's equity interests in the 3,000,233 shares of Common Stock issued upon the early exercise of Mr. Mandel-Brehm's stock options. The Mandel-Brehm Promissory Note bears interest on the unpaid principal balance at the rate per annum equal to the long-term Applicable Federal Rate as defined in Section 1274(d) of the Internal Revenue Code of 1986, as amended, or the Code, in effect on the first day of each calendar year, which was initially 1.35%. The Mandel-Brehm Promissory Note is due and payable by the earliest to occur of (i) August 9, 2026, (ii) the date that we first become subject to the reporting requirements of the Exchange Act with respect to any class of our securities, or (iii) an event of default, as defined in the Mandel-Brehm Promissory Note.

In August 2021, we entered into a secured promissory note, or the Gold Promissory Note, with Kelly Gold, our Chief Financial Officer, pursuant to which we loaned to Ms. Gold $127,999.82 to fund the payment associated with the early exercise of options held by Ms. Gold for 678,497 shares of Common Stock. The Gold Promissory Note was secured by a pledge to us of Ms. Gold's equity interests in the 678,497 shares of Common Stock issued upon the early exercise of Ms. Gold's stock options. The Gold Promissory Note bears interest on the unpaid principal balance at the rate per annum equal to the long-term Applicable Federal Rate, as defined in the Code, in effect on the first day of each calendar year, which was initially 1.35%. The Gold Promissory Note is due and payable by the earliest to occur of (i) August 9, 2026, (ii) the date that we first become subject to the reporting requirements of the Exchange Act with respect to any class of our securities, or (iii) an event of default, as defined in the Gold Promissory Note.

In August 2021, we entered into a secured promissory note, or the Bumcrot Promissory Note, with David Bumcrot, our Chief Scientific Officer, pursuant to which we loaned to Mr. Bumcrot $127,999.82 to fund the payment associated with the early exercise of options held by Mr. Bumcrot for 678,497 shares of Common Stock. The Bumcrot Promissory Note was secured by a pledge to us of Mr. Bumcrot's equity interests in the 678,497 shares of Common Stock issued upon the early exercise of Mr. Bumcrot's stock options. The Bumcrot Promissory Note bears interest on the unpaid principal balance at the rate per annum equal to the long-term Applicable Federal Rate, as defined in the Code, in effect on the first day of each calendar year, which was initially 1.35%. The Bumcrot Promissory Note is due and payable by the earliest to occur of (i) August 9, 2026, (ii) the date that we first become subject to the reporting requirements of the Exchange Act with respect to any class of our securities, or (iii) an event of default, as defined in the Bumcrot Promissory Note.

Employment arrangements

We have entered into employment agreements or offer letter agreements with certain of our executive officers. For more information regarding such employment agreements, see "Executive compensation—Agreements with our named executive officers."

Indemnification agreements

Our amended and restated certificate of incorporation that will be in effect upon the closing of this offering will contain provisions limiting the liability of directors and executive officers, and our amended and restated bylaws will provide that we will indemnify each of our directors and executive officers to the fullest extent permitted under Delaware law. Our amended and restated certificate of incorporation and amended and restated bylaws will also provide our board of directors with discretion to indemnify our employees and other agents when determined appropriate by the board.

In addition, we have entered into or intend to enter into indemnification agreements with each of our directors and executive officers. For more information regarding these agreements, see "Executive compensation—Limitations on liability and indemnification."

Related person transaction policy

Our board of directors intends to adopt a written related person transaction policy, to be effective upon the effectiveness of the registration statement of which this prospectus forms a part, setting forth the policies and procedures for the review and approval or ratification of related person transactions. This policy will cover, with certain exceptions set forth in Item 404 of Regulation S-K under the Securities Act, any transaction, arrangement or relationship, or any series of similar transactions, arrangements or relationships, in which we were or are to be a participant, where the amount involved exceeds in any fiscal year the lesser of (i) $120,000 or (ii) one percent of the average of our total assets at year end for the last two completed fiscal years and a related person had, has or will have a direct or indirect material interest, including, without limitation, purchases of goods or services by or from the related person or entities in which the related person has a material interest, indebtedness, guarantees of indebtedness and employment by us of a related person. In reviewing and approving any such transactions, our audit committee is tasked with considering all relevant facts and circumstances, including, but not limited to, whether the transaction is on terms comparable to those that could be obtained in an arm's length transaction and the extent of the related person's interest in the transaction. All of the transactions described in this section occurred prior to the adoption of this policy.

Principal stockholders

The following table sets forth certain information regarding beneficial ownership of our capital stock as of , 2024 by:

- each person, or group of affiliated persons, known by us to beneficially own more than 5% of our common stock;
- each of our directors;
- our named executive officer; and
- all of our current executive officers and directors as a group.

We have determined beneficial ownership in accordance with the rules of the SEC. Under these rules, beneficial ownership includes any shares of common stock as to which the individual or entity has sole or shared voting power or investment power. Percentage ownership of our common stock before this offering is based on shares of common stock outstanding as of , 2024, after giving effect to the automatic conversion of all of our convertible preferred stock into shares of our common stock immediately prior to the closing of this offering, as if such conversion had occurred as of , 2024, assuming an initial public offering price of $ per share, which is the midpoint of the price range set forth on the cover page of this prospectus. Percentage ownership of our common stock after this offering is based on shares of our common stock outstanding as of , 2024, after giving effect to the transactions described above and our issuance of shares of our common stock in this offering. In computing the number of shares beneficially owned by an individual or entity and the percentage ownership of that person, shares of common stock subject to options held by such person that are currently exercisable or will become exercisable within 60 days of , 2024 are considered outstanding, although these shares are not considered outstanding for purposes of computing the percentage ownership of any other person. Unless noted otherwise, the address of all listed stockholders is c/o One Kendall Square, Building 1400 West, 3rd Floor, Cambridge, Massachusetts 02139.

Except as indicated by the footnotes below, we believe, based on information furnished to us, that each of the stockholders listed has sole voting and investment power with respect to the shares beneficially owned by the stockholder unless noted otherwise, subject to community property laws where applicable.

Name of beneficial owner	Number of shares beneficially owned	Percentage of shares beneficially owned	
		Before offering	After offering
Greater than 5% stockholders			
Entities affiliated with 5AM Ventures(1)		%	%
AH Bio Fund I, L.P.(2)		%	%
Everest Aggregator, LP(3)		%	%
Entities affiliated with Kaiser Permanente Group Trust(4)		%	%
Entities affiliated with Northpond Ventures, LLC(5)		%	%
Entities affiliated with Polaris Partners(6)		%	%
SMRS-TOPE LLC(7)		%	%
Named executive officers and directors			
Josh Mandel-Brehm(8)		%	%
David Bumcrot, PhD(9)		%	%
Kelly Gold(10)		%	%
Steven Holtzman(11)		%	%
James Boylan(12)		%	%
Jorge Conde(13)		%	%

Name of beneficial owner	Number of shares beneficially owned	Percentage of shares beneficially owned	
		Before offering	After offering
Ingo Chakravarty(14)		%	%
Michael Higgins(15)		%	%
Amir Nashat, ScD(16)		%	%
Paula Ragan, PhD(17)		%	%
Andy Schwab(18)		%	%
Ravi I. Thadhani, MD, MPH(19)		%	%
Richard Young, PhD(20)		%	%
All current executive officers and directors as a group (13 persons)		%	%

* Represents beneficial ownership of less than one percent.

(1) Consists of (i) shares of common stock issuable upon conversion of Series B convertible preferred stock held by 5AM Ventures VI, L.P., or 5AM Ventures VI, and (ii) shares of common stock issuable upon conversion of Series B convertible preferred stock held by 5AM Opportunities II, L.P., or 5AM Opportunities, and, together with 5AM Ventures VI, 5AM Ventures. 5AM Partners VI, LLC, or Partners VI, is the sole general partner of 5AM Ventures VI and 5AM Opportunities II (GP), LLC, or Opportunities II GP, is the sole general partner of 5AM Opportunities. Dr. Kush M. Parmar and Andrew J. Schwab are the managing members of each Partners VI and Opportunities II GP and may be deemed to have shared voting and investment power over the securities beneficially owned by 5AM Ventures VI and 5AM Opportunities. Each of Partners VI, Opportunities II GP, Dr. Parmar and Mr. Schwab disclaims beneficial ownership of such securities except to the extent of its or his respective pecuniary interest therein. The principal business address of 5AM Ventures is 4 Embarcadero Center, Suite 3110, San Francisco, California 94111.

(2) Consists of shares of common stock issuable upon the conversion of Series A Prime convertible preferred stock and (ii) shares of common stock issuable upon conversion of Series B convertible preferred stock held of record by AH Bio Fund I, L.P., for itself and as nominee for AH Bio Fund I-B, L.P., or, collectively, the AH Bio Fund I Entities. AH Equity Partners Bio I, L.L.C., or AH Bio I EP, is the general partner of the AH Bio Fund I Entities. The managing members of AH Bio I EP are Marc Andreessen and Ben Horowitz. AH Bio I EP has sole voting and dispositive power with regard to the shares held by the AH Bio Fund I Entities. The address for each of these entities and individuals is 2865 Sand Hill Road, Suite 101, Menlo Park, CA 94025.

(3) Consists of shares of common stock issuable upon conversion of Series B convertible preferred stock held by Everest Aggregator, LP, or Everest Aggregator. Everest Aggregator is a limited partnership affiliated with Enavate Sciences. Enavate Sciences GP, LLC, or Enavate GP, is the general partner of Everest Aggregator. Voting, investment and dispositive power with respect to the shares held by Everest Aggregator is held by the managers of Enavate GP collectively, including James Boylan, a member of our board of directors. Mr. Boylan disclaims beneficial ownership of the shares held by Everest Aggregator. The principal business address of Everest Aggregator is 106 West 56th Street, New York, New York 10019.

(4) Consists of shares of common stock held by entities affiliated with Kaiser Permanente Group Trust. The principal business address of Kaiser Permanente Group Trust is One Kaiser Plaza—Ordway Building, Oakland, California 94612.

(5) Consists of shares of common stock issuable upon conversion of Series A Prime convertible preferred stock held by Northpond Ventures II, LP, or Northpond Fund II, and shares of common stock issuable upon conversion of Series B convertible preferred stock held by Northpond Ventures, LP, or Northpond Fund I. The general partner of Northpond Fund II is Northpond Ventures II GP, LLC, or Northpond II GP, and the general partner of Northpond Fund I is Northpond Ventures GP, LLC, or Northpond GP. Voting and dispositive decisions with respect to the securities held by Northpond Fund I and Northpond Fund II are made by Michael Rubin, the managing member of Northpond GP and Northpond II GP. Ingo Chakravarty, a member of our board of directors, is an Operating Partner of Northpond Ventures, LLC. Mr. Chakravarty has no voting or dispositive power with respect to the securities held by Northpond Fund I and Northpond Fund II.

(6) Consists of shares of common stock issuable upon the conversion of Series A Prime convertible preferred stock held by the Polaris Funds and (ii) shares of common stock issuable upon the conversion of Series B convertible preferred stock held by the Polaris Funds. Polaris GP VIII is the general partner of each of the Polaris Funds and may be deemed to have sole voting and dispositive power with respect to the shares held by the Polaris Funds. Amir Nashat, ScD, a member of our board of directors, David Barrett, Brian Chee and Bryce Youngren (collectively, the Polaris GP VIII Managing Members) are the managing members of Polaris GP VIII. Each of the Polaris GP VIII Managing Members, in their capacities with respect to Polaris GP VIII, may be deemed to have shared voting and dispositive power with respect to the shares held by the Polaris Funds. The principal business address of Polaris Partners is One Marina Drive, 8th Floor, Boston, Massachusetts 02210.

(7) Consists of shares of common stock held by entities affiliated with SMRS-TOPE LLC. The principal business address of SMRS-TOPE LLC is c/o HarbourVest Partners, L.P., One Financial Center, Boston, Massachusetts 02111.

(8) Consists of (i) shares of common stock and (ii) shares of common stock underlying outstanding stock options exercisable within 60 days of , 2024.

(9) Consists of (i) shares of common stock and (ii) shares of common stock underlying outstanding stock options exercisable within 60 days of , 2024.

(10) Consists of (i) shares of common stock and (ii) shares of common stock underlying outstanding stock options exercisable within 60 days of , 2024.

(11) Consists of (i) shares of common stock and (ii) shares of common stock underlying outstanding stock options exercisable within 60 days of , 2024.

(12) Consists of (i) shares of common stock and (ii) shares of common stock underlying outstanding stock options exercisable within 60 days of , 2024.

(13) Consists of (i) shares of common stock and (ii) shares of common stock underlying outstanding stock options exercisable within 60 days of , 2024.

(14) Consists of (i) shares of common stock and (ii) shares of common stock underlying outstanding stock options exercisable within 60 days of , 2024.

(15) Consists of (i) shares of common stock and (ii) shares of common stock underlying outstanding stock options exercisable within 60 days of , 2024.

(16) Consists of (i) shares of common stock and (ii) shares of common stock underlying outstanding stock options exercisable within 60 days of , 2024.

(17) Consists of (i) shares of common stock and (ii) shares of common stock underlying outstanding stock options exercisable within 60 days of , 2024.

(18) Consists of (i) shares of common stock and (ii) shares of common stock underlying outstanding stock options exercisable within 60 days of , 2024.

(19) Consists of (i) shares of common stock and (ii) shares of common stock underlying outstanding stock options exercisable within 60 days of , 2024.

(20) Consists of (i) shares of common stock and (ii) shares of common stock underlying outstanding stock options exercisable within 60 days of , 2024.

Description of capital stock

The following description of our capital stock and certain provisions of our Restated Charter and Restated Bylaws as they will be in effect immediately prior to the completion of this offering are summaries and are qualified by reference to our Restated Charter and Restated Bylaws. Copies of these documents are filed as exhibits to the registration statement of which this prospectus is a part.

General

Upon the completion of this offering, our Restated Charter will authorize us to issue up to shares of common stock, $0.0001 par value per share, and shares of preferred stock, $0.0001 par value per share, all of which shares of preferred stock will be undesignated. Our board of directors may establish the rights and preferences of the preferred stock from time to time.

As of , 2024, we had outstanding shares of common stock held by stockholders of record. As of , 2024, after giving effect to the automatic conversion of all of the outstanding shares of our convertible preferred stock and convertible preferred stock warrants, including shares of our Series A Prime convertible preferred stock and shares of our Series B convertible preferred stock into shares of common stock, based on an assumed initial public offering price of $ per share, which is the midpoint of the price range set forth on the cover page of this prospectus upon completion of this offering, there would have been shares of common stock issued and outstanding (including shares of unvested restricted common stock), held by stockholders of record, and no shares of preferred stock outstanding.

Common stock

Holders of our common stock are entitled to one vote for each share held on all matters submitted to a vote of stockholders and do not have cumulative voting rights. An election of directors by our stockholders shall be determined by a majority of the votes cast by the stockholders entitled to vote on the election, except in the case of a contested election, in which case the election shall be determined by a plurality of the votes cast by the stockholders entitled to vote on the election. Holders of common stock are entitled to receive proportionately any dividends as may be declared by our board of directors, subject to any preferential dividend rights of any series of preferred stock that we may designate and issue in the future.

In the event of our liquidation or dissolution, the holders of common stock are entitled to receive proportionately our net assets available for distribution to stockholders after the payment of all debts and other liabilities and subject to the prior rights of any outstanding preferred stock. Holders of common stock have no preemptive, subscription, redemption or conversion rights. Our outstanding shares of common stock are, and the shares offered by us in this offering will be, when issued and paid for, validly issued, fully paid and nonassessable. The rights, preferences and privileges of holders of common stock are subject to and may be adversely affected by the rights of the holders of shares of any series of preferred stock that we may designate and issue in the future.

Options and restricted shares

As of , 2024, there were options to purchase shares of our common stock outstanding, of which were vested and exercisable as of that date. For additional information regarding the terms of our 2016 Plan, see the sections titled “Executive and director compensation—Equity incentive plans.”

Preferred stock

As of , 2024, there were shares of our preferred stock outstanding, consisting of shares of our Series A Prime convertible preferred stock and shares of our Series B convertible preferred stock. All currently outstanding shares of convertible preferred stock will be converted into an aggregate of shares of common stock immediately prior to the closing of this offering.

Under the terms of our Restated Charter that will be in effect immediately prior to the completion of this offering, our board of directors is authorized to direct us to issue shares of preferred stock in one or more series without stockholder approval. Our board of directors has the discretion to determine the rights, preferences, privileges and restrictions, including voting rights, dividend rights, conversion rights, redemption privileges and liquidation preferences, of each series of preferred stock.

The purpose of authorizing our board of directors to issue preferred stock and determine its rights and preferences is to eliminate delays associated with a stockholder vote on specific issuances. The issuance of preferred stock, while providing flexibility in connection with possible acquisitions, future financings and other corporate purposes, could have the effect of making it more difficult for a third-party to acquire, or could discourage a third-party from seeking to acquire, a majority of our outstanding voting stock. Upon the completion of this offering, there will be no shares of preferred stock outstanding, and we have no present plans to issue any shares of preferred stock.

We have no present plans to issue any shares of preferred stock following the completion of this offering.

Warrants

As of , 2024, there were outstanding immediately exercisable warrants to purchase up to 1,602 shares of our Series A Prime convertible preferred stock at an exercise price of $0.9998556 per share. Upon completion of this offering, the warrant to purchase shares of Series A Prime convertible preferred stock will become automatically exercisable for the purchase of an aggregate of shares of our common stock, which equals the number of shares of our common stock into which 1,602 shares of our Series A Prime convertible preferred stock would have been automatically converted in connection with this offering had such shares been outstanding prior to the completion of this offering, at an exercise price of $ per share.

Registration rights

Upon the completion of this offering, holders of shares of our common stock, which includes all of the shares of common stock issuable upon the automatic conversion of the convertible preferred stock outstanding immediately prior to the closing of this offering, will be entitled to the following rights with respect to the registration of such shares for public resale under the Securities Act, pursuant to an investors' rights agreement by and among us and certain investors. These shares are collectively referred to herein as "registrable securities." The registration of shares of common stock as a result of the following rights being exercised would enable holders to trade these shares without restriction under the Securities Act when the applicable registration statement is declared effective.

Demand registration rights

At any time beginning one hundred eighty (180) days following the effective date of the registration statement of which this prospectus is a part, the holders of a majority of registrable securities then outstanding have the right to demand that we file a registration statement covering at least forty percent (40%) of the registrable securities then outstanding (or a lesser percent if the anticipated aggregate offering price, net of selling expenses, would exceed $10 million). These registration rights are subject to specified conditions and limitations, including the right of the underwriters, if any, to limit the number of shares included in any such registration under specified circumstances. Upon such a request, we are required to effect the registration as soon as practicable, but in any event no later than 60 days after the receipt of such request; provided, however, that we will not be required to effect such a registration if, among other things, we have already effected two registrations for the holders of registrable securities in response to these demand registration rights. An aggregate of shares of common stock will be entitled to these demand registration rights.

Piggyback registration rights

If we propose to register any of our securities under the Securities Act either for our own account or for the account of other stockholders, the holders of registrable securities will each be entitled to notice of the registration

and will be entitled to include their shares of common stock in the registration statement. These piggyback registration rights are subject to specified conditions and limitations, including the right of the underwriters to limit the number of shares included in any such registration under specified circumstances. An aggregate of shares of common stock will be entitled to these piggyback registration rights.

Registration on Form S-3

At any time after we become eligible to file a registration statement on Form S-3, the holders of at least twenty percent (20%) of registrable securities then outstanding will be entitled to request to have such shares registered by us on a Form S-3 registration statement. These Form S-3 registration rights are subject to other specified conditions and limitations, including the condition that the anticipated aggregate offering price, net of certain selling expenses, is at least $3.0 million. Upon receipt of this request, the holders of registrable securities will each be entitled to participate in this registration. Upon such a request, we are required to effect the registration as soon as practicable, but in any event no later than 45 days after the receipt of such request; provided, however, that we will not be required to effect such a registration if, among other things, we have already effected two registrations on Form S-3 for the holders of registrable securities in response to these demand registration rights within the preceding 12 months. An aggregate of shares of common stock will be entitled to these Form S-3 registration rights.

Expenses of registration

We are required to pay all expenses, including fees and expenses of one counsel to represent the selling stockholders (up to $50,000 total), relating to any demand, piggyback or Form S-3 registration, other than underwriting discounts and commissions, stock transfer taxes and any additional fees of counsel for the selling stockholders, subject to specified conditions and limitations. We are not required to pay registration expenses if a demand registration request is withdrawn at the request of a majority of holders of registrable securities to be registered, unless holders of a majority of the registrable securities agree to forfeit their right to one demand registration.

The amended and restated investors' rights agreement contains customary cross-indemnification provisions, pursuant to which we are obligated to indemnify the selling stockholders in the event of material misstatements or omissions in the applicable registration statement attributable to us, and the selling stockholders are obligated to indemnify us for material misstatements or omissions in the registration statement attributable to them, subject to certain limitations.

Termination of registration rights

The registration rights granted under the investors' rights agreement will terminate with respect to any particular stockholder upon the earlier of (a) the closing of a deemed liquidation event, as defined in our certificate of incorporation, (b) the fifth anniversary of the closing of this offering and (c) with respect to each stockholder, at such time such stockholder is able to sell all of its shares pursuant to Rule 144 or another similar exemption under the Securities Act during a three-month period without registration.

Anti-takeover effects of our Restated Charter and Restated Bylaws

Section 203 of the Delaware General Corporation Law

Our Restated Charter and Restated Bylaws, which will be in effect prior to the consummation of this offering, will contain certain provisions that are intended to enhance the likelihood of continuity and stability in the composition of our board of directors but which may have the effect of delaying, deferring or preventing a future takeover or change in control of us unless such takeover or change in control is approved by our board of directors.

These provisions include:

Classified board. Our Restated Charter will provide that our board of directors will be divided into three classes of directors, with the classes as nearly equal in number as possible. As a result, approximately one-third of our

board of directors will be elected each year. The classification of directors will have the effect of making it more difficult for stockholders to change the composition of our board of directors. Our Restated Charter will also provide that, subject to any rights of holders of preferred stock to elect additional directors under specified circumstances, the number of directors will be fixed exclusively pursuant to a resolution adopted by our board of directors. Upon completion of this offering, we expect that our board of directors will have directors.

Action by written consent; special meetings of stockholders. Our Restated Charter will provide that stockholder action can be taken only at an annual or special meeting of stockholders and cannot be taken by written consent in lieu of a meeting. Our Restated Charter and the Restated Bylaws will also provide that, except as otherwise required by law, special meetings of the stockholders can only be called pursuant to a resolution adopted by a majority of our board of directors. Except as described above, stockholders will not be permitted to call a special meeting or to require our board of directors to call a special meeting.

Removal of directors. Our Restated Charter will provide that our directors may be removed only for cause by the affirmative vote of at least a majority of the voting power of our outstanding shares of capital stock, voting together as a single class. This requirement of a supermajority vote to remove directors could enable a minority of our stockholders to prevent a change in the composition of our board of directors.

Advance notice procedures. Our Restated Bylaws will establish an advance notice procedure for stockholder proposals to be brought before an annual meeting of our stockholders, including proposed nominations of persons for election to our board of directors. Stockholders at an annual meeting will only be able to consider proposals or nominations specified in the notice of meeting or brought before the meeting by or at the direction of our board of directors or by a stockholder who was a stockholder of record on the record date for the meeting, who is entitled to vote at the meeting and who has given our Secretary timely written notice, in proper form, of the stockholder's intention to bring that business before the meeting. Although the Restated Bylaws will not give our board of directors the power to approve or disapprove stockholder nominations of candidates or proposals regarding other business to be conducted at a special or annual meeting, the Restated Bylaws may have the effect of precluding the conduct of certain business at a meeting if the proper procedures are not followed or may discourage or deter a potential acquirer from conducting a solicitation of proxies to elect its own slate of directors or otherwise attempting to obtain control of us.

Supermajority approval requirements. The DGCL generally provides that the affirmative vote of a majority of the shares entitled to vote on any matter is required to amend a corporation's certificate of incorporation or bylaws, unless either a corporation's certificate of incorporation or bylaws requires a greater percentage. Our Restated Charter and Restated Bylaws will provide that the affirmative vote of holders of at least 75% of the total votes eligible to be cast in the election of directors will be required to amend, alter, change or repeal specified provisions. This requirement of a supermajority vote to approve amendments to our Restated Charter and Restated Bylaws could enable a minority of our stockholders to exercise veto power over any such amendments.

Authorized but unissued shares. Our authorized but unissued shares of common stock and preferred stock will be available for future issuance without stockholder approval. These additional shares may be utilized for a variety of corporate purposes, including future public offerings to raise additional capital, corporate acquisitions and employee benefit plans. The existence of authorized but unissued shares of common stock and preferred stock could render more difficult or discourage an attempt to obtain control of a majority of our common stock by means of a proxy contest, tender offer, merger or otherwise.

Exclusive forum. Our Restated Charter will provide that, subject to limited exceptions, the Court of Chancery of the State of Delaware (or, if, and only if, the Court of Chancery of the State of Delaware dismisses a Covered Claim (as defined in our Restated Charter) for lack of subject matter jurisdiction, any other state or federal court in the State of Delaware that does have subject matter jurisdiction) will, to the fullest extent permitted by applicable law, be the sole and exclusive forum for Covered Claims. This provision would not apply to claims brought to enforce a duty or liability created by the Exchange Act.

Our Restated Charter will further provide that the federal district courts of the United States of America will be the exclusive forum for resolving any complaint asserting a cause of action arising under the Securities Act. In addition, our Restated Charter will provide that any person or entity purchasing or otherwise acquiring any interest in the shares of capital stock of the company will be deemed to have notice of and consented to these choice-of-forum provisions and waived any argument relating to the inconvenience of the forums in connection with any Covered Claim.

The choice of forum provisions to be contained in our Restated Charter may limit a stockholder's ability to bring a claim in a judicial forum that it finds favorable for disputes with us or any of our directors, officers, other employees or stockholders, which may discourage lawsuits with respect to such claims, although our stockholders will not be deemed to have waived our compliance with federal securities laws and the rules and regulations thereunder. While the Delaware courts have determined that such choice of forum provisions are facially valid, it is possible that a court of law in another jurisdiction could rule that the choice of forum provisions to be contained in our Restated Charter are inapplicable or unenforceable if they are challenged in a proceeding or otherwise, which could cause us to incur additional costs associated with resolving such action in other jurisdictions. See the section titled "Risk factors—Risks related to this offering and our common stock—Our Restated Charter will designate the state or federal courts within the State of Delaware as the exclusive forum for certain types of actions and proceedings that may be initiated by our stockholders, which could limit our stockholders' ability to obtain a favorable judicial forum for disputes with us or our directors, officers or employees."

Section 203 of the DGCL

Upon completion of this offering, we will be subject to the provisions of Section 203 of the DGCL. In general, Section 203 prohibits a publicly held Delaware corporation from engaging in a "business combination" with an "interested stockholder" for a three-year period following the time that this stockholder becomes an interested stockholder, unless the business combination is approved in a prescribed manner. A "business combination" includes, among other things, a merger, asset or stock sale or other transaction resulting in a financial benefit to the interested stockholder. An "interested stockholder" is a person who, together with affiliates and associates, owns, or did own within three years prior to the determination of interested stockholder status, 15% or more of the corporation's voting stock.

Under Section 203, a business combination between a corporation and an interested stockholder is prohibited unless it satisfies one of the following conditions: before the stockholder became interested, the corporation's board of directors approved either the business combination or the transaction which resulted in the stockholder becoming an interested stockholder; upon consummation of the transaction which resulted in the stockholder becoming an interested stockholder, the interested stockholder owned at least 85% of the voting stock of the corporation outstanding at the time the transaction commenced, excluding for purposes of determining the voting stock outstanding, shares owned by persons who are directors and also officers, and employee stock plans, in some instances; or at or after the time the stockholder became interested, the business combination was approved by the board of directors of the corporation and authorized at an annual or special meeting of the stockholders by the affirmative vote of at least two-thirds of the outstanding voting stock which is not owned by the interested stockholder.

A Delaware corporation may "opt out" of these provisions with an express provision in its original certificate of incorporation or an express provision in its certificate of incorporation or bylaws resulting from a stockholders' amendment approved by at least a majority of the outstanding voting shares. We have not opted out of these provisions. As a result, mergers or other takeover or change in control attempts of us may be discouraged or prevented.

Transfer agent and registrar

The transfer agent and registrar for our common stock is Equiniti Trust Company, LLC. The transfer agent's address is 6201 15th Avenue, Brooklyn, New York 11219.

Listing

We have applied for listing of our common stock on the Nasdaq Global Market under the trading symbol “CAMP.”

Limitations of liability and indemnification matters

For a discussion of liability and indemnification, see the section titled “Executive and director compensation—Limitations of liability and indemnification.”

Shares eligible for future sale

Immediately prior to this offering, there was no public market for our common stock, and no predictions can be made about the effect, if any, that market sales of our common stock or the availability of such shares for sale will have on the market price prevailing from time to time. Nevertheless, future sales of our common stock in the public market, or the perception that such sales may occur, could adversely affect the market price of our common stock and could impair our ability to raise capital through future sales of our securities. See the section titled "Risk factors—Risks related to this offering and ownership of our common stock—A significant portion of our total outstanding shares is restricted from immediate resale but may be sold into the market in the near future, which could cause the market price of our common stock to decline significantly, even if our business is doing well." Furthermore, although we have applied to have our common stock approved for listing on Nasdaq, we cannot assure you that there will be an active public trading market for our common stock.

Upon the completion of this offering, based on the number of shares of our common stock outstanding as of , 2024, assuming an initial public offering price of $ per share, which is the midpoint of the price range set forth on the cover page of this prospectus, and after giving effect to the automatic conversion of all of the shares of our convertible preferred stock and convertible preferred stock warrants into an aggregate of shares of our common stock, based on an assumed initial public offering price of $ per share, which is the midpoint of the price range set forth on the cover page of this prospectus immediately prior to the completion of this offering, we will have an aggregate of shares of our common stock outstanding (or shares of our common stock if the underwriters exercise in full their option to purchase additional shares). Of these shares of our common stock, all of the shares sold in this offering (or shares if the underwriters exercise in full their option to purchase additional shares) will be freely tradable without restriction or further registration under the Securities Act, except for any shares purchased by our "affiliates," as that term is defined in Rule 144 under the Securities Act, whose sales would be subject to the Rule 144 resale restrictions described below, other than the holding period requirement.

All remaining shares of common stock held by existing stockholders immediately prior to the completion of this offering will be "restricted securities" as such term is defined in Rule 144. These restricted securities are eligible for public sale only if they are registered under the Securities Act or if they qualify for an exemption from registration under Rules 144 or 701 under the Securities Act, which are summarized below. We expect that substantially all of these shares will be subject to the 180-day lock-up period under the lock-up agreements described below. Upon expiration of the lock-up period, we estimate that approximately shares of our common stock will be available for sale in the public market, subject in some cases to applicable volume limitations under Rule 144.

Lock-up agreements

We and each of our directors and executive officers and the holders of substantially all of our outstanding capital stock have entered into lock-up agreements with the underwriters or otherwise agreed, among other things and subject to certain exceptions, not to sell or transfer any common stock or securities convertible into, exchangeable for, exercisable for, or repayable with common stock, for 180 days after the date of this prospectus without first obtaining the written consent of J.P. Morgan Securities LLC and Leerink Partners LLC.

Upon the expiration of the lock-up period, substantially all of the shares subject to such lock-up restrictions will become eligible for sale, subject to the limitations discussed above. For a further description of these lock-up agreements, please see the section titled "Underwriting."

After the date of the initial public filing of the prospectus, certain of our employees, including our executive officers, and/or directors may enter into written trading plans that are intended to comply with Rule 10b5-1 under the Exchange Act. Sales under these trading plans would not be permitted until the expiration of the lock-up agreements relating to the offering described above.

Rule 144

Affiliate resales of restricted securities

In general, beginning 90 days after the effective date of the registration statement of which this prospectus is a part, a person who is an affiliate of ours, or who was an affiliate at any time during the 90 days before a sale, who has beneficially owned shares of our common stock for at least six months would be entitled to sell (subject to the lock-up agreement referred to above, if applicable) in "broker's transactions" or certain "riskless principal transactions" or to market makers, a number of shares within any three-month period that does not exceed the greater of:

- 1% of the number of shares of our common stock then outstanding, which will equal approximately shares (or shares if the underwriters exercise in full their option to purchase additional shares) of our common stock immediately after this offering; or
- the average weekly trading volume in shares of our common stock on Nasdaq during the four calendar weeks preceding the filing of a notice on Form 144 with respect to such sale.

An "affiliate" is a person that directly, or indirectly through one or more intermediaries, controls or is controlled by, or is under common control with an issuer. Affiliate resales under Rule 144 are also subject to the availability of current public information about us. In addition, if the number of shares being sold under Rule 144 by an affiliate during any three-month period exceeds 5,000 shares or has an aggregate sale price in excess of $50,000, the seller must file a notice on Form 144 with the SEC concurrently with either the placing of a sale order with the broker or the execution directly with a market maker.

Non-affiliate resales of restricted securities

In general, beginning 90 days after the effective date of the registration statement of which this prospectus is a part, a person who is not an affiliate of ours at the time of sale, and has not been an affiliate at any time during the three months preceding a sale, and who has beneficially owned shares of our common stock for at least six months but less than a year, is entitled to sell such shares subject only to the availability of current public information about us (as well as the lock-up agreement referred to above, if applicable). If such person has held our shares for at least one year, such person can resell under Rule 144(b)(1) without regard to any Rule 144 restrictions, including the 90-day public company requirement and the current public information requirement.

Non-affiliate resales are not subject to the manner of sale, volume limitation or notice filing provisions of Rule 144.

Rule 701

In general, under Rule 701, any of an issuer's employees, directors, officers, consultants or advisors who purchases shares from the issuer in connection with a compensatory stock or option plan or other written agreement before the effective date of a registration statement under the Securities Act is entitled to sell such shares 90 days after such effective date in reliance on Rule 144. An affiliate of the issuer can resell shares in reliance on Rule 144 without having to comply with the holding period requirement, and non-affiliates of the issuer can resell shares in reliance on Rule 144 without having to comply with the current public information and holding period requirements.

The SEC has indicated that Rule 701 will apply to typical options granted by an issuer before it becomes subject to the reporting requirements of the Exchange Act, along with the shares acquired upon exercise of such options, including exercises after an issuer becomes subject to the reporting requirements of the Exchange Act.

Equity plans

We intend to file one or more registration statements on Form S-8 under the Securities Act to register all shares of our common stock subject to outstanding options, RSUs and shares of our common stock issued or issuable

under our incentive plans. We expect to file the registration statement covering shares offered pursuant to our incentive plans shortly after the date of this prospectus, permitting the resale of such shares by non-affiliates in the public market without restriction under the Securities Act and the sale by affiliates in the public market, subject to compliance with the resale provisions of Rule 144.

Registration rights

Upon the completion of this offering, the holders of shares of our common stock or their transferees, after giving effect to the automatic conversion of all of the shares of our convertible preferred stock into shares of our common stock, will be entitled to various rights with respect to the registration of these shares under the Securities Act. Registration of these shares under the Securities Act would result in these shares becoming fully tradable without restriction under the Securities Act immediately upon the effectiveness of the registration, except for shares purchased by affiliates. See the section titled “Description of capital stock—Registration rights” for additional information. Shares covered by a registration statement will be eligible for sale in the public market upon the expiration or release from the terms of the lock-up agreement.

Certain material U.S. federal income tax consequences to non U.S. holders

The following is a summary of the material U.S. federal income tax considerations relating to the purchase, ownership and disposition of our common stock by Non-U.S. Holders (defined below). This summary does not purport to be a complete analysis of all the potential tax considerations relevant to Non-U.S. Holders of our common stock. This summary is based upon the Code, the Treasury regulations promulgated or proposed thereunder and administrative and judicial interpretations thereof, all as of the date hereof and all of which are subject to change at any time, possibly on a retroactive basis.

This discussion is limited to Non-U.S. Holders that hold our common stock as a "capital asset" within the meaning of Section 1221 of the Code (generally, property held for investment). For purposes of this summary, a "Non-U.S. Holder" means a beneficial owner of common stock that for U.S. federal income tax purposes is not classified as a partnership and is not:

- an individual who is a citizen or resident of the United States;
- a corporation or any other organization taxable as a corporation for U.S. federal income tax purposes, created or organized in or under the laws of the United States, any state thereof or the District of Columbia;
- an estate, the income of which is included in gross income for U.S. federal income tax purposes regardless of its source; or
- a trust if (1) a U.S. court is able to exercise primary supervision over the trust's administration and one or more U.S. persons have the authority to control all of the trust's substantial decisions or (2) the trust has a valid election in effect under applicable U.S. Treasury regulations to be treated as a U.S. person.

This discussion does not address all U.S. federal income tax consequences relevant to a Non-U.S. Holder's particular circumstances, including the impact of the alternative minimum tax or the Medicare contribution tax on net investment income. In addition, it does not address consequences relevant to Non-U.S. Holders subject to special rules, including, without limitation:

- U.S. expatriates and former citizens or long term residents of the United States;
- persons holding our common stock as part of a hedge, straddle or other risk reduction strategy or as part of a conversion transaction or other integrated investment;
- banks, insurance companies and other financial institutions;
- brokers, dealers or traders in securities;
- "controlled foreign corporations," "passive foreign investment companies," and corporations that accumulate earnings to avoid U.S. federal income tax;
- partnerships or other entities or arrangements treated as partnerships or other pass-through entities for U.S. federal income tax purposes (and investors therein);
- tax-exempt organizations or governmental organizations;
- persons deemed to sell our common stock under the constructive sale provisions of the Code;
- persons who hold or receive our common stock pursuant to the exercise of any employee stock option or otherwise as compensation;
- tax-qualified retirement plans;
- persons who hold common stock that constitutes "qualified small business stock" under Section 1202 of the Code, or "Section 1244 stock" under Section 1244 of the Code;
- persons who acquired our common stock in a transaction subject to the gain rollover provisions of the Code (including Section 1045 of the Code);

- persons that acquired our common stock pursuant to the exercise of warrants or conversion rights under convertible instruments;
- persons who have elected to mark securities to market;
- persons that own, or have owned, actually or constructively, more than 5% of our common stock;
- "qualified foreign pension funds" as defined in Section 897(l)(2) of the Code and entities all of the interests of which are held by qualified foreign pension funds; and
- persons subject to special tax accounting rules as a result of any item of gross income with respect to our common stock being taken into account in an applicable financial statement.

If an entity that is classified as a partnership for U.S. federal income tax purposes holds our common stock, the tax treatment of persons treated as its partners for U.S. federal income tax purposes will generally depend upon the status of the partner and the activities of the partnership. Partnerships and other entities that are classified as partnerships for U.S. federal income tax purposes and persons holding our common stock through a partnership or other entity classified as a partnership for U.S. federal income tax purposes are urged to consult their own tax advisors.

There can be no assurance that the IRS will not challenge one or more of the tax consequences described herein, and we have not obtained, nor do we intend to obtain a ruling from the IRS with respect to the U.S. federal income tax consequences to a Non-U.S. Holder of the purchase, ownership or disposition of our common stock.

THIS SUMMARY IS FOR GENERAL INFORMATION ONLY AND IS NOT INTENDED TO BE TAX ADVICE. NON-U.S. HOLDERS ARE URGED TO CONSULT THEIR TAX ADVISORS CONCERNING THE U.S. FEDERAL INCOME TAXATION, STATE, LOCAL AND NON-U.S. TAXATION AND OTHER TAX CONSEQUENCES TO THEM OF THE PURCHASE, OWNERSHIP AND DISPOSITION OF OUR COMMON STOCK.

Distributions of our common stock

We do not currently expect to make distributions with respect to our common stock. If we make a distribution of cash or property with respect to our common stock, any such distributions generally will constitute dividends for U.S. federal income tax purposes to the extent of our current and accumulated earnings and profits, if any, as determined under U.S. federal income tax principles. If a distribution exceeds our current and accumulated earnings and profits, the excess will constitute a return of capital and will first reduce the holder's adjusted tax basis in our common stock, but not below zero. Any remaining excess will be treated as capital gain, subject to the tax treatment described below in "—Gain on Sale, Exchange or Other Taxable Disposition of Our Common Stock." Any such distribution would also be subject to the discussion below under the section titled "—Additional Withholding and Reporting Requirements."

Dividends paid to a Non-U.S. Holder generally will be subject to a 30% U.S. federal withholding tax unless such Non-U.S. Holder provides us or our agent, as the case may be, with the appropriate IRS Form W-8, such as:

- IRS Form W-8BEN or W-8BEN-E (or successor form) certifying, under penalties of perjury, a reduction in withholding under an applicable income tax treaty, or
- IRS Form W-8ECI (or successor form) certifying that a dividend paid on our common stock is not subject to withholding tax because it is effectively connected with a trade or business in the United States of the Non-U.S. Holder (in which case such dividend generally will be subject to regular graduated U.S. tax rates as described below).

The certification requirement described above must be provided to us or our agent prior to the payment of dividends and must be updated periodically. The certification also may require a Non-U.S. Holder that provides an IRS form or that claims treaty benefits to provide its U.S. taxpayer identification number. Special certification and other requirements apply in the case of certain Non-U.S. Holders that hold shares of our common stock through intermediaries or are pass-through entities for U.S. federal income tax purposes.

Each Non-U.S. Holder is urged to consult its own tax advisor about the specific methods for satisfying these requirements. A claim for exemption will not be valid if the person receiving the applicable form has actual knowledge or reason to know that the statements on the form are false.

If dividends are effectively connected with a trade or business in the United States of a Non-U.S. Holder (and, if required by an applicable income tax treaty, are attributable to a permanent establishment maintained by such Non-U.S. Holder in the United States), the Non-U.S. Holder, although exempt from the withholding tax described above (provided that the certifications described above are satisfied), generally will be subject to U.S. federal income tax on such dividends on a net income basis in the same manner as if it were a resident of the United States. In addition, if a Non-U.S. Holder is treated as a corporation for U.S. federal income tax purposes, the Non-U.S. Holder may be subject to an additional "branch profits tax" equal to 30% (unless reduced by an applicable income treaty) of its earnings and profits in respect of such effectively connected dividend income.

Non-U.S. Holders that do not timely provide us or our agent with the required certification, but which are eligible for a reduced rate of U.S. federal withholding tax pursuant to an income tax treaty, may obtain a refund or credit of any excess amount withheld by timely filing an appropriate claim for refund with the IRS.

Gain on sale, exchange or other taxable disposition of our common stock

Subject to the discussion below under the section titled "—Additional withholding and reporting requirements," in general, a Non-U.S. Holder will not be subject to U.S. federal income tax or withholding tax on gain realized upon such holder's sale, exchange or other taxable disposition of shares of our common stock, unless (1) such Non-U.S. Holder is an individual who is present in the United States for 183 days or more in the taxable year of disposition, and certain other conditions are met, (2) we are or have been a "United States real property holding corporation," as defined in the Code, or a USRPHC, at any time within the shorter of the five-year period preceding the disposition and the Non-U.S. Holder's holding period in the shares of our common stock, and certain other requirements are met, or (3) such gain is effectively connected with the conduct by such Non-U.S. Holder of a trade or business in the United States (and, if required by an applicable income tax treaty, is attributable to a permanent establishment maintained by such Non-U.S. Holder in the United States).

If the first exception applies, the Non-U.S. Holder generally will be subject to U.S. federal income tax at a rate of 30% (or at a reduced rate under an applicable income tax treaty) on the amount by which such Non-U.S. Holder's capital gains allocable to U.S. sources exceed capital losses allocable to U.S. sources during the taxable year of the disposition. If the third exception applies, the Non-U.S. Holder generally will be subject to U.S. federal income tax with respect to such gain on a net income basis in the same manner as if it were a resident of the United States and a Non-U.S. Holder that is a corporation for U.S. federal income tax purposes may also be subject to a branch profits tax with respect to any earnings and profits attributable to such gain at a rate of 30% (or at a reduced rate under an applicable income tax treaty).

With respect to the second exception, generally, a corporation is a USRPHC only if the fair market value of its U.S. real property interests (as defined in the Code) equals or exceeds 50% of the sum of the fair market value of its worldwide real property interests plus its other assets used or held for use in a trade or business. We believe that we are not, and do not anticipate becoming, a USRPHC. Even if we became a USRPHC, a Non-U.S. Holder would not be subject to U.S. federal income tax on a sale, exchange or other taxable disposition of our common stock by reason of our status as USRPHC so long as our common stock is regularly traded on an established securities market at any time during the calendar year in which the disposition occurs and such Non-U.S. Holder does not own and is not deemed to own (directly, indirectly or constructively) more than 5% of our common stock at any time during the shorter of the five year period ending on the date of disposition and the holder's holding period.

Additional withholding and reporting requirements

Sections 1471 through 1474 of the Code, and related Treasury Regulations, together with other Treasury Department and IRS guidance issued thereunder, and intergovernmental agreements, legislation, rules and other official guidance adopted pursuant to such intergovernmental agreements, commonly referred to as FATCA,

impose a U.S. federal withholding tax of 30% on certain payments, including dividends paid on our common stock, paid to (1) a "foreign financial institution" (as defined under FATCA) unless such institution furnishes proper documentation (typically on IRS Form W-8BEN-E) evidencing either (i) an exemption from FATCA withholding, (ii) its compliance (or deemed compliance) with specified due diligence, reporting, withholding and certification obligations under FATCA or (iii) residence in a jurisdiction that has entered into an intergovernmental agreement with the United States relating to FATCA and compliance with the diligence and reporting requirements of the intergovernmental agreement and local implementing rules; or (2) a "non-financial foreign entity" (as defined under FATCA) that does not furnish proper documentation, typically on IRS Form W-8BEN-E, evidencing either (i) an exemption from FATCA or (ii) adequate information regarding substantial United States beneficial owners of such entity (if any). An intergovernmental agreement between the United States and an applicable foreign country may modify these requirements.

The IRS and the Department of Treasury have issued proposed regulations on which taxpayers may rely providing that these withholding rules will not apply to the gross proceeds of a sale or other disposition of shares of our common stock. Prospective investors should consult their own tax advisors regarding the effect of FATCA on their ownership and disposition of our common stock.

Backup withholding and information reporting

We must report annually to the IRS and to each Non-U.S. Holder the gross amount of the distributions on our common stock paid to the holder and the tax withheld, if any, with respect to the distributions. Non-U.S. Holders may have to comply with specific certification procedures (such as the provision of a properly completed W-8BEN or W-8BEN-E) to establish that the holder is not a United States person (as defined in the Code) in order to avoid backup withholding at the applicable rate, currently 24%, with respect to dividends on our common stock. Dividends paid to Non-U.S. Holders subject to the U.S. withholding tax, as described above under the section titled "—Distributions on our common stock," generally will be exempt from U.S. backup withholding.

Information reporting and backup withholding will generally apply to the proceeds of a disposition of our common stock by a Non-U.S. Holder effected by or through the U.S. office of any broker, U.S. or foreign, unless the holder certifies its status as a Non-U.S. Holder and satisfies certain other requirements, or otherwise establishes an exemption. Generally, information reporting and backup withholding will not apply to a payment of disposition proceeds to a Non-U.S. Holder where the transaction is effected outside the United States through a non-U.S. office of a broker. However, for information reporting purposes, dispositions effected through a non-U.S. office of a broker with substantial U.S. ownership or operations generally will be treated in a manner similar to dispositions effected through a U.S. office of a broker. Prospective investors should consult their own tax advisors regarding the application of the information reporting and backup withholding rules to them, including the availability of and procedure for obtaining an exemption from backup withholding.

Copies of information returns may be made available to the tax authorities of the country in which the Non-U.S. Holder resides or, in which the Non-U.S. Holder is incorporated, under the provisions of a specific treaty or agreement.

Backup withholding is not an additional tax. Any amounts withheld under the backup withholding rules from a payment to a Non-U.S. Holder can be refunded or credited against the Non-U.S. Holder's U.S. federal income tax liability, if any, provided that an appropriate claim is timely filed with the IRS.

Underwriting

We are offering the shares of common stock described in this prospectus through a number of underwriters. J.P. Morgan Securities LLC, Leerink Partners LLC, Piper Sandler & Co. and William Blair & Company, L.L.C. are acting as joint book-running managers of the offering and as representatives of the underwriters. We have entered into an underwriting agreement with the underwriters. Subject to the terms and conditions of the underwriting agreement, we have agreed to sell to the underwriters, and each underwriter has severally agreed to purchase, at the public offering price less the underwriting discounts and commissions set forth on the cover page of this prospectus, the number of shares of common stock listed next to its name in the following table:

Name	Number of shares
J.P. Morgan Securities LLC	
Leerink Partners LLC	
Piper Sandler & Co.	
William Blair & Company, L.L.C.	
Total	

The underwriters are committed to purchase all the common stock offered by us if they purchase any shares. The underwriting agreement also provides that if an underwriter defaults, the purchase commitments of non-defaulting underwriters may also be increased or the offering may be terminated.

The underwriters propose to offer the common stock directly to the public at the initial public offering price set forth on the cover page of this prospectus and to certain dealers at that price less a concession not in excess of $ per share. After the initial offering of the shares to the public, if all of the common stock is not sold at the initial public offering price, the underwriters may change the offering price and the other selling terms. Sales of any shares made outside of the United States may be made by affiliates of the underwriters.

The underwriters have an option to buy up to additional shares of common stock from us to cover sales of shares by the underwriters which exceed the number of shares specified in the table above. The underwriters have 30 days from the date of this prospectus to exercise this option to purchase additional shares. If any shares are purchased with this option to purchase additional shares, the underwriters will purchase shares in approximately the same proportion as shown in the table above. If any additional shares of common stock are purchased, the underwriters will offer the additional shares on the same terms as those on which the shares are being offered.

The underwriting fee is equal to the public offering price per share of common stock less the amount paid by the underwriters to us per share of common stock. The underwriting fee is $ per share. The following table shows the per share and total underwriting discounts and commissions to be paid to the underwriters assuming both no exercise and full exercise of the underwriters' option to purchase additional shares.

	Without option to purchase additional shares exercise	With full option to purchase additional shares exercise
Per Share	$	$
Total	$	$

We estimate that the total expenses of this offering, including registration, filing and listing fees, printing fees and legal and accounting expenses, but excluding the underwriting discounts and commissions, will be approximately $. We have also agreed to reimburse the underwriters for expenses relating to the clearance of this offering with the Financial Industry Regulatory Authority, Inc., up to $.

A prospectus in electronic format may be made available on the web sites maintained by one or more underwriters, or selling group members, if any, participating in the offering. The underwriters may agree to allocate a number

of shares to underwriters and selling group members for sale to their online brokerage account holders. Internet distributions will be allocated by the representatives to underwriters and selling group members that may make Internet distributions on the same basis as other allocations.

We have agreed that we will not (i) offer, pledge, sell, contract to sell, sell any option or contract to purchase, purchase any option or contract to sell, grant any option, right or warrant to purchase, lend or otherwise transfer or dispose of, directly or indirectly, or submit to, or file with, the Securities and Exchange Commission, or the SEC, a registration statement under the Securities Act relating to, any shares of our common stock or securities convertible into or exercisable or exchangeable for any shares of our common stock, or publicly disclose the intention to make any offer, sale, pledge, loan, disposition or filing, or (ii) enter into any hedging, swap or other arrangement that transfers all or a portion of the economic consequences associated with the ownership of any shares of common stock or any such other securities (regardless of whether any of these transactions are to be settled by the delivery of shares of common stock or such other securities, in cash or otherwise), without the prior written consent of J.P. Morgan Securities LLC and Leerink Partners LLC for a period of 180 days after the date of this prospectus, other than the shares of our common stock to be sold in this offering.

The restrictions described above do not apply to: (i) the issuance of shares of common stock or securities convertible into or exercisable for shares of our common stock pursuant to the conversion or exchange of convertible or exchangeable securities or the exercise of warrants or options (including net exercise) or the settlement of RSUs (including net settlement), in each case outstanding on the date of the underwriting agreement and described in this prospectus; (ii) grants of stock options, stock awards, restricted stock, RSUs, or other equity awards and the issuance of shares of our common stock or securities convertible into or exercisable or exchangeable for shares of our common stock (whether upon the exercise of stock options or otherwise) to our employees, officers, directors, advisors, or consultants pursuant to the terms of an equity compensation plan in effect as of the closing of this offering and described in this prospectus, provided that such recipients enter into a lock-up agreement with the underwriters; (iii) the issuance of up to % of the outstanding shares of our common stock, or securities convertible into, exercisable for, or which are otherwise exchangeable for, our common stock, immediately following the closing of this offering, in acquisitions or other similar strategic transactions, provided that such recipients enter into a lock-up agreement with the underwriters; or (iv) our filing of any registration statement on Form S-8 relating to securities granted or to be granted pursuant to any plan in effect on the date of the underwriting agreement and described in this prospectus or any assumed benefit plan pursuant to an acquisition or similar strategic transaction.

Our directors and executive officers, and substantially all of our shareholders (such persons, the “lock-up parties”) have entered into lock-up agreements with the underwriters prior to the commencement of this offering pursuant to which each lock-up party, with limited exceptions, for a period of 180 days after the date of this prospectus (such period, the “restricted period”), may not (and may not cause any of their direct or indirect affiliates to), without the prior written consent of J.P. Morgan Securities LLC and Leerink Partners LLC, (1) offer, pledge, sell, contract to sell, sell any option or contract to purchase, purchase any option or contract to sell, grant any option, right or warrant to purchase, lend or otherwise transfer or dispose of, directly or indirectly, any shares of our common stock or any securities convertible into or exercisable or exchangeable for our common stock (including, without limitation, common stock or such other securities which may be deemed to be beneficially owned by such lock-up parties in accordance with the rules and regulations of the SEC and securities which may be issued upon exercise of a stock option or warrant (collectively with the common stock, the “lock-up securities”)), (2) enter into any hedging, swap or other agreement or transaction that transfers, in whole or in part, any of the economic consequences of ownership of the lock-up securities, whether any such transaction described in clause (1) or (2) above is to be settled by delivery of lock-up securities, in cash or otherwise, (3) make any demand for, or exercise any right with respect to, the registration of any lock-up securities, or (4) publicly disclose the intention to do any of the foregoing. Such persons or entities have further acknowledged that these undertakings preclude them from engaging, during the restricted period, in any hedging or other transactions or arrangements (including, without limitation, any short sale or the purchase or sale of, or entry into, any put or call option, or combination thereof, forward, swap or any other derivative transaction or instrument, however described or defined) designed or intended, or which could reasonably be expected to lead to or result in, a sale or disposition

or transfer (whether by the lock-up party or by any other person) of any economic consequences of ownership, in whole or in part, directly or indirectly, of any lock-up securities, whether any such transaction or arrangement (or instrument provided for thereunder) would be settled by delivery of lock-up securities, in cash or otherwise.

The restrictions described in the immediately preceding paragraph and contained in the lock-up agreements between the underwriters and the lock-up parties do not apply, subject in certain cases to various conditions, to certain transactions, including (a) transfers of lock-up securities: (i) as a bona fide gift or gifts, or for bona fide estate planning purposes, or as a charitable contribution; (ii) by will, other testamentary document or intestacy; (iii) to any immediate family member of the lock-up party or any trust for the direct or indirect benefit of the lock-up party or any immediate family member, or if the lock-up party is a trust, to a trustor, trustee (or co-trustee) or beneficiary of the trust or to the estate of a beneficiary of the trust; (iv) to a corporation, partnership, limited liability company or other entity of which the lock-up party and/or its immediate family members are the legal and beneficial owner of all of the outstanding equity securities or similar interests; (v) to a nominee or custodian of a person or entity to whom a disposition or transfer would be permissible under clauses (i) through (iv); (vi) in the case of a corporation, partnership, limited liability company, trust or other business entity, (A) to another corporation, partnership, limited liability company, trust or other business entity that is an affiliate (as defined in Rule 405 promulgated under the Securities Act of 1933) of the lock-up party, or to any investment fund or other entity controlling, controlled by, managing or managed by or under common control with the lock-up party or its affiliates (including, for the avoidance of doubt, where the lock-up party is a partnership, to its general partner or a successor partnership or fund, or any other funds managed by such general partnership, partnership or fund) or (B) as part of a distribution, transfer or disposition without consideration to direct or indirect members, retired members, shareholders, partners, former partners, beneficiaries or other equity holders of the lock-up party; (vii) by operation of law or pursuant to an order of a court or regulatory agency (including a qualified domestic order, divorce settlement, divorce decree or separation agreement); (viii) to us from an employee or other service provider of ours upon death, disability or termination of employment or service relationship, in each case, of such employee or service provider, including without limitation, pursuant to a right of first refusal or an option to repurchase that we have with respect to transfers of such lock-up securities or other securities of ours; (ix) as part of a sale or transfer of lock-up securities acquired in this offering or in open market transactions after the completion of this offering; (x) to us in connection with the vesting, settlement or exercise of restricted stock units, options, warrants or other rights to purchase shares of our common stock (including, in each case, and without limitation, by way of “net” or “cashless” exercise), including, without limitation, for the payment of exercise price and tax and remittance payments due as a result of the vesting, settlement, or exercise of such restricted stock units, options, warrants or rights; or (xi) pursuant to a bona fide third-party tender offer, merger, consolidation or other similar transaction approved by our board of directors and made to all shareholders involving a change in control, provided that if such transaction is not completed, all such lock-up securities would remain subject to the restrictions in the immediately preceding paragraph; (b) exercise of outstanding options, settlement of restricted stock units or other equity awards, or exercise of warrants pursuant to plans or other equity compensation arrangements described in this prospectus, provided that any lock-up securities received upon such exercise, vesting or settlement would be subject to restrictions similar to those in the immediately preceding paragraph; (c) conversion of outstanding preferred stock, warrants to acquire preferred stock, or convertible securities into shares of our common stock or warrants to acquire shares of our common stock, provided that any common stock or warrant received upon such conversion would be subject to restrictions similar to those in the immediately preceding paragraph; and (d) the establishment or modification of trading plans under Rule 10b5-1 under the Exchange Act for the transfer of lock-up securities, provided that (1) such plans do not provide for the transfer of lock-up securities during the restricted period and (2) any public announcement or filing under the Exchange Act regarding such plan includes the restrictions set forth in the immediately preceding paragraph.

J.P. Morgan Securities LLC and Leerink Partners LLC, in their sole discretion, may release the securities subject to any of the lock-up agreements with the underwriters described above, in whole or in part at any time.

We have agreed to indemnify the underwriters against certain liabilities, including liabilities under the Securities Act.

We have applied to have our common stock approved for listing on the Nasdaq Global Market under the symbol “CAMP.”

In connection with this offering, the underwriters may engage in stabilizing transactions, which involves making bids for, purchasing and selling shares of common stock in the open market for the purpose of preventing or retarding a decline in the market price of the common stock while this offering is in progress. These stabilizing transactions may include making short sales of common stock, which involves the sale by the underwriters of a greater number of shares of common stock than they are required to purchase in this offering, and purchasing shares of common stock on the open market to cover positions created by short sales. Short sales may be “covered” shorts, which are short positions in an amount not greater than the underwriters’ option to purchase additional shares referred to above, or may be “naked” shorts, which are short positions in excess of that amount. The underwriters may close out any covered short position either by exercising their option to purchase additional shares, in whole or in part, or by purchasing shares in the open market. In making this determination, the underwriters will consider, among other things, the price of shares available for purchase in the open market compared to the price at which the underwriters may purchase shares through the option to purchase additional shares. A naked short position is more likely to be created if the underwriters are concerned that there may be downward pressure on the price of the common stock in the open market that could adversely affect investors who purchase in this offering. To the extent that the underwriters create a naked short position, they will purchase shares in the open market to cover the position.

The underwriters have advised us that, pursuant to Regulation M of the Securities Act, they may also engage in other activities that stabilize, maintain or otherwise affect the price of the common stock, including the imposition of penalty bids. This means that if the representatives of the underwriters purchase common stock in the open market in stabilizing transactions or to cover short sales, the representatives can require the underwriters that sold those shares as part of this offering to repay the underwriting discount received by them.

These activities may have the effect of raising or maintaining the market price of the common stock or preventing or retarding a decline in the market price of the common stock, and, as a result, the price of the common stock may be higher than the price that otherwise might exist in the open market. If the underwriters commence these activities, they may discontinue them at any time. The underwriters may carry out these transactions on the Nasdaq Global Market, in the over-the-counter market or otherwise.

Prior to this offering, there has been no public market for our common stock. The initial public offering price will be determined by negotiations between us and the representatives of the underwriters. In determining the initial public offering price, we and the representatives of the underwriters expect to consider a number of factors including:

- the information set forth in this prospectus and otherwise available to the representatives;
- our prospects and the history and prospects for the industry in which we compete;
- an assessment of our management;
- our prospects for future earnings;
- the general condition of the securities markets at the time of this offering;
- the recent market prices of, and demand for, publicly traded common stock of generally comparable companies; and
- other factors deemed relevant by the underwriters and us.

Neither we nor the underwriters can assure investors that an active trading market will develop for our common stock, or that the shares will trade in the public market at or above the initial public offering price.

Other than in the United States, no action has been taken by us or the underwriters that would permit a public offering of the securities offered by this prospectus in any jurisdiction where action for that purpose is required. The securities offered by this prospectus may not be offered or sold, directly or indirectly, nor may this prospectus

or any other offering material or advertisements in connection with the offer and sale of any such securities be distributed or published in any jurisdiction, except under circumstances that will result in compliance with the applicable rules and regulations of that jurisdiction. Persons into whose possession this prospectus comes are advised to inform themselves about and to observe any restrictions relating to the offering and the distribution of this prospectus. This prospectus does not constitute an offer to sell or a solicitation of an offer to buy any securities offered by this prospectus in any jurisdiction in which such an offer or a solicitation is unlawful.

Notice to prospective investors in the European Economic Area

In relation to each Member State of the European Economic Area, each a Relevant State, no shares of our common stock have been offered or will be offered pursuant to the offering to the public in that Relevant State prior to the publication of a prospectus in relation to the shares of our common stock which has been approved by the competent authority in that Relevant State or, where appropriate, approved in another Relevant State and notified to the competent authority in that Relevant State, all in accordance with the Prospectus Regulation, except that offers of Shares may be made to the public in that Relevant State other than at any time under the following exemptions under the Prospectus Regulation:

(a) to any legal entity which is a qualified investor as defined under the Prospectus Regulation;

(b) to fewer than 150 natural or legal persons (other than qualified investors as defined under the Prospectus Regulation), subject to obtaining the prior consent of the underwriters for any such offer; or

(c) in any other circumstances falling within Article 1(4) of the Prospectus Regulation,

provided that no such offer of shares of common stock shall require us or any underwriter to publish a prospectus pursuant to Article 3 the Prospectus Regulation or supplement a prospectus pursuant to Article 23 of the Prospectus Regulation and each person who initially acquires any shares of common stock or to whom any offer is made will be deemed to have represented, acknowledged and agreed to and with each of the underwriters and us that it is a “qualified investor” within the meaning of Article 2(e) of the Prospectus Regulation.

In the case of any shares of common stock being offered to a financial intermediary as that term is used in the Prospectus Regulation, each such financial intermediary will be deemed to have represented, acknowledged and agreed that the shares of common stock acquired by it in the offer have not been acquired on a non-discretionary basis on behalf of, nor have they been acquired with a view to their offer or resale to, persons in circumstances which may give rise to an offer of any shares of common stock to the public other than their offer or resale in a Relevant State to qualified investors as so defined or in circumstances in which the prior consent of the underwriters has been obtained to each such proposed offer or resale.

For the purposes of this provision, the expression an “offer to the public” in relation to any shares of common stock in any Relevant State means the communication in any form and by any means of sufficient information on the terms of the offer and any Shares to be offered so as to enable an investor to decide to purchase or subscribe for any Shares, and the expression “Prospectus Regulation” means Regulation (EU) 2017/1129.

Notice to prospective investors in the United Kingdom

No shares of common stock have been offered or will be offered pursuant to the offering to the public in the United Kingdom prior to the publication of a prospectus in relation to the shares of our common stock which either (i) has been approved by the Financial Conduct Authority or (ii) is to be treated as if it had been approved by the Financial Conduct Authority in accordance with the transitional provisions in Article 74 (transitional provisions) of the Prospectus (Amendment etc.) (EU Exit) Regulations 2019/1234, except that the share of our common stock may be offered to the public in the United Kingdom at any time:

(a) to any legal entity which is a qualified investor as defined under Article 2 of the UK Prospectus Regulation;

(b) to fewer than 150 natural or legal persons (other than qualified investors as defined under Article 2 of the UK Prospectus Regulation), subject to obtaining the prior consent of representatives for any such offer; or

(c) in any other circumstances falling within Section 86 of the FSMA,

provided that no such offer of the share of our common stock shall require us or any representative to publish a prospectus pursuant to Section 85 of the FSMA or supplement a prospectus pursuant to Article 23 of the UK Prospectus Regulation.

For the purposes of this provision, the expression an "offer to the public" in relation to the shares of our common stock in the United Kingdom means the communication in any form and by any means of sufficient information on the terms of the offer and any shares of our common stock to be offered so as to enable an investor to decide to purchase or subscribe for any shares of our common stock and the expression "UK Prospectus Regulation" means Regulation (EU) 2017/1129 as it forms part of domestic law by virtue of the European Union (Withdrawal) Act 2018.

In addition, in the United Kingdom, this document is being distributed only to, and is directed only at, and any offer subsequently made may only be directed at persons who are "qualified investors" (as defined in the Prospectus Regulation) (i) who have professional experience in matters relating to investments falling within Article 19(5) of the Financial Services and Markets Act 2000 (Financial Promotion) Order 2005, as amended, or the Order, and/or (ii) who are high net worth companies (or persons to whom it may otherwise be lawfully communicated) falling within Article 49(2)(a) to (d) of the Order (all such persons together being referred to as "relevant persons") or otherwise in circumstances which have not resulted and will not result in an offer to the public of the Shares in the United Kingdom within the meaning of the Financial Services and Markets Act 2000.

Any person in the United Kingdom that is not a relevant person should not act or rely on the information included in this document or use it as basis for taking any action. In the United Kingdom, any investment or investment activity that this document relates to may be made or taken exclusively by relevant persons.

Notice to prospective investors in Canada

The shares of common stock may be sold only to purchasers purchasing, or deemed to be purchasing, as principal that are accredited investors, as defined in National Instrument 45-106 Prospectus Exemptions or subsection 73.3(1) of the Securities Act (Ontario), and are permitted clients, as defined in National Instrument 31-103 Registration Requirements, Exemptions and Ongoing Registrant Obligations. Any resale of the shares of common stock must be made in accordance with an exemption from, or in a transaction not subject to, the prospectus requirements of applicable securities laws.

Securities legislation in certain provinces or territories of Canada may provide a purchaser with remedies for rescission or damages if this prospectus (including any amendment thereto) contains a misrepresentation, provided that the remedies for rescission or damages are exercised by the purchaser within the time limit prescribed by the securities legislation of the purchaser's province or territory. The purchaser should refer to any applicable provisions of the securities legislation of the purchaser's province or territory for particulars of these rights or consult with a legal advisor.

Pursuant to section 3A.3 of National Instrument 33-105 Underwriting Conflicts, or NI 33-105, the underwriters are not required to comply with the disclosure requirements of NI 33-105 regarding underwriter conflicts of interest in connection with this offering.

Notice to prospective investors in Switzerland

The shares of common stock may not be publicly offered in Switzerland and will not be listed on the SIX Swiss Exchange, or the SIX, or on any other stock exchange or regulated trading facility in Switzerland. This document does not constitute a prospectus within the meaning of, and has been prepared without regard to the disclosure standards for issuance prospectuses under art. 652a or art. 1156 of the Swiss Code of Obligations or the disclosure standards for listing prospectuses under art. 27 ff. of the SIX Listing Rules or the listing rules of any other stock exchange or regulated trading facility in Switzerland. Neither this document nor any other offering or

marketing material relating to the shares of common stock or the offering may be publicly distributed or otherwise made publicly available in Switzerland.

Neither this document nor any other offering or marketing material relating to the offering, us, or the shares of common stock have been or will be filed with or approved by any Swiss regulatory authority. In particular, this document will not be filed with, and the offer of shares of common stock will not be supervised by, the Swiss Financial Market Supervisory Authority, or FINMA, and the offer of shares of common stock has not been and will not be authorized under the Swiss Federal Act on Collective Investment Schemes, or CISA. The investor protection afforded to acquirers of interests in collective investment schemes under the CISA does not extend to acquirers of shares of common stock.

Notice to prospective investors in Hong Kong

The shares of common stock have not been offered or sold, and will not be offered or sold, in Hong Kong, by means of any document, other than (a) to "professional investors" as defined in the Securities and Futures Ordinance (Cap. 571 of the laws of Hong Kong), or the SFO, and any rules made thereunder; or (b) in other circumstances which do not result in the document being a "prospectus" as defined in the Companies (Winding Up and Miscellaneous Provisions) Ordinance (Cap. 32) of Hong Kong, or the CO, or which do not constitute an offer to the public within the meaning of the CO. No advertisement, invitation or document relating to the shares of common stock has been or may be issued or has been or may be in the possession of any person for the purposes of issue, whether in Hong Kong or elsewhere, which is directed at, or the contents of which are likely to be accessed or read by, the public of Hong Kong (except if permitted to do so under the securities laws of Hong Kong) other than with respect to shares of common stock which are or are intended to be disposed of only to persons outside Hong Kong or only to "professional investors" as defined in the SFO and any rules made thereunder.

Notice to prospective investors in Singapore

Each representative has acknowledged that this prospectus has not been registered as a prospectus with the Monetary Authority of Singapore. Accordingly, each representative has represented and agreed that it has not offered or sold any shares of common stock or caused the shares of common stock to be made the subject of an invitation for subscription or purchase and will not offer or sell any shares of common stock or cause the shares of common stock to be made the subject of an invitation for subscription or purchase, and has not circulated or distributed, nor will it circulate or distribute, this prospectus or any other document or material in connection with the offer or sale, or invitation for subscription or purchase, of the shares of common stock, whether directly or indirectly, to any person in Singapore other than:

(a) to an institutional investor (as defined in Section 4A of the Securities and Futures Act (Chapter 289) of Singapore, as modified or amended from time to time, or the SFA) pursuant to Section 274 of the SFA;

(b) to a relevant person (as defined in Section 275(2) of the SFA) pursuant to Section 275(1) of the SFA, or any person pursuant to Section 275(1A) of the SFA, and in accordance with the conditions specified in Section 275 of the SFA; or

(c) otherwise pursuant to, and in accordance with the conditions of, any other applicable provision of the SFA.

Where the shares of common stock are subscribed or purchased under Section 275 of the SFA by a relevant person which is:

(a) a corporation (which is not an accredited investor (as defined in Section 4A of the SFA)) the sole business of which is to hold investments and the entire share capital of which is owned by one or more individuals, each of whom is an accredited investor; or

(b) a trust (where the trustee is not an accredited investor) whose sole purpose is to hold investments and each beneficiary of the trust is an individual who is an accredited investor, securities or securities-based derivatives contracts (each term as defined in Section 2(1) of the SFA) of that corporation or the beneficiaries'

rights and interest (howsoever described) in that trust shall not be transferred within six months after that corporation or that trust has acquired the shares of common stock pursuant to an offer made under Section 275 of the SFA except:

(i) to an institutional investor or to a relevant person, or to any person arising from an offer referred to in Section 275(1A) or Section 276(4)(i)(B) of the SFA;

(ii) where no consideration is or will be given for the transfer;

(iii) where the transfer is by operation of law;

(iv) as specified in Section 276(7) of the SFA; or

(v) as specified in Regulation 37A of the Securities and Futures (Offers of Investments) (Securities and Securities-based Derivatives Contracts) Regulations 2018.

Singapore SFA Product Classification—In connection with Section 309B of the SFA and the CMP Regulations 2018, unless otherwise specified before an offer of the shares of common stock, we have determined, and hereby notify all relevant persons (as defined in Section 309A(1) of the SFA), that the shares of common stock are "prescribed capital markets products" (as defined in the CMP Regulations 2018) and Excluded Investment Products (as defined in MAS Notice SFA 04-N12: Notice on the Sale of Investment Products and MAS Notice FAA-N16: Notice on Recommendations on Investment Products).

Notice to prospective investors in Japan

The shares of common stock have not been and will not be registered pursuant to Article 4, Paragraph 1 of the Financial Instruments and Exchange Act. Accordingly, none of the shares of common stock nor any interest therein may be offered or sold, directly or indirectly, in Japan or to, or for the benefit of, any "resident" of Japan (which term as used herein means any person resident in Japan, including any corporation or other entity organized under the laws of Japan), or to others for re-offering or resale, directly or indirectly, in Japan or to or for the benefit of a resident of Japan, except pursuant to an exemption from the registration requirements of, and otherwise in compliance with, the Financial Instruments and Exchange Act and any other applicable laws, regulations and ministerial guidelines of Japan in effect at the relevant time.

Notice to prospective investors in the United Arab Emirates

The shares of common stock have not been, and are not being, publicly offered, sold, promoted or advertised in the United Arab Emirates (including the Dubai International Financial Centre, or the DIFC) other than in compliance with the laws of the United Arab Emirates (and the DIFC) governing the issue, offering and sale of securities. Further, this prospectus does not constitute a public offer of securities in the United Arab Emirates (including the DIFC) and is not intended to be a public offer. This prospectus has not been approved by or filed with the Central Bank of the United Arab Emirates, the Securities and Commodities Authority or the Dubai Financial Services Authority, or the DFSA.

Notice to prospective investors in Israel

This prospectus does not constitute a prospectus under the Israeli Securities Law, 5728—1968, or the Israeli Securities Law, and has not been filed with or approved by the Israel Securities Authority. In Israel, this prospectus is being distributed only to, and is directed only at, and any offer of the shares of common stock is directed only at, (i) a limited number of persons in accordance with the Israeli Securities Law and (ii) investors listed in the first addendum, or the Addendum, to the Israeli Securities Law, consisting primarily of joint investment in trust funds, provident funds, insurance companies, banks, portfolio managers, investment advisors, members of the Tel Aviv Stock Exchange, underwriters, venture capital funds, entities with equity in excess of NIS 50 million and "qualified individuals," each as defined in the Addendum (as it may be amended from time to time), or, collectively referred to as qualified investors (in each case, purchasing for their own account or, where permitted under the

Addendum, for the accounts of their clients who are investors listed in the Addendum). Qualified investors are required to submit written confirmation that they fall within the scope of the Addendum, are aware of the meaning of same and agree to it.

Notice to prospective investors in Australia

This prospectus:

(a) does not constitute a disclosure document or a prospectus under Chapter 6D.2 of the Corporations Act 2001 (Cth), or the Corporations Act;

(b) has not been, and will not be, lodged with the Australian Securities and Investments Commission, or ASIC, as a disclosure document for the purposes of the Corporations Act and does not purport to include the information required of a disclosure document for the purposes of the Corporations Act; and

(c) may only be provided in Australia to select investors who are able to demonstrate that they fall within one or more of the categories of investors, or Exempt Investors, available under section 708 of the Corporations Act.

The shares of common stock may not be directly or indirectly offered for subscription or purchased or sold, and no invitations to subscribe for or buy the shares of common stock may be issued, and no draft or definitive offering memorandum, advertisement or other offering material relating to any shares of common stock may be distributed in Australia, except where disclosure to investors is not required under Chapter 6D of the Corporations Act or is otherwise in compliance with all applicable Australian laws and regulations. By submitting an application for the shares of common stock, you represent and warrant to us that you are an Exempt Investor.

As any offer of shares of common stock under this document will be made without disclosure in Australia under Chapter 6D.2 of the Corporations Act, the offer of those securities for resale in Australia within 12 months may, under section 707 of the Corporations Act, require disclosure to investors under Chapter 6D.2 if none of the exemptions in section 708 applies to that resale. By applying for the shares of common stock you undertake to us that you will not, for a period of 12 months from the date of issue of the shares of common stock, offer, transfer, assign or otherwise alienate those shares of common stock to investors in Australia except in circumstances where disclosure to investors is not required under Chapter 6D.2 of the Corporations Act or where a compliant disclosure document is prepared and lodged with ASIC.

Notice to prospective investors in China

This prospectus will not be circulated or distributed in the PRC and the shares of common stock will not be offered or sold, and will not be offered or sold to any person for re-offering or resale directly or indirectly to any residents of the PRC except pursuant to any applicable laws and regulations of the PRC. Neither this prospectus nor any advertisement or other offering material may be distributed or published in the PRC, except under circumstances that will result in compliance with applicable laws and regulations.

Notice to prospective investors in Korea

The shares of common stock have not been and will not be registered under the Financial Investments Services and Capital Markets Act of Korea and the decrees and regulations thereunder, or the FSCMA, and the shares of common stock have been and will be offered in Korea as a private placement under the FSCMA. None of the shares of common stock may be offered, sold or delivered directly or indirectly, or offered or sold to any person for re-offering or resale, directly or indirectly, in Korea or to any resident of Korea except pursuant to the applicable laws and regulations of Korea, including the FSCMA and the Foreign Exchange Transaction Law of Korea and the decrees and regulations thereunder, or FETL. Furthermore, the purchaser of the shares of common stock shall comply with all applicable regulatory requirements (including but not limited to requirements under the FETL) in connection with the purchase of the shares of common stock. By the purchase of the shares of common stock,

the relevant holder thereof will be deemed to represent and warrant that if it is in Korea or is a resident of Korea, it purchased the shares of common stock pursuant to the applicable laws and regulations of Korea.

Notice to prospective investors in Saudi Arabia

This document may not be distributed in the Kingdom of Saudi Arabia except to such persons as are permitted under the Offers of Securities Regulations as issued by the board of the Saudi Arabian Capital Market Authority, or CMA, pursuant to resolution number 2-11-2004 dated 4 October 2004 as amended by resolution number 1-28-2008, as amended, or the CMA Regulations. The CMA does not make any representation as to the accuracy or completeness of this document and expressly disclaims any liability whatsoever for any loss arising from, or incurred in reliance upon, any part of this document. Prospective purchasers of the securities offered hereby should conduct their own due diligence on the accuracy of the information relating to the securities. If you do not understand the contents of this document, you should consult an authorized financial adviser.

Notice to prospective investors in the Dubai International Financial Centre

This document relates to an Exempt Offer in accordance with the Markets Rules 2012 of the DFSA. This document is intended for distribution only to persons of a type specified in the Markets Rules 2012 of the DFSA. It must not be delivered to, or relied on by, any other person. The DFSA has no responsibility for reviewing or verifying any documents in connection with Exempt Offers. The DFSA has not approved this prospectus supplement nor taken steps to verify the information set forth herein and has no responsibility for this document. The securities to which this document relates may be illiquid and/or subject to restrictions on their resale. Prospective purchasers of the securities offered should conduct their own due diligence on the securities. If you do not understand the contents of this document, you should consult an authorized financial advisor.

In relation to its use in the DIFC, this document is strictly private and confidential and is being distributed to a limited number of investors and must not be provided to any person other than the original recipient, and may not be reproduced or used for any other purpose. The interests in the securities may not be offered or sold directly or indirectly to the public in the DIFC.

Notice to prospective investors in Bermuda

Shares of common stock may be offered or sold in Bermuda only in compliance with the provisions of the Investment Business Act of 2003 of Bermuda which regulates the sale of securities in Bermuda. Additionally, non-Bermudian persons (including companies) may not carry on or engage in any trade or business in Bermuda unless such persons are permitted to do so under applicable Bermuda legislation.

Notice to prospective investors in the British Virgin Islands

The shares of common stock are not being, and may not be offered, to the public or to any person in the British Virgin Islands for purchase or subscription by or on behalf of the company. The shares of common stock may be offered to companies incorporated under the BVI Business Companies Act, 2004 (British Virgin Islands), or the BVI Companies, but only where the offer will be made to, and received by, the relevant BVI Company entirely outside of the British Virgin Islands.

Notice to prospective investors in Malaysia

No prospectus or other offering material or document in connection with the offer and sale of the shares of common stock has been or will be registered with the Securities Commission of Malaysia, or the Commission, for the Commission's approval pursuant to the Capital Markets and Services Act 2007. Accordingly, this prospectus and any other document or material in connection with the offer or sale, or invitation for subscription or purchase, of the shares of common stock may not be circulated or distributed, nor may the shares of common stock be offered or sold, or be made the subject of an invitation for subscription or purchase, whether directly or indirectly, to persons in Malaysia other than (i) a closed end fund approved by the Commission, (ii) a holder of

a Capital Markets Services Licence, (iii) a person who acquires the shares of common stock, as principal, if the offer is on terms that the shares of common stock may only be acquired at a consideration of not less than RM250,000 (or its equivalent in foreign currencies) for each transaction, (iv) an individual whose total net personal assets or total net joint assets with his or her spouse exceeds RM3 million (or its equivalent in foreign currencies), excluding the value of the primary residence of the individual, (v) an individual who has a gross annual income exceeding RM300,000 (or its equivalent in foreign currencies) per annum in the preceding twelve months, (vi) an individual who, jointly with his or her spouse, has a gross annual income of RM400,000 (or its equivalent in foreign currencies), per annum in the preceding twelve months, (vii) a corporation with total net assets exceeding RM10 million (or its equivalent in a foreign currencies) based on the last audited accounts, (viii) a partnership with total net assets exceeding RM10 million (or its equivalent in foreign currencies), (ix) a bank licensee or insurance licensee as defined in the Labuan Financial Services and Securities Act 2010, (x) an Islamic bank licensee or takaful licensee as defined in the Labuan Financial Services and Securities Act 2010, and (xi) any other person as may be specified by the Commission; provided that, in the each of the preceding categories (i) to (xi), the distribution of the shares of common stock is made by a holder of a Capital Markets Services Licence who carries on the business of dealing in securities. The distribution in Malaysia of this prospectus is subject to Malaysian laws. This prospectus does not constitute and may not be used for the purpose of public offering or an issue, offer for subscription or purchase, invitation to subscribe for or purchase any securities requiring the registration of a prospectus with the Commission under the Capital Markets and Services Act 2007.

Notice to prospective investors in Taiwan

The shares of common stock have not been and will not be registered with the Financial Supervisory Commission of Taiwan pursuant to relevant securities laws and regulations and may not be sold, issued or offered within Taiwan through a public offering or in circumstances which constitutes an offer within the meaning of the Securities and Exchange Act of Taiwan that requires a registration or approval of the Financial Supervisory Commission of Taiwan. No person or entity in Taiwan has been authorized to offer, sell, give advice regarding or otherwise intermediate the offering and sale of the shares of common stock in Taiwan.

Notice to prospective investors in South Africa

Due to restrictions under the securities laws of South Africa, the shares of common stock are not offered, and the offer shall not be transferred, sold, renounced or delivered, in South Africa or to a person with an address in South Africa, unless one or other of the following exemptions applies:

Section 96(1)(a): the offer, transfer, sale, renunciation or delivery is to:

(a) persons whose ordinary business, or part of whose ordinary business, is to deal in securities, as principal or agent;

(b) the South African Public Investment Corporation;

(c) persons or entities regulated by the Reserve Bank of South Africa;

(d) authorised financial service providers under South African law;

(e) financial institutions recognised as such under South African law;

(f) a wholly-owned subsidiary of any person or entity contemplated in (iii), (iv) or (v), acting as agent in the capacity of an authorized portfolio manager for a pension fund, or as manager for a collective investment scheme (in each case duly registered as such under South African law); or

(g) any combination of the persons in (i) to (vi); or

Section 96(1)(b): the total contemplated acquisition cost of the securities, for a single addressee acting as principal is equal to or greater than ZAR1,000,000 or such higher amount as may be promulgated by notice in the Government Gazette of South Africa pursuant to section 96(2)(a) of the South African Companies Act.

Information made available in this prospectus should not be considered as “advice” as defined in the South African Financial Advisory and Intermediary Services Act, 2002.

Other relationships

Certain of the underwriters and their affiliates have provided in the past to us and our affiliates and may provide from time to time in the future certain commercial banking, financial advisory, investment banking and other services for us and such affiliates in the ordinary course of their business, for which they have received and may continue to receive customary fees and commissions. In addition, from time to time, certain of the underwriters and their affiliates may effect transactions for their own account or the account of customers, and hold on behalf of themselves or their customers, long or short positions in our debt or equity securities or loans, and may do so in the future.

Legal matters

The validity of the shares of common stock offered by this prospectus will be passed upon for us by Ropes & Gray LLP, Boston, Massachusetts. Certain legal matters will be passed upon for the underwriters by Paul Hastings LLP, New York, New York.

Experts

The consolidated financial statements of CAMP4 Therapeutics Corporation as of December 31, 2023 and 2022 and for the years then ended appearing in this prospectus and registration statement have been audited by Ernst & Young, LLP, independent registered public accounting firm, as set forth in their report thereon (which contains an explanatory paragraph describing conditions that raise substantial doubt about the company's ability to continue as a going concern as described in Note 1 to the consolidated financial statements) appearing elsewhere herein, and are included in reliance upon such report given upon the authority of such firm as experts in accounting and auditing.

Where you can find more information

We have filed with the SEC a registration statement on Form S-1 under the Securities Act with respect to the shares of common stock being offered by this prospectus. This prospectus, which constitutes part of the registration statement, does not contain all of the information set forth in the registration statement or the exhibits and schedules filed therewith. For further information with respect to our company and the common stock offered by this prospectus, we refer you to the registration statement and the exhibits and schedules filed thereto.

Statements contained in this prospectus as to the contents of any contract or any other document that is filed as an exhibit to the registration statement are not necessarily complete, and each such statement is qualified in all respects by reference to the full text of such contract or other document filed as an exhibit to the registration statement. The SEC also maintains an internet website that contains reports, proxy statements and other information about registrants, like us, that file electronically with the SEC. The address of that site is www.sec.gov.

Upon the effectiveness of the registration statement, we will be subject to the informational requirements of the Exchange Act and, in accordance with the Exchange Act, will file reports, proxy and information statements and other information with the SEC. Such annual, quarterly and special reports, proxy and information statements and other information can be accessed at the SEC's website referenced above. We also intend to make this information available on the investor relations section of our website, which is located at www.camp4tx.com. Information contained on or accessible through our website is not a part of this prospectus, and the inclusion of our website address in this prospectus is an inactive textual reference only.

CAMP4 Therapeutics Corporation
Index to consolidated financial statements

Report of Independent Registered Public Accounting Firm

To the Stockholders and the Board of Directors of CAMP4 Therapeutics Corporation

Opinion on the Financial Statements

We have audited the accompanying consolidated balance sheets of CAMP4 Therapeutics Corporation (the Company) as of December 31, 2023 and 2022, the related consolidated statements of operations and comprehensive loss, convertible preferred stock and stockholders' deficit and cash flows for the years then ended, and the related notes (collectively referred to as the "consolidated financial statements"). In our opinion, the consolidated financial statements present fairly, in all material respects, the financial position of the Company at December 31, 2023 and 2022, and the results of its operations and its cash flows for the years then ended in conformity with U.S. generally accepted accounting principles.

The Company's Ability to Continue as a Going Concern

The accompanying consolidated financial statements have been prepared assuming that the Company will continue as a going concern. As discussed in Note 1 to the consolidated financial statements, the Company has suffered recurring losses from operations, and has stated that substantial doubt exists about the Company's ability to continue as a going concern. Management's evaluation of the events and conditions and management's plans regarding these matters are also described in Note 1. The consolidated financial statements do not include any adjustments that might result from the outcome of this uncertainty. Our opinion is not modified with respect to this matter.

Basis for Opinion

These financial statements are the responsibility of the Company's management. Our responsibility is to express an opinion on the Company's financial statements based on our audits. We are a public accounting firm registered with the Public Company Accounting Oversight Board (United States) (PCAOB) and are required to be independent with respect to the Company in accordance with the U.S. federal securities laws and the applicable rules and regulations of the Securities and Exchange Commission and the PCAOB.

We conducted our audits in accordance with the standards of the PCAOB and in accordance with auditing standards generally accepted in the United States of America. Those standards require that we plan and perform the audit to obtain reasonable assurance about whether the financial statements are free of material misstatement, whether due to error or fraud. The Company is not required to have, nor were we engaged to perform, an audit of its internal control over financial reporting. As part of our audits we are required to obtain an understanding of internal control over financial reporting but not for the purpose of expressing an opinion on the effectiveness of the Company's internal control over financial reporting. Accordingly, we express no such opinion.

Our audits included performing procedures to assess the risks of material misstatement of the financial statements, whether due to error or fraud, and performing procedures that respond to those risks. Such procedures included examining, on a test basis, evidence regarding the amounts and disclosures in the financial statements. Our audits also included evaluating the accounting principles used and significant estimates made by management, as well as evaluating the overall presentation of the financial statements. We believe that our audits provide a reasonable basis for our opinion.

/s/ Ernst & Young LLP

We have served as the Company's auditor since 2017.

Boston, Massachusetts
June 14, 2024

CAMP4 Therapeutics Corporation
Consolidated balance sheets
(In thousands, except share and per share amounts)

	December 31,	
Assets	**2023**	**2022**
Current assets:		
Cash and cash equivalents	$ 38,380	$ 83,190
Prepaid expenses and other current assets	1,633	1,219
Total current assets	40,013	84,409
Restricted cash	1,624	1,346
Property and equipment, net	4,797	5,648
Operating lease right-of-use assets	7,764	10,770
Finance lease right-of-use assets	748	376
Total assets	$ 54,946	$ 102,549
Liabilities, Convertible Preferred Stock and Stockholders' Deficit		
Current liabilities:		
Accounts payable	$ 1,042	$ 2,151
Accrued expenses and other current liabilities	3,302	3,425
Operating lease liabilities, current portion	2,704	2,227
Finance lease liabilities, current portion	354	143
Financing liability, current portion	405	—
Total current liabilities	7,807	7,946
Operating lease liabilities, net of current portion	8,487	9,880
Finance lease liabilities, net of current portion	148	145
Financing liability, net of current portion	85	—
Other long-term liabilities	2	2
Total liabilities	16,529	17,933
Commitments and contingencies (Note 7)		
Convertible preferred stock, $0.0001 par value per share; 149,673,284 shares authorized as of December 31, 2023 and 2022, 130,648,426 shares issued and outstanding as of December 31, 2023 and 2022; liquidation value of $162,885 as of December 31, 2023 and 2022	162,147	162,147
Stockholders' deficit:		
Common stock, $0.0001 par value per share; 210,000,000 shares authorized as of December 31, 2023 and 2022; 11,509,269, and 11,559,826 shares issued, 5,168,193 and 4,002,103 shares outstanding as of December 31, 2023 and 2022, respectively	1	1
Additional paid-in capital	36,231	33,139
Accumulated deficit	(159,962)	(110,671)
Total stockholders' deficit	(123,730)	(77,531)
Total liabilities, convertible preferred stock and stockholders' deficit	$ 54,946	$ 102,549

The accompanying notes are an integral part of these consolidated financial statements.

CAMP4 Therapeutics Corporation
Consolidated statements of operations and comprehensive loss
(In thousands, except for share and per share data)

	Year ended December 31,	
	2023	2022
Revenue		
Research and collaboration revenue	$ 350	$ —
Operating expenses		
Research and development	40,616	34,771
General and administrative	11,613	10,230
Total operating expenses	52,229	45,001
Loss from operations	(51,879)	(45,001)
Other income (expense), net:		
Interest income	2,808	904
Other expense	(220)	(95)
Total other income (expense), net	2,588	809
Net loss attributable to common stockholders and comprehensive loss	$ (49,291)	(44,192)
Net loss per share attributable to common stockholders, basic and diluted	$ (11.13)	$ (12.61)
Weighted average shares of common stock outstanding, basic and diluted	4,429,564	3,503,242

The accompanying notes are an integral part of these consolidated financial statements.

CAMP4 Therapeutics Corporation
Consolidated statements of convertible preferred stock and stockholders' deficit
(In thousands, except share amounts)

	Convertible preferred stock		Common stock		Additional paid-in capital	Accumulated deficit	Total stockholders' deficit
	Shares	Amount	Shares	Amount			
Balance at January 1, 2022	62,389,791	$ 61,952	3,017,624	$ 1	$ 31,707	$ (66,479)	$ (34,771)
Issuance of common stock	—	—	224,245	—	55	—	55
Issuance of Series B convertible preferred stock, net of issuance costs of $309	68,258,635	100,195	—	—	—	—	—
Vesting of restricted common stock	—	—	760,234	—	—	—	—
Stock-based compensation expense	—	—	—	—	1,377	—	1,377
Net loss	—	—	—	—	—	(44,192)	(44,192)
Balance at January 1, 2023	130,648,426	$ 162,147	4,002,103	$ 1	$ 33,139	$ (110,671)	$ (77,531)
Vesting of restricted common stock	—	—	683,390	—	—	—	—
Issuance of common stock	—	—	482,700	—	185	—	185
Stock-based compensation expense	—	—	—	—	2,907	—	2,907
Net loss	—	—	—	—	—	(49,291)	(49,291)
Balance at December 31, 2023	130,648,426	$ 162,147	5,168,193	$ 1	$ 36,231	$ (159,962)	$ (123,730)

The accompanying notes are an integral part of these consolidated financial statements.

CAMP4 Therapeutics Corporation
Consolidated statements of cash flows
(In thousands)

	Year ended December 31,	
	2023	2022
Operating Activities		
Net loss	$ (49,291)	$ (44,192)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation and amortization	1,678	878
Stock-based compensation expense	2,907	1,377
Non-cash lease expense	1,728	1,678
Non-cash interest expense	92	12
Changes in operating assets and liabilities:		
Prepaid and other current assets	(420)	(630)
Accounts payable	(1,115)	1,331
Accrued expenses and other liabilities	(136)	1,891
Operating lease assets and liabilities	402	(888)
Net cash used in operating activities	(44,155)	(38,543)
Investing Activities		
Purchases of property and equipment	(678)	(4,025)
Net cash used in investing activities	(678)	(4,025)
Financing Activities		
Proceeds from issuance of convertible preferred stock, net of issuance costs	—	100,195
Proceeds from exercise of common stock options	185	55
Proceeds from financing obligation, net of issuance costs	706	—
Principal payments on financing obligation	(268)	—
Principal payments on finance leases	(322)	(93)
Net cash provided by financing activities	301	100,157
Net (decrease) increase in cash, cash equivalent and restricted cash	(44,532)	57,589
Cash, cash equivalents and restricted cash at beginning of year	84,536	26,947
Cash, cash equivalents and restricted cash at end of period	$ 40,004	$ 84,536
Supplemental disclosure of cash flow information:		
Operating lease right-of-use asset obtained in exchange for lease liabilities	$ 1,397	$ 12,449
Finance lease right-of-use asset obtained in exchange for lease liabilities	$ 504	$ 369
Purchases of property and equipment in accounts payable and accrued expenses	$ 12	$ 295

The accompanying notes are an integral part of these consolidated financial statements.

CAMP4 Therapeutics Corporation
Notes to consolidated financial statements

1. Description of business and basis of presentation

Description of business

CAMP4 Therapeutics Corporation, formerly Marauder Therapeutics, Inc., and its subsidiary (collectively, the "Company"), is a clinical-stage biopharmaceutical company pioneering the discovery and development of regulatory RNA-based therapeutics with the goal of upregulating gene expression and restoring healthy protein levels to treat a broad range of genetic diseases. The Company is initially focusing on genetic diseases of the central nervous system and liver. The Company was organized in September 2015 and began operations in 2016.

Basis of presentation and principles of consolidation

The accompanying consolidated financial statements have been prepared in conformity with accounting principles generally accepted in the United States of America ("US GAAP"). Any reference in these notes to applicable guidance is meant to refer to the authoritative standards of US GAAP as found in the Accounting Standards Codification ("ASC") and Accounting Standards Updates ("ASU") of the Financial Accounting Standards Board ("FASB").

The Company's consolidated financial statements include the accounts of CAMP4 Therapeutics Corporation and its wholly owned subsidiary, CAMP4 Therapeutics Pty Ltd ("CAMP4 AUS"), which was established on September 15, 2023. All intercompany balances and transactions have been eliminated in consolidation.

Liquidity and going concern

As of December 31, 2023, the Company had approximately $38.4 million of cash and cash equivalents and working capital of approximately $32.2 million. The Company has a relatively limited operating history, and the revenue and income potential of the Company's business and market are unproven. The Company has experienced net losses and negative cash flows from operations since its inception and, as of December 31, 2023, the Company had an accumulated deficit of $160.0 million. During the year ended December 31, 2023, the Company incurred a net loss of $49.3 million and had negative cash flows from operations of $44.2 million. The Company will continue to incur significant costs and expenses related to its ongoing operations until it successfully develops, obtains regulatory approval for and gains market acceptance of a product candidate and achieves revenues adequate to support the Company's operations.

From inception to December 31, 2023, the Company has funded its operations primarily through the issuance of convertible preferred stock and revenues from its license and collaboration agreements. The Company's current capital resources, which consist of cash and cash equivalents, will not be sufficient to fund operations through at least the next twelve months from the date the accompanying consolidated financial statements are issued based on its current operating plan. As the Company continues to pursue its business plan, it expects to finance its operations through potential public or private equity offerings, debt financings or other capital sources, including current or potential future collaborations, licenses and other similar arrangements. However, there can be no assurance that any additional financing or strategic arrangements will be available to the Company on acceptable terms, if at all. If events or circumstances occur such that the Company does not obtain additional funding, it may be necessary to significantly reduce its scope of operations to reduce the current rate of spending through actions such as reductions in staff and the need to delay, limit, reduce or terminate product development or future commercialization efforts or grant rights to develop and market product candidates that it would otherwise prefer to develop and market itself, which could have a material adverse effect on the Company's business, results of operations or financial condition.

The accompanying consolidated financial statements have been prepared on a going concern basis, which contemplates the realization of assets and satisfaction of liabilities in the ordinary course of business. The financial

statements do not include any adjustments relating to the recoverability and classification of recorded asset amounts or the amounts and classification of liabilities that might result from the outcome of this uncertainty.

2. Summary of significant accounting policies

Use of estimates

The preparation of consolidated financial statements in conformity with US GAAP requires management to make estimates assumptions, and judgments that affect the reported amounts of assets, liabilities, expenses and related disclosures in the accompanying notes. The Company bases its estimates, assumptions and judgments on historical experience when available and on various factors that it believes to be reasonable under the circumstances as of the date of the accompanying consolidated financial statements, including the fair value of common stock, stock-based compensation expense, accrued expenses, lease accounting and the recoverability of the Company's net deferred tax assets and related valuation allowance. In addition, other factors may affect estimates, including the expected business and operational changes, the sensitivity and volatility associated with the assumptions used in developing estimates, and whether historical trends are expected to be representative of future trends. The estimation process often may yield a range of potentially reasonable estimates of the ultimate future outcomes, and management must select an amount that falls within that range of reasonable estimates. Actual results could differ materially from the estimates and assumptions used in the preparation of the accompanying consolidated financial statements under different assumptions or conditions.

Cash and cash equivalents

The Company considers all highly liquid investments and instruments with original maturities of 90 days or less that can be liquidated without prior notice or penalty to be cash equivalents. Cash equivalents primarily represent funds invested in readily available money market accounts. As of December 31, 2023 and 2022, the Company had cash and cash equivalents balances deposited at one major financial institution.

Restricted cash

In connection with its operating leases, the Company is required to maintain security deposits totaling $1.5 million, which were issued in the form of letters of credit with a bank. As of December 31, 2023 and 2022, the Company held cash in this amount in separate restricted bank accounts as collateral for the letters of credit. The restricted cash balance is classified as long-term restricted cash on the accompanying consolidated balance sheets. In addition, the Company held less than $0.1 million of cash in money market accounts as of each of December 31, 2023 and 2022 as collateral for the Company's credit card obligation and increased letter of credit due to an amendment to the leases.

The following table provides a reconciliation of cash, cash equivalents and restricted cash reported in the consolidated balance sheets to the corresponding amounts shown in the consolidated statements of cash flows:

	December 31,	
	2023	2022
Cash and cash equivalents	$38,380	$83,190
Restricted cash	1,624	1,346
Total cash, cash equivalents and restricted cash	$40,004	$84,536

Concentration of credit risks

Financial instruments that subject the Company to significant concentrations of credit risk consist primarily of cash and cash equivalents. The Company deposits cash and cash equivalents with high credit quality financial institutions in the United States. These deposits are held in checking and money market accounts and may, from time to time, exceed the federally insured amounts. The Company has not experienced any losses in such accounts.

The Company believes it is not exposed to any significant risk in its cash and cash equivalents. The primary objectives of the Company's investment portfolio are the preservation of capital and maintenance of liquidity.

The Company is subject to risks common to companies in the biopharmaceutical industry, including, but not limited to, risks related to the successful development and commercialization of product candidates, fluctuations in operating results and financial risks, the ability to successfully raise additional funds when needed, protection of proprietary rights and patent risks, patent litigation, compliance with government regulations, dependence on key personnel and collaboration partners, dependence on third-party manufacturers and competition from competing products in the marketplace.

Fair value measurements

The Company applies fair value measurements to record fair value adjustments to certain assets and liabilities and to determine fair value disclosures. Fair value is measured as the price that would be received to sell an asset or paid to transfer a liability in an orderly transaction between market participants at the measurement date. A fair value measurement assumes that the transaction to sell the asset or transfer the liability occurs in the principal market for the asset or liability or, in the absence of a principal market, the most advantageous market. A framework is used for measuring fair value utilizing a three-tier hierarchy that prioritizes the inputs to valuation techniques used to measure fair value. The hierarchy gives the highest priority to unadjusted quoted prices in active markets for identical assets or liabilities (Level 1) and the lowest priority to unobservable inputs (Level 3).

The three levels of the fair value hierarchy are as follows:

***Level* 1—**Observable inputs such as unadjusted quoted prices in active markets that are accessible at the measurement date for identical unrestricted assets or liabilities the Company has the ability to access;

***Level* 2—**Inputs (other than quoted prices included within Level 1) that are observable for the asset or liability, either directly or indirectly, for substantially the full term of the asset or liability. These include quoted prices for similar assets or liabilities in active markets and quoted prices for identical or similar assets or liabilities in markets that are not active; and

***Level* 3—**Unobservable inputs that are supported by little or no market activity and that are significant to determining the fair value of the assets or liabilities, including pricing models, discounted cash flow methodologies and similar techniques.

Financial instruments are categorized in their entirety based on the lowest level of input that is significant to the fair value measurement. The assessment of the significance of a particular input to the fair value measurement requires judgment and considers factors specific to the investment. To the extent that the valuation is based on models or inputs that are less observable or unobservable in the market, the determination of fair value requires more judgment. Accordingly, the degree of judgment exercised by the Company in determining fair value is greatest for instruments categorized in Level 3. The Company reviews the fair value hierarchy classification at each reporting date. Changes in the ability to observe valuation inputs may result in a reclassification of levels for certain assets or liabilities within the fair value hierarchy. The Company did not have any transfers of assets and liabilities between the levels of the fair value measurement hierarchy during the years presented.

Foreign currency remeasurement

The Company's reporting currency and the functional currency of its foreign subsidiary, CAMP4 AUS, is the United States Dollar ("USD"). At the date a foreign currency denominated transaction is recognized, each asset, liability, revenue, expense, gain or loss arising from the transaction is measured initially in USD based on the exchange rate in effect at that date. Subsequently, at each balance sheet date, balances related to monetary assets and liabilities are adjusted to reflect the current exchange rate, which is the rate at which the related receivable or payable could be settled at that date.

Foreign exchange transaction gains and losses are included in other income (expense), net in the accompanying consolidated statements of operations and comprehensive loss and were immaterial for the years ended December 31, 2023 and 2022.

Comprehensive loss

There were no differences between net loss and comprehensive loss presented in the consolidated statements of operations for the years ended December 31, 2023 and 2022.

Property and equipment, net

Property and equipment are stated at cost less accumulated depreciation. Expenditures for maintenance and repairs are charged to expense as incurred, whereas major betterments are capitalized as additions to property and equipment. Depreciation is calculated using the straight-line method over the following estimated useful lives of the assets:

Description	Useful life
Computer and software	Three years
Laboratory equipment	Five years
Furniture and fixtures	Seven years
Leasehold improvements	Shorter of asset life or remaining lease term

Impairment of long-lived assets

The Company evaluates its long-lived assets, which consist of property and equipment, operating lease right-of-use assets, and finance lease right-of-use assets, for impairment at least annually and whenever events or changes in circumstances indicate that the carrying value of an asset may not be recoverable. Factors that the Company considers in deciding when to perform an impairment review include significant underperformance of the business in relation to expectations, significant negative industry or economic trends and significant changes or planned changes in the use of the assets. If the asset is considered to be impaired, the amount of any impairment is measured as the difference between the carrying value and the fair value of the impaired asset. The Company recognized no impairment losses for the years ended December 31, 2023 and 2022.

Commitments and contingencies

Contractual commitments

The Company enters into contracts in the normal course of business with contract research organizations (“CROs”), contract manufacturing organizations (“CMOs”), academic institutions and other third parties for preclinical and clinical research studies, testing and manufacturing services. These contracts generally do not contain minimum purchase commitments and are cancellable by the Company upon prior written notice, although purchase orders for preclinical materials are generally non-cancellable. Payments due upon cancellation consist primarily of payments for services provided or expenses incurred, including non-cancellable obligations from the Company’s service providers, up to the date of cancellation or upon the completion of a manufacturing run.

Guarantees and indemnifications

Indemnification obligations

The Company has entered into indemnification agreements with its officers and directors that require the Company to indemnify such individuals for certain events or occurrences while each such officer or director is, or was, serving at the Company’s request in such capacity. The maximum potential future payments the Company could be required to make is, in many cases, unlimited. The Company has directors’ and officers’ liability insurance coverage that limits its exposure and enables the Company to recover a portion of any future amounts paid.

The Company leases office and laboratory space under operating leases. The Company has standard indemnification arrangements under the leases that require it to indemnify the landlords against all costs, expenses, fines, suits, claims, demands, liabilities and actions directly resulting from any breach, violation or nonperformance of any covenant or condition of the Company’s leases.

In the ordinary course of its business, the Company enters into indemnification agreements with certain suppliers and business partners pursuant to which the Company has certain indemnification obligations limited to the costs, expenses, fines, suits, claims, demands, liabilities and actions directly resulting from the Company's gross negligence or willful misconduct, and in certain instances, breaches, violations or nonperformance of covenants or conditions under the agreements.

As of December 31, 2023 and 2022, the Company had not experienced any material losses related to these indemnification obligations, and no material claims with respect thereto were outstanding. The Company does not expect significant claims related to these indemnification obligations and, consequently, concluded that the fair value of these obligations is negligible, and no related reserves were established.

The Company is subject to the possibility of loss contingencies arising in the ordinary course of business. Management considers the likelihood of loss related to an asset, or the incurrence of a liability, as well as its ability to reasonably estimate the amount of the loss, in determining loss contingencies. An estimated loss contingency is accrued when it is probable that an asset has been impaired, or a liability has been incurred and the amount of loss can be reasonably estimated. The Company regularly evaluates current information available to determine whether such accruals should be adjusted and whether new accruals are required.

Legal proceedings

From time to time, the Company may become involved in litigation relating to claims arising from the ordinary course of business. Management believes that there are no claims or actions pending against the Company currently, the ultimate disposition of which would have a material adverse effect on the Company's consolidated results of operations, financial condition or cash flows.

Leases

In accordance with ASC 842, *Leases*, the Company determines if an arrangement is or contains a lease at inception. A contract is or contains a lease if the contract conveys the right to control the use of an identified asset for a period of time in exchange for consideration. The Company classifies leases at the lease commencement date as operating or finance leases and records a right-of-use asset and a lease liability on the consolidated balance sheet for all leases with an initial lease term of greater than 12 months. Leases with an initial term of 12 months or less are not recorded in the balance sheet, but payments are recognized as expense on a straight-line basis over the lease term.

A lease qualifies as a finance lease if any of the following criteria are met at the inception of the lease: (i) there is a transfer of ownership of the leased asset to the Company by the end of the lease term, (ii) the Company holds an option to purchase the leased asset that it is reasonably certain to exercise, (iii) the lease term is for a major part of the remaining economic life of the leased asset, (iv) the present value of the sum of lease payments equals or exceeds substantially all of the fair value of the leased asset, or (v) the nature of the leased asset is specialized to the point that it is expected to provide the lessor no alternative use at the end of the lease term. All other leases are recorded as operating leases.

The Company enters into contracts that contain both lease and non-lease components. Non-lease components may include maintenance, utilities and other operating costs. The Company combines the lease and non-lease components of fixed costs in its lease arrangements as a single lease component. Variable costs, such as utilities or maintenance costs, are not included in the measurement of right-of-use assets and lease liabilities, but rather are expensed when the event determining the amount of variable consideration to be paid occurs.

Finance and operating lease assets and liabilities are recognized at the lease commencement date based on the present value of the lease payments over the lease term using the discount rate implicit in the lease. If the rate implicit is not readily determinable, the Company utilizes an estimate of its incremental borrowing rate based upon the available information at the lease commencement date. Operating lease assets are further adjusted for prepaid or accrued lease payments. Operating lease assets are expensed using the straight-line method as an operating expense over the lease term. Finance lease assets are amortized to depreciation expense using the

straight-line method. Finance lease payments are bifurcated into (i) a portion that is recorded as imputed interest expense and (ii) a portion that reduces the finance liability associated with the lease.

Certain of the Company's leases provide a lease incentive in the form of reimbursable leasehold improvements. Due to the unpredictability of the payout of leasehold improvement reimbursements, the Company recognizes a reduction to the right-of-use asset and the lease liability once it has incurred costs that qualify as reimbursable by the lessor. The reduction to the right-of-use asset is recognized prospectively over the remainder of the lease term.

Certain of the Company's leases include options to extend or terminate the lease. The amounts determined for the Company's right-of-use assets and lease liabilities generally do not assume that renewal options or early-termination provisions, if any, are exercised, unless it is reasonably certain that the Company will exercise such options.

In addition, the Company examines other contracts with suppliers, vendors and outside parties to identify whether such contracts contain an embedded lease and, as applicable, records such embedded leases in accordance with ASC 842, *Leases*.

Financing obligation (failed sale-leaseback)

In accordance with ASC 842, *Leases*, for potential sale-leaseback transactions, the Company assesses the contract to identify if a sale occurred in accordance with ASC 606. Sale-and-leaseback transactions occur when the Company sells assets to a third-party and simultaneously leases them back. The resulting leases that qualify for sale-and-leaseback accounting are evaluated and accounted for as operating leases. A transaction that does not qualify for sale-and-leaseback accounting as a result of finance lease classification or the failure to meet certain revenue recognition criteria is accounted for as a financing transaction. For a financing transaction, the Company will retain the assets sold within Property, plant and equipment, net and record a financing obligation equal to the amount of cash proceeds received. Rental payments under such transactions are recognized as a reduction of the financing obligation and as interest expense using an effective interest method. To date, the Company has entered into one failed sale-leaseback transaction. See additional discussion in *Note 7. Commitments and Contingencies*.

Revenue recognition and accounting for collaboration agreements

Revenue from contracts with customers

The Company recognizes revenue in accordance with ASC Topic 606, *Revenue from Contracts with Customers* ("ASC 606"). Under ASC 606, an entity recognizes revenue when its customer obtains control of promised goods or services, in an amount that reflects the consideration that the entity expects to receive in exchange for those goods or services. In determining the appropriate amount of revenue to be recognized as it fulfills its obligations under its agreements, the Company performs the following steps: (i) identification of the promised goods or services in the contract; (ii) determination of whether the promised goods or services are performance obligations, including whether they are distinct in the context of the contract; (iii) measurement of the transaction price, including the constraint on variable consideration; (iv) allocation of the transaction price to the performance obligations; and (v) recognition of revenue when (or as) the Company satisfies each performance obligation.

As part of the accounting for revenue from contracts with customers, the Company uses judgment to determine: (a) the performance obligations based on the determination under step (ii) above; (b) the transaction price under step (iii) above; and (c) the recognition of revenue as services are performed under step (v) above. The Company also uses judgment to determine whether development milestones or other variable consideration, with the exception of royalties and sales-based milestones, should be included in the transaction price as described further below.

The Company applies the five-step model to contracts when the arrangement is not a collaboration pursuant to ASC Topic 808, *Collaborative Arrangements* ("ASC 808"), and it is probable that the Company will collect the

consideration it is entitled to in exchange for the goods or services it transfers to the customer. At contract inception, once the contract is determined to be within the scope of ASC 606, the Company assesses the goods or services promised within each contract and determines those that are performance obligations. The Company then recognizes as revenue the amount of the transaction price that is allocated to the respective performance obligation when (or as) the performance obligation is satisfied.

Collaborative agreements

The Company analyzes its collaboration agreements to assess whether they are within the scope of ASC 808 by determining whether such arrangements involve joint operating activities performed by parties that are both active participants in the activities and exposed to significant risks and rewards that are dependent on the commercial success of such activities. To the extent the arrangement is within the scope of ASC 808, the Company assesses whether aspects of the arrangement between the Company and the collaboration partner are within the scope of other accounting literature. If the Company concludes that some or all aspects of the arrangement represent a transaction with a customer, the Company accounts for those aspects of the arrangement within the scope of ASC 606. If the Company concludes that some or all aspects of the arrangement are within the scope of ASC 808 and do not represent a transaction with a customer, the Company recognizes the Company's share of the allocation of the shared costs incurred with respect to the jointly conducted activities as a component of the related expense in the period incurred.

Research and development expenses

Research and development ("R&D") expenses consist of costs incurred for R&D of its lead product candidate, CMP-CPS-001, and are recorded to operating expenses when incurred. The Company's R&D expenses consist primarily of costs incurred in performing R&D activities, including personnel-related expenses such as salaries, stock-based compensation and benefits, facilities costs, depreciation and external costs of outside vendors engaged to conduct clinical and preclinical development activities and to manufacture CMP-CPS-001. The Company accrues expenses related to development activities performed by third parties based on an evaluation of services received and efforts expended pursuant to the terms of the contractual arrangements. Payments under some of these contracts depend on preclinical trial milestones. Non-refundable advance payments for goods and services that will be used over time for research and development are deferred and capitalized as research and development prepaid expenses on our consolidated balance sheets. The capitalized amounts are recognized as an expense as the goods are delivered or as the related services are performed. In accruing service fees, the Company estimates the time period over which services will be performed and the level of effort to be expended in each period. If the actual timing of the performance of services or the level of effort varies from the estimate, the Company will adjust the accrual or prepaid expense accordingly. Costs to acquire technologies to be used in R&D that have not reached technological feasibility and have no alternative future use are also expensed as incurred.

General and administrative expenses

General and administrative ("G&A") expenses consist primarily of personnel-related expenses, including salaries, bonuses, benefits, travel and stock-based compensation expenses for employees in executive, accounting and finance, business development, human resources, legal, and other administrative functions. Other significant G&A expenses include allocated facility-related costs, legal fees relating to corporate and intellectual property matters, professional fees for accounting, audit and tax services, consulting fees and insurance costs. G&A costs are expensed as incurred.

Patent costs

All patent-related costs incurred in connection with filing and prosecuting patent applications are expensed as incurred due to the uncertainty about the recovery of the expenditure. Amounts are classified as G&A expenses.

Offering costs

The Company complies with the requirements of ASC 340, *Other Assets and Deferred Costs*, with regards to offering costs. Prior to the completion of an offering of common stock, direct offering costs are capitalized as deferred offering costs. The deferred offering costs are charged to additional paid-in capital for offerings of common stock or as a reduction in the carrying value of preferred stock for offerings of preferred stock. As of December 31, 2023 and 2022, the Company had no deferred offering costs recorded. During the year ended December 31, 2022, the Company recorded $0.3 million of offering costs related to the Series B convertible stock issuance and recognized this amount as a reduction in the carrying value of the Series B convertible preferred stock.

Stock-based compensation

The Company periodically grants equity-based payment awards in the form of stock options to employees, directors and non-employees and records stock-based compensation expenses for awards of stock-based payments based on their estimated fair value at the grant date. The Company recognizes stock-based compensation expense for all equity-based payments, including stock options. Stock-based compensation costs are calculated based on the estimated fair value of the underlying option using the Black-Scholes option-pricing model on the date of grant for stock options and are recognized as expense in the accompanying consolidated statements of operations and comprehensive loss on a straight-line basis over the requisite service period, which is typically the vesting period. Determining the appropriate fair value model and related input assumptions requires judgment, including estimating the fair value of the Company's common stock and stock price volatility.

Given the absence of a public trading market, the fair value of the Company's common stock is determined by the Company's Board of Directors (the "Board") at the time of each option grant by considering a number of objective and subjective factors. These factors include the valuation of a select group of representative public companies within the industry that focus on biotechnology that the Board believes is comparable to the Company's operations; operating and financial performance; the lack of liquidity of the common stock and trends in the broader economy and biotechnology industry also impact the determination of the fair value of the common stock.

The other inputs to the Black-Scholes option-pricing model include the following:

- The risk-free interest rate used is based on the published U.S. Department of Treasury interest rates in effect at the time of stock option grant for zero coupon U.S. Treasury notes with maturities approximating each grant's expected term;
- The dividend yield is zero as the Company has not paid dividends and does not anticipate paying a cash dividend in the foreseeable future;
- The expected term for options granted is calculated using the simplified method and represents the average time that options are expected to be outstanding based on the mid-point between the vesting date and the end of the contractual term of the award; and
- Expected volatility is derived from the historical volatilities of a select group of representative companies, for a look-back period commensurate with the expected term of the stock options, as the Company has no trading history of common stock.

The Company recognizes forfeitures related to stock-based compensation awards as they occur.

The Company classifies stock-based compensation expense in the consolidated statement of operations and comprehensive loss in the same manner in which the award recipients' payroll costs are classified or in which the award recipients' service payments are classified.

Income taxes

The Company accounts for income taxes in accordance with ASC 740, *Income Taxes* ("ASC 740"). ASC 740 requires the use of the asset and liability method of accounting for income taxes. The current or deferred tax

consequences of a transaction are measured by applying the provisions of enacted tax laws to determine the amount of taxes payable currently or in future years. Deferred tax assets and liabilities are determined based on the difference between the consolidated financial statements and tax basis of assets and liabilities and expected future tax consequences of events that have been included in the consolidated financial statements or tax returns using enacted tax rates in effect for the year in which the differences are expected to reverse. Under this method, a valuation allowance is used to offset deferred tax assets if, based upon the available evidence, it is more likely than not that some or all of the deferred tax assets may not be realized. Management evaluates the recoverability of deferred taxes and the adequacy of the valuation allowance at each reporting period (see Note 11, *Income Taxes*).

The Company follows the provisions of ASC 740 relative to accounting for uncertain tax positions. These provisions provide guidance on the recognition, de-recognition and measurement of potential tax benefits associated with tax positions. For those benefits to be recognized, a tax position must be more likely than not to be sustained upon examination by taxing authorities. The Company had no reserves related to uncertain tax positions as of December 31, 2023 and 2022. As applicable, the Company recognizes accrued penalties and interest related to unrecognized tax benefits in the provision for income taxes. At December 31, 2023 and 2022, the Company did not accrue any potential interest or penalties.

The Company is required to file federal and state income tax returns in the U.S. and foreign income tax returns in Australia. The preparation of tax returns requires the Company to interpret the applicable tax laws and regulations in effect in such jurisdictions, which could affect the amount of tax paid by the Company.

The Company's income tax returns are based on calculations and assumptions that are subject to examination by the Internal Revenue Service and other tax authorities. In addition, the calculation of the Company's tax liabilities involves dealing with uncertainties in the application of complex tax regulations.

Classification of convertible preferred stock

The Company's convertible preferred stock is classified as temporary equity in the accompanying consolidated balance sheets and is excluded from stockholders' deficit as the potential redemption of such stock is outside the Company's control. The convertible preferred stock is not redeemable except for in the event of a liquidation, dissolution or winding up of the Company. Costs incurred in connection with the issuance of convertible preferred stock are recorded as a reduction of gross proceeds from issuance. The Company does not accrete the carrying values of the preferred stock to the redemption values since the occurrence of these events was not considered probable as of December 31, 2023 and 2022. Subsequent adjustments of the carrying values to the ultimate redemption values will be made only when it becomes probable that these events will occur.

Net loss per share attributable to common stockholders

The Company determined all of its convertible preferred stock qualifies as participating securities, as defined in ASC 260, *Earnings Per Share* ("ASC 260"). Under ASC 260, securities are considered participating securities if the securities may participate in undistributed earnings with common stock, whether that participation is conditioned upon the occurrence of a specified event or not. In accordance with ASC 260, a company is required to use the two-class method when computing net income (loss) per share when a company has securities that qualify as participating securities. The two-class method is an earnings allocation formula that determines net income (loss) per share for each class of common stock and participating security according to dividends declared (or accumulated) and participation rights in undistributed earnings. Diluted net income (loss) per share for the Company's common stock is computed using the more dilutive of the two-class method or the if-converted method.

Basic net income (loss) per share attributable to common stockholders is computed by dividing the net income (loss) attributable to common stockholders by the weighted average number of shares of common stock outstanding for the period. Diluted net income (loss) attributable to common stockholders is computed by adjusting net income (loss) per share attributable to common stockholders to reallocate undistributed earnings based on the potential impact of dilutive securities. Diluted net income (loss) per share attributable to common stockholders is

computed by dividing the diluted net income (loss) attributable to common stockholders by the weighted average number of shares of common stock outstanding for the period, including potential dilutive common shares. For purpose of this calculation, outstanding options to purchase common stock, unvested restricted stock awards, and shares of convertible preferred stock are considered potential dilutive common shares. The Company has generated a net loss in all periods presented, and therefore the basic and diluted net loss per share attributable to common stockholders are the same as the inclusion of the potentially dilutive securities would be anti-dilutive.

Segment information

Operating segments are defined as components of an enterprise (business activity from which it earns revenue and incurs expenses) about which discrete financial information is available and regularly reviewed by the chief operating decision maker in deciding how to allocate resources and in assessing performance. The Company's chief operating decision maker is its Chief Executive Officer. The chief operating decision maker reviews consolidated operating results to make decisions about allocating resources and assessing performance for the entire company. The Company views its operations and manages its business as one operating segment.

Emerging growth company status

The Company is an emerging growth company ("EGC") as defined in the Jumpstart Our Business Startups Act of 2012, as amended (the "JOBS Act"), and may take advantage of certain exemptions from various reporting requirements that are applicable to other public companies that are not EGCs. The Company may take advantage of these exemptions until it is no longer an EGC under Section 107 of the JOBS Act and has elected to use the extended transition period for complying with new or revised accounting standards. As a result of this election, the Company's consolidated financial statements may not be comparable to companies that comply with public company FASB standards' effective dates.

Recently adopted accounting pronouncements

In June 2016, the FASB issued ASU No. 2016-13, Financial Instruments — Credit Losses (Topic 326): *Measurement of Credit Losses on Financial Instruments*, which has been subsequently amended by ASU No. 2018-19, ASU No. 2019-04, ASU No. 2019-05, ASU No. 2019-10, ASU No. 2019-11, ASU No. 2020-02, and ASU 2022-02 ("ASU 2016-1"3). The Company adopted ASU 2016-13 on January 1, 2023 using the modified retrospective approach. The Company's consolidated financial statements for prior-year periods have not been revised and are reflective of the credit loss requirements which were in effect for that period. The adoption of ASU 2016-13 did not have a material impact on the Company's consolidated financial statements and related disclosures.

Recently issued accounting standards

Accounting standards not listed below were assessed and determined not to be applicable or are expected to have minimal impact on the Company's consolidated financial statements.

In December 2023, the FASB issued ASU 2023-09, Income Taxes ("Topic 740"): *Improvements to Income Tax Disclosures*. The guidance includes the requirement that public business entities, on an annual basis, disclose specific categories in the rate reconciliation and provide additional information for reconciling items that meet a quantitative threshold (if the effect of those reconciling items is equal to or greater than 5% of the amount computed by multiplying pretax income (or loss) by the applicable statutory income tax rate). It also requires that all entities disclose, on an annual basis, the amount of income taxes paid (net of refunds received) disaggregated by federal (national), state, and foreign taxes and the amount of income taxes paid (net of refunds received) disaggregated by individual jurisdictions in which income taxes paid (net of refunds received) is equal to or greater than 5% of total income taxes paid (net of refunds received) and requires that all entities disclose income (or loss) from continuing operations before income tax expense (or benefit) disaggregated between domestic and foreign and income tax expense (or benefit) from continuing operations disaggregated by federal (national), state, and foreign. Lastly, the guidance eliminates the requirement for all entities to disclose the nature and estimate of the range of the reasonably possible change in the unrecognized tax benefits balance in the

next 12 months or make a statement that an estimate of the range cannot be made. For public business entities, the guidance is effective for annual periods beginning after December 15, 2024. Early adoption is permitted for annual consolidated financial statements that have not yet been issued or made available for issuance. The guidance should be applied on a prospective basis. Retrospective application is permitted. The Company is currently evaluating the impact that this guidance may have on its consolidated financial statements.

3. Fair value measurements

The following tables present the financial instruments carried at fair value on a recurring basis as of December 31, 2023 and 2022, respectively, in accordance with the ASC 820 hierarchy (in thousands):

	Fair value measurements at December 31, 2023			
	Level 1	Level 2	Level 3	Total
Assets				
Cash equivalents	$ 37,074	$ —	$ —	$ 37,074

	Fair value measurements at December 31, 2022			
	Level 1	Level 2	Level 3	Total
Assets				
Cash equivalents	$ 82,377	$ —	$ —	$ 82,377

The Company's carrying amounts reflected in the consolidated balance sheet for prepaid expenses and other current assets, accounts payable and accrued expenses and other liabilities are shown at their historical values which approximate their fair values.

4. Prepaid expenses and other current assets

Prepaid expenses and other current assets consisted of the following (in thousands):

	December 31,	
	2023	2022
Variable lease expenses	$ 105	$ 16
Federal R&D tax credit receivable	442	678
Software and subscriptions	287	158
Research and development (R&D)	480	162
Other	318	206
Prepaid expenses and other current assets	$1,633	$1,219

5. Property and equipment, net

Property and equipment consisted of the following (in thousands):

	December 31,	
	2023	2022
Laboratory equipment	$ 3,322	$ 2,757
Computer and software	938	921
Furniture and fixtures	524	492
Leasehold improvements	4,518	4,518
Total property and equipment	9,302	8,687
Less: accumulated depreciation and amortization	(4,505)	(3,039)
Property and equipment, net	$ 4,797	$ 5,648

The Company incurred depreciation and amortization expense of $1.7 million and $0.9 million for the years ended December 31, 2023 and 2022, respectively. Depreciation and amortization expense for the years ended December 31, 2023 and 2022 includes $0.1 million and less than $0.1 million of finance lease right-of-use asset amortization, respectively. See additional discussion in *Note 7. Commitments and Contingencies*.

6. Accrued expenses and other current liabilities

Accrued expenses and other current liabilities consisted of the following (in thousands):

	December 31,	
	2023	2022
External research and development expenses	$ 601	$ 571
Employee compensation and benefits	1,937	1,777
Professional fees and other general and administrative expenses	475	818
Other	289	259
	$3,302	$3,425

7. Commitments and contingencies

Operating leases

The Company currently leases approximately 30,000 square feet of office space and laboratory space in Cambridge, Massachusetts and approximately 5,300 square feet of office and lab space in Boulder, Colorado. The office and laboratory space lease in Cambridge, Massachusetts expires on June 30, 2027. The lease provides a lease incentive in the form of reimbursable leasehold improvements of up to $3.6 million. As of December 31, 2023, the Company had capitalized $4.5 million of leasehold improvement costs to date under this lease, of which $3.6 million was reimbursed through the lease incentive. During the years ended December 2023 and 2022, the Company received $2.7 million and $0.9 million, respectively, as reimbursements of improvement costs. Amounts received for lease incentives are included in the changes in operating lease assets and liabilities line in the consolidated statement of cash flows. As of December 31, 2023, this operating lease accounted for $6.4 million of operating lease right-of-use assets, $2.5 million of current operating lease liabilities and $7.4 million of non-current operating lease liabilities.

In September 2023, the Company leased certain office and laboratory space under an operating lease in Boulder, Colorado for approximately 5,300 square feet of space. The five-year lease commenced on September 1, 2023. The office and laboratory space in Boulder, Colorado expires on September 30, 2028. As the rate implicit in this lease agreement was not readily determinable, the Company used its incremental borrowing rate of 7.12% as of the commencement date of the lease. At commencement of the lease, the Company recorded $1.4 million of operating right-of-use assets, $0.2 million of current operating lease liabilities and $1.2 million of non-current operating lease liabilities As of December 31, 2023, this operating lease accounted for $1.3 million of operating lease right-of-use assets, $0.2 million of current operating lease liabilities and $1.1 million of non-current operating lease liabilities.

The table below summarizes the Company's operating lease costs for the years ended December 31, 2023 and 2022 (in thousands except for lease terms and borrowing rates):

	Year ended December 31,	
	2023	2022
Lease cost		
Operating lease cost	$ 2,489	$ 2,629
Short-term lease cost	119	46
Variable lease expense	1,213	1,018
Total lease cost	$ 3,821	$ 3,693
Other information		
Cash paid for amounts included in the measurement of lease liabilities, included in operating cash flows	$ 601	$ 1,972
Weighted-average remaining lease term	3.7	4.5
Weighted-average incremental borrowing rate	6.72%	6.66%

Maturities of lease liabilities as of December 31, 2023 were as follows (in thousands):

Year ending December 31,	
2024	$ 3,356
2025	3,455
2026	3,558
2027	1,980
2028	263
Total lease payment	12,612
Less: amount representing imputed interest	(1,421)
Total future minimum lease obligations	$11,191

Finance leases

The Company leases certain specialized lab equipment under several finance lease agreements with maturities ranging from November 2024 to November 2028. As of December 31, 2023, these finance leases account for $0.7 million of finance lease right-of-use assets, $0.4 million of current finance lease liabilities and $0.1 million of non-current finance lease liabilities.

The table below summarizes the Company's finance lease costs for the years ended December 31, 2023 and 2022 (in thousands except for lease terms and borrowing rates):

		Year ended December 31,	
	Classification	2023	2022
Finance lease cost			
Amortization of right-of-use assets	Depreciation and amortization	$ 146	$ 69
Interest on lease liabilities	Other Expense	33	12
Total finance lease cost		$ 179	$ 81
Other information			
Cash paid for amounts included in the measurement of lease liabilities, included in operating cash flows		$ 322	$ 94
Weighted-average remaining lease term		1.8	2.0
Weighted-average incremental borrowing rate		8.14%	6.62%

Maturities of finance lease liabilities as of December 31, 2023 were as follows (in thousands):

Year ending December 31,	
2024	$381
2025	98
2026	31
2027	31
Total lease payment	541
Less: amount representing imputed interest	(39)
Total future minimum lease obligations	$502

Financing obligation

In April 2023, the Company (seller-lessee) sold certain laboratory equipment to an unrelated third-party (buyer-lessor) and simultaneously entered into a 26-month lease agreement for the laboratory equipment with the buyer-lessor through June 2025. The lease requires monthly payments of less than $0.1 million and provides a fixed price repurchase option at the end of the lease term of $0.1 million.

The repurchase option precludes accounting for the transfer of the asset to the buyer-lessor as a sale under ASC 842 since the exercise price of the repurchase option is fixed and, therefore, is not the fair value of the asset on the exercise date of the option. Thus, the agreement is considered a financing transaction (i.e., failed sale-leaseback) as the Company is reasonably certain to exercise the repurchase option at the end of the lease. The net proceeds received amounted to $0.7 million, which is recorded as a financing liability in the Company's consolidated balance sheet. The Company imputes interest at a rate of 0.86% on a monthly basis. For the year ended December 31, 2023, the Company recorded less than $0.1 million of interest expense related to this financing transaction in other expense in the consolidated statement of operations and comprehensive loss.

Legal proceedings

A liability for loss contingencies arising from claims, assessments, litigation, fines, penalties and other sources is recorded in the consolidated financial statements if it is determined that it is probable that a loss has been incurred and the amount (or range) of the loss can be reasonably estimated. There are no matters currently outstanding for which any liabilities have been accrued or require disclosure.

8. Collaboration and license agreements

In-license agreements

Children's Medical Center Corporation

In April 2018, the Company entered into a development and license agreement (the "CMCC Agreement") with Children's Medical Center Corporation ("CMCC"). The agreement allows the Company to use CMCC's proprietary intellectual property to conduct research, development and commercialization of products utilizing CMCC's proprietary intellectual property in return for specified payments. The proprietary intellectual property licensed pursuant to this agreement is related to certain legacy programs the Company is not pursuing and was subsequently sublicensed to Fulcrum Therapeutics, Inc. ("Fulcrum"), as described below. As part of the agreement, the Company issued a total of 169,624 shares of common stock to CMCC and its affiliates based on the fair value of the common stock on the date of issuance.

The Company is obligated to pay potential development milestone payments under the terms of the CMCC Agreement of up to $7.7 million for the first licensed target, $3.9 million for the second licensed target and $1.9 million for the third licensed target upon the achievement of certain specified contingent events. If commercial sales of a licensed product commence, the Company will pay CMCC royalties at percentage rates ranging in the

low- to mid-single digits on net sales of licensed products in countries where such product is protected by patent rights. The Company incurred $0.03 million of royalties owed to CMCC in both 2023 and 2022 under the agreement and recorded the amounts in R&D expense in the consolidated statement of operations and comprehensive loss. Further, under the terms of the CMCC Agreement, the Company is required to pay 10% of any upfront payment received under a sublicensing agreement entered into prior to the initiation of the first investigational new drug study. As such, the Company recorded $0.04 million of sublicense costs for the year ended December 31, 2023, which is presented in R&D expenses on the consolidated statements of operations and comprehensive loss. The Company re-evaluates the likelihood of achieving future milestones at the end of each reporting period. As of December 31, 2023, the Company determined that the likelihood of achieving future milestones was not probable.

Whitehead Institute for Biomedical Research

In October 2019, the Company entered into a patent license agreement with the Whitehead Institute for Biomedical Research, or the Whitehead Institute, which was subsequently amended on December 14, 2021, or the Whitehead First Amendment, and on November 7, 2023, or the Whitehead Second Amendment. Under the agreement, the Company was granted a worldwide, royalty-bearing, sublicensable license under certain patent rights owned or controlled by the Whitehead Institute. As part of the agreement, the Company paid an initial $0.1 million license issuance fee, and the Company is obligated to pay annual license maintenance fees of up to $0.07 million for the term of the agreement. In addition, with each of the Whitehead First Amendment and Whitehead Second Amendment, the Company paid the Whitehead Institute license amendment issuance fees of $0.02 million. The Company is obligated to pay potential development milestone payments under the terms of the agreement upon the achievement of certain specified contingent events. The Company is also obligated to pay tiered royalties at percentage rates ranging from less than one percent to the mid-single digits of net sales or of running royalties of net sales, subject to specified reductions, upon the achievement of certain contingent events. The Company incurred $0.06 million and $0.04 million of license maintenance fees and $0.02 million and $0 of license amendment issuance fees owed to the Whitehead Institute in 2023 and 2022, respectively, under the amended agreement and recorded the amounts in our research and development expense in our consolidated statement of operations and comprehensive loss.

Sublicense agreement

Fulcrum Therapeutics, Inc.

In July 2023, the Company entered into a license agreement (the “Fulcrum Agreement”) with Fulcrum. Under the Fulcrum Agreement, the Company granted an exclusive license related to the Company’s intellectual property (“IP”) and granted a sublicense for IP obtained through the CMCC Agreement. In exchange for the license rights, Fulcrum paid the Company a $0.35 million upfront payment. In the event that Fulcrum achieves certain development and commercial milestones, Fulcrum will be obligated to pay the Company one-time milestone payments ranging from $1.0 million to $20.0 million (with respect to a Tier 1 Product, as defined in the Fulcrum Agreement) or $0.6 million to $12.0 million (with respect to a Tier 2 Product, as defined in the Agreement), depending on the milestone achieved. In addition, the Fulcrum Agreement includes both potential nominal minimum annual royalty payments as well as sales-based royalties upon commercialization of up to the low-double digits.

The Company assessed this arrangement in accordance with ASC 606 and concluded that the contract counterparty, Fulcrum, is a customer. In accordance with ASC 606, the Company determined that there is one performance obligation in the Fulcrum Agreement, consisting of the exclusive and non-exclusive license rights to Fulcrum. The transaction price was comprised of the fixed consideration of $0.35 million and was recognized upon transfer of control of the licenses at a point in time upon contract execution. The arrangement includes significant variable consideration primarily in the form of milestone payments, which is fully constrained at the inception of the contract. All variable consideration is remeasured at each financial reporting date. At December 31, 2023, the Company determined the variable consideration was fully constrained. The related constraint on each element of variable consideration is reassessed each reporting period.

The sales-based royalty fee is considered variable consideration and will be recognized as revenue as such sales occur. The sales-based royalty fee qualifies for the royalty constraint exception and does not require an estimate of the future transaction price.

During the year ended December 31, 2023, the Company recorded $0.35 million in license revenue pursuant to the Fulcrum Agreement.

Collaborative arrangement

Eli Lilly and Company

In July 2023, the Company executed a Material Transfer Agreement ("MTA") with Eli Lilly and Company ("Eli Lilly"). As part of the MTA, the Company and Eli Lilly agreed to perform research and development activities to generate up to three antisense oligonucleotides ("ASOs") in accordance with a prescribed workplan. For the year ended December 31, 2023, the Company received $0.4 million from Eli Lilly related to the MTA. The Company evaluated the MTA under ASC 808 and concluded that it is a collaboration arrangement. The Company and Eli Lilly are jointly overseeing the research and development activities under the MTA and are active participants in the research and development activities. In addition, both parties are exposed to the significant risks and potential rewards under the MTA. During the year ended December 31, 2023, the Company recorded $0.5 million as a reduction in R&D expense in the consolidated statement of operations and comprehensive loss. Additionally, the Company had an unbilled receivable of $0.1 million recorded within prepaid expenses and other current assets on the consolidated balance sheet as of December 31, 2023.

9. Convertible preferred stock and stockholders' deficit

Convertible preferred stock

As of December 31, 2023 and 2022, the Company's Series A Prime convertible preferred stock and Series B convertible preferred stock have been classified as temporary equity in the accompanying consolidated balance sheets.

Convertible preferred stock consisted of the following as of December 31, 2023 and 2022 (in thousands, except share amounts):

	Authorized shares	Shares issued and outstanding	Liquidation Value	Common stock issuable upon conversion
Series A Prime	68,173,692	62,389,791	$ 62,381	62,389,791
Series B	81,499,592	68,258,635	$ 100,504	68,258,635

Series B Convertible Preferred Stock

In 2022, the Company entered into a securities purchase agreement (the "Series B Agreement") to sell shares of Series B convertible preferred stock (the "Series B Preferred Stock") at $1.4724 per share. From June through July 2022, the Company issued 68,258,635 shares of Series B convertible preferred stock to existing and new investors for gross cash proceeds of $100.5 million, less issuance costs of $0.3 million, resulting in net proceeds of $100.2 million.

Rights, preferences, privileges and restrictions

Voting rights

Each preferred stockholder is entitled to cast the number of votes equal to the number of whole shares of common stock into which the shares of preferred stock held by such holder are convertible at the time of such

vote**.** All preferred stockholders are entitled to vote on all matters upon which holders of common stock have the right to vote, other than matters that must by law be voted by class or series vote.

Conversion rights

Each share of convertible preferred stock is convertible at the option of the holder at any time into a share of common stock. Each share of convertible preferred stock is convertible into that number of common shares as is determined by dividing the applicable initial purchase price) of such share by the applicable conversion price. The conversion rate is subject to adjustment upon the occurrence of certain events, including diluting issues of shares, stock splits, stock combinations, certain dividends and distributions, a merger and a reorganization.

All shares of the convertible preferred stock are automatically convertible into shares of common stock, in a firm commitment underwritten public offering pursuant to an effective registration statement under the Securities Act of 1933, as amended, covering the offer and sale of common stock for the account of the Company which results in at least $75.0 million of gross proceeds to the Company.

Dividend rights

Preferred stockholders are entitled to receive, when and as declared by the Board of Directors, preferential non-cumulative cash dividends at a rate of 6% per annum of the original issue price per share. Such dividends are payable only when and if declared by the Company's board of directors. No such dividends have been declared or paid through December 31, 2023.

Liquidation preference

In the event of any voluntary or involuntary liquidation, dissolution, or winding up of the Company, the holders of shares of the preferred stock shall be paid an amount per share first out of the assets and legally available funds of the Company available for distribution to holders of the Company's capital stock of all classes, an amount equal to the greater of the original issue price, plus all declared dividends accrued but unpaid with respect to each such shares, as adjusted for any stock dividend, stock split, recapitalization, or other similar event. After payment of all preferential amounts to the holders of preferred shares, any assets and funds of the Company that remain available for distribution shall be distributed ratably among the holders of the common stock.

Redemption rights

The holders of the shares of the Preferred Stock may redeem their shares for the original issue price per share and any declared dividends upon a Deemed Liquidation Event, as defined per the terms of the applicable preferred stock agreement.

Common stock

The Company is authorized to issue up to 210,000,000 shares of common stock at December 31, 2023 and 2022, respectively, of which 11,509,269 and 11,559,826 shares were issued at December 31, 2023 and 2022, respectively; 5,168,193 and 4,002,103 shares were outstanding as of December 31, 2023 and 2022, respectively.

Voting, dividend and liquidation rights of the holders of the common stock are subject to and qualified by the rights, powers and preferences of the holders of the convertible preferred stock.

Voting

Each holder of outstanding shares of common stock is entitled to one vote in respect of each share. The holders of outstanding shares of common stock, voting together as a single class, are entitled to elect one director. The number of authorized shares of common stock may be increased or decreased by the affirmative vote of a majority of the outstanding shares of common stock and preferred stock voting together as a single class.

Dividends

Subject to the payment in full of all preferential dividends to which the holders of the preferred stock are entitled, the holders of common stock are entitled to receive dividends out of funds legally available therefor at such times and in such amounts as the board of directors may determine in its sole discretion, with holders of preferred stock and common stock sharing pari passu in such dividends.

Liquidation rights

After payment in full of all preferential amounts to which the holders of preferred stock are entitled upon any voluntary or involuntary liquidation, dissolution or winding-up of the Company or deemed liquidation event of the Company, all of the remaining assets of the Company available for distribution to the stockholders shall be distributed among the holders of the preferred stock and common stock, pro rata based on the number of shares held by each such holder on an as converted to common stock basis.

Reserved shares

As of December 31, 2023, the Company reserved the following shares of common stock for issuance upon conversion of the outstanding convertible preferred stock and exercise of stock options:

	December 31, 2023
Conversion of convertible preferred stock	130,648,426
Stock options available for issuance	13,195,448
Stock options outstanding	25,514,335
Warrants	1,602
Restricted stock vesting	832,840
Total	170,192,651

10. Stock-based compensation

In 2016, the Company adopted the Marauder Therapeutics, Inc. 2016 Stock Option and Grant Plan (the “Plan”). All of the Company’s employees, officers, directors, consultants and advisors are eligible to be granted options, restricted stock units and other stock-based awards under the terms of the Plan. When the Plan was initially established, it provided for the grant of 212,030 shares of common stock. During 2018, the Board of Directors approved an increase to 15,884,027 shares of common stock available under the Plan. During 2021, the Board of Directors approved an increase to 17,070,142 shares of common stock available under the Plan. During 2022, the Board of Directors approved another increase to 42,656,671 shares of common stock available under the Plan. During the year ended December 31, 2023, there were 13,195,448 shares of common stock remaining and available for issuance under the 2016 Plan.

The Company may grant options to purchase authorized but unissued shares of the Company’s common stock. Options granted under the 2016 Plan include incentive stock options that can be granted only to the Company’s employees and non-statutory stock options that can be granted to the Company’s employees, consultants, advisors and directors.

The exercise prices, vesting and other restrictions of the awards to be granted under the 2016 Plan is determined by the board of directors, except that no stock option may be issued with an exercise price less than the fair market value of the common stock at the date of the grant or have a term in excess of ten years. Options granted under the 2016 Plan are exercisable in whole or in part at any time subsequent to vesting, which is typically over a four-year period.

Stock options

The Company estimated the fair value of stock options using the Black-Scholes valuation model. The weighted-average assumptions used in the Black-Scholes option pricing model to determine the fair value of the stock options granted were as follows:

	Year ended December 31,	
	2023	2022
Expected volatility	92.24%	84.73%
Risk-free interest rate	3.84%	3.45%
Expected dividend yield	0%	0%
Expected term (in years)	5.97	5.92

The weighted average fair value of stock options granted during the years ended December 31, 2023 and 2022 as determined by the Black-Scholes option pricing model was $0.60 and $0.51 per share, respectively. The total intrinsic value of options exercised during the years ended December 31, 2023 and 2022 was $203 and $52, respectively.

The Company issued $1.0 million of promissory notes to certain executives during the year ended December 31, 2021 in order for them to early exercise stock options. Management concluded the promissory notes are recourse in form but non-recourse in substance as the Company does not intend to seek repayment beyond the shares issued. The promissory notes are therefore treated as an option for accounting purposes and are not recorded on the consolidated balance sheet. Stock-based compensation expense is recorded, accordingly. The exercise price used in determining the fair value of the stock options includes the interest earned on the notes and the expected term is five years, reflecting the term of the notes. The early exercised shares are not outstanding for accounting purposes before repayment of the notes.

The following table summarizes stock option activity for the year ended December 31, 2023 (in thousands, except share and per share amounts):

	Number of outstanding options	Weighted average exercise price	Weighted average remaining contractual term	Aggregate intrinsic value
			(Years)	
Balance at December 31, 2022	27,496,583	$ 0.58	9.19	6,140
Granted	2,144,445	$ 0.78		
Forfeited	(3,643,993)	$ 0.60		203
Exercises	(482,700)	$ 0.38		
Balance at December 31, 2023	25,514,335	$ 0.54	8.05	$ 6,893
Vested and expected to vest at December 31, 2022	25,514,335	$ 0.54	8.05	$ 6,893
Exercisable at December 31, 2023	11,717,871	$ 0.41	7.33	$ 4,678

The Company has recorded stock-based compensation expense related to stock options of $2.8 million and $1.2 million for the years ended December 31, 2023 and 2022, respectively. The Company has an aggregate $6.4 million of gross unrecognized stock-based compensation expense as of December 31, 2023 remaining to be recognized over a weighted average period of 2.6 years.

A summary of restricted stock award activity for the year ended December 31, 2023 is a follows:

	Number of shares	Weighted average fair value
Unvested at December 31, 2022	1,516,230	$ 0.20
Granted	—	—
Vested	(683,390)	0.22
Forfeited	—	—
Balance at December 31, 2023	832,840	$ 0.18

All restricted common stock awards were initially issued at a price determined to be fair value on the date of grant. The Company recognizes forfeitures of restricted common stock as they occur. As of December 31, 2023, total unrecognized stock-based compensation expense relating to unvested restricted common stock was $0.2 million. This amount is expected to be recognized over a weighted average period of 1.3 years. The fair value of shares that vested during the years ended December 31, 2023 and 2022 was $0.5 million and $0.5 million, respectively.

Stock-based compensation expense related to stock options and restricted stock recorded in the accompanying consolidated statements of operations is as follows (in thousands):

	Year ended December 31,	
	2023	2022
Research and development	$ 1,555	$ 676
General and administrative	1,352	701
	$ 2,907	$ 1,377

The Company has not recognized and does not expect to recognize in the near future, any tax benefit related to employee stock-based compensation expense as a result of the full valuation allowance related to its net deferred tax assets.

11. Income taxes

A reconciliation of the expected income tax benefit computed using the federal statutory income tax rate to the Company's effective income tax rate is as follows for the years ended December 31, 2023 and 2022:

	Year ended December 31,	
	2023	2022
Income tax computed at federal statutory rate	21.00%	21.00%
State taxes, net of federal benefit	7.49	6.72
Foreign rate differential	0.04	—
Research and development credit	4.03	3.08
Valuation allowance	(31.99)	(30.30)
Permanent differences	(0.57)	(0.50)
Effective income tax rate	(0.00)%	(0.00)%

The Company's deferred tax assets at December 31, 2023 and 2022, consisted of the following (in thousands):

	Year ended December 31,	
	2023	2022
Deferred tax assets:		
Net operating losses	$ 18,936	$ 15,927
R&D credit	7,239	4,555
Capitalized Sec. 59 (e) R&D expenditures	2,337	2,822
Operating lease liabilities	3,052	3,353
Capitalized research and development costs	17,413	8,281
Other	2,735	1,930
Total gross deferred tax assets	51,712	36,868
Deferred tax liabilities:		
Operating lease right-of-use assets	(2,118)	(3,025)
Other	—	(19)
Total gross deferred tax liabilities	(2,118	(3,044)
Net deferred tax assets	49,594	33,824
Valuation allowance	(49,594)	(33,824)
Net deferred tax asset	$ —	$ —

As of December 31, 2023 and 2022, the Company had a federal net operating loss carryforward of $69.8 million and $58.8 million, respectively, which may be available to offset future income tax liabilities. Of the $69.8 million of federal net operating loss carryforwards, approximately $4.8 million will begin to expire in 2036 and approximately $64.9 million are carried forward indefinitely. As of December 31, 2023 and 2022, the Company had state net operating loss ("NOL") carryforwards of $66.7 million and $56.7 million, respectively, which will begin to expire in 2036.

As of December 31, 2023 and 2022, the Company had federal research and development tax credit carryforwards of $5.2 million and $3.2 million, respectively, which begin to expire in 2036. As of December 31, 2023 and 2022, the Company had state research and development tax credit carryforwards of $2.6 million and $1.7 million, respectively, which begin to expire in 2032. As of December 31, 2023 and 2022, the Company had capitalized research and development costs of $17.4 million and $8.3 million, respectively, as required by the Tax Cuts and Jobs Act of 2017.

Future realization of the tax benefits of existing temporary differences and NOL carryforwards ultimately depends on the existence of sufficient taxable income within the carryforward period. As of December 31, 2023, the Company performed an evaluation to determine whether a valuation allowance was needed. The Company considered all available evidence, both positive and negative, which included the results of operations for the current and preceding years. The Company determined that it is more likely than not that all of the deferred tax assets will not be realized. Accordingly, the Company maintained a full valuation allowance as of December 31, 2023. The valuation allowance increased in fiscal years 2023 and 2022 by $15.8 million and $13.4 million, respectively, due to the increase in the deferred tax assets by the same amount, primarily due to NOL carryforwards and capitalized research and development costs.

The utilization of NOLs and tax credit carryforwards to offset future taxable income may be subject to an annual limitation as a result of ownership changes that have occurred previously or may occur in the future. Under Sections 382 and 383 of the Internal Revenue Code of 1986 ("IRC"), a corporation that undergoes an ownership change may be subject to limitation on its ability to utilize its pre-change NOLs and other tax attributes otherwise available to offset future taxable income and / or tax liability. An ownership change is defined as a cumulative change of more than 50% in the ownership positions of certain stockholders during a rolling three- year period.

The Company has not completed a formal study to determine if any ownership changes within the meaning of IRC Section 382 and 383 have occurred as of December 31, 2023. An ownership change would restrict its ability to use its NOLs or tax credit carryforwards and could require the Company to pay federal or state income taxes earlier than would be required if such limitation were not in effect.

For the year ended December 31, 2023, the Company generated research credits but has not conducted a study to document the qualified activities. This study may result in an adjustment to the Company's research and development credit carryforwards; however, until a study is completed and any adjustment is known, no amounts have been recognized as an uncertain tax position. A full valuation allowance has been provided against the Company's research and development credits and, if an adjustment is required, this adjustment would be offset by an adjustment to the deferred income tax asset established for the research and development credit carryforwards and the valuation allowance.

12. Net loss per share attributable to common stockholders

Basic and diluted net loss per share was calculated as follows (in thousands, except share and per share amounts):

	Year ended December 31,	
	2023	2022
Numerator:		
Net loss attributable to common stockholders	$ (49,291)	$ (44,192)
Denominator:		
Weighted-average common shares outstanding, basic and diluted	4,429,564	3,503,242
Net loss per share attributable to common stockholders, basic and diluted	$ (11.13)	$ (12.61)

The Company's potentially dilutive securities, which include convertible preferred stock, outstanding stock options, unvested restricted common stock, and convertible preferred stock warrants, have been excluded from the computation of diluted net loss per share as the effect would be to reduce the net loss per share. Therefore, the weighted-average number of common shares outstanding used to calculate both basic and diluted net loss per share attributable to common stockholders is the same.

The Company excluded the following from the computation of diluted net loss per share attributable to common stockholders at December 31, 2023 and 2022 because including them would have had an anti-dilutive effect:

	Years ended December 31,	
	2023	2022
Conversion of preferred stock	130,648,426	130,648,426
Stock options outstanding	25,514,335	27,496,583
Conversion of preferred stock warrant	1,602	1,602
Unvested restricted common stock	832,840	1,516,230
	156,997,203	159,662,841

13. Employee benefit plan

On January 1, 2017, the Company's board of directors approved the Company's 401(k) retirement plan (the "401(k) Plan"). Employees of the Company are eligible to participate in the 401(k) Plan. Participants may contribute up to 100% of their annual compensation to the 401(k) Plan, subject to statutory limitations. Effective January 1, 2022, under the 401(k) Plan "Safe Harbor Match", the Company matches one hundred percent (100%) of the first three percent (3%) of employee contributions and these contributions vest in full at the time of match.

For the year ended December 31, 2023 and 2022, the Company made matching contributions of $0.4 million and $0.3 million, respectively.

14. Related parties

In September 2015, the Company entered into consulting agreements with its two founders, related parties who hold shares of the Company's common stock, to provide R&D and strategic planning services. For the years ended December 31, 2023 and 2022, the Company recognized R&D expense totaling $0.3 million and $0.3 million, respectively, related to work performed under the founder agreements. The Company had no amounts due to the founders at both December 31, 2023 and 2022, respectively. For the years ended December 31, 2023 and 2022, the Company recognized stock-based compensation expense totaling $0.2 million and less than $0.1 million, related to the consulting agreements, respectively.

In March 2019, the Company entered into a consulting agreement with an executive consultant, a related party who holds shares of the Company's common stock. For the years ended December 31, 2023 and 2022, the Company recognized G&A expense totaling $0.1 million and $0.1 million, respectively, related to work performed under the consulting agreement. The Company had no amounts due to the consultant at both December 31, 2023 and 2022, respectively. For the years ended December 31, 2023 and 2022, the Company recognized stock-based compensation expense totaling $0.2 million and less than $0.1 million, respectively, related to the consulting agreement.

15. Subsequent events

The Company evaluated subsequent events through June 14, 2024, the date on which the December 31, 2023 consolidated financial statements were issued. No subsequent events requiring disclosure were identified.

Through and including , 2024 (the 25th day after the commencement of this offering), all dealers that buy, sell or trade shares of our common stock, whether or not participating in this offering, may be required to deliver a prospectus. This delivery requirement is in addition to the dealers' obligation to deliver a prospectus when acting as underwriters and with respect to their unsold allotments or subscriptions.

shares

Common Stock

J.P. Morgan **Leerink Partners** **Piper Sandler**

William Blair

Part II

Information not required in prospectus

Item 13. Other expenses of issuance and distribution.

The following table indicates the costs and expenses, other than the underwriting discounts and commissions, payable by the registrant in connection with the offering described in this registration statement. All amounts are estimated except the Securities and Exchange Commission, or the SEC, registration fee, the Financial Industry Regulatory Authority, Inc., or FINRA, filing fee and the Nasdaq Global Market initial listing fee.

	Amount
SEC registration fee	$ *
FINRA filing fee	*
Nasdaq Global Market initial listing fee	*
Accountants' fees and expenses	*
Legal fees and expenses	*
Blue sky fees and expenses	*
Transfer agent's fees and expenses	*
Printing and engraving expenses	*
Miscellaneous	*
Total expenses	**$** *

* To be provided by amendment

Item 14. Indemnification of directors and officers.

As permitted by Section 102(b)(7) of the DGCL, we plan to include in our Restated Charter a provision to eliminate the personal liability of our directors for monetary damages for breach of their fiduciary duties as directors, subject to certain exceptions. In addition, our Restated Charter and Restated Bylaws will provide that we are required to indemnify our officers and directors under certain circumstances, including those circumstances in which indemnification would otherwise be discretionary, and we are required to advance expenses to our officers and directors as incurred in connection with proceedings against them for which they may be indemnified, in each case except to the extent that the DGCL prohibits the elimination or limitation of liability of directors for breaches of fiduciary duty.

Section 145(a) of the DGCL provides that a corporation shall have the power to indemnify any person who was or is a party or is threatened to be made a party to any threatened, pending or completed action, suit or proceeding, whether civil, criminal, administrative or investigative (other than an action by or in the right of the corporation) by reason of the fact that the person is or was a director, officer, employee or agent of the corporation, or is or was serving at the request of the corporation as a director, officer, employee or agent of another corporation, partnership, joint venture, trust or other enterprise, against expenses (including attorneys' fees), judgments, fines and amounts paid in settlement actually and reasonably incurred by the person in connection with such action, suit or proceeding if the person acted in good faith and in a manner the person reasonably believed to be in or not opposed to the best interest of the corporation, and, with respect to any criminal action or proceeding, had no reasonable cause to believe the person's conduct was unlawful. The termination of any action, suit or proceeding by judgment, order, settlement, conviction or upon a plea of nolo contendere or its equivalent shall not, of itself, create a presumption that the person did not act in good faith and in a manner which the person reasonably believed to be in or not opposed to the best interests of the corporation, and, with respect to any criminal action or proceeding, had reasonable cause to believe that the person's conduct was unlawful.

Section 145(b) of the DGCL provides that a corporation shall have the power to indemnify any person who was or is a party or is threatened to be made a party to any threatened, pending or completed action or suit by or in the

right of the corporation to procure a judgment in its favor by reason of the fact that the person is or was a director, officer, employee or agent of the corporation, or is or was serving at the request of the corporation as a director, officer, employee or agent of another corporation, partnership, joint venture, trust or other enterprise against expenses (including attorneys' fees) actually and reasonably incurred by the person in connection with the defense or settlement of such action or suit if the person acted in good faith and in a manner the person reasonably believed to be in or not opposed to the best interests of the corporation and except that no indemnification shall be made with respect to any claim, issue or matter as to which such person shall have been adjudged to be liable to the corporation unless and only to the extent that the Court of Chancery or the court in which such action or suit was brought shall determine upon application that, despite the adjudication of liability but in view of all the circumstances of the case, such person is fairly and reasonably entitled to indemnity for such expenses which the Court of Chancery or such other court shall deem proper.

We have entered into indemnification agreements with our directors and, prior to the completion of this offering, intend to enter into indemnification agreements with certain of our officers. These indemnification agreements will provide broader indemnity rights than those provided under the DGCL and our Restated Charter. These indemnification agreements are not intended to deny or otherwise limit third-party or derivative suits against us or our directors or officers, but to the extent a director or officer were entitled to indemnity or contribution under the indemnification agreement, the financial burden of a third-party suit would be borne by us, and we would not benefit from derivative recoveries against the director or officer. Such recoveries would accrue to our benefit but would be offset by our obligations to the director or officer under the indemnification agreement.

The underwriting agreement will provide that the underwriters are obligated, under certain circumstances, to indemnify our directors, officers and controlling persons against certain liabilities, including liabilities under the Securities Act.

We maintain directors' and officers' liability insurance for the benefit of our directors and officers.

Item 15. Recent sales of unregistered securities.

The following list sets forth information regarding all unregistered securities sold by us in the three years preceding the filing of this registration statement. None of the following transactions involved any underwriters, underwriting discounts or commissions, or any public offering. Unless otherwise specified above, we believe these transactions were exempt from registration under the Securities Act in reliance on Sections 3(a)(9) and 4(a)(2) of the Securities Act (and Regulation D or Regulation S promulgated thereunder) or Rule 701 promulgated under Section 3(b) of the Securities Act as transactions by an issuer not involving any public offering or under benefit plans and contracts relating to compensation as provided under Rule 701. The recipients of the securities in each of these transactions represented their intentions to acquire the securities for investment only and not with a view to or for sale in connection with any distribution thereof, and appropriate legends were placed on the share certificates issued in these transactions. All recipients had adequate access, through their relationships with us, to information about us. The sales of these securities were made without any general solicitation or advertising.

(a) Issuances of common stock, stock options and restricted shares pursuant to our equity compensation plans

In 2021, we granted stock options to purchase an aggregate of 8,203,100 shares of our common stock at a weighted-average exercise price of $0.21 to employees, directors and consultants. We also issued 64,259 shares of our common stock upon the exercise of stock options at a weighted-average exercise price of $0.29 per share.

In 2022, we granted stock options to purchase an aggregate of 17,562,710 shares of our common stock at a weighted-average exercise price of $0.71 to employees, directors and consultants. We also issued 224,245 shares of our common stock upon the exercise of stock options at a weighted-average exercise price of $0.19 per share.

In 2023, we granted stock options to purchase an aggregate of 2,144,445 shares of our common stock at a weighted-average exercise price of $0.78 to employees, directors and consultants. We also issued 482,700 shares of our common stock upon the exercise of stock options at a weighted-average exercise price of $0.38 per share.

Since January 1, 2024, we have granted stock options to purchase an aggregate of shares of our common stock at a weighted-average exercise price of $ to employees, directors and consultants.

In 2021, we issued 2,416,085 shares of restricted stock to certain directors and consultants for services rendered to the Company. The restricted stock awards vest in equal monthly installments over 48 months, starting on March 31, 2021. We also issued 190,827 shares of restricted stock to Ravi I. Thadhani, a member of our board of directors, for services. 25% of the restricted stock award vested on March 31, 2022, and the remaining 75% vests in equal monthly installments over the 36-month period that follows.

(b) Issuances of preferred stock

In March 2021 with subsequent closings through October 2021, we issued and sold an aggregate of 212,264,148 shares of our Series A Prime convertible preferred stock at a purchase price of $0.2120 per share for aggregate gross proceeds of $45.0 million.

In June 2022 and July 2022, we issued and sold an aggregate of 68,258,635 shares of our Series B convertible preferred stock at a purchase price of $1.4724 per share for aggregate gross proceeds of $100.5 million.

Item 16. Exhibits and financial statement schedules.

(a) Exhibits.

The exhibits listed below are filed as part of this registration.

Exhibit no.	Description of exhibit
1.1*	Form of Underwriting Agreement.
3.1*	Form of Amended and Restated Certificate of Incorporation of the Registrant (to be effective prior to the completion of this offering).
3.2*	Form of Amended and Restated Bylaws of the Registrant (to be effective prior to the completion of this offering).
4.1*	Third Amended and Restated Investors' Rights Agreement, by and among the Registrant and certain of its stockholders, dated June 3, 2022.
4.2*	Specimen Stock Certificate.
5.1*	Opinion of Ropes & Gray LLP.
10.1+**	Patent License Agreement by and between CAMP4 Therapeutics Corporation and the Whitehead Institute for Biomedical Research, dated as of October 23, 2019.
10.2+**	First Amendment to Patent License Agreement by and between CAMP4 Therapeutics Corporation and the Whitehead Institute for Biomedical Research, dated as of December 14, 2021.
10.3+**	Second Amendment to Patent License Agreement by and between CAMP4 Therapeutics Corporation and the Whitehead Institute for Biomedical Research, dated as of November 7, 2023.
10.4#*	Amended and Restated 2016 Stock Option and Grant Plan, and form of award agreements thereunder.
10.5#*	2024 Equity Incentive Plan.
10.6#*	Form of Non-Qualified Stock Option Award Agreement for Non-Employee Directors under the 2024 Equity Incentive Plan.
10.7#*	Form of Incentive Stock Option Award Agreement under the 2024 Equity Incentive Plan.
10.8#*	Form of Non-Qualified Stock Option Award Agreement under the 2024 Equity Incentive Plan.
10.9#*	Form of Restricted Stock Unit Award Agreement under the 2024 Equity Incentive Plan.
10.10#*	2024 Employee Stock Purchase Plan.
10.11#*	Form of Indemnification Agreement between the Registrant and each of its directors and executive officers.
21.1*	Subsidiaries of the Registrant.

Exhibit no.	Description of exhibit
23.1*	Consent of Ernst & Young LLP, independent registered public accounting firm
23.2*	Consent of Ropes & Gray LLP (included in Exhibit 5.1).
24.1*	Power of Attorney (included on signature page).
107*	Filing Fee Table.

* To be filed by amendment.

\# Indicates management contract or compensatory plan.

\+ Portions of this exhibit (indicated by asterisks) have been redacted pursuant to Item 601 of Regulation S-K because they are both not material and the registrant customarily and actually treats such information as private or confidential.

** Previously filed.

(b) Financial statement schedules.

Schedules not listed above have been omitted because the information required to be set forth therein is not applicable or is shown in the financial statements or notes thereto.

Item 17. Undertakings.

The undersigned Registrant hereby undertakes to provide to the underwriters at the closing specified in the underwriting agreement, certificates in such denominations and registered in such names as required by the underwriters to permit prompt delivery to each purchaser.

Insofar as indemnification for liabilities arising under the Securities Act may be permitted to directors, officers and controlling persons of the Registrant pursuant to the foregoing provisions, or otherwise, the Registrant has been advised that in the opinion of the SEC such indemnification is against public policy as expressed in the Securities Act and is, therefore, unenforceable. In the event that a claim for indemnification against such liabilities (other than the payment by the Registrant of expenses incurred or paid by a director, officer or controlling person of the Registrant in the successful defense of any action, suit or proceeding) is asserted by such director, officer or controlling person in connection with the securities being registered, the Registrant will, unless in the opinion of its counsel the matter has been settled by controlling precedent, submit to a court of appropriate jurisdiction the question of whether such indemnification by it is against public policy as expressed in the Securities Act and will be governed by the final adjudication of such issue.

The undersigned Registrant hereby undertakes that:

(1) For purposes of determining any liability under the Securities Act of 1933, the information omitted from the form of prospectus filed as part of this Registration Statement in reliance upon Rule 430A and contained in a form of prospectus filed by the Registrant pursuant to Rule 424(b)(1) or (4) or 497(h) under the Securities Act shall be deemed to be part of this Registration Statement as of the time it was declared effective.

(2) For the purpose of determining any liability under the Securities Act of 1933, each post-effective amendment that contains a form of prospectus shall be deemed to be a new registration statement relating to the securities offered therein, and the offering of such securities at that time shall be deemed to be the initial bona fide offering thereof.

Signatures

Pursuant to the requirements of the Securities Act of 1933, the registrant has duly caused this registration statement to be signed on its behalf by the undersigned, thereunto duly authorized in the City of Cambridge, Commonwealth of Massachusetts, on this day of , 2024.

CAMP4 Therapeutics Corporation

By: _____

Josh Mandel-Brehm
President and Chief Executive Officer

KNOW ALL BY THESE PRESENTS, that each person whose signature appears below hereby constitutes and appoints Josh Mandel-Brehm and Kelly Gold, and each of them, as his or her true and lawful agents, proxies and attorneys-in-fact, with full power of substitution and resubstitution, for him or her and in his or her name, place and stead, in any and all capacities, to (i) act on, sign and file with the Securities and Exchange Commission any and all amendments (including post-effective amendments) to this registration statement together with all schedules and exhibits thereto and any subsequent registration statement filed pursuant to Rule 462(b) under the Securities Act of 1933, as amended, together with all schedules and exhibits thereto, (ii) act on, sign and file such certificates, instruments, agreements and other documents as may be necessary or appropriate in connection therewith, (iii) act on and file any supplement to any prospectus included in this registration statement or any such amendment or any subsequent registration statement filed pursuant to Rule 462(b) under the Securities Act of 1933, as amended, and (iv) take any and all actions which may be necessary or appropriate to be done, as fully for all intents and purposes as he might or could do in person, hereby approving, ratifying and confirming all that such agent, proxy and attorney-in-fact or any of his substitutes may lawfully do or cause to be done by virtue thereof.

Pursuant to the requirements of the Securities Act of 1933, this registration statement has been signed by the following persons in the capacities and on the dates indicated.

Signature	Title	Date
_____ Josh Mandel-Brehm	President, Chief Executive Officer and Director *(Principal Executive Officer)*	, 2024
_____ Kelly Gold	Chief Financial Officer (*Principal Financial Officer and Principal Accounting Officer*)	, 2024
_____ Steven Holtzman	Director and Chair	, 2024
_____ James Boylan	Director	, 2024
_____ Jorge Conde	Director	, 2024

Signature	Title	Date
Ingo Chakravarty	Director	, 2024
Michael Higgins	Director	, 2024
Amir Nashat, ScD	Director	, 2024
Paula Ragan, PhD	Director	, 2024
Andy Schwab	Director	, 2024
Ravi I. Thadhani, MD, MPH	Director	, 2024
Richard Young, PhD	Director	, 2024