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UNITED STATES
SECURITIES AND EXCHANGE COMMISSION

Washington, D.C. 20549

FORM 10-Q

(Mark One)

☒ **QUARTERLY REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934**

For the quarterly period ended September 30, 2024

or

☐ **TRANSITION REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934**

For the transition period from _____ to _____

Commission File Number: 001-42365

CAMP4 Therapeutics Corporation

(Exact name of registrant as specified in its charter)

Delaware	**81-1152476**
(State or other jurisdiction of incorporation or organization)	**(I.R.S. Employer Identification Number)**

One Kendall Square
Building 1400 West, 3rd Floor
Cambridge, Massachusetts 02139
(Address of Principal Executive Offices)

(617) 651-8867
(Registrant's telephone number)

Indicate by check mark whether the registrant (1) has filed all reports required to be filed by Section 13 or 15(d) of the Securities Exchange Act of 1934 during the preceding 12 months (or for such shorter period that the registrant was required to file such reports), and (2) has been subject to such filing requirements for the past 90 days. Yes ☐ No ☒

Indicate by check mark whether the registrant has submitted electronically every Interactive Data File required to be submitted pursuant to Rule 405 of Regulation S-T (§232.405 of this chapter) during the preceding 12 months (or for such shorter period that the registrant was required to submit such files). Yes ☒ No ☐

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 accelerated 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 13(a) of the Exchange Act. ☐

Indicate by check mark whether the registrant is a shell company (as defined in Rule 12b-2 of the Exchange Act). Yes ☐ No ☒

Securities registered pursuant to Section 12(b) of the Act:

Title of Each Class	Trading symbol	Name of Exchange on which registered
Common stock, par value $0.0001 per share	**CAMP**	**Nasdaq Global Market**

As of November 18, 2024, there were 20,161,073 shares of the registrant's common stock, par value $0.0001 per share, outstanding.

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SPECIAL NOTE REGARDING FORWARD-LOOKING STATEMENTS

This Quarterly Report on Form 10-Q ("Quarterly Report") 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 Quarterly Report, 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 our CMP-CPS-001 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;
- the sufficiency of our existing cash and cash equivalents 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 Jumpstart Our Business Startups Act of 2012 (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 Quarterly Report. 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 Quarterly Report, 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 Quarterly Report 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 Quarterly Report.

SUMMARY RISK FACTORS

Our business is subject to a number of risks of which you should be aware before making an investment decision. These risks are discussed more fully in Part II, Item 1A. "Risk Factors" in this Quarterly Report. These risks include the following:

- 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 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;
- 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;

- Prior to the completion of our IPO, there was no public market for our common stock. An active, liquid, and orderly market for our common stock may not develop or be sustained, or we may in the future fail to satisfy the continued listing requirements of Nasdaq; 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 adequately address these and other risks we face, our business, results of operations, financial condition and prospects may be harmed.

NOTE REGARDING TRADEMARKS

"CAMP4," "RAP Platform," "RNA Actuator" and our other registered or common law trademarks, trade names or service marks appearing in this Quarterly Report are the property of CAMP4 Therapeutics Corporation and are registered as trademarks in the U.S. and other countries. This Quarterly Report also contains references to trademarks belonging to other entities. Solely for convenience, trademarks and trade names referred to in this Quarterly Report, 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.

PART I —FINANCIAL INFORMATION

Item 1. Financial Statements

CAMP4 Therapeutics Corporation

Condensed Consolidated Balance Sheets
(In thousands, except share and per share amounts)

	As of September 30, 2024 (Unaudited)	As of December 31, 2023
Current assets:		
Cash and cash equivalents	$ 2,528	$ 38,380
Accounts receivable	1,000	—
Prepaid expenses and other current assets	1,976	1,633
Total current assets	5,504	40,013
Restricted cash	1,624	1,624
Deferred offering costs	3,324	—
Property and equipment, net	3,831	4,797
Operating lease right-of-use assets, net	6,479	7,764
Finance lease right-of-use assets, net	602	748
Total assets	$ 21,364	$ 54,946
Current liabilities:		
Accounts payable	$ 3,873	$ 1,042
Accrued expenses	3,677	3,302
Deferred revenue, short-term	1,000	—
Operating lease liabilities, current portion	2,919	2,704
Finance lease liabilities, current portion	170	354
Financing liability, current portion	190	405
Total current liabilities	11,829	7,807
Long-term liabilities:		
Operating lease liabilities, net of current portion	6,274	8,487
Finance lease liabilities, net of current portion	87	148
Financing liability, net of current portion	—	85
Other long-term liabilities	2	2
Total liabilities	18,192	16,529
Commitments and contingencies (Note 7)		
Convertible preferred stock, $0.0001 par value; 149,673,284 shares authorized as of September 30, 2024 and December 31, 2023; 130,648,426 shares issued and outstanding as of September 30, 2024 and December 31, 2023; liquidation preference of $162,885 as of September 30, 2024 and December 31, 2023	162,147	162,147
Stockholders' deficit:		
Common stock, $0.0001 par, 210,000,000 shares authorized as of September 30, 2024 and December 31, 2023, 1,036,537 and 1,026,057 shares issued, 1,005,930 and 460,704 shares outstanding as of September 30, 2024 and December 31, 2023, respectively	2	1
Additional paid-in capital	39,497	36,231
Accumulated deficit	(198,474)	(159,962)
Total stockholders' deficit	(158,975)	(123,730)
Total liabilities, convertible preferred stock, and stockholders' deficit	$ 21,364	$ 54,946

The accompanying notes are an integral part of these condensed consolidated financial statements.

CAMP4 Therapeutics Corporation

Condensed Consolidated Statements of Operations and Comprehensive Loss
(In thousands, except for share and per share data)

	Three months ended September 30,		Nine months ended September 30,	
	2024	2023	2024	2023
	(Unaudited)	(Unaudited)	(Unaudited)	(Unaudited)
Revenue				
Research and collaboration revenue	$ —	$ 350	$ —	$ 350
Operating Expenses:				
Research and development	9,702	9,819	28,821	29,955
General and administrative	3,814	2,869	10,233	8,798
Total operating expenses	13,516	12,688	39,054	38,753
Loss from operations	(13,516)	(12,338)	(39,054)	(38,403)
Other income (expense), net:				
Interest income	94	689	720	2,239
Other expense	(62)	(47)	(178)	(137)
Total other income, net	32	642	542	2,102
Net loss attributable to common stockholders and comprehensive loss	$ (13,484)	$ (11,696)	$ (38,512)	$ (36,301)
Net loss per share attributable to common stockholders, basic and diluted	$ (24.19)	$ (29.21)	$ (76.50)	$ (94.62)
Weighted-average shares of common stock outstanding, basic and diluted	557,437	400,426	503,455	383,653

The accompanying notes are an integral part of these condensed consolidated financial statements.

CAMP4 Therapeutics Corporation

Condensed Consolidated Statements of Convertible Preferred Stock and Stockholders' Deficit
(In thousands, except share amounts)
(Unaudited)

	Convertible preferred stock		Common stock		Additional paid-in capital	Accumulated deficit	Total stockholders' deficit
	Shares	Amount	Shares	Amount			
Balance at December 31, 2023	130,648,426	$ 162,147	460,704	$ 1	$ 36,231	$ (159,962)	$ (123,730)
Issuance of common stock	—	—	971	—	2	—	2
Vesting of restricted common stock	—	—	14,589	—	—	—	—
Stock-based compensation expense	—	—	—	—	856	—	856
Net loss	—	—	—	—	—	(12,456)	(12,456)
Balance at March 31, 2024	130,648,426	$ 162,147	476,264	$ 1	$ 37,089	$ (172,418)	$ (135,328)
Issuance of common stock	—	—	88	—	—	—	—
Vesting of restricted common stock	—	—	14,525	—	—	—	—
Stock-based compensation expense	—	—	—	—	786	—	786
Net loss	—	—	—	—	—	(12,572)	(12,572)
Balance at June 30, 2024	130,648,426	$ 162,147	490,877	$ 1	$ 37,875	$ (184,990)	$ (147,114)
Issuance of common stock	—	—	500,531	1	30	—	31
Vesting of restricted common stock	—	—	14,522	—	—	—	—
Stock-based compensation expense	—	—	—	—	1,592	—	1,592
Net loss	—	—	—	—	—	(13,484)	(13,484)
Balance at September 30, 2024	130,648,426	$ 162,147	1,005,930	$ 2	$ 39,497	$ (198,474)	$ (158,975)

	Convertible preferred stock		Common stock		Additional paid-in capital	Accumulated deficit	Total stockholders' deficit
	Shares	Amount	Shares	Amount			
Balance at December 31, 2022	130,648,426	$ 162,147	356,759	$ 1	$ 33,139	$ (110,671)	$ (77,531)
Issuance of common stock	—	—	2,751	—	7	—	7
Vesting of restricted common stock	—	—	16,331	—	—	—	—
Stock-based compensation expense	—	—	—	—	763	—	763
Net loss	—	—	—	—	—	(12,506)	(12,506)
Balance at March 31, 2023	130,648,426	$ 162,147	375,841	$ 1	$ 33,909	$ (123,177)	$ (89,267)
Issuance of common stock	—	—	1,818	—	4	—	4
Vesting of restricted common stock	—	—	15,111	—	—	—	—
Stock-based compensation expense	—	—	—	—	786	—	786
Net loss	—	—	—	—	—	(12,100)	(12,100)
Balance at June 30, 2023	130,648,426	$ 162,147	392,770	$ 1	$ 34,699	$ (135,277)	$ (100,577)
Issuance of common stock	—	—	232	—	1	—	1
Vesting of restricted common stock	—	—	14,739	—	—	—	—
Stock-based compensation expense	—	—	—	—	722	—	722
Net loss	—	—	—	—	—	(11,696)	(11,696)
Balance at September 30, 2023	130,648,426	$ 162,147	407,741	$ 1	$ 35,422	$ (146,973)	$ (111,550)

The accompanying notes are an integral part of these condensed consolidated financial statements.

CAMP4 Therapeutics Corporation

Condensed Consolidated Statements of Cash Flows
(In thousands)

	Nine months ended September 30,	
	2024	2023
	(Unaudited)	(Unaudited)
Operating activities		
Net loss	$ (38,512)	$ (36,301)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation and amortization	1,278	1,243
Stock-based compensation expense	3,235	2,271
Non-cash lease expense	1,285	1,310
Non-cash interest expense	68	62
Change in operating assets and liabilities:		
Prepaid expenses and other current assets	(343)	(333)
Accounts payable	1,024	(965)
Accrued expenses and other liabilities	(310)	(698)
Operating lease assets and liabilities	(1,999)	1,039
Net cash used in operating activities	(34,274)	(32,372)
Investing activities		
Purchases of property and equipment	(178)	(435)
Net cash used in financing activities	(178)	(435)
Financing activities		
Proceeds from issuance of common stock	33	12
Proceeds from financing obligation, net of transaction costs	—	706
Principal payments on financing obligation	(345)	(153)
Principal payments on finance leases	(268)	(201)
Payments of deferred offering costs	(820)	—
Net cash (used in) provided by financing activities	(1,400)	364
Net decrease in cash, cash equivalents and restricted cash	(35,852)	(32,443)
Cash, cash equivalents and restricted cash – beginning of year	40,004	84,536
Cash, cash equivalents and restricted cash – end of period	$ 4,152	$ 52,093
Supplemental disclosures of cash flow information:		
Operating lease right-of-use asset obtained in exchange for lease liabilities	$ —	$ 1,397
Finance lease right-of-use asset obtained in exchange for lease liabilities	$ —	$ 368
Deferred offering costs in accounts payable and accrued expenses	$ 2,504	$ —
Purchase of property and equipment in accounts payable and accrued expenses	$ —	$ 56

The accompanying notes are an integral part of these condensed consolidated financial statements.

CAMP4 Therapeutics Corporation
Notes to Condensed Consolidated Financial Statements
(Unaudited)

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 ("U.S. GAAP"). Any reference in these notes to applicable guidance is meant to refer to the authoritative standards of U.S. GAAP as found in the Accounting Standards Codification ("ASC") and Accounting Standards Updates ("ASU") of the Financial Accounting Standards Board ("FASB").

These unaudited condensed consolidated financial statements have been prepared on the same basis as the annual consolidated financial statements and, in the opinion of management, reflect all adjustments, which include only normal recurring adjustments, necessary to present fairly the Company's consolidated financial position, consolidated results of operations, and consolidated cash flows for the interim periods presented. The results of operations for the three and nine months ended September 30, 2024 are not necessarily indicative of the results to be expected for the year ending December 31, 2024 or for any other future annual or interim period.

The condensed consolidated balance sheet as of December 31, 2023 included herein was derived from the audited consolidated financial statements as of that date which was included in the prospectus (the "IPO Prospectus") filed on October 11, 2024 pursuant to Rule 424(b) under the Securities Act of 1933, as amended ("the Securities Act") with the Securities and Exchange Commission ("the SEC"). These unaudited condensed consolidated financial statements should be read in conjunction with the Company's audited consolidated financial statements for the year ended December 31, 2023 contained in the IPO Prospectus.

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.

Reverse-Stock Split

On October 3, 2024, the Company effected a one-for-11.2158 reverse stock split of its issued and outstanding shares of common stock and a proportional adjustment to the existing conversion ratios for each series of the Company's convertible preferred stock. Accordingly, all share and per share amounts for all periods presented in the accompanying condensed consolidated financial statements and notes thereto have been adjusted retroactively, where applicable, to reflect this reverse stock split and adjustment of the preferred stock conversion ratios. The par value and the number of authorized shares of the convertible preferred stock and common stock were not adjusted in connection with the reverse stock split.

Liquidity and Going Concern

As of September 30, 2024, the Company had approximately $2.5 million of unrestricted cash and cash equivalents and working capital of approximately $0.6 million. The Company has experienced net losses and negative cash flows from operations since its inception and, as of September 30, 2024, the Company had an accumulated deficit of $198.5 million. The revenue and income potential of the Company's business and market are unproven. During the nine months ended September 30, 2024, the Company incurred a net loss of $38.5 million and had negative cash flows from operations of $34.3 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.

On October 15, 2024, upon the closing of the Company's initial public offering (the "IPO"), the Company received aggregate proceeds, net of underwriting discounts and commissions, of $69.8 million. In addition, on November 1, 2024, the Company received additional proceeds of $6.6 million pursuant to the partial exercise by the underwriters of their option to purchase additional shares in the IPO. Collectively, the Company received aggregate net proceeds of $76.4 million from the IPO and the subsequent partial exercise by the underwriters of their option to purchase additional shares ("IPO Proceeds"). The IPO Proceeds, together with the Company's existing cash and cash equivalents, will be sufficient to fund the Company's planned operations for at least 12 months beyond the issuance date of these condensed financial statements. As such, substantial doubt does not exist about the Company's ability to continue as a going concern for the one year period following the date that these unaudited condensed financial statements and accompanying notes were issued. The Company will eventually require additional funding in order to fund its planned operations. If the Company is unable to obtain additional funding before achieving sufficient profitability and positive cash flows from operations, if ever, the Company will be forced to delay, reduce or eliminate some or all of its research and development programs, which could adversely affect its business prospects, or the Company may be unable to continue operations. Although management continues to pursue plans to obtain additional funding before achieving sufficient profitability and positive cash flows from operations, there is no assurance that the Company will be successful in obtaining sufficient funding on terms acceptable to the Company to fund continuing operations, if at all.

2. Summary of Significant Accounting Policies

Use of Estimates

The preparation of condensed consolidated financial statements in conformity with U.S. 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 condensed 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 condensed 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 September 30, 2024 and December 31, 2023, 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 September 30, 2024 and December 31, 2023, 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 condensed consolidated balance sheets. In addition, the Company held cash of $0.1 million as of September 30, 2024 and December 31, 2023, in money market accounts as collateral for the Company's credit card obligation and increased letter of credit due to an amendment to the lease.

The following table provides a reconciliation of cash, cash equivalents and restricted cash reported in the condensed consolidated balance sheets to the corresponding amounts shown in the condensed consolidated statements of cash flows (in thousands):

	September 30, 2024	September 30, 2023
Cash and cash equivalents	$ 2,528	$ 50,469
Restricted cash	1,624	1,624
Total cash, cash equivalents, and restricted cash	$ 4,152	$ 52,093

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 patents, patent litigation, compliance with government regulations, dependence on key personnel and collaboration partners, dependence on third-party manufacturers and competition from other 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 periods 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 condensed consolidated statements of operations and comprehensive loss and were immaterial for the three and nine months ended September 30, 2024 and 2023.

Comprehensive Loss

There were no differences between net loss and comprehensive loss presented in the condensed consolidated statements of operations for the three and nine months ended September 30, 2024 and 2023.

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 did not recognize any impairment losses for the three and nine months ended September 30, 2024 and 2023.

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 September 30, 2024 and December 31, 2023, 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 condensed 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 condensed 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 condensed consolidated 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.

During the three and nine months ended September 30, 2024 and 2023, the Company did not recognize any revenue under ASC 606.

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 the Company's condensed 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. Pursuant to ASC 340-10-S99-1, initial public offering costs directly attributable to an offering of equity securities are deferred and charged against the gross proceeds of the offering as a reduction of additional paid-in capital. Deferred offering costs consist of professional and registration fees that are directly related to the proposed public offering. Upon the completion of the IPO, the deferred offering costs were expensed immediately as a charge to operating expenses in the Company's consolidated statements of operations and comprehensive loss. As of September 30, 2024 and December 31, 2023, the Company recorded deferred offering costs of $3.3 million and $0, respectively, as presented on the condensed consolidated balance sheets.

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 condensed 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 prior to the Company's IPO in October 2024, the fair value of the Company's common stock was 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 had no trading history of its common stock prior to the IPO.

The Company recognizes forfeitures related to stock-based compensation awards as they occur.

The Company classifies stock-based compensation expense in the condensed 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.

Classification of Convertible Preferred Stock

The Company's convertible preferred stock is classified as temporary equity in the accompanying condensed 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 September 30, 2024 and December 31, 2023. 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 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 condensed consolidated financial statements may not be comparable to companies that comply with public company FASB standards' effective dates.

Recently Issued Accounting Standards

Accounting standards not listed below were assessed and determined not to be applicable or are expected to have a minimal impact on the Company's condensed 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 September 30, 2024 and December 31, 2023, respectively, in accordance with the ASC 820 hierarchy (in thousands):

	Fair Value Measurements at September 30, 2024			
	Level 1	Level 2	Level 3	Total
Assets				
Cash equivalents	$ 735	$ —	$ —	$ 735

	Fair Value Measurements at December 31, 2023			
	Level 1	Level 2	Level 3	Total
Assets				
Cash equivalents	$ 37,074	$ —	$ —	$ 37,074

The Company's carrying amounts reflected in the condensed 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):

	September 30, 2024	December 31, 2023
Variable lease expenses	$ 7	$ 105
Federal R&D tax credit receivable	108	442
Software and subscription expenses	252	287
Research and development (R&D) expenses	1,319	480
Other	290	318
Total prepaid expenses and other current assets	$ 1,976	$ 1,633

5. Property and Equipment, Net

Property and equipment consisted of the following (in thousands):

	September 30, 2024	December 31, 2023
Laboratory equipment	$ 3,488	$ 3,322
Computer and software	938	938
Furniture and fixtures	524	524
Leasehold improvements	4,518	4,518
Total property and equipment	9,468	9,302
Less: accumulated depreciation and amortization	(5,637)	(4,505)
Total property and equipment, net	$ 3,831	$ 4,797

The Company incurred depreciation and amortization expense of $0.4 million and $1.3 million for the three and nine months ended September 30, 2024, respectively. Depreciation and amortization expense for the three and nine months ended September 30, 2024 included less than $0.1 million and $0.1 million of finance lease right-of-use asset amortization, respectively. The Company incurred depreciation and amortization expense of $0.4 million and $1.2 million for the three and nine months ended September 30, 2023, respectively. Depreciation and amortization expense for the three and nine months ended September 30, 2023 included less than $0.1 million and $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):

	September 30, 2024	December 31, 2023
Payroll and employee related expenses	$ 1,703	$ 1,937
Professional fees and other general and administrative expenses	1,211	475
Research and development expenses	394	601
Other	369	289
Total accrued expenses	$ 3,677	$ 3,302

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 September 30, 2024 and 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. Amounts received for lease incentives are included in the changes in operating lease assets and liabilities line in the condensed consolidated statement of cash flows. As of September 30, 2024, this operating lease accounted for $5.3 million of operating lease right-of-use assets, $2.7 million of current operating lease liabilities and $5.3 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 lease for this office and laboratory space in Boulder, Colorado expires on September 30, 2028. As of September 30, 2024, this operating lease accounted for $1.2 million of operating lease right-of-use assets, $0.3 million of current operating lease liabilities and $0.9 million of non-current operating lease liabilities.

The table below summarizes the Company's operating lease costs for the three and nine months ended September 30, 2024 and 2023 (in thousands except for lease terms and borrowing rates):

	Three months ended September 30,		Nine months ended September 30,	
	2024	2023	2024	2023
Operating lease costs:				
Lease expense	$ 609	$ 554	$ 1,827	$ 1,880
Short-term lease expense	14	31	40	88
Variable lease expense	344	286	1,017	862
Total operating lease costs	$ 967	$ 871	$ 2,884	$ 2,830
Other information:				
Cash paid for amounts included in the measurement of lease liabilities, including in operating cash flows	$ 770	$ 755	$ 2,523	$ 2,449
Weighted-average remaining lease term (in years)	2.9	3.9	2.9	3.9
Weighted-average incremental borrowing rate	6.72 %	6.72 %	6.72 %	6.72 %

Maturities of operating lease liabilities as of September 30, 2024 were as follows (in thousands):

Maturity of operating lease liabilities	
2024 remaining	$ 852
2025	3,455
2026	3,558
2027	1,980
2028	264
Total lease payments	10,109
Less: amount representing imputed interest	(916)
Total future minimum lease obligations	$ 9,193

Finance Leases

The Company leases certain specialized lab equipment under several finance lease agreements with maturities ranging from November 2024 to November 2028.

The table below summarizes the Company's finance lease costs for the three and nine months ended September 30, 2024 and 2023 (in thousands except for lease terms and borrowing rates):

		Three months ended September 30,		Nine months ended September 30,	
	Classification	2024	2023	2024	2023
Finance lease costs:					
Amortization of right-of-use assets	Depreciation and amortization	$ 49	$ 41	$ 146	$ 100
Interest on lease liabilities	Other expense	6	10	22	23
Total finance lease costs		$ 55	$ 51	$ 168	$ 123
Other information:					
Cash paid for amounts included in the measurement of lease liabilities, including in operating cash flows		$ 89	$ 89	$ 268	$ 201
Weighted-average remaining lease term		1.8	1.4	1.8	1.4
Weighted-average incremental borrowing rate		8.24 %	8.22 %	8.24 %	8.22 %

Maturities of finance lease liabilities as of September 30, 2024 were as follows (in thousands):

Maturity of finance lease liabilities		
2024 remaining	$	114
2025		98
2026		31
2027		31
Total lease payments	$	274
Less: amount representing imputed interest		(17)
Total future minimum lease obligations	$	257

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 condensed consolidated balance sheet. The Company imputes interest at a rate of 0.86% on a monthly basis. For each of the three and nine months ended in September 30, 2024 and 2023, the Company recorded less than $0.1 million of interest expense related to this financing transaction in other expense in the condensed consolidated statements 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 "Children's Medical Center Corporation 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., or Fulcrum, as described below. As part of the agreement, the Company issued a total of 15,123 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 Children's Medical Center Corporation 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 less than $0.1 million of royalties owed to CMCC under the agreement during each of the three and nine months ended September 30, 2024 and 2023, and recorded the amounts in R&D expense in the condensed 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 September 30, 2024, 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 (the "Whitehead Institute"), which was subsequently amended on December 14, 2021 (the "Whitehead First Amendment"), and on November 7, 2023 (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 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 the condensed consolidated statement of operations and comprehensive loss. The Company is also obligated to pay annual license maintenance fees for the term of the agreement, pursuant to which the Company has paid an aggregate of $0.20 million through September 30, 2024. In addition, the Company is 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 $0.27 million through September 30, 2024. The Company is obligated to pay potential development milestone payments to the Whitehead Institute of up to an aggregate of $1.9 million under the terms of the agreement upon the achievement of certain specified contingent events. In addition, if the Company successfully commercializes 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 seven years after the first commercial sale, in each case on a product-by-product and country-by-country basis. We incurred de minimis amounts of license maintenance and amendment issuance fees during each of the three and nine months ended September 30, 2024 and 2023 under the amended agreement and recorded the amounts in our research and development expense in our condensed consolidated statements 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 Children's Medical Center Corporation 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 granted 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 was fully constrained at the inception of the contract and at September 30, 2024. All variable consideration is remeasured and 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 three and nine months ended September 30, 2024, the Company did not record any license revenue pursuant to the Fulcrum Agreement. During the three and nine months ended September 30, 2023, the Company recorded $0.35 million of 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"), which was subsequently amended in July 2024. 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 three and nine months ended September 30, 2024, the Company received $0.2 million and $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 both parties are exposed to the significant risks and potential rewards under the MTA. For the three and nine months ended September 30, 2024, the Company recorded $0.1 million and $0.3 million, respectively, as a reduction in R&D expense in the condensed consolidated statement of operations and comprehensive loss. For the three and nine months ended September 30, 2023, the Company recorded $0.2 million and $0.2 million, respectively, as a reduction in R&D expense in the condensed consolidated statement of operations and comprehensive loss. Additionally, the Company had an unbilled receivable of less than $0.1 million and $0.1 million recorded within prepaid expenses and other current assets on the condensed consolidated balance sheets as of September 30, 2024 and December 31, 2023, respectively.

Research and Collaboration Agreement

BioMarin Pharmaceutical Inc.

On September 26, 2024, the Company entered into a Collaboration and License Agreement (the "BioMarin Agreement") with BioMarin Pharmaceutical Inc. ("BioMarin"), pursuant to which the Company and BioMarin agreed to collaborate with respect to the research and discovery of regulatory RNA-targeting ASOs using the Company's proprietary RAP Platform to modulate the expression of two undisclosed genetic targets under two distinct programs. Under the terms of the agreement, BioMarin will pay an upfront, nonrefundable payment of $1.0 million, and will reimburse the Company for the research activities the Company will conduct for each program under an agreed research plan and budget. On a per-program basis, the Company will be eligible to receive up to $5.0 million in future contingent preclinical milestones, up to $75.0 million in future contingent development and regulatory milestones and up to $105.0 million in commercial sales milestones. The Company will be further eligible to receive tiered royalties at percentage rates ranging from low-single digits to the high-single digits of net sales, subject to specified reductions, until either the last-to-expire valid claim of patent covering the product, ten years after the first commercial sale, or the expiration of any applicable regulatory exclusivity obtained for the product, in each case on a product-by-product and country-by-country basis. The agreement may be terminated in its entirety or on a program-by-program basis for convenience by BioMarin. The agreement may also be terminated by either the Company or BioMarin under certain other circumstances, including material breach, as set forth in the agreement. The notice periods for termination provisions range from 30 days to 270 days depending on the reason of termination.

The Company assessed this arrangement in accordance with ASC 606 and concluded that the contract counterparty, BioMarin, is a customer. In accordance with ASC 606, the Company determined that there is one performance obligation in the BioMarin Agreement, consisting of the bundle of R&D activities to be provided by the Company and both the exclusive and non-exclusive license rights granted to BioMarin under the arrangement. The transaction price is comprised of the fixed consideration of $1.0 million for the upfront fee along with the research activity reimbursements under the agreed upon research plan and budget. The associated revenue will be recognized over time based upon a cost-to-cost method, i.e., the input method, as the transfer of control occurs over a period of time and this is the best measure of progress towards satisfying the performance obligation. The estimated remaining period over which revenue is expected to be recognized is 12 months from September 30, 2024. The arrangement includes significant variable consideration primarily in the form of milestone payments, which is fully constrained at the inception of the contract and at September 30, 2024. All variable consideration is remeasured and 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 three and nine months ended September 30, 2024, the Company did not record any research and collaboration revenue pursuant to the BioMarin Agreement as no R&D activities were provided by the Company. At contract execution, the Company had the unconditional right to bill BioMarin for the non-refundable upfront fee of $1.0 million. The $1.0 million upfront fee billing is presented within accounts receivable and short-term deferred revenue on the condensed consolidated balance sheet as of September 30, 2024.

9. Convertible Preferred Stock and Stockholders' Deficit

Convertible Preferred Stock

As of September 30, 2024 and December 31, 2023, the Company's Series A Prime and Series B convertible preferred stock have been classified as temporary equity in the accompanying condensed consolidated balance sheets.

Convertible preferred stock consisted of the following as of September 30, 2024 and December 31, 2023 (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	5,562,653
Series B	81,499,592	68,258,635	$ 100,504	6,085,929

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 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.

In conjunction with the closing of the Company's IPO on October 15, 2024, all shares of preferred stock, including 62,389,791 shares of Series A Prime convertible preferred stock and 68,258,635 shares of Series B convertible preferred stock, converted automatically into an aggregate of 11,648,582 shares of common stock.

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 September 30, 2024.

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

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 September 30, 2024, the Company reserved the following shares of common stock for issuance upon conversion of the outstanding convertible preferred stock and exercise of stock options:

	As of September 30, 2024
Shares reserved for convertible preferred stock	11,648,582
Shares reserved for future issuance under the 2016 Stock Incentive Plan	869,646
Shares reserved for stock option exercises	2,078,470
Shares reserved for warrants	142
Shares reserved for restricted stock vesting	30,607
Total	14,627,447

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 18,904 shares of common stock. During 2018, the Board of Directors approved an increase to 1,416,218 shares of common stock available under the Plan. During 2021, the Board of Directors approved an increase to 1,521,972 shares of common stock available under the Plan. During 2022, the Board of Directors approved another increase to 3,803,266 shares of common stock available under the Plan. As of September 30, 2024, there were 869,646 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 following table summarizes stock option activity for the nine months ended September 30, 2024 (in thousands, except share and per share amounts):

	Number of Options	Weighted-Average Exercise Price	Weighted-Average Remaining Contractual Term	Aggregate Intrinsic Value
Balance at December 31, 2023	2,273,221	$ 6.06	8.05	$ 6,893
Options granted	398,212	$ 10.33		
Options cancelled	(91,373)	$ 7.18		
Options exercised (1)	(501,590)	$ 0.06		$ 6,378
Balance at September 30, 2024	2,078,470	$ 7.80	7.92	$ 10,353
Vested and expected to vest as of September 30, 2024	2,078,470	$ 7.80	7.92	$ 10,353
Exercisable at September 30, 2024	1,002,581	$ 6.62	7.15	$ 6,177

(1) The exercised options for the three months ended September 30, 2024, include 388,488 stock options deemed outstanding common shares in connection with the forgiveness of secured promissory notes issued to certain executive officers, as further described in the IPO Prospectus.

The Company recorded stock-based compensation expense related to stock options of $1.6 million and $3.2 million for the three and nine months ended September 30, 2024, respectively. The Company recorded stock-based compensation expense related to stock options of $0.7 million and $2.3 million for the three and nine months ended September 30, 2023, respectively. The Company has an aggregate $7.2 million of gross unrecognized stock-based compensation expense as of September 30, 2024 remaining to be recognized over a weighted average period of 2.5 years.

A summary of restricted stock award activity for the nine months ended September 30, 2024 is as follows:

	Number of Shares	Weighted Average Fair Value
Unvested at December 31, 2023	74,243	$ 2.02
Granted	—	—
Vested	(43,636)	$ 2.13
Forfeited	—	—
Unvested at September 30, 2024	30,607	$ 1.86

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 September 30, 2024, total unrecognized stock-based compensation expense relating to unvested restricted common stock was less than $0.1 million. This amount is expected to be recognized over a weighted average period of 0.5 years. The fair value of shares that vested during the three and nine months ended September 30, 2024 was $0.2 million and $0.5 million, respectively.

Stock-based compensation expense related to stock options and restricted stock recorded in the accompanying condensed consolidated statements of operations is as follows (in thousands):

	Three months ended September 30,		Nine months ended September 30,	
	2024	2023	2024	2023
Research and development	$ 519	$ 368	$ 1,424	$ 1,229
General and administrative	1,073	354	1,811	1,042
Total	$ 1,592	$ 722	$ 3,235	$ 2,271

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

No provision for federal, state, or foreign income taxes has been recorded for the three and nine months ended September 30, 2024 and 2023. The Company has incurred net operating losses for all the periods presented and has not reflected any benefit for such net operating loss carryforwards in the accompanying condensed consolidated financial statements due to uncertainty around utilizing these tax attributes within their respective carryforward periods. The Company has recorded a full valuation allowance against all of its deferred tax assets as it is not more likely than not that such assets will be realized in the near future. The Company's policy is to recognize interest expense and penalties related to income tax matters as tax expense. For the three and nine months ended September 30, 2024 and 2023, the Company has not recognized any interest or penalties related to income taxes.

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):

	Three months ended September 30,		Nine months ended September 30,	
	2024	2023	2024	2023
Numerator:				
Net loss attributable to common stockholders	$ (13,484)	$ (11,696)	$ (38,512)	$ (36,301)
Denominator:				
Weighted-average common shares outstanding, basic and diluted	557,437	400,426	503,455	383,653
Net loss per share attributable to common stockholders, basic and diluted	$ (24.19)	$ (29.21)	$ (76.5)	$ (94.62)

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 September 30, 2024 and 2023 because including them would have had an anti-dilutive effect:

	September 30,	
	2024	2023
Conversion of outstanding convertible preferred stock	11,648,582	11,648,582
Options to purchase common stock	2,078,470	2,284,676
Unvested restricted common stock	30,607	46,181
Conversion of preferred stock warrant	142	142
Total	13,757,801	13,957,292

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 three and nine months ended September 30, 2024, the Company made matching contributions of less than $0.1 million and $0.3 million, respectively. For the three and nine months ended September 30, 2023, the Company made matching contributions of less than $0.1 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 three and nine months ended September 30, 2024, the Company recognized R&D expense of less than $0.1 million and $0.2 million, respectively, related to work performed under the founder agreements. For the three and nine months ended September 30, 2023, the Company recognized R&D expense of less than $0.1 million and $0.2 million, respectively, related to work performed under the founder agreements. For both the three and nine months ended September 30, 2024, the Company recognized stock-based compensation expense of less than $0.1 million and $0.1 million, related to the consulting agreements, respectively. For both the three and nine months ended September 30, 2023, the Company recognized stock-based compensation expense of less than $0.1 million and $0.2 million, related to the consulting agreements, respectively. The Company had no amounts due to the founders at September 30, 2024.

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 both the three and nine months ended September 30, 2024, the Company recognized G&A expense totaling less than $0.1 million, related to work performed under the consulting agreement. For both the three and nine months ended September 30, 2023, the Company recognized G&A expense of less than $0.1 million, related to work performed under the consulting agreement. For the three and nine months ended September 30, 2024, the Company recognized stock-based compensation expense of less than $0.1 million and $0.1 million, related to the consulting agreement. For the three and nine months ended September 30, 2023, the Company recognized stock-based compensation expense of less than $0.1 million and $0.2 million, related to the consulting agreement. The Company had no amounts due to the consultant at September 30, 2024.

Item 2. Management's Discussion and Analysis of Financial Condition and Results of Operations

The following discussion and analysis of our financial condition and results of operations and the unaudited interim condensed consolidated financial statements and related notes included in this Quarterly Report on Form 10-Q ("Quarterly Report) should be read in conjunction with the financial statements and related notes thereto as of and for the year ended December 31, 2023 contained in the prospectus (the "IPO Prospectus") filed on October 11, 2024 pursuant to Rule 424(b) under the Securities Act of 1933, as amended (the "Securities Act"), with the Securities and Exchange Commission (the "SEC"). This discussion and analysis and other parts of this Quarterly Report contain forward-looking statements. Our actual results and the timing of selected events could differ materially from those described in or implied by these forward-looking statements. Factors that could cause or contribute to such differences include, but are not limited to, those discussed in the sections of this Quarterly Report titled "Special Note Regarding Forward-Looking Statements" and "Risk Factors," under Part II, Item 1A.

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 ("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 ("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 ("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 common stock, including in our initial public offering ("IPO"), which closed on October 15, 2024, as well as through revenues from our license and collaboration agreements. Through September 30, 2024, we have received gross proceeds of $188.3 million from the sale of our convertible preferred stock. In addition, through September 30, 2024, 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 $13.5 million and $11.7 million for the three months ended September 30, 2024 and 2023, respectively, and were $38.5 million and $36.3 million for the nine months ended September 30, 2024 and 2023, respectively. As of September 30, 2024, we had an accumulated deficit of $198.5 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 our IPO on October 15, 2024, 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 program in SYNGAP1-related disorders;
- conduct preclinical studies and clinical trials for 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 third party manufacturers for preclinical and clinical drug supply supporting 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 as planned 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 or future product candidates, which we expect will take a number of years and may never occur. As a result, we will need substantial additional funding 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 and potential future collaborations, license agreements, 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 intend to develop and market ourselves, or even cease operations.

As of September 30, 2024, we had cash and cash equivalents of $2.5 million. On October 15, 2024, upon the closing of the IPO, we received aggregate proceeds, net of underwriting discounts and commissions, of $69.8 million. In addition, on November 1, 2024, we received additional proceeds of $6.6 million pursuant to the partial exercise by the underwriters of their option to purchase additional shares in the IPO. Collectively, we received aggregate net proceeds of $76.4 million from the IPO and the subsequent partial exercise by the underwriters of their option to purchase additional shares (the "IPO Proceeds"). Based on our current operating plan, we estimate that our existing cash and cash equivalents as of the date of this Quarterly Report, including the IPO Proceeds, will be sufficient to fund our projected operating expenses and capital expenditure requirements through at least 12 months from the date of this Quarterly Report. 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 Quarterly Report.

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 supply as well as commercial supply 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 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:

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 (the "Whitehead Institute"), which was subsequently amended on December 14, 2021 (the "Whitehead First Amendment"), and on November 7, 2023 (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 condensed 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 $0.20 million through September 30, 2024. 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 $0.27 million through September 30, 2024. We are obligated to pay potential development milestone payments to the Whitehead Institute of up to an aggregate of $1.9 million 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 seven years after the first commercial sale, in each case on a product-by-product and country-by-country basis. We incurred de minimis amounts of license maintenance and amendment issuance fees during each of the three and nine months ended September 30, 2024 and 2023 under the amended agreement and recorded the amounts in our research and development expense in our condensed consolidated statements of operations and comprehensive loss.

Children's Medical Center Corporation

In April 2018, we entered into a development and license agreement (the "Children's Medical Center Corporation Agreement") with Children's Medical Center Corporation ("CMCC"). The agreement allows us 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 we are not pursuing and which were subsequently sublicensed to Fulcrum Therapeutics, Inc. ("Fulcrum"), as described below. As part of the agreement, we issued a total of 15,123 shares of common stock to CMCC and 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 Children's Medical Center Corporation 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 less than $0.1 million of royalties owed to CMCC under the agreement during each of the three and nine months ended September 30, 2024 and 2023, and recorded the amounts in research and development ("R&D") expense in the condensed consolidated statements of operations and comprehensive loss. We re-evaluate the likelihood of achieving future milestones at the end of each reporting period. As of September 30, 2024, we determined that the likelihood of achieving future milestones was not probable.

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 Children's Medical Center Corporation 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 each of the three and nine months ended September 30, 2024, we did not record any license revenue pursuant to the Fulcrum Agreement. During the three and nine months ended September 30, 2023, we recorded $0.35 million of license revenue pursuant to the Fulcrum Agreement.

Collaborative Arrangement

Eli Lilly and Company

In July 2023, we executed a Material Transfer Agreement ("MTA") with Eli Lilly and Company ("Eli Lilly"), which was subsequently amended in July 2024. 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 nine months ended September 30, 2024, we received $0.4 million from Eli Lilly related to the MTA. For the three and nine months ended September 30, 2024, we recorded $0.1 million and $0.3 million, respectively, as a reduction in R&D expense in the condensed consolidated statement of operations and comprehensive loss. For the three and nine months ended September 30, 2023, we recorded $0.2 million and $0.2 million, respectively, as a reduction in R&D expense in the condensed consolidated statement of operations and comprehensive loss.

Research and Collaboration Agreement

BioMarin Pharmaceutical, Inc.

On September 26, 2024, we entered into a Collaboration and License Agreement (the "BioMarin Agreement") with BioMarin Pharmaceutical Inc. ("BioMarin"), pursuant to which we and BioMarin agreed to collaborate with respect to the research and discovery of regulatory RNA-targeting ASOs using our proprietary RAP Platform to modulate the expression of two undisclosed genetic targets under two distinct programs. Under the terms of the BioMarin Agreement, BioMarin will pay an upfront, nonrefundable payment of $1.0 million, and will reimburse us for the research activities we will conduct for each program under an agreed research plan and budget. On a per-program basis, we will be eligible to receive up to $5.0 million in future contingent preclinical milestones, up to $75.0 million in future contingent development and regulatory milestones and up to $105.0 million in commercial sales milestones. We will be further eligible to receive tiered royalties at percentage rates ranging from the low single digits to the high single digits of net sales, subject to specified reductions, until either the last-to-expire valid claim of a patent covering the product, ten years after the first commercial sale, or the expiration of any applicable regulatory exclusivity obtained for the product, in each case on a product-by-product and country-by-country basis. The agreement may be terminated in its entirety or on a program-by-program basis for convenience by BioMarin. The agreement may also be terminated by either us or BioMarin under certain other circumstances, including material breach, as set forth in the agreement. The notice periods for termination provisions range from 30 days to 270 days depending on the reason for termination.

During the three and nine months ended September 30, 2024, we did not record any research collaboration and license revenue pursuant to the BioMarin Agreement.

Components of Our Results of Operations

Revenue

For the three and nine months ended September 30, 2023, we 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 three and nine months ended September 30, 2024. 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) 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 ("CROs"), contract manufacturing organizations ("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 academic 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 allocated 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, audit, 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.

Other (Expense)

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

Results of Operations

Comparison of the Three Months Ended September 30, 2024 and 2023

The following table summarizes our results of operations for the three months ended September 30, 2024 and 2023 (in thousands):

	Three months ended September 30,		
	2024	2023	Change ($)
Revenue			
Research and collaboration revenue	$ —	$ 350	$ (350)
Operating Expenses:			
Research and development	$ 9,702	$ 9,819	$ (117)
General and administrative	3,814	2,869	945
Total operating expenses	13,516	12,688	828
Loss from operations	(13,516)	(12,338)	(1,178)
Other income (expense), net:			
Interest income	94	689	(595)
Other expense	(62)	(47)	(15)
Total other income, net	32	642	(610)
Net loss and comprehensive loss	$ (13,484)	$ (11,696)	$ (1,788)

Research and Collaboration Revenue

We did not recognize any research and collaboration revenue during the three months ended September 30, 2024. We recognized $0.35 million in research and collaboration revenue during the three months ended September 30, 2023. The decrease 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 three months ended September 30, 2024 and 2023 (in thousands):

	Three months ended September 30,		
	2024	2023	Change ($)
Clinical and pre-clinical expenses	$ 4,511	$ 5,053	$ (542)
Personnel-related expenses	2,941	2,979	(38)
Professional fees	558	332	226
Facility-related and other expenses	1,692	1,455	237
Total research and development expenses	$ 9,702	$ 9,819	$ (117)

Research and development expenses were $9.7 million for the three months ended September 30, 2024 compared to $9.8 million for the three months ended September 30, 2023. The decrease of $0.1 million in R&D expenses for the three months ended September 30, 2024 as compared to the prior period was primarily due to a decrease of $0.5 million in workforce-related expenses, offset in part by an increase of $0.2 million in lab operations and information technology expenses, and an increase of $0.2 million in professional and consulting fees associated with preclinical, regulatory and clinical affairs and continued development of our lead product candidate.

General and Administrative Expenses

The following table summarizes our general and administrative expenses for the three months ended September 30, 2024 and 2023 (in thousands):

	Three months ended September 30,		
	2024	2023	Change ($)
Personnel-related expenses	2,419	$ 1,893	$ 526
Professional and consultant fees	903	546	357
Facilities-related fees and other related costs	492	430	62
Total general and administrative expenses	$ 3,814	$ 2,869	$ 945

General and administrative expenses were $3.8 million for the three months ended September 30, 2024 compared to $2.9 million for the three months ended September 30, 2023. The increase of $0.9 million in general and administrative expenses for the three months ended September 30, 2024 as compared to the prior period was primarily due to an increase of $0.5 million related to stock-based compensation associated with the forgiveness of promissory notes originally issued to certain executive officers, and an increase in accounting and consulting fees and fees paid to prosecute patents of $0.4 million.

Other Income (Expense), Net

Other income, net was less than $0.1 million for the three months ended September 30, 2024 compared to $0.6 million for the three months ended September 30, 2023. The decrease of $0.6 million was primarily due to a decrease in interest income on lower average cash equivalent balances in 2024.

Results of Operations

Comparison of the Nine Months Ended September 30, 2024 and 2023

The following table summarizes our results of operations for the nine months ended September 30, 2024 and 2023 (in thousands):

	Nine months ended September 30,		
	2024	2023	Change ($)
Revenue			
Research and collaboration revenue	$ —	$ 350	$ (350)
Operating expenses:			
Research and development	$ 28,821	$ 29,955	$ (1,134)
General and administrative	10,233	8,798	1,435
Total operating expenses	39,054	38,753	301
Loss from operations	(39,054)	(38,403)	(651)
Other income (expense), net:			
Interest income	720	2,239	(1,519)
Other expense	(178)	(137)	(41)
Total other income, net	542	2,102	(1,560)
Net loss and comprehensive loss	$ (38,512)	$ (36,301)	$ (2,211)

Research and Collaboration Revenue

We did not recognize any research and collaboration revenue during the nine months ended September 30, 2024. We recognized $0.35 million in research and collaboration revenue during the nine months ended September 30, 2023. The decrease 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 nine months ended September 30, 2024 and 2023 (in thousands):

	Nine months ended September 30, 2024	2023	Change ($)
Clinical and pre-clinical expenses	$ 13,030	$ 14,183	$ (1,153)
Personnel-related expenses	9,197	10,365	(1,168)
Professional fees	1,665	1,087	578
Facility-related and other expenses	4,929	4,320	609
Total research and development expenses	$ 28,821	$ 29,955	$ (1,134)

Research and development expenses were $28.8 million for the nine months ended September 30, 2024 compared to $30.0 million for the nine months ended September 30, 2023. The decrease of $1.2 million in R&D expenses for the nine months ended September 30, 2024 as compared to the prior period was primarily due to a decrease of $1.2 million in workforce-related expenses and a decrease of $1.2 million in preclinical contract research spend due to fewer external preclinical research activities, offset in part by an increase of $0.6 million in lab operations and information technology expenses, and an increase of $0.6 million in professional and consulting fees associated with preclinical, regulatory and clinical affairs and continued development of our lead product candidate.

General and Administrative Expenses

The following table summarizes our general and administrative expenses for the nine months ended September 30, 2024 and 2023 (in thousands):

	Nine months ended September 30, 2024	2023	Change ($)
Personnel-related expenses	5,978	$ 5,364	$ 614
Professional and consultant fees	2,801	1,984	817
Facilities-related fees and other related costs	1,454	1,450	4
Total general and administrative expenses	$ 10,233	$ 8,798	$ 1,435

General and administrative expenses were $10.2 million for the nine months ended September 30, 2024 compared to $8.8 million for the nine months ended September 30, 2023. The increase of $1.4 million in general and administrative expenses for the nine months ended September 30, 2024 as compared to prior period was primarily due to an increase of $0.6 million related to stock-based compensation associated with the forgiveness of promissory notes originally issued to certain executive officers and an increase in accounting and consulting fees and fees paid to maintain patents of $0.8 million.

Other Income (Expense), Net

Other income, net was less than $0.5 million for the nine months ended September 30, 2024 compared to $2.1 million for the nine months ended September 30, 2023. The decrease of $1.6 million was primarily due to a decrease in interest income on lower average cash equivalent balances in 2024.

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. Through September 30, 2024, 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 September 30, 2024, we have received aggregate gross proceeds of $188.3 million from the sale of shares of our convertible preferred stock. In addition, through September 30, 2024, we have recognized $17.4 million in research and collaboration revenue through our collaboration and license agreements. As of September 30, 2024, we had cash and cash equivalents of $2.5 million.

On October 15, 2024, upon the closing of our IPO, we received aggregate proceeds, net of underwriting discounts and commissions, of $69.8 million. In addition, on November 1, 2024, we received additional proceeds of $6.6 million pursuant to the partial exercise by the underwriters of their option to purchase additional shares in the IPO. Collectively, we received IPO Proceeds of $76.4 million. Based on our current operating plan, we estimate that our existing cash and cash equivalents as of the date of this Quarterly Report, including the IPO Proceeds, will be sufficient to fund our projected operating expenses and capital expenditure requirements through at least 12 months from the date this Quarterly Report was issued. 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 Quarterly Report.

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 or may receive 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.

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 two 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, 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 condensed consolidated financial statements as of September 30, 2024 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 condensed consolidated financial statements as of September 30, 2024 for additional details related to our finance leases.

Restricted Cash

In connection with our 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 condensed consolidated financial statements as of September 30, 2024 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

For the Nine Months Ended September 30, 2024 and 2023

The following table provides information regarding our cash flows for the nine months ended September 30, 2024 and 2023 (in thousands):

	Nine months ended September 30,	
	2024	2023
Net cash used in operating activities	$ (34,274)	$ (32,372)
Net cash used in investing activities	(178)	(435)
Net cash (used in) provided by financing activities	(1,400)	364
Net (decrease) increase in cash and cash equivalents	$ (35,852)	$ (32,443)

Operating Activities

During the nine months ended September 30, 2024, operating activities used $34.2 million of cash, primarily resulting from our net loss of $38.5 million and net cash used in changes in our operating assets and liabilities of $1.6 million, partially offset by non-cash charges of $5.9 million, including depreciation and amortization, stock-based compensation expense and non-cash operating lease expense.

During the nine months ended September 30, 2023, operating activities used $32.4 million of cash, primarily resulting from our net loss of $36.3 million and net cash used in changes in our operating assets and liabilities of $1.0 million, partially offset by non-cash charges of $4.9 million, including depreciation and amortization, stock-based compensation expense and non-cash operating lease expense.

Investing Activities

During the nine months ended September 30, 2024, net cash used in investing activities was $0.2 million, due to purchases of property and equipment.

During the nine months ended September 30, 2023, net cash used in investing activities was $0.4 million, due to purchases of property and equipment.

Financing Activities

During the nine months ended September 30, 2024, net cash used in financing activities was $1.4 million, consisting primarily of $0.8 million of deferred offering cost payments and $0.6 million of finance lease principal payments and repayments for our financing liability.

During the nine months ended September 30, 2023, net cash provided by financing activities was $0.4 million, consisting primarily of net proceeds of $0.7 million from a financing obligation, offset by $0.2 million of finance lease principal payments and less than $0.2 million of repayments for our financing liability related to such financing obligation.

Critical Accounting Policies and Significant Judgments and Estimates

Our management's discussion and analysis of our financial condition and results of operations are based on our consolidated financial statements, which have been prepared in accordance with U.S. generally accepted accounting principles ("GAAP"). The preparation of our financial statements and related disclosures requires us to make estimates and judgments that affect the reported amounts of assets, liabilities, expenses, and the disclosure of our contingent liabilities in our financial statements. We base our estimates on historical experience, known trends and events and various other factors that 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.

There have been no significant changes to our critical accounting estimates from those described in Management's Discussion and Analysis of Financial Condition and Results of Operations - Critical Accounting Policies and Significant Estimates and Judgments and our audited financial statements as of and for the year ended December 31, 2023 as included in the IPO Prospectus.

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 condensed consolidated financial statements.

Emerging Growth Company and Smaller Reporting Company Status

We are an emerging growth company, as defined in the Jumpstart Our Business Startups Act of 2012, as amended (the "JOBS Act"), and we may remain an emerging growth company until December 31, 2029 or until such earlier time that we are no longer an emerging growth company. 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 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) December 31, 2029.

We are also a "smaller reporting company," meaning that the market value of our shares held by non-affiliates 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 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.

Item 3. Quantitative and Qualitative Disclosures About Market Risk

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 Quarterly Report.

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 Quarterly Report.

Item 4. Controls and Procedures

Evaluation of Disclosure Controls and Procedures

Our management, with the participation of our principal executive officer and our principal financial officer, evaluated the effectiveness of our disclosure controls and procedures as defined in Rules 13a-15(e) and 15d-15(e) under the Securities Exchange Act of 1934 as of September 30, 2024. The term "disclosure controls and procedures," as defined in Rules 13a-15(e) and 15d-15(e) under the Exchange Act, means controls and other procedures of a company that are designed to ensure that information required to be disclosed by us in the reports that we file or submits under the Exchange Act is recorded, processed, summarized and reported, within the time periods specified in the SEC's rules and forms. Disclosure controls and procedures include, without limitation, controls and procedures designed to ensure that information required to be disclosed by a company in the reports that it files or submits under the Exchange Act is accumulated and communicated to the company's management, including its principal executive and principal financial officers, or persons performing similar functions, as appropriate to allow timely decisions regarding required disclosure. Management recognizes that any controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving their objectives and management necessarily applies its judgment in evaluating the cost-benefit relationship of possible controls and procedures. Based on the evaluation of our disclosure controls and procedures as of September 30, 2024, our Chief Executive Officer and Chief Financial Officer concluded that, as of such date, our disclosure controls and procedures were effective at the reasonable assurance level.

Changes in Internal Control Over Financial Reporting

There were no changes in our internal control over financial reporting (as defined in Rules 13a-15(f) and 15d-15(f) under the Exchange Act) during the quarter ended September 30, 2024 that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

PART II — OTHER INFORMATION

Item 1. 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.

Item 1A. Risk Factors

A description of the risks and uncertainties associated with our business and industry is set forth below. 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 Quarterly Report on Form 10-Q ("Quarterly Report"), including in the unaudited condensed consolidated financial statements and the related notes contained in this Quarterly Report and in the section titled "Management's Discussion and Analysis of Financial Condition and Results of Operations." 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 year ended December 31, 2023 and the nine months ended September 30, 2024, we reported net losses of $49.3 million and $$38.5 million, respectively. As of September 30, 2024, we had an accumulated deficit of $$198.5 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 ("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 program for 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.

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.

Based on our current operating plan, we believe that our existing cash and cash equivalents as of September 30, 2024, together with the net proceeds from our initial public offering and subsequent exercise in part by the underwriters of their option to purchase additional shares (collectively, the "IPO Proceeds"), which we received in October and November 2024, will be sufficient to fund our operating expenses and capital expenditure requirements for at least 12 months from the filing date of this Quarterly Report. 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. We do not expect that our existing cash and cash equivalents, including the IPO Proceeds, will be sufficient to complete development of any of our product candidates, or any future product candidates we may identify, and we will require substantial capital in order 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;
- the timing and payment of milestone, royalty or other payments we must make or may receive 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.

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 (the "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 ("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 (the "FDA") of an investigational new drug ("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 ("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 (the "EC") following a favorable assessment performed by the European Medicines Agency (the "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 ("GCPs"), current Good Laboratory Practices ("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 ("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 be successful in upregulating the expression of their target genes and may nonetheless fail to 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 achieve the optimal levels of gene upregulation or, notwithstanding such upregulation, may not be effective in achieving a meaningful clinical result in 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 ("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 ("CROs") and clinical trial sites;
- delays in opening clinical trial sites or obtaining required approval from institutional review boards ("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 ("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 ("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 ("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 N-acetylglutamate synthesase ("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 certain of our future product candidates in combination with other therapies, which exposes us to additional risks.

We may develop certain of our 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 any product candidate we develop, we may be unable to obtain approval of or market such product candidate.

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 (the "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 (the "PREA"), a new drug application (an "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 (an "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 the Food and Drug Omnibus Reform Act of 2022 ("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 have received orphan drug designation for CMP-CPS-001 for the treatment of UCDs, and we may pursue orphan drug designation for certain of our other product candidates. We may not be able to obtain or maintain the benefits of orphan drug designation, including potential orphan drug exclusivity, and even if we do, that exclusivity may not prevent regulatory authorities from approving other competing products.

The FDA granted orphan drug designation to CMP-CPS-001 for the treatment of UCDs in September 2024; however, we may not be able to obtain or maintain the benefits of such designation, including potential orphan drug exclusivity. Additionally, we may seek orphan drug designation for certain of our other product candidates in the future; 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 decision of the U.S. Court of Appeals for the 11th Circuit in Catalyst Pharms., Inc. v. Becerra, 14 F.4th 1299 (11th Cir. 2021) has created uncertainty regarding the scope of orphan drug exclusivity. Although the FDA subsequently announced that it intends to continue to apply its longstanding interpretation of the regulations to matters outside of the scope of the Catalyst order and 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. 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.

We have received rare pediatric disease designation from the FDA for CMP-CPS-001 for the treatment of UCDs; however, there is no guarantee that a marketing application for CMP-CPS-001, if approved, will qualify for a rare pediatric disease priority review voucher.

Under the Rare Pediatric Disease Priority Review Voucher ("PRV") program, a sponsor of an NDA that receives approval for a drug for a "rare pediatric disease" may qualify for a rare pediatric disease PRV that can be redeemed to obtain priority review for a subsequent application. Under the current statutory sunset provisions, a rare pediatric disease product application may be eligible for a rare pediatric disease PRV if the drug receives rare pediatric disease designation before September 30, 2024 and receives marketing approval before September 30, 2026. While we have obtained rare pediatric disease designation for CMP-CPS-001 for the treatment of UCDs, it is unlikely that this product candidate will be approved before September 30, 2026. If approval is not obtained by then, we will not be eligible for a rare pediatric disease PRV, unless Congress further reauthorizes the program beyond the current sunset date. Additionally, designation of a drug for a rare pediatric disease does not guarantee that an NDA for such product candidate will meet the criteria for a "rare pediatric disease priority product application" or be eligible for a rare pediatric disease PRV at the time the application is approved. The FDA may determine that a marketing application does not meet the eligibility criteria for a rare pediatric disease PRV for a number of reasons, including:

- the rare pediatric disease that received such designation no longer meets the definition of a "rare pediatric disease";
- the marketing application contains an active ingredient (including any ester or salt of the active ingredient) that has been previously approved in a marketing application;
- the marketing application is not deemed eligible for priority review;
- the marketing application does not rely on clinical data derived from studies examining a pediatric population and dosages of the product intended for that population (that is, if the marketing application does not contain sufficient clinical data to allow for adequate labeling for use by the full range of affected pediatric patients); or
- the marketing application is approved for a different adult indication than the rare pediatric disease for which our product candidates are designated.

Rare pediatric disease designation does not lead to faster development or regulatory review of the product or increase the likelihood that will receive marketing approval.

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 FDCA, 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.

The U.S. Supreme Court's June 2024 decision in Loper Bright Enterprises v. Raimondo overturned the longstanding Chevron doctrine, under which courts were required to give deference to regulatory agencies' reasonable interpretations of ambiguous federal statutes. The Loper decision could result in additional legal challenges to regulations and guidance issued by federal agencies, including the FDA, on which we rely. Any such legal challenges, if successful, could have a material impact on our business. Additionally, the Loper decision may result in increased regulatory uncertainty, inconsistent judicial interpretations, and other impacts to the agency rulemaking process, any of which could adversely impact our business and operations. We cannot predict the likelihood, nature or extent of government regulation that may arise from future legislation or administrative action or as a result of legal challenges, either in the United States or abroad. If we are slow or unable to adapt to changes in existing requirements or the adoption of new requirements or policies, or if we are not able to maintain regulatory compliance, our business could be materially harmed.

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 (“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 ("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 ("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 ("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 (the "Affordable Care Act"), which became law in the 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 (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 (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 (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 ("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, although the bill was passed in the House on September 9, 2024. 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 industry in the United States. 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 (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 (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 (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 (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 U.S.-based companies. Compliance with such regulations may limit our exclusive rights and limit our ability to contract with non-U.S. manufacturers.

We have in-licensed certain patents and patent applications that were generated through the use of U.S. government funding or grants, and we may acquire or license in the future intellectual property rights that have been generated through the use of U.S. government funding or grants. Pursuant to the Bayh-Dole Act of 1980, the U.S. government has certain rights in inventions developed with government funding. These U.S. government rights include a non-exclusive, non-transferable, irrevocable worldwide license to use inventions for any governmental purpose. In addition, the U.S. 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 U.S. government exercised its march-in rights in our current or future intellectual property rights generated through the use of U.S. 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 U.S. government for the exercise of such rights. The U.S. 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 U.S. 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 U.S. 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 U.S. industry may limit our ability to contract with non-U.S. 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 U.S. 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. 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 have entered into employment offer letters with each of our executive officers, 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 September 30, 2024, we had 53 full-time employees. As we continue development and pursue the potential commercialization of our product candidates, 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 include property, general liability, employee benefits liability, workers' compensation, clinical trial liability, cyber liability, fiduciary 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 ("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 $66.7 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 (the "Tax Act"), as amended by the Coronavirus Aid, Relief, and Economic Security Act (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 Ownership of Our Common Stock

Prior to the completion of our IPO, there was no public market for our common stock. An active, liquid, and orderly market for our common stock may not develop or be sustained, or we may in the future fail to satisfy the continued listing requirements of Nasdaq.

Prior to the completion of our IPO, there was no public market for our common stock. Our common stock only recently began trading on the Nasdaq Global Market ("Nasdaq"), and we can provide no assurance that we will be able to develop an active trading market for our common stock or that an active trading market will be sustained. If an active market for our common stock is not sustained, it may be difficult for you to sell your shares at the time you wish to sell them or at a price that you 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 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 your ability to sell or purchase our common stock when you 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.

Since shares of our common stock were sold in our IPO in October 2024 at a price of $11.00 per share and through November 12, 2024, the price per share of our common stock has ranged from $7.80 to $12.30. 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 price at which they initially purchased the common stock. 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.

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 entered into by holders of substantially all of our common stock outstanding immediately prior to our IPO, 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. As of November 1, 2024, we had 20,155,303 shares of common stock outstanding. Of these shares, the 7,463,762 shares we sold in our IPO and subsequent exercise in part by the underwriters of their option to purchase additional shares may be resold in the public market immediately, unless held 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 lock-up and other legal restrictions described in our IPO Prospectus lapse.

Our executive officers, directors, and principal stockholders, if they choose to act together, have the ability to significantly influence all matters submitted to stockholders for approval.

As of the closing date of our IPO, our executive officers, directors and their affiliates, in the aggregate, own approximately 91.0% of our outstanding common stock. As a result, such persons, acting together, 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.

In connection with our IPO, our directors and executive officers and the holders of substantially all of our outstanding securities prior to our IPO 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 our IPO 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. After the lock-up agreements expire, these shares of common stock will be eligible for sale in the public market, except that shares held by directors, executive officers and other affiliates will be subject to volume limitations under Rule 144 under the Securities Act of 1933, as amended (the "Securities Act").

In addition, as of September 30, 2024, 2,078,470 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 there are any sales of these shares of common stock, or if it is perceived that they will be sold, in the public market, the trading price of our common stock could decline.

The holders of 11,648,582 shares of our outstanding common stock, or approximately 59.7% of our total outstanding common stock based on shares outstanding as of September 30, 2024, are 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. 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 (the "JOBS Act"). We are also a "smaller reporting company," as defined in Rule 12b-2 under the Securities Exchange Act of 1934 (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 ("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 ("Restated Charter"), our amended and restated bylaws ("Restated Bylaws") 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 Restated Charter 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 (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 Restated Charter, 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 Restated Charter designates 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 Restated Charter provides 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) is, to the fullest extent permitted by applicable law, the sole and exclusive forum for the following types of claims, including: (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 Restated Charter or Restated Bylaws, (iv) any claim to interpret, apply, enforce or determine the validity of our Restated Charter or 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 does not apply to claims brought to enforce a duty or liability created by the Exchange Act.

Our Restated Charter further provides that the federal district courts of the United States of America are the exclusive forum for resolving any complaint asserting a cause of action arising under the Securities Act. In addition, our Restated Charter provides 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 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 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. The choice of forum provisions may also impose additional litigation costs on stockholders who assert that the provisions are not enforceable or invalid.

General Risk Factors

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

As a public company, we incur significant legal, accounting, and other expenses that we did not incur prior to our IPO. We are subject to the reporting requirements of the Exchange Act, which 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 that, as a result of the rules and regulations applicable to public companies, we will incur substantial legal and financial compliance costs. These 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 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 United States 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 is dependent in part on the research and reports that securities or industry analysts publish about us, our business, our market, or our competitors. A limited number of securities and industry analysts publish research on our company. If a larger number of securities or industry analysts do not commence coverage of our company, the trading price of our stock would be negatively affected. 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 annual report for our fiscal year ending December 31, 2025. 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.

We may subject to 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.

Item 2. Unregistered Sales of Equity Securities and Use of Proceeds

(a) Sales of Unregistered Securities

From July 1, 2024 through September 30, 2024, we issued to employees options to purchase an aggregate of 132,886 shares of our common stock at a weighted-average exercise price of $12.79 per share under our Amended and Restated 2016 Stock Option and Grant Plan (the "2016 Plan"), and we issued and sold to employees an aggregate of 9,421 shares of our common stock under the 2016 Plan upon the exercise of stock options at a weighted-average exercise price of $3.09.

The options and common stock issued upon the exercise of options described above were issued under our 2016 Plan in reliance on the exemption provided by Rule 701 promulgated under the Securities Act as sales and offers under compensatory benefit plans and contracts relating to compensation in compliance with Rule 701, or in reliance on Section 4(a)(2), as transactions by an issuer not involving a public offering. The recipients of securities in these transactions had adequate access, through employment, business or other relationships, to information about us. The foregoing transactions did not involve any underwriters, underwriting discounts or commissions, or any public offering. On October 16, 2024, we filed a registration statement on Form S-8 under the Securities Act to register all of the shares of our common stock subject to outstanding options and all shares of our common stock otherwise issuable pursuant to our equity compensation plans.

(b) Use of Proceeds from Public Offering of Common Stock

On October 10, 2024, our Registration Statement on Form S-1 (File No. 333-282241), was declared effective in connection with our IPO, pursuant to which we sold an aggregate of 6,820,000 shares of our common stock at a price to the public of $11.00 per share. The underwriters of our IPO were J.P. Morgan Securities LLC, Leerink Partners LLC, Piper Sandler & Co. and William Blair & Company, L.L.C.

Our IPO closed on October 15, 2024. In connection with the IPO, we granted the underwriters a 30-day option to purchase an additional 1,023,000 shares of common stock. On November 1, 2024, pursuant to the partial exercise by the underwriters of such option, we issued an additional 643,762 shares of common stock. We received aggregate gross proceeds of $82.1 million in connection with the IPO and subsequent exercise of the underwriters' option and aggregate net proceeds of $76.4 million after deducting underwriting discounts and commissions and expenses payable by us. In connection with our IPO, no payments were made by us to directors, officers or persons owning ten percent or more of our ordinary shares or to their associates or to our affiliates. There has been no material change in the planned use of proceeds from our IPO as described in our IPO Prospectus filed pursuant to Rule 424(b)(4) under the Securities Act with the SEC on October 11, 2024. We are holding the balance of the net proceeds in cash and cash equivalents.

(c) Issuer Purchases of Equity Securities

None.

Item 3. Defaults Upon Senior Securities

Not applicable.

Item 4. Mine Safety Disclosures

Not applicable.

Item 5. Other Information

No "Rule 10b5-1 plans" or "non-Rule 10b5-1 trading arrangements", as each term is defined in Item 408(a) of Regulation S-K, were adopted, modified, or terminated by officers or directors of the Company.

Item 6. Exhibits

See Exhibit Index.

EXHIBIT INDEX

Exhibit No.	Description
3.1	Fifth Amended and Restated Certificate of Incorporation of CAMP4 Therapeutics Corporation (incorporated by reference to Exhibit 3.1 to the Form 8-K filed on October 15, 2024, File No. 001-423665)
3.2	Amended and Restated Bylaws of CAMP4 Therapeutics Corporation (incorporated by reference to Exhibit 3.2 to the Form 8-K filed on October 15, 2024, File No. 001-423665)
10.1	CAMP4 Therapeutics Corporation 2024 Equity Incentive Plan (incorporated by reference to Exhibit 4.2 to the Form S-8 filed on October 16, 2024, File No. 333-282676)
10.2	CAMP4 Therapeutics Corporation 2024 Employee Stock Purchase Plan (incorporated by reference to Exhibit 4.3 to the Form S-8 filed on October 16, 2024, File No. 333-282676)
31.1†	Certification of Chief Executive Officer Pursuant to Section 302 of the Sarbanes-Oxley Act of 2002
31.2†	Certification of Chief Financial Officer Pursuant to Section 302 of the Sarbanes-Oxley Act of 2002
32.1†*	Certification of Chief Executive Officer Pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002
32.2†*	Certification of Chief Financial Officer Pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002
101.INS†	XBRL Instance Document – the instance document does not appear in the Interactive Data File because its XBRL tags are embedded within the Inline XBRL document
101.SCH†	XBRL Taxonomy Extension Schema Document
101.CAL†	XBRL Taxonomy Extension Calculation Linkbase Document
101.DEF†	XBRL Taxonomy Extension Definition Linkbase Document
101.LAB†	XBRL Taxonomy Extension Label Linkbase Document
101.PRE†	XBRL Taxonomy Extension Presentation Linkbase Document
104†	Cover Page Interactive Data File (formatted in Inline XBRL and contained in Exhibit 101)

† Filed herewith.

* This certification will not be deemed "filed" for purposes of Section 18 of the Securities Exchange Act of 1934, as amended (the "Exchange Act"), or otherwise subject to the liability of that section. Such certification will not be deemed to be incorporated by reference into any filing under the Securities Act of 1933, as amended, or the Exchange Act, except to the extent specifically incorporated by reference into such filing.

SIGNATURES

Pursuant to the requirements of the Securities Exchange Act of 1934, the Registrant has duly caused this report to be signed on its behalf by the undersigned, duly authorized.

Date: November 21, 2024

CAMP4 Therapeutics Corporation

By: /s/ Josh Mandel-Brehm
Name: Josh Mandel-Brehm
Title: President and Chief Executive Officer
(Principal Executive Officer)

By: /s/ Kelly Gold
Name: Kelly Gold
Title: Chief Financial Officer
(Principal Financial Officer and Principal Accounting Officer)

Exhibit 31.1

CERTIFICATION PURSUANT TO RULES 13a-14(a) AND 15d-14(a) UNDER THE SECURITIES EXCHANGE ACT OF 1934, AS ADOPTED PURSUANT TO SECTION 302 OF THE SARBANES-OXLEY ACT OF 2002

I, Josh Mandel-Brehm, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of CAMP4 Therapeutics Corporation;

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4. The registrant's other certifying officer(s) and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) for the registrant and have:

    a. Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

    b. [Paragraph omitted pursuant to SEC Release Nos. 33-8238/34-47986 and 33-8392/34-49313];

    c. Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

    d. Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and

5. The registrant's other certifying officer(s) and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

    a. All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

    b. Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Dated: November 21, 2024

By: /s/ Josh Mandel-Brehm
Name: Josh Mandel-Brehm
Title: President and Chief Executive Officer
(Principal Executive Officer)

Exhibit 31.2

CERTIFICATION PURSUANT TO RULES 13a-14(a) AND 15d-14(a) UNDER THE SECURITIES EXCHANGE ACT OF 1934, AS ADOPTED PURSUANT TO SECTION 302 OF THE SARBANES-OXLEY ACT OF 2002

I, Kelly Gold, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of CAMP4 Therapeutics Corporation;

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4. The registrant's other certifying officer(s) and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) for the registrant and have:

    a. Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

    b. [Paragraph omitted pursuant to SEC Release Nos. 33-8238/34-47986 and 33-8392/34-49313];

    c. Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

    d. Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and

5. The registrant's other certifying officer(s) and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

    a. All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

    b. Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Dated: November 21, 2024

By: /s/ Kelly Gold
Name: Kelly Gold
Title: Chief Financial Officer
(Principal Financial Officer and Principal Accounting Officer)

Exhibit 32.1

CERTIFICATION PURSUANT TO 18 U.S.C. SECTION 350,
AS ADOPTED PURSUANT TO
SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002

Pursuant to 18 U.S.C. § 1350, as created by Section 906 of the Sarbanes-Oxley Act of 2002, the undersigned officer of CAMP4 Therapeutics Corporation (the "Company") hereby certifies, to the best of my knowledge, that:

(i) the accompanying Quarterly Report on Form 10-Q of the Company for the fiscal quarter ended September 30, 2024 (the "Report") fully complies with the requirements of Section 13(a) or Section 15(d) of the Securities Exchange Act of 1934; and

(ii) the information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

Dated: November 21, 2024

By: /s/ Josh Mandel-Brehm
Name: Josh Mandel-Brehm
Title: President and Chief Executive Officer
(Principal Executive Officer)

Exhibit 32.2

CERTIFICATION PURSUANT TO 18 U.S.C. SECTION 350,
AS ADOPTED PURSUANT TO
SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002

Pursuant to 18 U.S.C. § 1350, as created by Section 906 of the Sarbanes-Oxley Act of 2002, the undersigned officer of CAMP4 Therapeutics Corporation (the "Company") hereby certifies, to the best of my knowledge, that:

(i) the accompanying Quarterly Report on Form 10-Q of the Company for the fiscal quarter ended September 30, 2024 (the "Report") fully complies with the requirements of Section 13(a) or Section 15(d) of the Securities Exchange Act of 1934; and

(ii) the information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

Dated: November 21, 2024

By: /s/ Kelly Gold
Name: Kelly Gold
Title: Chief Financial Officer
(Principal Financial Officer and Principal Accounting Officer)