

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

FORM 8-K

CURRENT REPORT
Pursuant to Section 13 or 15(d)
of the Securities Exchange Act of 1934
Date of Report (Date of earliest event reported): January 7, 2025

CAMP4 THERAPEUTICS CORPORATION
(Exact name of registrant as specified in its charter)

Delaware
(State or other jurisdiction
of incorporation)

001-42365
(Commission
File Number)

81-1152476
(IRS Employer
Identification No.)

One Kendall Square
Building 1400 West, 3rd Floor
Cambridge, MA
(Address of principal executive offices)

02139
(Zip Code)

(Registrant's telephone number, including area code): (617) 651-8867

Not Applicable
(Former name or former address, if changed since last report)

Check the appropriate box below if the Form 8-K filing is intended to simultaneously satisfy the filing obligation of the registrant under any of the following provisions (see General Instruction A.2. below):

- Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)
- Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12)
- Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b))
- Pre-commencement communications pursuant to Rule 13e-4(c) under the Exchange Act (17 CFR 240.13e-4(c))

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

Title of each class	Trading Symbol(s)	Name of each exchange on which registered
Common Stock, par value \$0.0001 per share	CAMP	The Nasdaq Global Market

Indicate by check mark whether the registrant is an emerging growth company as defined in Rule 405 of the Securities Act of 1933 (§230.405 of this chapter) or Rule 12b-2 of the Securities Exchange Act of 1934 (§240.12b-2 of this chapter).

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.

Item 7.01 Regulation FD Disclosure.

From time to time, Camp4 Therapeutics Corporation (the "Company") intends to conduct meetings with third parties in which its current corporate slide presentation is presented. A copy of this slide presentation, dated January 2025, is furnished as Exhibit 99.1 to this Current Report on Form 8-K. The information responsive to Item 7.01 of this Form 8-K and Exhibit 99.1 hereto shall not be deemed "filed" for purposes of Section 18 of the Securities Exchange Act of 1934 (the "Exchange Act") or otherwise subject to the liabilities of that section, nor shall it be deemed incorporated by reference in any filing under the Securities Act of 1933, as amended, or the Exchange Act, except as expressly set forth by specific reference in such a filing.

Item 8.01 Other Events.

On January 7, 2025, the Company issued a press release reporting safety data from all four cohorts of the single ascending dose, or SAD, portion of its ongoing Phase 1 clinical trial of CMP-CPS-001 in healthy volunteers and highlighting certain other recent corporate updates and key upcoming milestones. The full text of this press release is filed as Exhibit 99.2 to this Current Report on Form 8-K and is incorporated herein by reference. The information contained on the websites referenced in the press release is not incorporated herein.

Item 9.01 Financial Statements and Exhibits.

(d) Exhibits

Exhibit No.	Description
99.1	Slide presentation, dated January 2025.
99.2	Press release, dated January 7, 2025.
104	Cover Page Interactive Data File (embedded within the Inline XBRL document).

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 hereunto duly authorized.

CAMP4 THERAPEUTICS CORPORATION

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

Date: January 7, 2025



Pioneering a new class of RNA medicines to increase targeted gene expression

Corporate Overview
January 2025

Forward-Looking Statements

This presentation contains forward-looking statements that are based on the beliefs and assumptions and on information currently available to CAMP4's management. All statements other than statements of historical fact contained in this presentation are forward-looking statements. Forward-looking statements include information concerning the initiation, timing, progress and results of preclinical and clinical trials of CAMP4's product candidates, the timing or likelihood of regulatory filings and approvals for any of its product candidates, and estimates regarding CAMP4's expenses, future revenues, and future capital requirements. In some cases, you can identify forward-looking statements because they contain words such as "may," "might," "will," "would," "shall," "should," "expects," "plans," "anticipates," "could," "intends," "target," "projects," "contemplates," "believes," "estimates," "looks," "seeks," "predicts," "potential," "ongoing," or "continue" or the negative of these words or other similar terms or expressions that concern our expectations, strategy, plans or intentions, although not all forward-looking statements are accompanied by such words. Forward-looking statements involve known and unknown risks, uncertainties and other factors that may cause CAMP4's actual results, performance or achievements to be materially different from any future results, performance or achievements expressed or implied by the forward-looking statements. This information was factually accurate on the date it was published. CAMP4 assumes no duty to update the information to reflect subsequent developments, except as required by law.

The safety and efficacy of CAMP4's product candidates and/or uses under investigation have not been established. There is no guarantee that any of our product candidates will receive regulatory authority approval or become commercially available in any country for the uses being investigated or that any such product candidate will achieve a particular revenue level. In particular, CAMP4's expectations could be affected by, among other things, uncertainties involved in the development of new therapeutic products; unexpected clinical trial results or unexpected new clinical data; unexpected regulatory actions or delays or government regulation generally; CAMP4's ability to obtain or maintain patent or other proprietary intellectual property protection; competition in general; CAMP4's ability to establish and maintain collaborations, strategic relationships and supply arrangements, or to realize the intended benefits from such relationships or arrangements; whether CAMP4's cash resources will be sufficient to fund its foreseeable and unforeseeable operating expenses and capital expenditure requirements; CAMP4's ability to raise additional funding on favorable terms, or at all; the rate and degree of market acceptance and clinical utility of CAMP4's product candidates; the ability and willingness of our third-party collaborators to continue research, development and manufacturing activities relating to our product candidates; the accuracy of our data analyses or estimates for the potential and market for our products; and government, industry, and general public pricing and other political pressures. The actual results may vary from the anticipated results and the variations may be material. Other factors that may cause the Company's actual results to differ from current expectations are discussed in the Company's filings with the SEC, including the sections titled "Risk factors," "Management's discussion and analysis of financial condition and results of operations" and "Special note regarding forward-looking statements" in the Company's most recent Quarterly Report on Form 10-Q filed with the SEC on November 21, 2024. You are cautioned not to place undue reliance on these forward-looking statements, which speak only as of the date this presentation is given. Except as required by law, CAMP4 undertakes no obligation to publicly update any forward-looking statements, whether as a result of new information, future events or otherwise.



Pioneering a new class of RNA medicines to increase targeted gene expression



There are prevalent diseases where gene upregulation is likely to have a meaningful clinical benefit



CAMP4 is the leader in gene regulatory RNA (regRNA) discovery and regRNA-targeting antisense oligonucleotide (ASO) therapies to upregulate gene expression to restore healthy protein levels



Our proprietary RAP Platform™ was built for the discovery of novel regRNAs that regulate the expression of every protein-coding gene



Our current focus is on metabolic and CNS genetic diseases where modest increases in protein expression can be clinically meaningful



Clinical data expected from MAD portion of ongoing Phase 1 study of CMP-CPS-001 for Urea Cycle Disorders in 2H '25 and advancement of SYNGAP1 program into GLP tox studies

IPO: OCT 2024

NASDAQ: CAMP

CASH RUNWAY INTO Q2 '26

HEADQUARTERS:
CAMBRIDGE, MA



World-class management team, experienced board and advisors



Josh Mandel-Brehm
President & CEO
Biogen polarispartners
genzyme



David Bumcrot, PhD
Chief Scientific Officer
editas Alnylam



Kelly Gold
Chief Financial Officer
Biogen Deutsche Bank



Yuri Maricich, MD
Chief Medical Officer
corixa. Cavion
PEAR



Michelle Gates
Chief People Officer
Akamai



Caleb Moore
Chief Business
Operations Officer
ACCELERON
genzyme CURIST



Satya Kuchimanchi, PhD
SVP, Technical Operations
TRIPLET3 Alnylam



Alla Sigova, PhD
VP, Head of Platform
SAIL Whitehead
Institute



Daniel Tardiff, PhD
VP, Head of Discovery
Pfizer Whitehead
Institute
Yumanity
INSERMUNO





Board of Directors

- James Boylan
- Ingo Chakravarty
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- Steven Holtzman
- Josh Mandel-Brehm
- Amir Nashat, ScD
- Paula Ragan, PhD
- Andy Schwab
- Ravi Thadhani, MD
- Rick Young, PhD
- Len Zon*, MD

*Board observer, co-founder



Advancing a pipeline in metabolic and CNS genetic diseases with the goal of a new clinical candidate every 12-18 months

Program	Indication	Target	Discovery & Preclinical	Phase 1	Phase 2	Phase 3	Anticipated Milestones	Commercial Rights
Metabolic diseases								
CMP-CPS-001	Urea Cycle Disorders	CPS1					Phase 1 MAD data in 2H'25	CAMP4
CNS diseases								
CMP-SYNGAP	SYNGAP1-related Disorders	SYNGAP1					Initiation of GLP tox studies in '25	CAMP4
New Named Program	Genetically defined Parkinson's disease (PD) and sporadic PD	GBA1						CAMP4
New Discovery Programs	CNS & Metabolic	Numerous	 Active discovery and development of multiple programs utilizing RAP Platform					CAMP4
Collaborations								
Strategic research collaboration leveraging CAMP4's RAP Platform advancing novel therapeutics that increase protein levels by targeting regRNA sequences for two genetic targets.								BIOMARIN



Agenda

CAMP4 overview

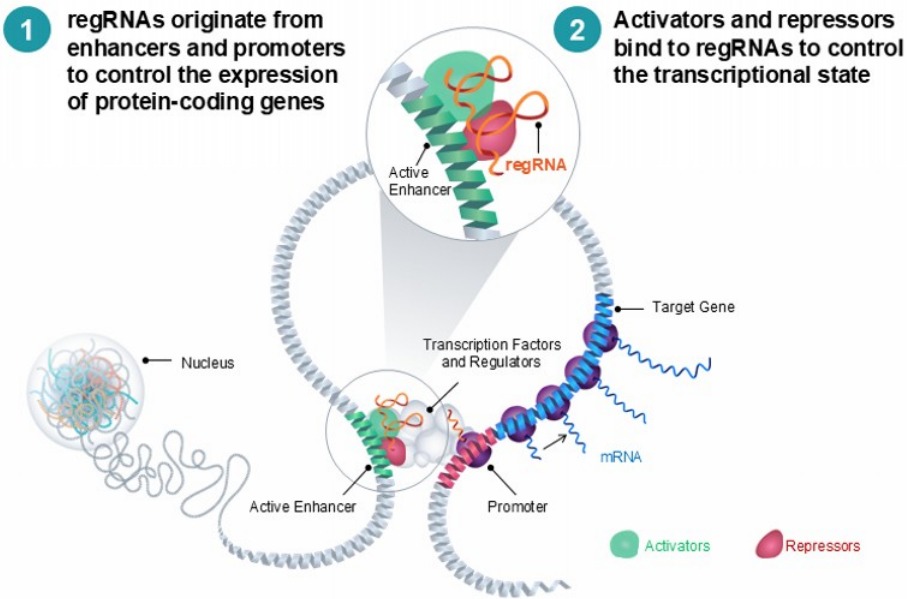
RAP Platform: regRNAs are master controllers of gene regulation

Lead metabolic program: Urea Cycle Disorders

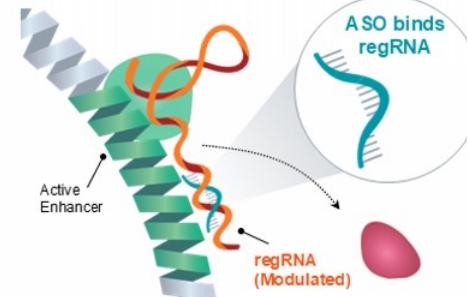
Lead CNS program: SYNGAP1-related Disorders



regRNAs play a central role in the regulation of every gene's expression



3 ASOs disrupt the interactions between repressors and regRNAs enabling increases in gene expression



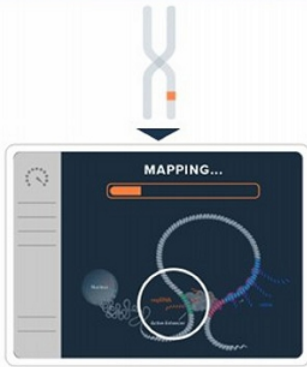
Increased mRNA expression
Addresses root cause of **haploinsufficient or partial loss-of-function diseases** by returning **targeted protein levels** to within a healthy range



Source: Sharp, Chakraborty, Henninger & Young, *RNA* 2022; Henninger, Oksuz, Srinivas et al., *Cell* 2021; Saribrelli & Lauberth, *NatStructMolBio* 2020; Oksuz, et al (Younglab), *Molecular Cell* 2023

CAMP4's proprietary RAP Platform™ catalogs thousands of regRNAs in any tissue and generates ASO leads to increase gene expression

1. Map candidate regRNAs



Next-gen sequencing and machine learning algorithms map regRNAs controlling every expressed gene.

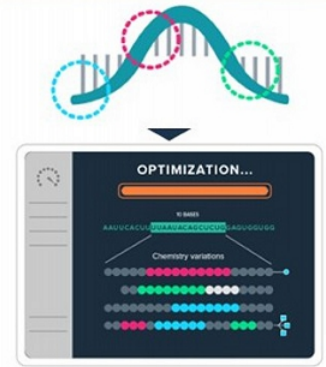
One of the most extensive, proprietary regRNA databases to date across wide range of tissue types.

2. Generate ASO leads



Rapidly pinpoint regions where ASO binding results in optimal upregulation of a target gene

3. Optimize leads



Therapeutic candidates are designed to integrate a range of chemical modifications and tissue-targeting delivery strategies optimizing potency and safety

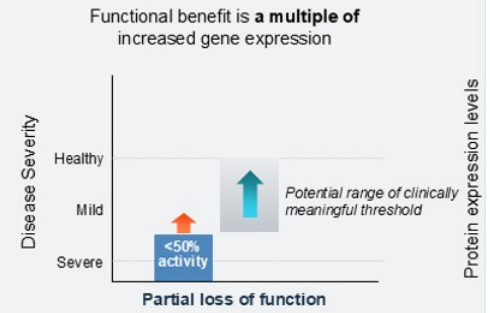
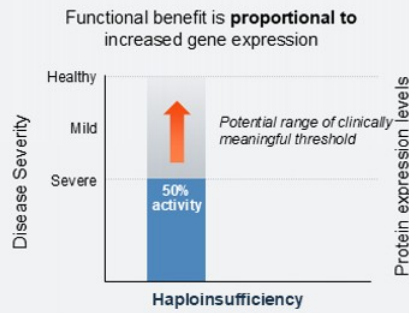


CAMP4 applies its RAP Platform to genetic diseases where modest increases in gene expression can be clinically meaningful

Platform Fit

Upregulating target gene by modest amount can provide clinically meaningful benefit

- ↑ Increase target gene
- ↑ Increase metabolic function



Translation and Druggability

- Ability to achieve delivery and target engagement in the desired cell type
- Compelling preclinical datasets in relevant disease models

Clinical and Regulatory

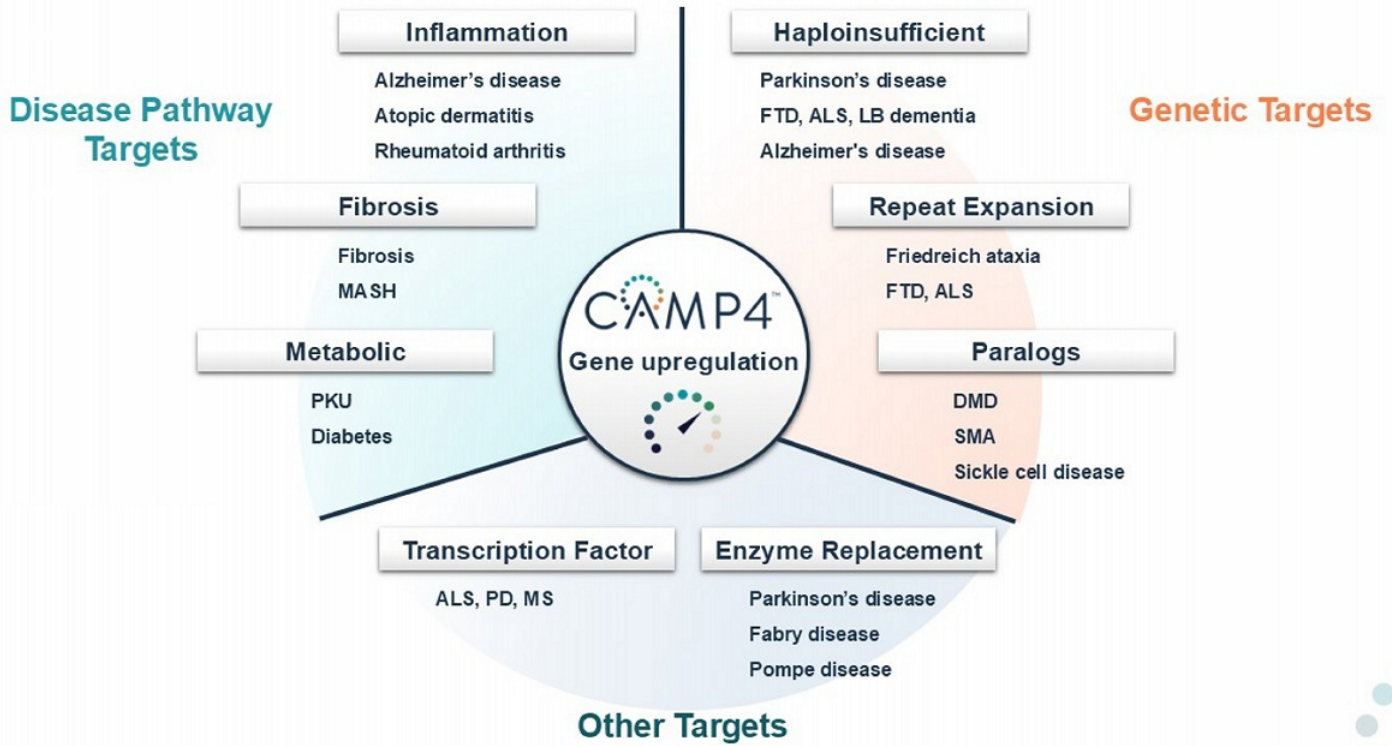
- Defined patient population
- Efficacy can be evaluated in Ph 1/2 based on availability of biomarkers
- Clear or established path to approval

Commercial Potential

- High unmet need, often life threatening
- Differentiated from competition, with attractive revenue potential



RAP Platform has potential to address a broad array of rare and prevalent diseases



Agenda

CAMP4 overview

RAP Platform: regRNAs are master controllers of gene regulation

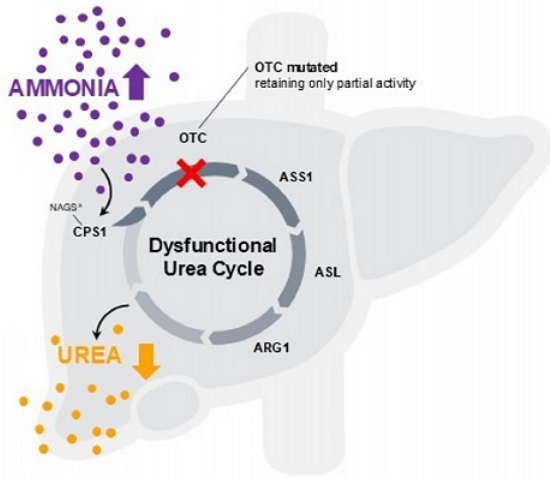
Lead metabolic program: Urea Cycle Disorders

Lead CNS program: SYNGAP1-related Disorders



Urea Cycle Disorders (UCDs) are a set of life-threatening inherited metabolic diseases characterized by the accumulation of toxic ammonia

Mutation in one of several urea cycle enzymes or transporters causes suboptimal ureagenesis (conversion of ammonia to urea)



UCD background

- Ammonia accumulates to dangerous levels without warning, posing a constant risk of life-threatening hyperammonemic crises and irreversible brain damage
- ~5,000 UCD patients in the U.S.; 3,700 severe patients*, 1,200+ symptomatic OTC female heterozygotes^{1, 2}
- OTC, ASL, ASS1 mutations account for 90% of all patients^{1, 3}

Current standard of care is symptomatic

- No mutation agnostic disease modifying treatments available
- Symptomatic therapies include nitrogen scavengers (3-4 pills / day) and a strict diet that borders on malnutrition
- Constant risk of hyperammonemic crises which can be caused by infection, lapse in diet or medications



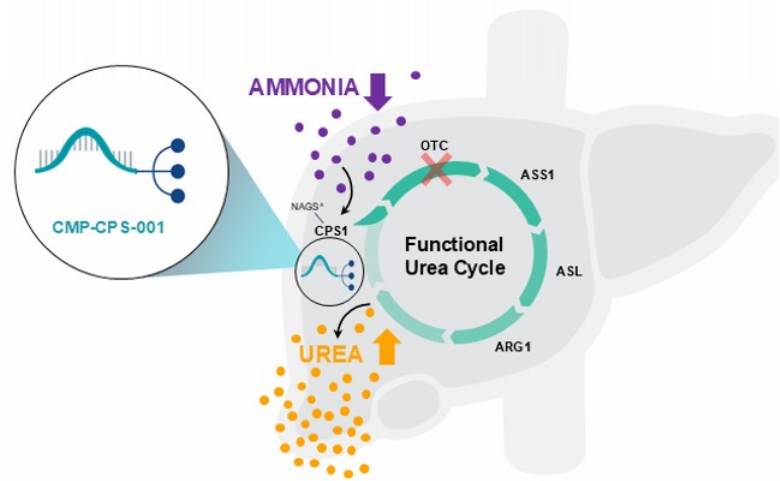
*Enzyme levels > 5% of normal, severe symptoms persist beyond the first month of life
*NAGS enzyme produces the co-factor NAG which activates CPS1

¹ Batshaw et al., Mol Genet Metab, 2014; ² Sen et al., Mol Genet Genomic Med, 2024; ³ Fosset et al., J Inher Metab Dis., 2019

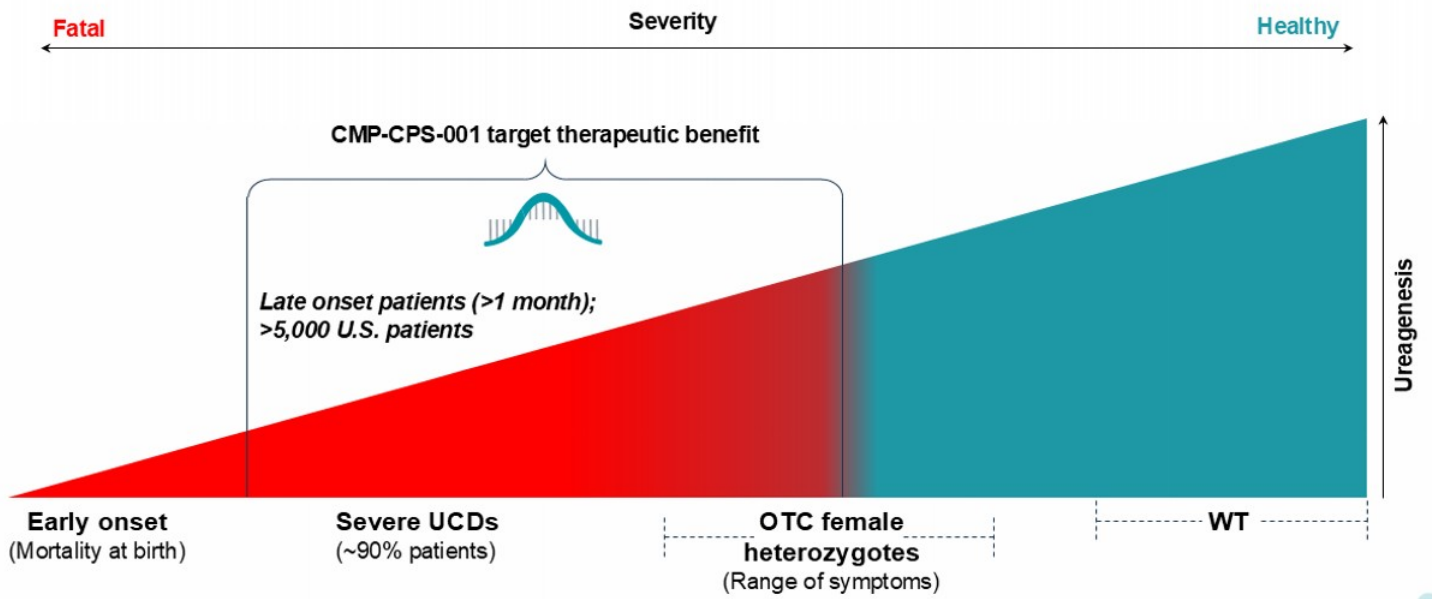
CAMP4 is targeting increased expression of CPS1, resulting in amplified ureagenesis and improved conversion of ammonia to urea

CPS1 is the gatekeeper of the urea cycle





CMP-CPS-001 is a GalNAc-conjugated ASO that binds to a CPS1-specific regRNA to increase CPS1 expression and upregulate the expression of multiple urea cycle enzymes to amplify the conversion of ammonia to urea, potentially addressing more than 90% of patients with late onset UCDs.



Clinical observations and precedent have found that modest increases in ureagenesis resulted in significant reductions in disease severity



CMP-CPS-001 has the potential to be the 1st disease modifying pan-UCD therapy

	 CMP-CPS-001* <i>Anticipated Profile</i>	Standard-of-Care (symptomatic)  RAVICTI® <small>(glycerolphenylbutyrate) Oral Liquid</small>	Pipeline  DTX301 (Ph3)  LUNAR-OTC (Ph2)	
Description	Increase ureagenesis via GalNAc-conjugated ASO 1x monthly (titratable), subcutaneous	Nitrogen-binding agent, "scavenger" 1-3x daily, oral liquid	AAV Gene Tx 1x infusion	mRNA Bi-weekly infusions
Population	<ul style="list-style-type: none"> ~90% of severe UCD patients (pediatric and adult) Symptomatic female OTC heterozygotes 	Broadly applicable	<ul style="list-style-type: none"> OTC-only ≥12 yrs old 	
	<ul style="list-style-type: none"> ✓ Prevent hyperammonemic crises ✓ Reduction, removal of scavengers ✓ Protein-restricted diet liberalization 	<ul style="list-style-type: none"> ❑ Hyperammonemic crises risk ❑ Significant pill burden ❑ Strict protein-restricted diet remains 		



*CMP-CPS-001 has been granted Rare Pediatric Disease Designation and Orphan Drug Designation



CMP-CPS-001 has the potential to be the first disease-modifying therapy for the treatment of the most prevalent UCDs by increasing ureagenesis

Compelling preclinical proof of concept

CAMP4 approach directly reduces toxic ammonia and increases ureagenesis

- **Human hepatocyte data:** Dose-dependent increase in CPS1 expression in normal and diseased OTC-d human cells
- **Otc-deficient mice data:** 20-30% ↑ ureagenesis compared to baseline, leading to ~50% ↓ ammonia (wild-type levels); ~1 month duration of action
- **Humanized mouse data:** 20-30% ↑ ureagenesis compared to baseline, leading to ~70% ↓ ammonia; ↑ CPS1 + downstream enzymes
- **Non-human primate data:** 40% ↑ ureagenesis

Phase 1 clinical design

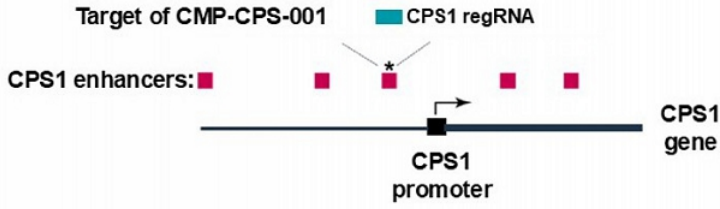
Ability to measure increases in ureagenesis in healthy volunteers can translate to improved ammonia clearance in UCD patients

- **Biomarker:** Urea cycle activity (ureagenesis) can be monitored in healthy volunteers and patients using the ureagenesis rate test (URT)
- **Phase 1 study:** CAMP4 is utilizing URT in SAD / MAD Phase 1 CMP-CPS-001 clinical study
- **Proof of concept:** Multiple companies have utilized the URT in healthy volunteers and in patients

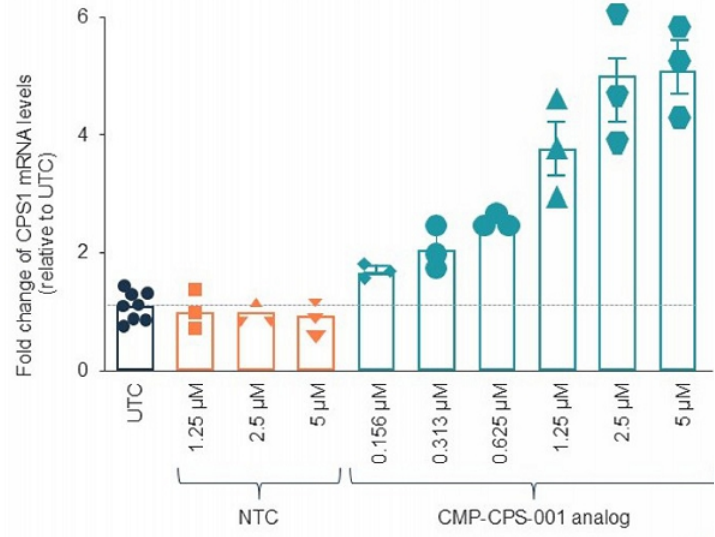


CMP-CPS-001 targets the key regRNA controlling CPS1 expression

RAP Platform identified CPS1 regRNA



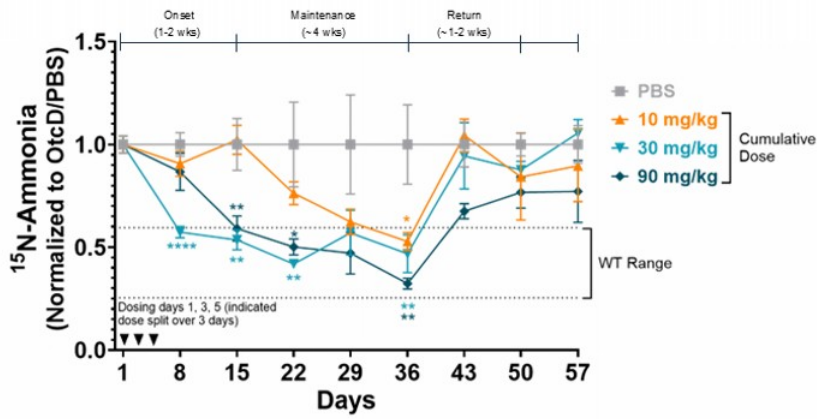
CMP-CPS-001 increases CPS1 mRNA in primary WT liver cells



Activity confirmed in OTC deficient donor cells with CMP-CPS-001 analog (clinical candidate without GalNAc)

NTC = non-targeting control

ASO targeting mouse *Cps1* regRNA in *Otc*-deficient mice reduces ammonia and supports once-monthly dosing

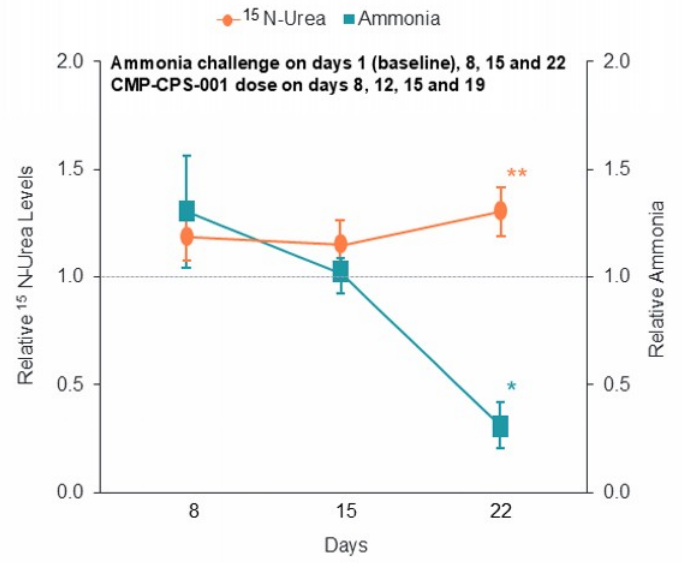
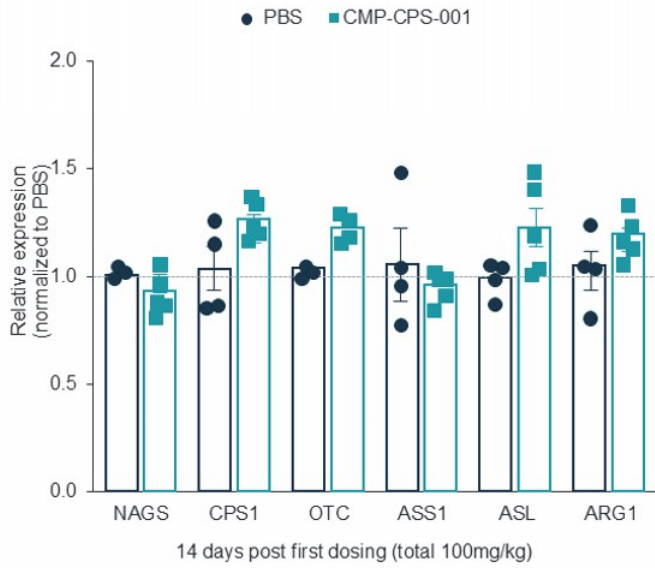


- The *Otc*^{spf-ash} mouse model carries a patient mutation in *Otc* that reduces mRNA levels
- *Otc* activity is 5%-10% of wild-type¹
- Model displays elevated ammonia relative to wild-type mice following an acute ammonia challenge
- ASO was shown to cause significant ~50% reduction in toxic ammonia (approx. WT levels)
- Correlated with ~20% increase in urea production (data not shown)
- Maximal effect achieved in 2-3 weeks
- Response persisted for ~1 month



CMP-CPS-001 increases ureagenesis in WT mice with humanized liver

Data confirm target engagement of CMP-CPS-001 in human hepatocytes in vivo and show increased expression of other urea cycle enzymes



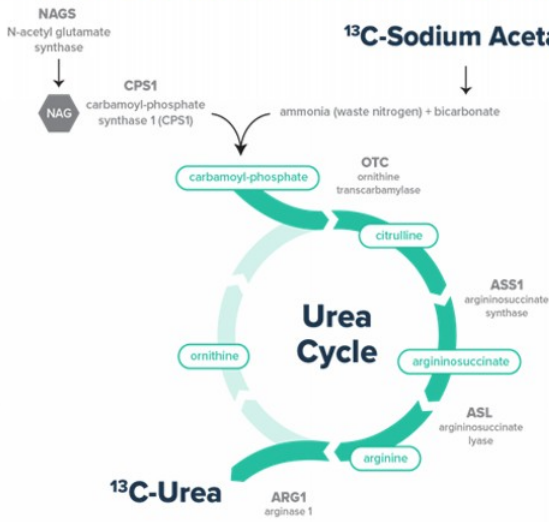
Error bars represent standard error of the mean; PBS denotes phosphate-buffered saline

** denotes P<0.01; * denotes P<0.05

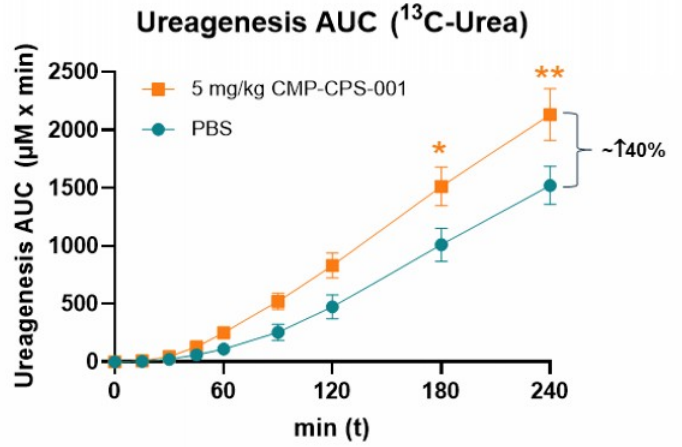


Using ureagenesis rate test (URT), CMP-CPS-001 was shown to increase ureagenesis up to 40% in wildtype cynomolgus monkeys

URT uses ^{13}C -labeled sodium acetate to measure ureagenesis



CMP-CPS-001 increases ureagenesis in NHPs



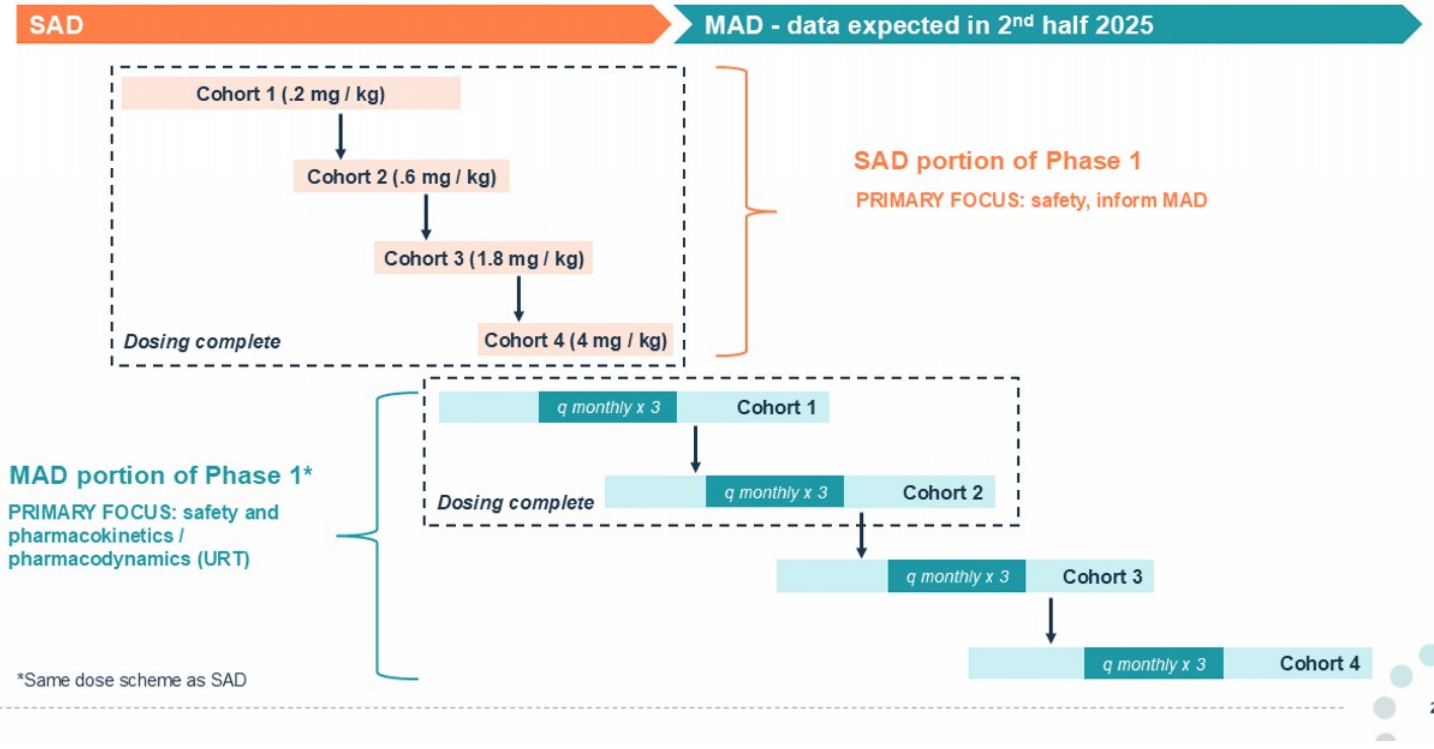
Using ^{13}C -Sodium Acetate for ureagenesis in WT NHP

NHP administered drug once-monthly. Ureagenesis assessed up to 30 days after final dose. Data shown is one week after a second dose.



Ongoing CMP-CPS-101 Phase 1 SAD / MAD clinical trial in healthy volunteers

n = 10-12 participants per cohort



Phase 1 SAD safety summary (all cohorts 1 – 4)

- SAD portion of study completed. Conducted planned interim analysis of safety data for 48 normal healthy volunteer participants.
 - 4 Cohorts of 12 participants each, randomized 3:1 investigational product to placebo; 32 individuals received CMP-CPS-001.
 - CMP-CPS-001 has been well tolerated, with no indication of a maximum tolerated dose at the tested dose levels. No safety trends of concern have been observed, including no treatment-emergent serious adverse events.
 - Safety results were favorable and consistent with profiles of approved liver-targeted ASOs and with expectations based on previously reviewed safety data from SAD Cohorts 1 & 2, which showed similar findings of all TEAEs being Grade 1 (mild) or Grade 2 (moderate) with no concerning safety trends.
 - All treatment emergent adverse events (TEAEs) were Grade 1 (mild) or Grade 2 (moderate). The most common TEAEs by participant and number were headache (six) and nausea (four).
- The Safety Review Committee has approved dose escalation and initiation of MAD Cohort 3.



Phase 1 has the potential to enable a combined Phase 2/3 registration study anticipated in 2026

Data Elements

1 Safety

- Vitals
- Cardiac monitoring
- Liver function tests
- Immunogenicity

2 Pharmacokinetics

- Plasma and urine measurements
- Compare human pharmacokinetic behavior to pre-clinical data observations
- Observe that human PK achieves levels expected to demonstrate efficacy on ammonia and ureagenesis in animal studies

3 Ureagenesis rate test (PD)

- Ureagenesis measures rates of flux through the urea cycle
- Rates of ureagenesis correlate with reduction in ammonia (approvable endpoint)
- URT utilized by other programs to correspond with clinically meaningful dropping of low protein diet and scavenger (supportive care measures)

Key Endpoint(s) in one or more anticipated Phase 2 / 3 Trials:

- Ammonia (recognized approvable endpoint)
- Diet liberalization
- Nitrogen Scavenger reduction
- Ureagenesis, glutamine
- Clinical events



Agenda

CAMP4 overview

RAP Platform: regRNAs are master controllers of gene regulation

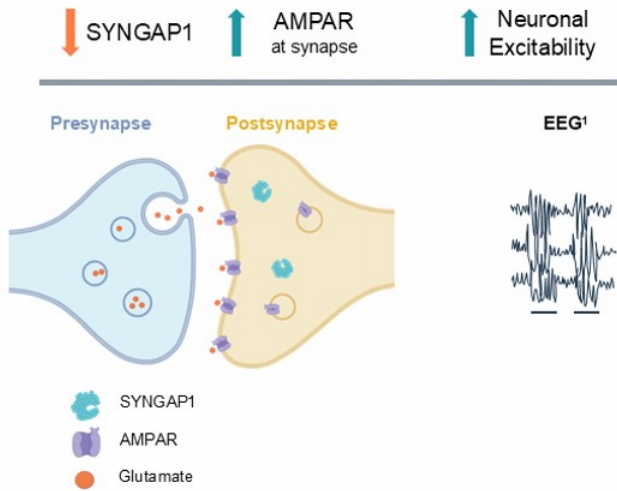
Lead metabolic program: Urea Cycle Disorders

Lead CNS program: SYNGAP1- related Disorders



SYNGAP1-related disorders, a severe genetic neurodevelopmental condition

Mutations in SYNGAP1 lead to decreased SYNGAP1 protein, causing increased synaptic firing



SYNGAP1 background

- Highly burdensome symptom array:
 - Intellectual disability, severe behavioral problems, ASD
 - Generalized epilepsy
 - Sleep problems
 - Impaired motor skills, gait abnormality
 - Impaired communication, speech problems
- ~10,000+ SYNGAP1 patients in the US¹⁻³

Current standard of care is symptomatic

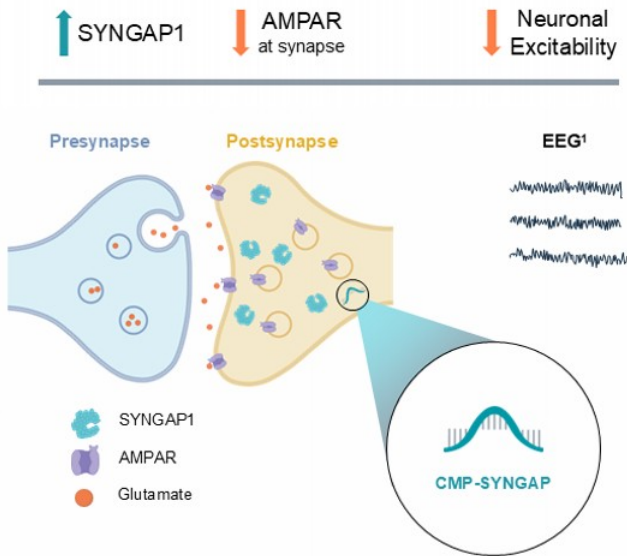
- No disease modifying treatments available
- Non-specific antiepileptics, sleep meds
- Constant patient care needed, caregiver worry about behavioral problems, agitation



¹ Illustrative depiction of Electroencephalogram

¹ López-Rivera et al., Brain, 2020
² Bahk et al., Int J Environ Res Public Health, 2019
³ Weldon et al., J Neurodev Disord, 2018

CAMP4 aims to increase SYNGAP1 protein levels to restore SYNGAP1 at the synapse and improve disease symptoms



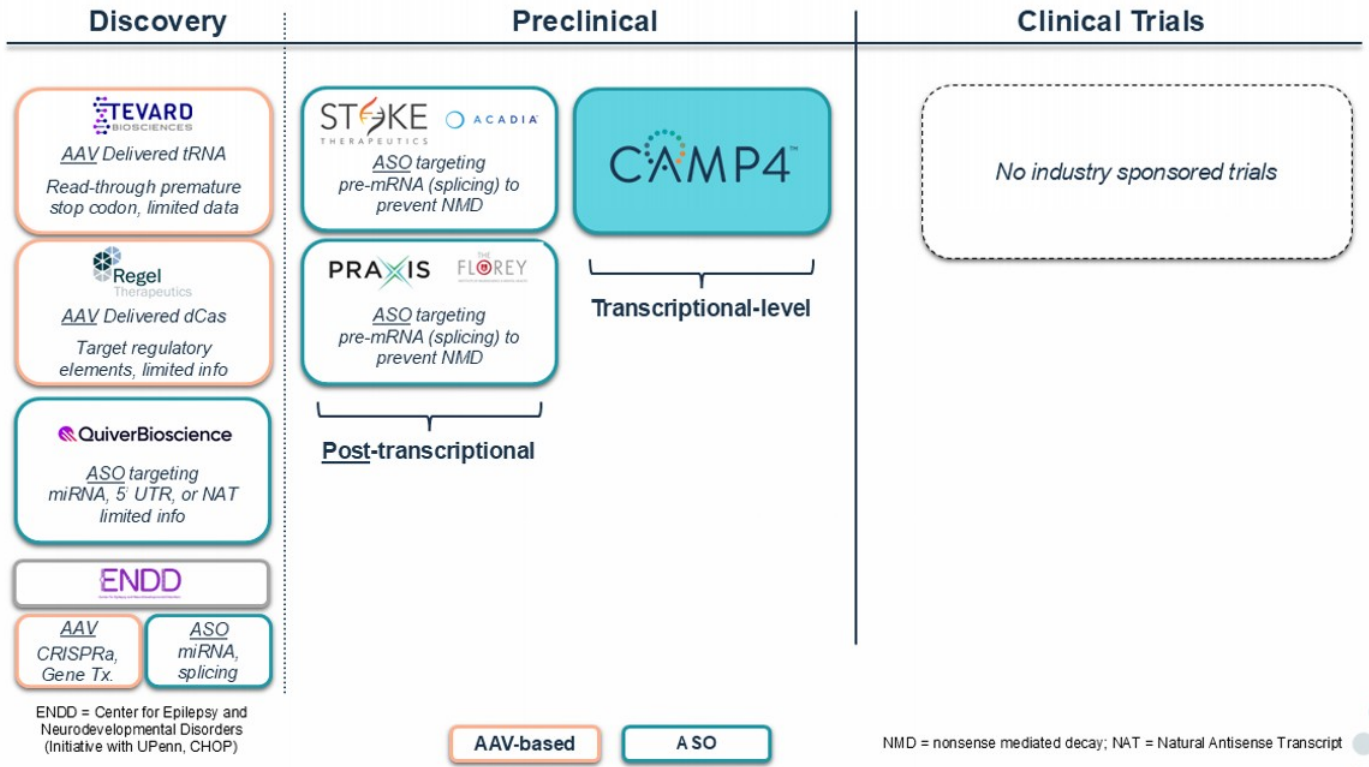
Restoring SYNGAP1 levels to treat disease

The **CMP-SYNGAP** program has identified lead ASOs that bind to a *SYNGAP1*-specific regRNA to increase *SYNGAP1* expression. Intrathecal administration of the clinical candidate will aim to restore SYNGAP1 towards wild-type levels, normalize synaptic function, and improve symptoms of patients with mutations in *SYNGAP1* causing haploinsufficiency.



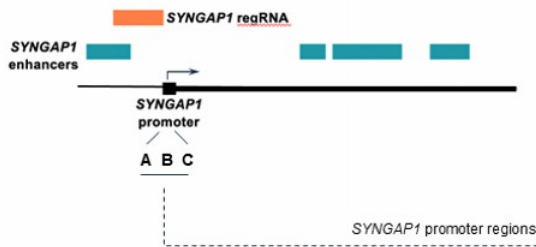
¹Illustrative depiction of Electroencephalogram

CAMP4 positioned as the only transcriptional-level ASO approach



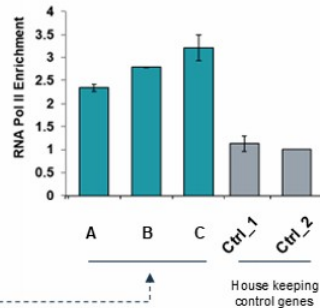
Targeting *SYNGAP1* regRNA demonstrated 2-fold increased expression of *SYNGAP1* mRNA and protein in vitro

1 Mapped *SYNGAP1* regRNA in iPSC neurons



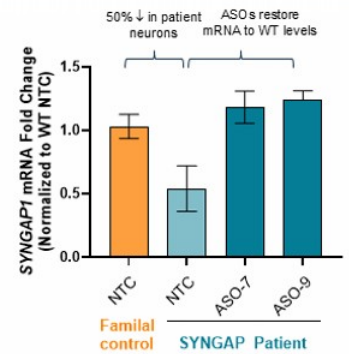
- Regulatory regions identified using RAP Platform

2 ASO binding to *SYNGAP1* regRNA directly increases transcription



- Increased RNA Polymerase II at *SYNGAP1* promoter (Chromatin Immunoprecipitation)
- All groups treated with regRNA-targeting ASO
- Data normalized to NTC for both control genes and *SYNGAP1*

3 ASOs targeting regRNA restore wildtype *SYNGAP1* mRNA levels in iPSC neurons

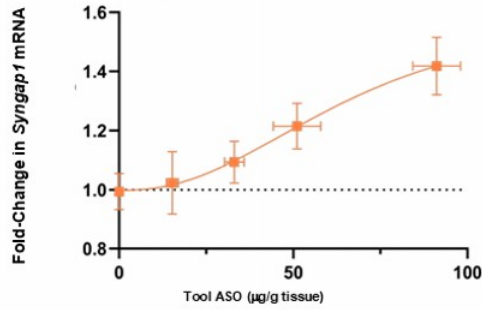


- iPSC-derived neurons from *SYNGAP1* patient and a familial control



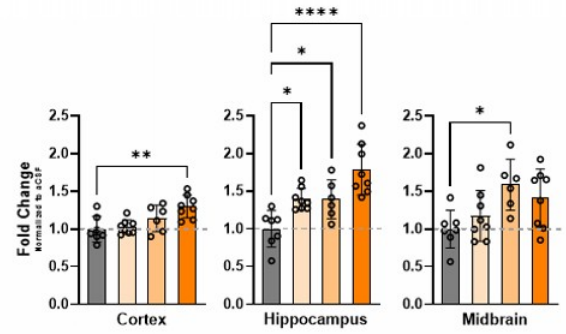
ASOs targeting SYNGAP1 regRNA demonstrated dose-responsive increase in mRNA and protein in vivo

Dose-responsive increase in *Syngap1* mRNA with mouse surrogate ASO



- Wild-type, neonatal mice
- Intracerebroventricular injection
- mRNA assessment at two weeks post-dose (showing midbrain)

Dose-responsive increase in SYNGAP1 protein with lead ASO in humanized mouse model



- Mouse model with human *SYNGAP1* replacement of mouse gene
- Intracerebroventricular injection
- Protein assessment at two weeks post-dose

- aCSF* (grey)
- 100 µg (dark orange)
- 50 µg (light orange)
- 25 µg (pale orange)

*Artificial CSF



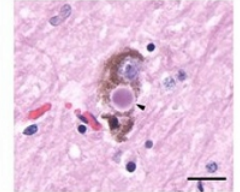
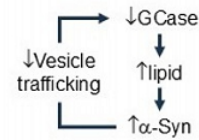
New discovery program: Increasing GBA1 expression for the potential treatment of Parkinson's disease

Significant unmet need

- 5-15% of Parkinson's disease (PD) patients have GBA1 (GCCase) mutations¹
- Frequency equates to ~100K GBA-PD patients in the US (~1M PD)^{1,2}
- Mutations result in ~50% reduction of GCCase activity³

GBA1 rationale

- GCCase and α -synuclein (hallmark disease protein for PD) are part of a positive feedback loop⁴
- GCCase activity also reduced in sporadic PD³
- Plan to explore therapeutic potential in both genetically defined GBA-PD and sporadic PD



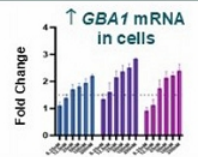
Image⁵

GBA1 upregulation in vitro

- ASOs identified that target GBA1 regRNA that lead to robust increases in expression in vitro

RAP Platform: regRNAs identified

ASO Synthesis & Screening



Biomarker

- Clinical development enabled with widely used rating scales (UPDRS) and biomarkers (GCCase in CSF, NfL, α -synuclein)



¹Sidransky et al., N Engl J Med, 2010; ²Parkinson's Foundation & APDA; ³Gegg et al., Annals of Neurology, 2012; ⁴Mazzulli et al., Cell, 2011; ⁵Credit: Tulemo, CC BY-SA 4.0, via Wikimedia Commons.

Pioneering a new class of RNA medicines to increase targeted gene expression



There are prevalent diseases where gene upregulation is likely to have a meaningful clinical benefit



CAMP4 is the leader in gene regulatory RNA (regRNA) discovery and regRNA-targeting antisense oligonucleotide (ASO) therapies to upregulate gene expression to restore healthy protein levels



Our proprietary RAP Platform™ was built for the discovery of novel regRNAs that regulate the expression of every protein-coding gene



Our current focus is on metabolic and CNS genetic diseases where modest increases in protein expression can be clinically meaningful



Clinical data expected from MAD portion of ongoing Phase 1 study of CMP-CPS-001 for Urea Cycle Disorders in 2H '25 and advancement of SYNGAP1 program into GLP tox studies

IPO: OCT 2024

NASDAQ: CAMP

CASH RUNWAY INTO Q2 '26

HEADQUARTERS:
CAMBRIDGE, MA





Thank you

CAMP4 Provides Corporate Updates and Highlights Key Upcoming Milestones

- Data from Single Ascending Dose (SAD) portion of Phase 1 study of CMP-CPS-001 for the treatment of urea cycle disorders (UCDs) demonstrates favorable safety results
 - Completed dosing in the first two Multiple Ascending Dose (MAD) cohorts, and initiated dosing in Cohort 3
 - MAD safety and key study biomarker data expected in 2H 2025
 - CAMP4 added to Russell 2000® Index
 - Initiation of new discovery program targeting a GBA1 regRNA for the treatment of Parkinson's disease (PD)
- Company to present at the 43rd Annual J.P. Morgan Healthcare Conference on Wednesday, January 15 at 3:45 p.m. PST

CAMBRIDGE, Mass., January 7, 2025 – CAMP4 Therapeutics Corporation (“CAMP4”) (Nasdaq: CAMP), a clinical-stage biotechnology company developing regRNA-targeting antisense oligonucleotide (ASO) therapies to upregulate gene expression to restore healthy protein levels, today provided corporate updates and key objectives for 2025.

“2024 was a transformative year for CAMP4, highlighted by our successful IPO, and we are entering 2025 with tremendous momentum and a clear path towards delivering on our goals,” said Josh Mandel-Brehm, Chief Executive Officer of CAMP4. “We are very pleased with the clinical progress of our lead program CMP-CPS-001 for urea cycle disorders, which was granted Rare Pediatric Disease Designation and Orphan Drug Designation, validating the urgency of our novel regRNA-targeting ASOs. Additionally, our strategic partnership with BioMarin highlights confidence in our RAP platform. These achievements, combined with the proceeds from the IPO, position us to continue to progress our pipeline development and execute on key milestones this year.”

“We are pleased to report the safety data from the SAD portion of our Phase 1 study of CMP-CPS-001 for UCDs, showing that the drug has been well-tolerated,” said Dr. Yuri Maricich, Chief Medical Officer of CAMP4. “We expect 2025 to be a pivotal year as we anticipate reporting MAD safety and key biomarker data in the second half of 2025. Results from these studies could enable us to advance the CMP-CPS-001 program into a registrational Phase 2/3 trial in 2026.”

2024 Key Highlights

- Completed IPO of 6,820,000 shares of common stock at an initial public offering price of \$11.00 per share. Aggregate gross proceeds to CAMP4 were approximately \$75.0 million. The underwriters also partially exercised their option to purchase an additional 643,762 shares of common stock for total offering gross proceeds of \$82.1 million.
 - Advanced Phase 1 clinical trial of CMP-CPS-001 for UCDs. The Phase 1 study is a randomized, double-blind, and placebo-controlled study designed to evaluate the safety, tolerability, and pharmacokinetics of CMP-CPS-001 in 96 healthy volunteers.
 - Completed planned interim analysis of all four SAD cohorts of the Phase 1 clinical trial of CMP-CPS-001 in 48 healthy volunteer participants. Safety results were favorable and consistent with the safety profile of approved liver-targeted ASOs, with all treatment emergent adverse events (TEAEs) being Grade 1 (mild) or Grade 2 (moderate). The two most common TEAEs across all cohorts were headache (six participants) and nausea (four participants). No safety trends of concern have been observed, and CMP-CPS-001 appears to be well-tolerated. Dosing is completed in the first two MAD cohorts, and the Safety Review Committee (SRC) has approved dose escalation to MAD Cohort 3, in which dosing has been initiated.
 - The FDA granted Rare Pediatric Disease Designation and Orphan Drug Designation to CMP-CPS-001 for the treatment of UCDs.
 - Entered strategic research collaboration agreement with BioMarin Pharmaceutical Inc., under which BioMarin has the right to select two targets identified by CAMP4's RAP Platform to advance into clinical development.
 - Company added to the Russell 2000® Index as part of the fourth quarter IPO additions.
 - Appointed John Maraganore, Ph.D., and Rachel Meyers, Ph.D., as strategic advisors.
-

Expected Milestones in 2025

- MAD safety, pharmacokinetic and key pharmacodynamic biomarker data in healthy volunteers in the second half of 2025.
- GLP toxicity studies of lead ASO candidate for neurodevelopmental disorders caused by SYNGAP1 mutations to be initiated this year.
- Advance a new discovery program targeting a GBA1 regRNA to increase gene expression for the treatment of Parkinson's disease (PD) caused by mutations in GBA1, with potential for application in sporadic PD.
- Company to focus on expanding its strategic partnerships to continue maximizing the value of its RAP platform.

About CAMP4 Therapeutics

CAMP4 is developing disease-modifying treatments for a broad range of rare and prevalent genetic diseases where increasing healthy protein levels may offer meaningful therapeutic benefits. Our approach allows for targeted gene upregulation by harnessing a fundamental mechanism of how genes are controlled. To increase gene expression, our therapeutic ASO drug candidates target regRNAs, which act locally on transcription factors and are the master regulators of gene expression. CAMP4's proprietary RAP Platform™ enables the mapping of regRNAs and generation of therapeutic candidates designed to target the regRNAs associated with genes underlying haploinsufficient and recessive partial loss-of-function disorders, of which there are more than 1,200, in which a modest increase in protein expression may have the potential to be clinically meaningful. Learn more about us at www.CAMP4tx.com and follow us on [LinkedIn](#) and [X](#).

Forward-Looking Statements

This press release contains forward-looking statements which involve risks, uncertainties and contingencies, many of which are beyond the control of the Company, which may cause actual results, performance, or achievements to differ materially from anticipated results, performance, or achievements. All statements other than statements of historical facts contained in this press release are 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," "contemplate," "believe," "estimate," "predict," "potential" or "continue" or the negative of these terms or other similar expressions, although not all forward-looking statements contain these words. Forward-looking statements include, but are not limited to, statements concerning CAMP4's plans, objectives, expectations and intentions; the timing and results of ongoing and future clinical trials, including expectations on the timing of reporting MAD data from the CMP-CPS-001 trial and advancing the CMP-CPS-001 program into a registration-enabling trial; the timing to initiate GLP toxicity studies relating to CAMP4's SYNGAP1 program; the timing to advance new discovery programs; its growth strategy; and cash runway guidance. The forward-looking statements in this press release speak only as of the date of this press release and are subject to a number of known and unknown risks, uncertainties and assumptions that could cause the Company's actual results to differ materially from those anticipated in the forward-looking statements, including, but not limited to: the Company's limited operating history, incurrence of substantial losses since the Company's inception and anticipation of incurring substantial and increasing losses for the foreseeable future; the Company's need for substantial additional financing to achieve the Company's goals; the uncertainty of clinical development, which is lengthy and expensive, and characterized by uncertain outcomes, and risks related to additional costs or delays in completing, or failing to complete, the development and commercialization of the Company's current product candidates or any future product candidates; delays or difficulties in the enrollment and dosing of patients in clinical trials; the impact of any significant adverse events or undesirable side effects caused by the Company's product candidates; potential competition, including from large and specialty pharmaceutical and biotechnology companies; the Company's ability to realize the benefits of the Company's current or future collaborations or licensing arrangements and ability to successfully consummate future partnerships; the Company's ability to obtain regulatory approval to commercialize any product candidate in the United States or any other jurisdiction, and the risk that any such approval may be for a more narrow indication than the Company seeks; the Company's dependence on the services of the Company's senior management and other clinical and scientific personnel, and the Company's ability to retain these individuals or recruit additional management or clinical and scientific personnel; the Company's ability to grow the Company's organization, and manage the Company's growth and expansion of the Company's operations; risks related to the manufacturing of the Company's product candidates, which is complex, and the risk that the Company's third-party manufacturers may encounter difficulties in production; the Company's ability to obtain and maintain sufficient intellectual property protection for the Company's product candidates or any future product candidates the Company may develop; the Company's reliance on third parties to conduct the Company's preclinical studies and clinical trials; the Company's compliance with the Company's obligations under the licenses granted to the Company by others, for the rights to develop and commercialize the Company's product candidates; risks related to the operations of the Company's suppliers; and other risks and uncertainties described in the section "Risk Factors" in the Company's Quarterly Report on Form 10-Q for the quarter ended September 30, 2024, as well as other information we file with the Securities and Exchange Commission. The forward-looking statements in this press release are inherently uncertain and are not guarantees of future events. Because forward-looking statements are inherently subject to risks and uncertainties, some of which cannot be predicted or quantified and some of which are beyond the Company's control, you should not unduly rely on these forward-looking statements. The events and circumstances reflected in the forward-looking statements may not be achieved or occur and actual future results, levels of activity, performance and events and circumstances could differ materially from those projected in the forward-looking statements. Moreover, the Company operates in an evolving environment. New risks and uncertainties may emerge from time to time, and management cannot predict all risks and uncertainties. Investors, potential investors, and others should give careful consideration to these risks and uncertainties. Except as required by applicable law, the Company does not undertake to publicly update or revise any forward-looking statements contained herein, whether as a result of any new information, future events, changed circumstances or otherwise.

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