

**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): March 5, 2026

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 2.02 Results of Operations and Financial Condition.

On March 5, 2026, CAMP4 Therapeutics Corporation (the "Company") issued a press release announcing its financial results for the year ended December 31, 2025. A copy of the press release is furnished as Exhibit 99.1 to this Current Report on Form 8-K.

The information in this Item 2.02 and Exhibit 99.1 attached hereto is being furnished and shall 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 liabilities of that Section, nor shall it be deemed incorporated by reference into any registration statement or other filing under the Securities Act of 1933, as amended (the "Securities Act"), or the Exchange Act, except as shall be expressly set forth by specific reference in such a filing.

Item 7.01 Regulation FD Disclosure.

From time to time, the Company presents and/or distributes slides and presentations to the investment community to provide updates and summaries of its business. On March 5, 2026, the Company updated its corporate presentation, which is available on the "Investors" section of the Company's website at <https://investors.camp4tx.com>. This presentation is also furnished as Exhibit 99.2 to this Current Report on Form 8-K.

The information in this Item 7.01 and Exhibit 99.2 attached hereto is being furnished and shall not be deemed "filed" for purposes of Section 18 of the Exchange Act or otherwise subject to the liabilities of that Section, nor shall it be deemed incorporated by reference into any registration statement or other filing under the Securities Act or the Exchange Act, except as shall be expressly set forth by specific reference in such a filing.

Item 9.01 Financial Statements and Exhibits.

(d) Exhibits

Exhibit No.	Description
99.1	Press release issued by CAMP4 Therapeutics Corporation on March 5, 2026.
99.2	Corporate presentation, dated March 2026.
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: March 5, 2026

CAMP4 Reports Full Year 2025 Financial Results and Corporate Highlights

GLP toxicology studies ongoing for CMP-002, with initiation of global Phase 1/2 clinical trial in SYNGAP1 patients expected as early as 2H 2026

Entered strategic collaboration with GSK to advance RNA-based therapeutic discoveries, received \$17.5 million upfront and eligible for milestone-based payments in addition to tiered royalties

Completed private placement with upfront gross proceeds of \$50 million, with potential for up to an additional \$50 million of gross proceeds, as well as underwritten offering of common stock of \$30 million in gross proceeds, extending cash runway into 2028

CAMBRIDGE, Mass., March 5, 2026 – CAMP4 Therapeutics Corporation (“CAMP4”) (Nasdaq: CAMP), a clinical-stage biopharmaceutical company developing a pipeline of regulatory RNA-targeting therapeutics designed to upregulate gene expression with the goal of restoring healthy protein levels to treat a broad range of genetic diseases, today announced financial results for the full year ended December 31, 2025, and provided recent corporate highlights.

“In 2025, we brought our SYNGAP1 program to the forefront of our pipeline and made significant progress against our goal of bringing a potential first-in-class treatment for SYNGAP1-related disorder into the clinic,” said Josh Mandel-Brehm, President and Chief Executive Officer of CAMP4. “Our GLP toxicology studies for CMP-002 are ongoing, and we continue to expect to initiate a global first-in-human Phase 1/2 clinical trial as early as the second half of 2026. We also made progress in our mission of developing potentially disease modifying medicines for patients with disorders marked by suboptimal gene expression by exploring new candidates for both in-house development and potential partnerships and signed a collaboration agreement with GSK to identify and develop ASO drug candidates for multiple gene targets relevant to neurodegenerative and kidney disease indications. Finally, we strengthened our balance sheet through a combination of equity financing and non-dilutive capital from collaboration partners to ensure that CAMP4 is well-capitalized to achieve its goals.”

Corporate Highlights:

- Presented compelling preclinical data for SYNGAP1 program showing that haploinsufficient SYNGAP1 mice treated with CMP-002 demonstrated an increase in SYNGAP1 protein levels and that treatment rescued multiple SYNGAP1-dependent behavioral phenotypes. This data further demonstrated that CMP-002 administration led to a significant increase in SYNGAP1 protein levels in relevant brain regions in non-human primates (NHPs).
- Initiated GLP toxicology studies for CMP-002 in support of future clinical trial applications, which could enable initiation of a global Phase 1/2 clinical trial in patients as early as H2 2026.
- Entered a strategic research, collaboration, and license agreement with GSK to identify and develop antisense nucleotide (ASO) drug candidates for multiple gene targets relevant to neurodegenerative and kidney disease indications. CAMP4 received a \$17.5 million cash upfront

payment and has the potential to receive up to \$440 million upon achievement of certain development and commercial milestones, in addition to tiered royalties on future product sales.

- Completed oversubscribed private placement for gross proceeds of up to \$100 million, \$50 million of which was received in the form of an upfront payment in September, as well as underwritten offering of common stock with gross proceeds of \$30 million in December to fund further development of CMP-002.
- Conducted analysis from single ascending dose (SAD) and multiple ascending dose (MAD) portions of the CMP-001 Phase 1 clinical trial and made strategic decision to pause further investment in the development of CMP-001 and explore potential partnership opportunities.

Full Year 2025 Financial Results

Cash and cash equivalents as of December 31, 2025, were \$109.5 million, compared to \$64.0 million as of December 31, 2024. The Company believes that its current cash and cash equivalents will be sufficient to fund its planned activities into 2028.

R&D Expenses: Research and development (“R&D”) expenses for the year ended December 31, 2025, were \$38.2 million, compared to \$38.8 million for the year ended December 31, 2024. The decrease was primarily driven by a gain on lease modification and decreased clinical costs associated with CMP-001.

G&A Expenses: General and administrative (“G&A”) expenses were \$17.4 million for the year ended December 31, 2025, compared to \$14.9 million for the year ended December 31, 2024. The change was primarily driven by an increase in professional expenses.

Net Loss: Net loss for the year ended December 31, 2025, was \$80.4 million compared to \$51.8 million for the year ended December 31, 2024. The increase in net loss was primarily driven by a \$29.8 million non-cash loss recognized due to a change in fair value of the derivative tranche liability related to our September private placement.

About CAMP4 Therapeutics

CAMP4 is developing disease-modifying treatments for a broad range of genetic diseases where amplifying healthy protein may offer therapeutic benefits. Our approach amplifies mRNA by harnessing a fundamental mechanism of how genes are controlled. To amplify mRNA, our therapeutic ASO drug candidates target regulatory RNAs (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. For more information, visit camp4tx.com.

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 the anticipated timing to advance the Company’s SYNGAP1 program into a clinical trial; the Company’s plan to pursue partnership opportunities to support the further development of CMP-001; the development of ASO drug candidates for multiple gene targets relevant to neurodegenerative and kidney disease indications; the potential of the Company’s platform technology; the Company’s receipt of future contingent milestones and/or royalties; the potential to receive up to \$50 million in additional gross proceeds in connection with the Company’s September 2025 private placement; the Company’s strategy, goals, business plans and focus; the therapeutic potential of the Company’s product candidates; and the Company’s 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 Annual Report on Form 10-K for the year ended December 31, 2025, as well as other information the Company files 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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CAMP4 Therapeutics Corporation
Consolidated Statements of Operations and Comprehensive Loss
(In thousands, except for share and per share data)

	Year ended December 31,	
	2025	2024
Revenue		
Research and collaboration revenue	\$ 3,498	\$ 652
Operating expenses		
Research and development	38,202	38,817
General and administrative	17,357	14,923
Impairment of right-of-use asset	494	—
Total operating expenses	56,053	53,740
Loss from operations	(52,555)	(53,088)
Other (expense) income, net:		
Interest income	2,174	1,330
Change in fair value of derivative tranche liability	(29,830)	—
Other expense	(192)	(33)
Total other (expense) income, net	(27,848)	1,297
Net loss	\$ (80,403)	\$ (51,791)
Net loss per share attributable to common stockholders, basic and diluted	\$ (2.65)	\$ (11.04)
Weighted average shares of common stock outstanding, basic and diluted	30,380,567	4,690,094

	December 31,	
	2025	2024
Condensed Balance Sheet Data		
<i>(in thousands)</i>		
Cash and cash equivalents	\$ 109,517	\$ 64,039
Working capital(1)	98,581	56,785
Total assets	117,808	78,307
Total liabilities	70,104	15,163
Accumulated deficit	(292,156)	(211,753)
Total stockholders' equity	47,704	63,144

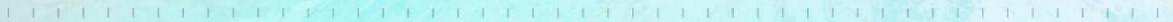
(1) Working capital is defined as total current assets less total current liabilities. See our consolidated financial statements and the related notes thereto included in our Annual Report on Form 10-K for the year ended December 31, 2025, for further details regarding our current assets and current liabilities.



Corporate Overview

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

MARCH 2026



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 and market and industry data" in the Company's most recent Annual Report on Form 10-K for the year ended December 31, 2025. 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.



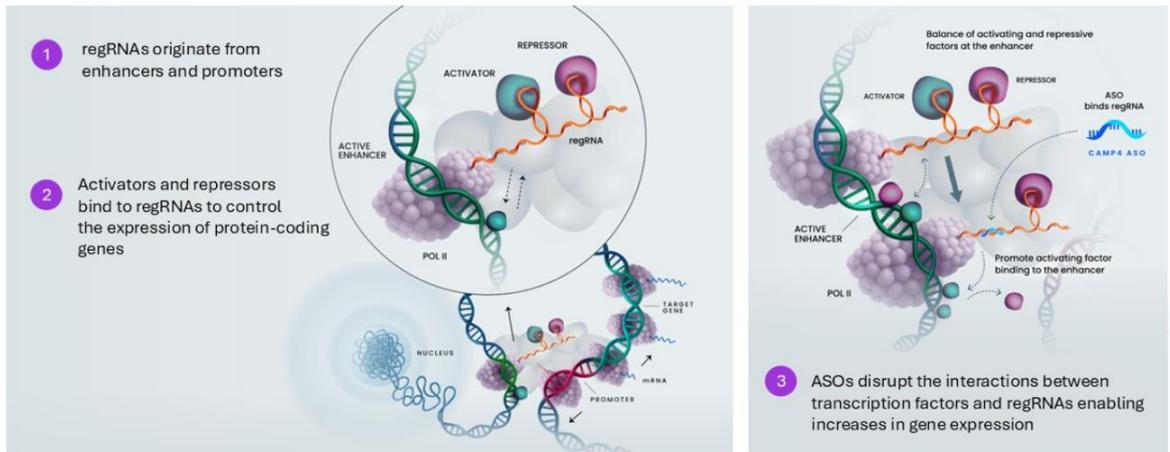
CAMP4: Targeted ASO therapeutics that selectively upregulate gene expression by modulating regulatory RNA

- Developing a targeted disease modifying therapy to address dire unmet need in SYNGAP1-related disorder
 - SYNGAP1 is a haploinsufficient CNS disorder, and an optimal target for CAMP4
 - CMP-002 is designed to increase SYNGAP protein levels, restore SYNGAP1 function and improve disease symptoms
 - >10,000 SYNGAP1 patients in the US; epi in line with rare diseases with similar unmet needs and large commercial markets
- Positioned to be first in the clinic for SYNGAP1
 - No disease modifying therapies are approved or in clinical development
 - Highly translatable preclinical models: Proof of concept data in humanized mice showed reversal of disease phenotype, primate data showed significant protein upregulation and broad ASO distribution across key brain regions believed to be critical to the disease
 - Expect to advance CMP-002 to a global Ph 1/2 study in patients as early as second half of 2026
- CNS-focused pipeline, leveraging BD to derive additional value from the platform
 - Proprietary RAP Platform[®] was built for the discovery of novel regRNAs that regulate the expression of every protein-coding gene that can be selectively drugged using state of the art ASO chemistry
 - Additional undisclosed development epileptic encephalopathy (DEE) programs in development, similar in phenotype to SYNGAP1
 - Strategic discovery partnership with GSK unlocks additional platform value beyond CNS and validate CAMP4's novel approach to gene upregulation



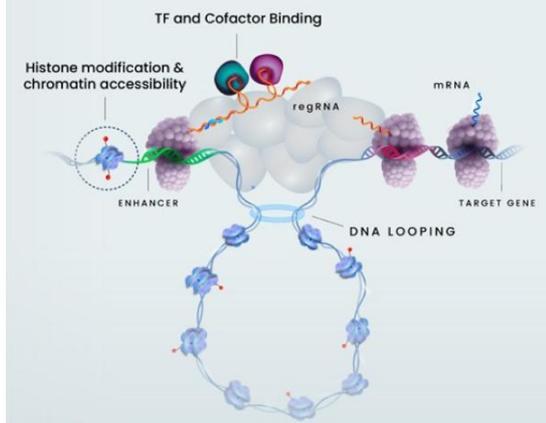
regRNAs play a central role in the regulation of every gene's expression

Increased mRNA addresses root cause of disease by returning targeted protein levels toward a healthy range



CAMP4's proprietary RAP Platform® catalogs thousands of regRNA targets and generates ASO candidates to increase gene expression

Genome-wide analyses of chromatin & RNA



1 Map candidate regRNAs

- Generate large scale genomic datasets for cells & tissues
- Deploy proprietary ML/AI platform to identify regulatory regions
- Capture and sequence predicted regRNAs
- Create proprietary catalogs containing tens of thousands of regRNAs across diverse cell and tissue types

2 Generate ASO leads

Screen regRNAs to rapidly identify leads that upregulate target genes



3 Optimize lead candidates

Optimize chemistry and sequences for activity, pharmacology, & safety



Our pipeline of CNS-focused upregulation programs

Program	Indication	Target	Discovery & Preclinical Development	Phase 1/2	Phase 3	Anticipated Milestones	Commercial Rights
CNS DISEASES							
CMP-002	SYNGAP1-related disorder	SYNGAP1				GLP tox studies ongoing Clinical initiation as early as H2 2026	CAMP4
New Discovery Programs	CNS	Numerous	Active discovery and development of multiple programs utilizing RAP Platform®.				CAMP4
METABOLIC DISEASES							
CMP-001	Urea Cycle Disorders	CPS1	Exploring potential partnership opportunities.				CAMP4
COLLABORATIONS							
Strategic research collaboration to identify and develop antisense oligonucleotide (ASO) drug candidates for multiple gene targets relevant to neurodegenerative and kidney disease indications.							GSK

SYNGAP1 patient journey: Tony and his family's experience highlights the dire unmet need for disease modifying therapy

PATIENT

Tony, 11 Years Old



TONY, 3



TONY, 8

Diagnostic Journey

- Developmental delays evident at 2, one seizure at 3
- EEG confirmed epilepsy, negative chromosomal microarray, variant confirmed by RNA Seq
- Pathogenic diagnosis at 4



7

Patient story and images used with permission: "Our Son Has a Rare Genetic Disorder, Life Is Risky for Us" Mike Craglia, Newsweek (January 25, 2023)

CAREGIVER + FAMILY BURDEN

Immense Caregiver Burden



TONY, 8

"His spontaneous aggression leads to bruises and scary moments for family members and makes it very challenging to find childcare."

"Requires transferring to a special school. Tony is getting stronger and the future is scary."

"My life is dedicated to this cause; as a parent, it's the number one thing I strive to do for my sons: alleviate Tony's suffering to help him live the best possible life"

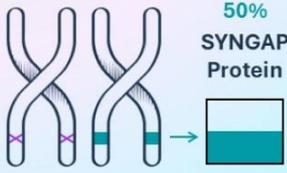
- Tony's Father



SYNGAP1 is a true haploinsufficiency with >10,000 US patients in need of therapy

Haploinsufficiency results in 50% of normal protein levels

SYNGAP1 Haploinsufficiency



50% SYNGAP Protein

>10,000 SYNGAP1 patients in the US



6.1–10 per 100K incidence rate^{1,2}

100% have intellectual disability, ~85% have seizures, potentially experiencing 10+ per day^{3,4,5}



High unmet need for disease-modifying therapy

ICD-10 code assigned in 2021 **F78.A1**



0 approved disease modifying therapies

Dire unmet need for a targeted disease modifying therapy to alter SYNGAP1's devastating disease course



JAELI, 16

Complex Symptoms



Developmental delay and/or intellectual disability

- 100% of patients^{1,2,3}



Generalized epilepsy

- ~85% of patients^{3,4,5}



Severe behavioral problems

- ~70% of patients^{1,5}



Sleep problems

- ~60% of patients^{2,5}



Limited communication

- ~30% non-verbal, single words⁴

No Approved Therapy

Non-specific treatments have limited impact on SYNGAP1 symptoms

- Anti-seizure medications
- Cannabinoids
- Sleep medications

Polypharmacy is common – Patient regimen⁶ example:

- Epidiolex
- Ravicti
- Sodium bicarb
- Amantadine

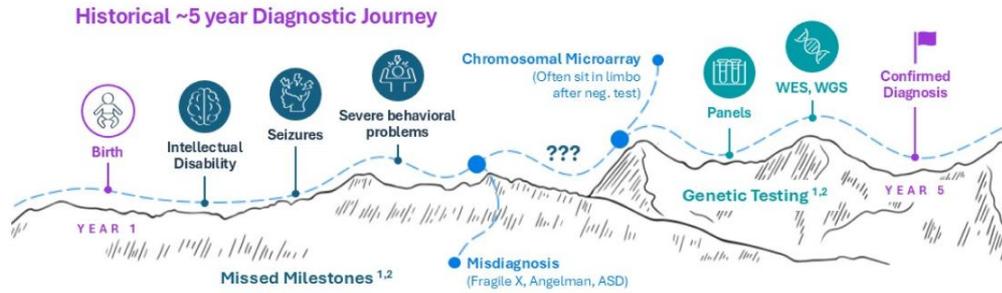
Constant patient care needed

- Caregivers vigilant at all times
- Significant lifelong cost of care



¹ Wiltrout, et al., *Epilepsia*, 2024; ² Jimenez-Gomez, et al., *J Neurodev Disord*, 2019; ³ Holder et al., *GeneReviews*, 2019; ⁴ SYNGAP-Related Epilepsy, *Epilepsy Foundation* (Accessed May 2025); ⁵ Viskamp et al., *Neurology*, 2019; ⁶ SYNGAP Research Fund (SRF)

Expanding awareness and testing is enabling faster diagnosis from ~5 years to ~1 year from time of first symptom or missed milestone



~21K patients across key global geographies (US + EU5); SYNGAP1 remains highly underdiagnosed

Major Market Prevalence



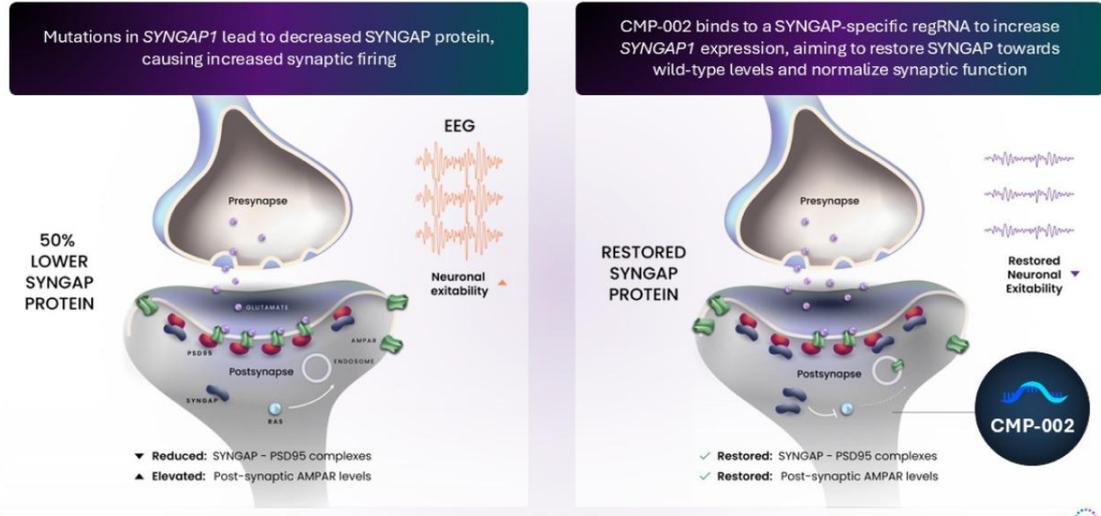
- Scaled annual incidence to prevalence ¹
- Prevalence may be larger; diagnosis rates may increase significantly with genetic testing awareness and utilization ²
- Third party market research triangulated across literature, rare disease analogs, KOL interviews, Komodo claims data
- Prevalence estimates support multi-billion \$ commercial potential

Increasing Diagnosis Rate



- Predict notable increase in diagnosis:
 - Increase in US claims, ICD-10 code added 2021 (+200 pt / yr)
 - [CURE SYNGAP1](#) global census (+50 pt / quarter)
 - SYNGAP1 has been added to many genetic testing panels ³
 - Increasing use of genetic testing in ASD, ID, DEE ^{4,5}

CAMP4 aims to increase SYNGAP protein levels, restore *SYNGAP1* function and improve disease symptoms



SYNGAP1 represents an ideal target for CAMP4; restoring SYNGAP protein has the potential to meaningfully improve patient outcomes

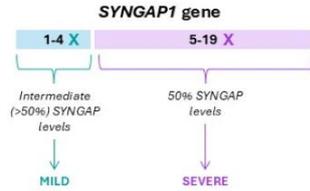


Opportunity in SYNGAP1 driven by unmet need and compelling preclinical data (presented at ASGCT May '25)

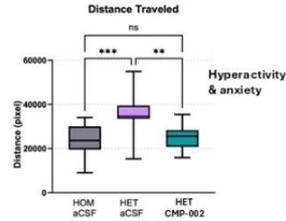
- Ultra-rare SYNGAP1 sub population with intermediate SYNGAP levels equate to milder disease state
- CMP-002 rescued functional defects in relevant human mouse model
- IT administration in NHPs well-tolerated and showed significant increase in SYNGAP in key brain regions

Precedent of ASO or siRNA activity in NHPs has translated to clinical efficacy when targeting genetic diseases

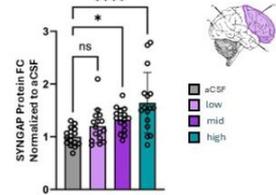
Milder disease severity (verbal responses, milder epilepsy) in minority of patients with intermediate SYNGAP levels



CMP-002 treatment rescued SYNGAP1 mouse model exhibiting disease-relevant phenotypes

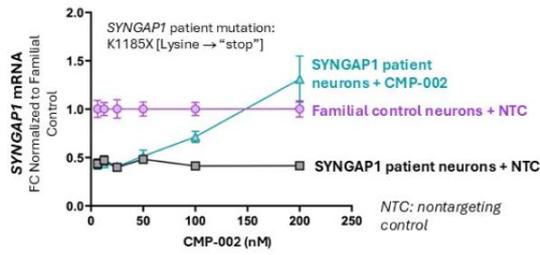


CMP-002 delivered intrathecally increased SYNGAP in disease-relevant brain regions in monkeys

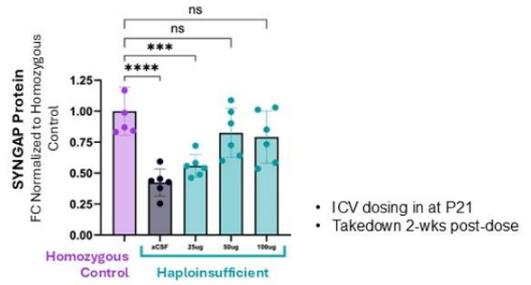


CMP-002 restores SYNGAP levels in models of haploinsufficiency

SYNGAP1 Patient iPSC-derived neurons

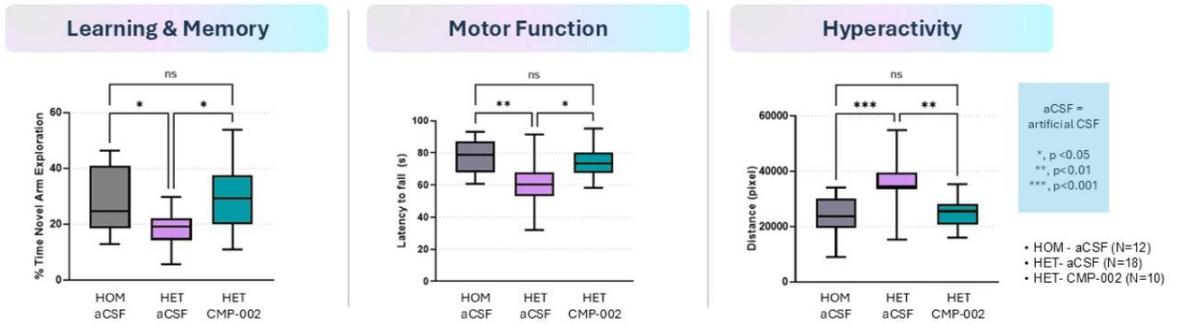


"Humanized" SYNGAP1 mouse



Dose-dependent increase in SYNGAP approaching wild-type levels → both ASO activity and regRNA function translates from human patient-derived neurons in a dish to neurons in an intact animal brain

Improved behavioral phenotypes in SYNGAP1 humanized haploinsufficient mice given CMP-002

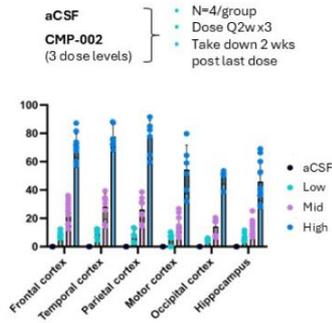


Neonatal mice administered CMP-002 and assessed within 3-weeks; protein restored to near-wild type levels

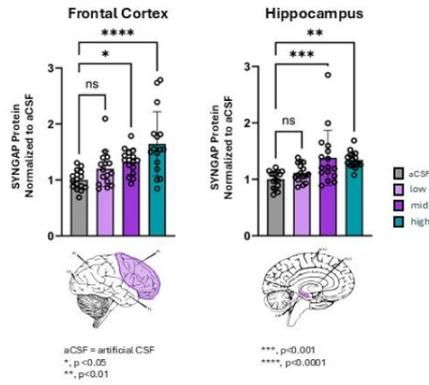
Restoring SYNGAP protein to near wild-type levels ameliorates multiple behavioral phenotypes caused by SYNGAP1 haploinsufficiency

Intrathecal administration of CMP-002 to cynomolgus monkeys achieves broad brain distribution to increase SYNGAP protein levels

ASO Concentrations



SYNGAP Protein Levels



Summary

IT administration in NHPs was **well-tolerated**

Broad ASO distribution throughout disease-relevant brain regions

↑ **SYNGAP protein** throughout brain

GLP tox studies **ongoing**

CAMP4 positioned to be first in the clinic for SYNGAP1

Standard-of-Care	<ul style="list-style-type: none">• No disease modifying therapies available• Patients currently managed symptomatically, with complications of polypharmacy
Natural History Study	<ul style="list-style-type: none">• Consortium-driven, funded, multi-site study* is ongoing, multiple advocacy / centers of excellence > 1 yr duration, 100 patients with 1,240 patient years of data obtainable
Center of Excellence	<ul style="list-style-type: none">• Global centers of excellence, currently nodes for translational work and natural history, with readiness to expand to trial sites for rapid clinical trial conductance
Path to Clinic	<ul style="list-style-type: none">• GLP toxicity studies ongoing• Potential for CMP-002 to be evaluated in a global Ph1/2 study in patients initiating as early as H2 2026
Path to Approval	<ul style="list-style-type: none">• Multiple, established paths to approval for a developmental epileptic encephalopathy (DEE)• Seizure quantification + neurodevelopmental scale(s) as well as multiple clinical scales accepted by regulators

Multiple regulatory precedents and validated measures to demonstrate disease-modifying effect for PoC in First-in-Human

Phase 1/2 Endpoint Categories



Regulatory Approval Paradigms

Seizure +

- Seizure as primary with secondary neurodevelopmental endpoints: fits with SYNGAP1 high seizure burden
- Multiple methodologies and regulatory-accepted scales
- Regulatory precedents across Dravet, Lennox-Gastaut, CDKL5

Combo – Composite Model (Rett Syndrome/ Angelman)

- Genetic neurodevelopmental disorders with seizures: Rett, Angelman paradigms use one-or-more endpoints to achieve PoC success
- In Rett, trofinetide approved on RSBG + CGI. FDA buy-in for 1 or more of 28 developmental milestones (DM) for Taysha
- In Angelman, Ultragenyx and Ionis: Bayley-4 and either cognition or receptive communication co-primaries

Development path and design to maximize speed and success

Ph 1/2 Optimized for:

- Rapid enrollment
- Efficacy assessment across multiple domains of disease
- Optimal biological dose Selection
- Optionality around endpoint precedent and potential expedited regulatory programs participation

First-in-Human Clinical Trial Design Approach

- Straight to MAD
- Population: enriched genotype and phenotype population
- Aim to start in pediatric patients
- Comprehensive assessments that map to natural history for supportive regulatory comparison
- Open-Label Extension to demonstrate long-term disease-modifying benefit

Potential Cohort Study Design Schematic



CAMP4 team has been pioneering the field of regRNAs



Josh Mandel-Brehm
President & CEO

Biogen polarispartners genzyme



Kelly Gold
Chief Financial Officer

Biogen Deutsche Bank



Dan Tardiff, PhD
Chief Scientific Officer

Pfizer Whitehead Institute Yamanity



Yuri Maricich, MD
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Building momentum and a unique value proposition

- We recently repositioned our pipeline to focus on SYNGAP1 and have funded the program to **accelerate the path to the clinic** as early as H2 2026

- SYNGAP1 represents a **major market opportunity** and is the cornerstone program for CAMP4
 - Future opportunity to build pipeline around additional developmental epileptic encephalopathies and haploinsufficient neurodevelopmental disorders

- CMP-002 poised to enter the clinic as early as second half of 2026, making it the **first potentially disease modifying medicine in the clinic** for SYNGAP1

- Leveraging RAP Platform® to **build CNS-focused pipeline** and drive value through BD
 - CNS is a target rich area for upregulation approach to address rare and prevalent diseases
 - RAP Platform® has been tested in >40 target genes associated with diseases across different tissues, generating opportunities for both pipeline expansion and high-value partnerships
 - Intend to pursue additional discovery partnerships to fully capitalize on platform's potential

Thank You

