

**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): May 14, 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 7.01 Regulation FD Disclosure.

On May 14, 2026, CAMP4 Therapeutics Corporation (the "Company") issued a press releases titled "CAMP4 Therapeutics to Present New Preclinical Data Demonstrating CMP-002 Improves Seizure Threshold and Severity in a Model of SYNGAP1-Related Disorder," a copy of which is furnished as Exhibit 99.1 hereto.

On May 14, 2026, the Company also updated its corporate slide presentation, a copy of which is furnished as Exhibit 99.2 hereto.

The information in this Item 7.01 and Exhibits 99.1 and 99.2 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 9.01 Financial Statements and Exhibits.

(d) Exhibits

Exhibit No.	Description
99.1	Press release issued by CAMP4 Therapeutics Corporation on May 14, 2026.
99.2	Slide presentation dated May 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: May 14, 2026

CAMP4 Therapeutics to Present New Preclinical Data Demonstrating CMP-002 Improves Seizure Threshold and Severity in a Model of SYNGAP1-Related Disorder

CMP-002 administration resulted in a statistically significant improvement in seizure phenotypes and parameters in a SYNGAP1 haploinsufficient mouse model

Results build upon prior preclinical evidence of neurodevelopmental benefit and suggest the potential for broader therapeutic impact

CAMBRIDGE, Mass., May 14, 2026 (GLOBE NEWSWIRE) – CAMP4 Therapeutics Corporation ("CAMP4" or "the Company") (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 the presentation of new preclinical data for CMP-002, the Company's lead investigational antisense oligonucleotide (ASO) therapeutic candidate for SYNGAP1-related disorder (SRD), at the TIDES Oligonucleotide & Peptide Therapeutics conference on May 14, 2026.

The new data demonstrate that CMP-002 administration produced a statistically significant improvement in both seizure threshold and severity of chemically-induced tonic-clonic seizures in mice haploinsufficient for SYNGAP1.

"SYNGAP1-related disorder is characterized by a constellation of neurological symptoms, of which seizures are among the most common, resulting in a devastating burden on patients and their families," said Daniel Tardiff, PhD, Chief Scientific Officer of CAMP4. "Our prior work established that CMP-002 can meaningfully restore motor and behavioral function in preclinical models, and these new seizure data are an important extension of that story. Given this evidence, we believe that by restoring SYNGAP1 protein towards healthy levels, CMP-002 may address a broad range of symptoms that define this disease. We look forward to sharing these findings with the broader oligonucleotide therapeutics community at TIDES."

Because SYNGAP1 haploinsufficient mice do not exhibit spontaneous and readily countable seizures, the study employed a seizure induction model using pentylenetetrazol (PTZ). PTZ is a GABA receptor antagonist that increases excitatory signaling and induces seizures. In the new study, PTZ was administered to induce tonic-clonic seizures one month after administration of CMP-002 to juvenile mice. SYNGAP1 haploinsufficient mice experienced a greater seizure burden compared to wild-type mice, while intervention with a single dose of CMP-002 led to a statistically significant resistance to the onset of tonic-clonic seizures following repeated PTZ administration, as well as a statistically significant decrease in seizure severity.

Together, these data suggest that restoring SYNGAP1 protein toward healthy levels with CMP-002 may improve both the neurodevelopmental and seizure phenotypes that define SRD, supporting the potential of CMP-002 to provide broad disease-modifying benefit.

CAMP4 expects to advance CMP-002 into a Phase 1/2 clinical trial in individuals with SYNGAP1-related disorder in the second half of 2026.

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.

About SYNGAP1-Related Disorder

SYNGAP1-related disorder (also referred to as SYNGAP1) is a rare, haploinsufficient CNS disorder caused by mutations in the SYNGAP1 gene, resulting in approximately 50% of normal SYNGAP protein levels. The condition affects over 10,000 individuals in the United States and is characterized by intellectual disability in 100% of patients, epilepsy in approximately 85%, severe behavioral problems in approximately 70%, sleep problems in approximately 60%, and limited communication, with approximately 30% of patients being non-verbal. There are currently no approved disease-modifying therapies for patients living with SYNGAP1.

About CMP-002

CMP-002 is CAMP4's lead investigational ASO therapeutic candidate designed to bind to a SYNGAP1-specific regRNA to increase SYNGAP1 gene expression and restore SYNGAP protein toward near wild-type levels. Administered intrathecally, CMP-002 has demonstrated dose-dependent increases in SYNGAP protein expression in patient-derived neurons, reversal of disease-relevant behavioral phenotypes in a humanized haploinsufficient mouse model, statistically significant improvement of seizure phenotypes and parameters in a chemically induced seizure mouse model, and broad brain distribution with significant SYNGAP protein upregulation in non-human primates. The Company expects to initiate a global Phase 1/2 clinical trial in SYNGAP1 patients in 2H 2026.

About TIDES Oligonucleotide & Peptide Therapeutics

TIDES is a leading annual conference focused on the development, manufacturing, and clinical advancement of oligonucleotide and peptide therapeutics. The conference convenes scientists, clinicians, and industry leaders to accelerate the translation of these modalities from discovery to the clinic.

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 CMP-002 into a clinical trial and the therapeutic potential of the Company's platform technology and product candidates. 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 uncertainty of preclinical and 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; 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; 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 platform technology and product candidates; 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 and Quarterly Report on Form 10-Q for the quarter ended March 31, 2026, 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.

Contacts

Investor Relations:

Sara Michelmore



Milestone Advisors

sara@milestone-advisorsllc.com

Media:

Sofia Bermudez

LifeSci Communications

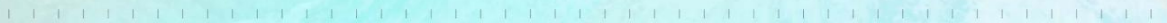
sbermudez@lifescicomms.com



Corporate Overview

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

MAY 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 and quarterly report on Form 10-Q for the quarter ended March 31, 2026. 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.

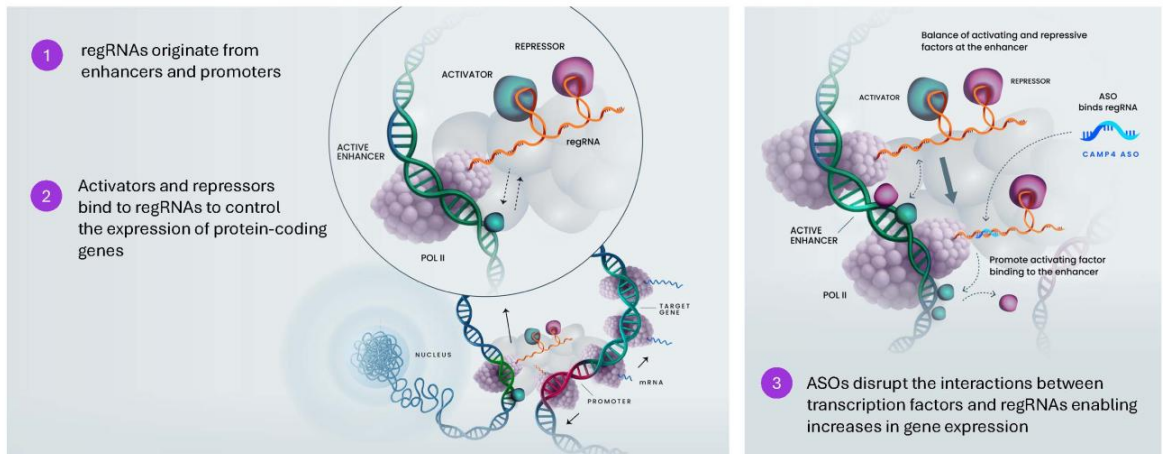


CAMP4: Targeted ASO therapeutics that selectively upregulate gene expression by modulating regulatory RNA

- 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 in the 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

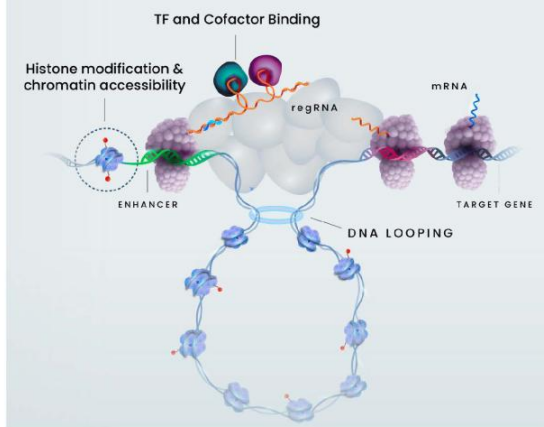
regRNAs play a central role in the regulation of every gene's expression

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

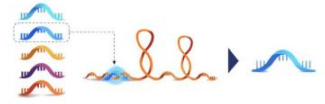


1 Map candidate regRNAs

- 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

CAREGIVER + FAMILY BURDEN

Immense Caregiver Burden



TONY, 8

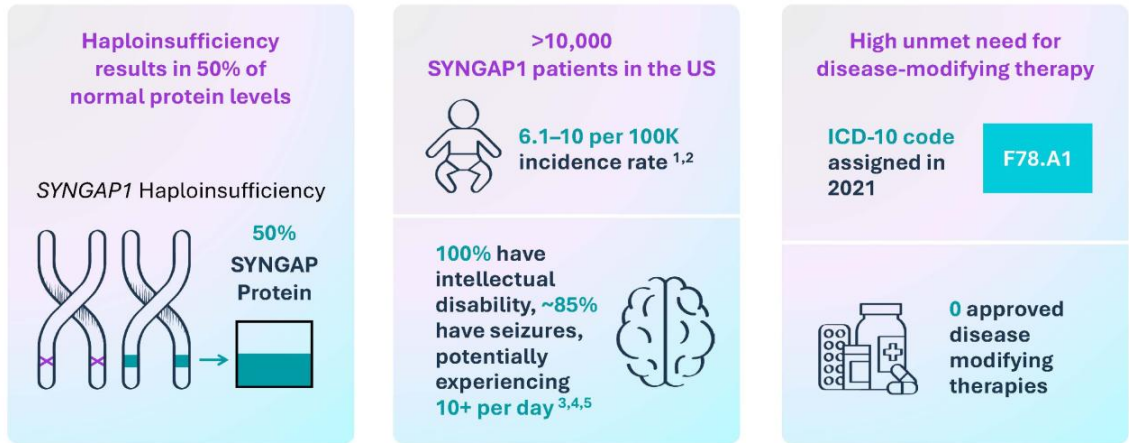
"His spontaneous aggression leads to bruises and scary moments for family members and makes it very challenging to find childcare."

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

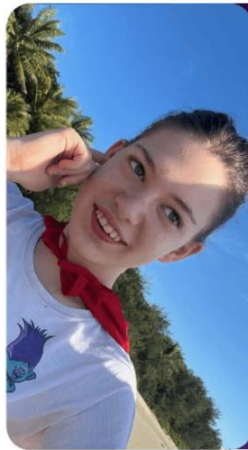
"My life is dedicated to this cause; as a parent, it's the number one thing I strive to do for my sons: alleviate Tony's suffering to help him live the best possible life"

- Tony's Father

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



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

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

Historical ~5 year Diagnostic Journey



Emerging Journey (~1 Year)



Enabled by:

- Rapid expansion of panels including SYNGAP1³
- ACMG now recommends WES / WGS in pediatric pts w DD⁴
- Recent examples from CURE SYNGAP1 of pts diagnosed <1 yr

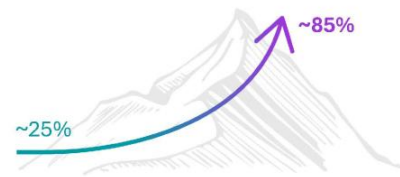
~21K patients across key global geographies (US + EU5); SYNGAP1 remains highly underdiagnosed

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

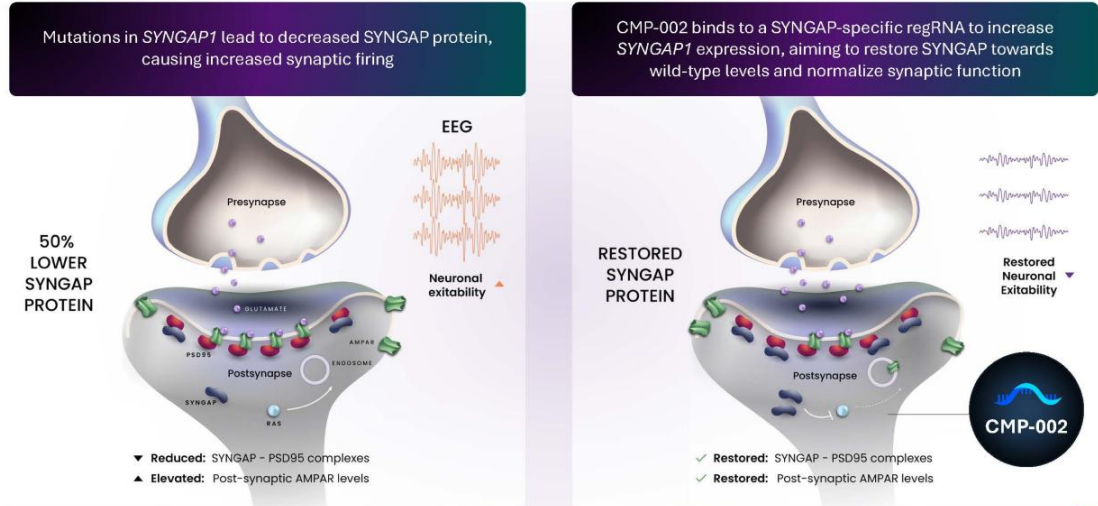
Increasing Diagnosis Rate



- 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}

DEE = Developmental Epileptic Encephalopathies, ID = Intellectual Disability, ASD = Autism Spectrum Disorders
 Sources: Trinity Life Sciences, Komodo claims data (2021-2025), CURE SYNGAP1; ¹ Scaled annual incidence to prevalence using country specific live births and adjusted for mortality estimates; incidence rates based on Lopez Rivera et al. (2020)
² Graglia et al (2025) ³ SYNGAP1 included on gene panels provided by Invitae, Ambry Genetics, Fulgent, GeneDx, Baylor Genetics, Prevention Genetics, Revvity ⁴ Balancour et al. (2013), (5) Sanders et al. (2018)

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

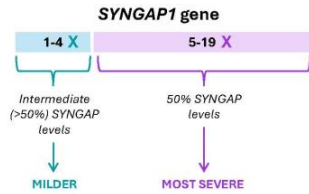


Opportunity in SYNGAP1 driven by unmet need and compelling preclinical data

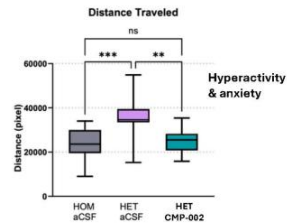
- Ultra-rare SYNGAP1 sub population with intermediate SYNGAP levels equate to milder disease state
- CMP-002 rescued functional defects in relevant human mouse model
- IT administration in NHPs well-tolerated and showed significant increase in SYNGAP in key brain regions

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

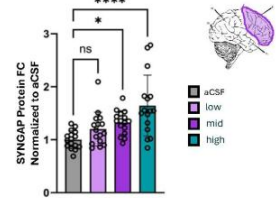
Milder disease severity (verbal responses, milder epilepsy) in minority of patients with intermediate SYNGAP levels



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

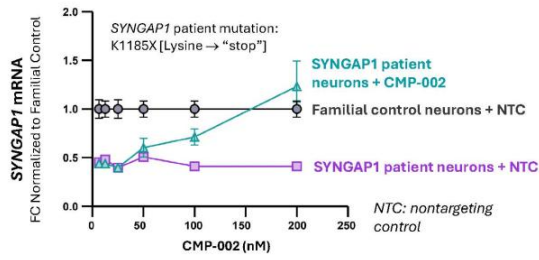


CMP-002 delivered intrathecally increased SYNGAP in disease-relevant brain regions in monkeys

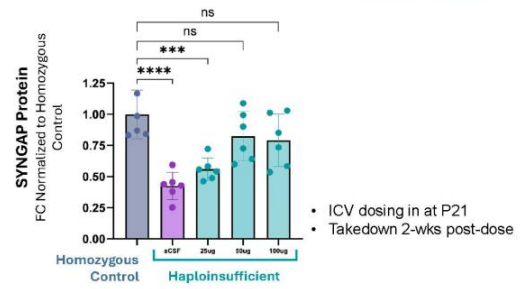


CMP-002 restores SYNGAP levels in models of haploinsufficiency

SYNGAP1 Patient iPSC-derived neurons

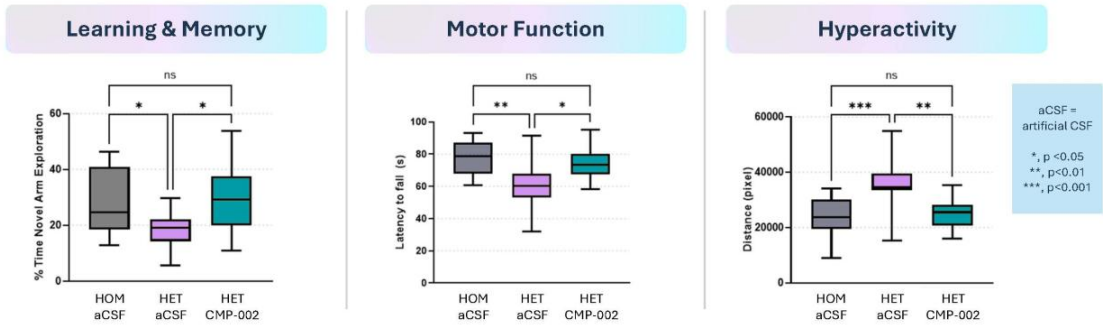


"Humanized" SYNGAP1 mouse



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

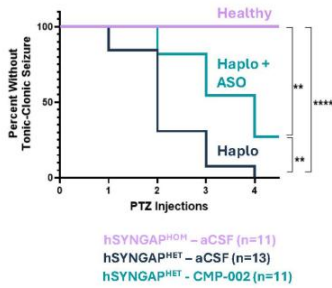
Restoring SYNGAP protein to near wild-type levels ameliorates multiple behavioral phenotypes caused by SYNGAP1 haploinsufficiency

CMP-002 reduces PTZ-induced seizures in SYNGAP1 humanized mouse

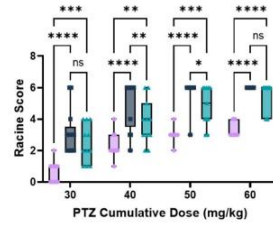
- Chemically-induced seizure model shown to be sensitized by SYNGAP1 haploinsufficiency
- Repeated IP administration of GABA antagonist with monitoring of seizures over 5-minute window
- Score when mice have seizures and their severity



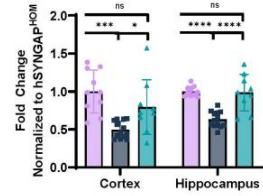
Seizure Threshold



Racine Score

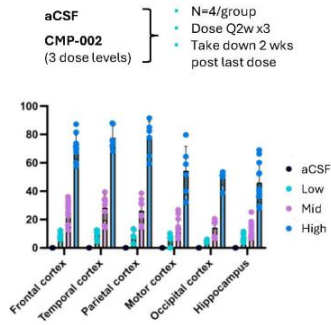


Protein Level

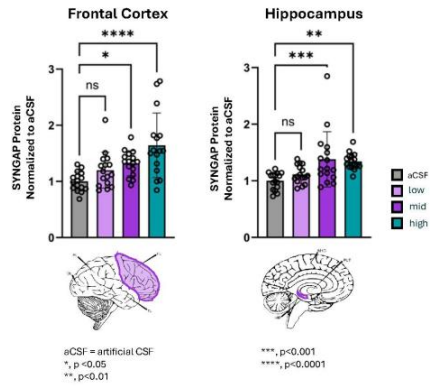


Intrathecal administration of CMP-002 to cynomolgus monkeys achieves broad brain distribution to increase SYNGAP protein levels

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

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">• Collaborating with CURE SYNGAP1 to support ongoing, multi-site study with multiple centers of excellence; 100 patients with 1,240 patient-years of data available
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">• First clinical trial regulatory filing submitted; filings with multiple global regulatory agencies planned throughout 2026• Planning to initiate global Ph1/2 study in patients in H2 2026
Path to Approval	<ul style="list-style-type: none">• Multiple, established paths to approval for a developmental epileptic encephalopathy (DEE)• Optionality on endpoints with regulatory approval precedent by regulators

Phase 1/2 study will assess key domains of SYNGAP1, utilizing validated measures to demonstrate PoC in First-in-Human

Phase 1/2 endpoint categories



Development path and design to maximize speed and success

Ph 1/2 key features:

- Global study for rapid enrollment
- Straight to MAD
- Efficacy assessments across all domains of disease
- Identify optimal biological dose selection
- Drive optionality for potential expedited regulatory programs participation

First-in-Human clinical trial design approach:

- Aim to start in pediatric patients
- Patient cohort study design: Screening → Baseline → Treatment period → Follow-up Period → Open Label Extension
- Endpoints mapped to natural history for additional control
- Key inclusion criteria: Enriched genotype representing majority of population (haploinsufficient); seizures, impaired sleep, inability to say phrases
- Open-Label Extension to demonstrate long-term disease-modifying benefit

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 Vumc



Yuri Maricich, MD
Chief Medical Officer

Corixa Cuvion PEAR



Caleb Moore
Chief Business
Operations Officer

genzyme ACCELERON CUBIST



Michelle Gates
Chief People Officer

Akamai



Alla Sigova, PhD
SVP, Head of Platform

SAIL Whitehead Institute



Satya Kuchimanchi, PhD
SVP, Technical Operations

TRIPLET Alynham

Board of Directors

Michael Higgins

Steven Holtzman

Josh Mandel-Brehm

Michael McLean

Amir Nashat, ScD

Andy Schwab

Murray Stewart, DM FRCP

Douglas Williams, PhD*

Rick Young, PhD

* Board Chair



Building momentum and a unique value proposition

- CAMP4 is advancing the first potentially disease modifying therapy for SYNGAP1 into a **Phase 1 / 2 clinical study in 2H '26** and well positioned to deliver **long-term pipeline value**

- 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

- 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

