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): September 28, 2026

CAMP4 THERAPEUTICS CORPORATION

(Exact name of registrant as specified in its charter)

Delaware	**001-42365**	**81-1152476**
(State or other jurisdiction of incorporation)	**(Commission File Number)**	**(IRS Employer Identification No.)**

100 Talcott Avenue **Suite 201** **Watertown, MA**	**02472**
(Address of principal executive offices)	**(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 September 28, 2026, CAMP4 Therapeutics Corporation (the "Company") held an Analyst Day meeting regarding its planned Phase 1/2 ASCEND clinical trial of CMP-002 in SYNGAP1-related disorder (the "ASCEND clinical trial") and its pipeline of product candidates. A copy of the data presentation used in connection with the Analyst Day meeting is furnished as Exhibit 99.1 to this Current Report on Form 8-K.

In addition, on September 28, 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 report is furnished pursuant to Item 7.01, including Exhibits 99.1 and 99.2 attached hereto, 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 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 filing.

Item 8.01 Other Events.

On September 28, 2026, the Company held an Analyst Day meeting at which it provided the following updates regarding the ASCEND clinical trial and the Company's pipeline of product candidates.

ASCEND Clinical Trial

The ASCEND clinical trial is a Phase 1/2, randomized, double-blind, placebo-controlled, multiple ascending dose ("MAD") study. Participants will be randomized 3:1 to receive intrathecal administration of CMP-002 or a sham procedure consisting of a pinprick. The clinical trial includes three MAD cohorts with a minimum of eight participants each, followed by an open-label extension study. The Company believes that the dose levels to be administered in each of the three cohorts are within the expected therapeutic range based on pharmacokinetic and pharmacodynamic modeling of its preclinical data. The primary goals of the clinical trial include demonstrating safety and tolerability, characterizing pharmacokinetics and selecting an optimal biological dose, and identifying relevant endpoints applicable to patients with SYNGAP-1-related disorder ("SRD") for subsequent study of CMP-002.

The ASCEND clinical trial is designed to enroll participants aged 2 to less than 18 years with genetically confirmed SRD. Key inclusion criteria include a clinical diagnosis of SRD with genetic confirmation of a protein-truncating mutation in SYNGAP1 exons 5-19, a haploinsufficiency phenotype characterized by intellectual disability, inability to speak in full phrases, sleep disturbance, refractory epilepsy (defined as daily seizures despite at least one ongoing anti-seizure medication) and a prior trial of at least two anti-seizure medications, and a stable anti-seizure medication regimen prior to baseline. Key exclusion criteria include non-truncating SYNGAP1 variants (including missense variants, microdeletions, variants located in exons 1-4 or variants of uncertain significance), a history of central nervous system disease unrelated to SRD, spinal abnormalities or other conditions that would make intrathecal dosing unsafe, and clinically significant laboratory abnormalities.

The ASCEND clinical trial is designed to include a 28-day screening period, a baseline assessment period of at least four weeks, a treatment period during which three doses will be administered over approximately three months, and a post-treatment follow-up period with assessments at multiple timepoints over approximately six months. Total participant time in the clinical trial is expected to be approximately 10 months. The clinical trial includes daily seizure diary completion,

actigraphy monitoring, safety and side effect review and functional assessments across multiple domains throughout the clinical trial.

The ASCEND clinical trial will also assess exploratory endpoints across six key domains of SYNGAP1-related disorder: seizures, sleep, motor function and gait, communication, behavior, and development and cognition. Seizure and sleep assessments will include 24-hour video-electroencephalography ("EEG"), seizure diaries, the Seizure-Related Impact Assessment Scale, actigraphy, and the Children's Sleep Habits Questionnaire. The clinical trial will utilize five validated scales to assess behavior, development, communication, and motor function: the Bayley Scales of Infant and Toddler Development, 4th Edition; the Vineland Adaptive Behavior Scales, 3rd Edition; the Aberrant Behavior Checklist, 2nd Edition; the Observer-Reported Communication Ability Measure; and the Gross Motor Function Measure. EEG-based measures will be utilized to assess multiple parameters.

The ASCEND clinical trial is planned to be conducted at more than 11 clinical trial sites across at least nine countries. Sites in the United Kingdom, Australia and Argentina have been approved by the relevant regulatory authorities. Sites in the European Union are under review by applicable regulatory and ethics authorities.

The Company expects to dose the first patient in the ASCEND clinical trial by the end of 2026. The Company is targeting a topline data readout from the ASCEND clinical trial in the first half of 2028.

Pipeline Updates

In addition, the Company provided an update on its broader pipeline of product candidates. The Company is advancing a preclinical program targeting SHANK3 for the treatment of Phelan-McDermid Syndrome, a neurodevelopmental disorder characterized by autism, developmental delay, intellectual disability, motor impairment and milestone regression, for which there are no approved disease-modifying treatments. The Company estimates that there are more than 45,000 patients with Phelan-McDermid Syndrome in the United States. The Company expects to designate a development candidate in the SHANK3 program in 2027. The Company also disclosed that it is evaluating programs for two additional early-stage haploinsufficient diseases of the central nervous system.

Forward-Looking Statements

This Current Report on Form 8-K contains forward-looking statements. All statements contained in this Current Report on Form 8-K that do not relate to matters of historical fact should be considered forward-looking statements, including, without limitation, statements regarding the Company's strategy, goals, business plans and focus; preclinical data, clinical development plans and the anticipated timing of availability of clinical data and results from the Company's planned ASCEND clinical trial; the Company's regulatory interactions with the FDA; the Company's expectations regarding the therapeutic potential of the Company's product candidates; the Company's estimates with respect to the addressable patient populations for its product candidates; the potential of the Company's platform technology; and the Company's expectations regarding its business prospects and results of operations. In some cases, you can identify forward-looking statements by terms such as "aim," "anticipate," "approach," "believe," "contemplate," "could," "designed", "estimate," "expect," "goal," "intend," "look," "may," "mission," "plan," "possible," "potential," "predict," "project," "pursue," "should,", "strive", "target," "will," "would," or the negative thereof and similar words and expressions. Forward-looking statements are based on management's current expectations, beliefs and assumptions and on information currently available to the Company. Such statements are neither promises nor guarantees, and involve a number of known and unknown risks, uncertainties and assumptions that may cause the Company's actual results, performance or achievements to be materially different from any expressed or implied by the forward-looking statements. Such risks and uncertainties include, but are 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 and risks related to additional

costs or delays in the development and commercialization of the Company's current 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; the Company's ability to realize the benefits of the Company's current or future collaborations or licensing arrangements; the Company's ability to obtain regulatory approval to commercialize its product candidates; 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; and the Company's reliance on third parties to conduct the Company's preclinical studies and clinical trials, as was the risks detailed under the heading "Risk Factors" included in the Company's Annual Report on Form 10-K for the fiscal year ended December 31, 2025 and in the Company's other filings with the U.S. Securities and Exchange Commission. The forward-looking statements in this Current Report on Form 8-K speak only as of the date of this Current Report on Form 8-K, and the Company undertakes no obligation to update or revise any of the statements. The Company's business is subject to substantial risks and uncertainties, including those referenced above. Investors, potential investors, and others should give careful consideration to these risks and uncertainties.

Item 9.01 Financial Statements and Exhibits.

(d) Exhibits

Exhibit No.	Description
99.1	Analyst Day presentation, dated September 28, 2026.
99.2	Corporate presentation, dated September 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: September 28, 2026


CAMP4
CAMP4 Analyst Event
September 28, 2026

Forward-Looking Statements

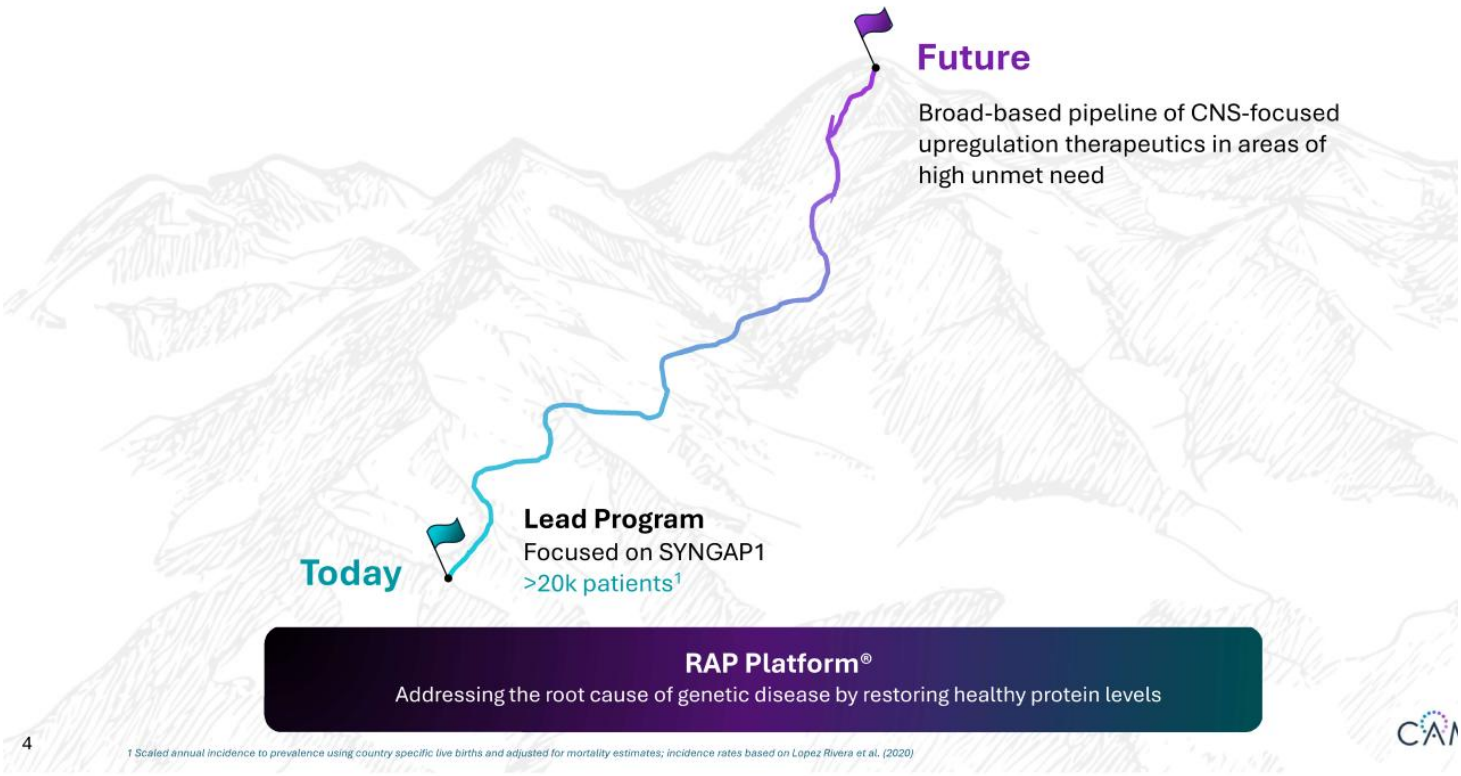
This presentation contains forward-looking statements that are based on the beliefs and assumptions and on information currently available to CAMP4's management. All statements other than statements of historical fact contained in this presentation are forward-looking statements. Forward-looking statements include information concerning the company, corporate strategy and operational and development plans; plans and expectations regarding the design of, and potential endpoints for, the company's clinical trials; the timing, progress and results of preclinical and clinical trials of CAMP4's product candidates; the timing or outcome of regulatory interactions, filings and approvals for any of its product candidates; expectations regarding the clinical and therapeutic potential of CAMP4's product candidates; estimates with respect to addressable patient populations and markets for the company's 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 June 30, 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.

Agenda

- Welcome & Opening remarks | Josh Mandel-Brehm, CEO
- Patient story: hearing from a SYNGAP1 parent | Beata Tarasiuk, CURE SYNGAP1 State Ambassador (AZ)
- ASCEND Phase 1/2 clinical trial | Dr. Dan Tardiff, CSO, and Dr. Yuri Maricich, CMO
- Patient advocacy | Mike Graglia, Co-Founder and Managing Director, CURE SYNGAP1
- KOL Fireside chat | Dr. Yuri Maricich & Dr. Stéphane Auvin, Epileptologist & Pediatric Neurologist
- Emerging pipeline and closing remarks | Josh Mandel-Brehm
- Analyst Q&A

Solving genetic diseases of the brain through upregulation



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)

Video: Patient story

ASCEND clinical trial in SYNGAP1

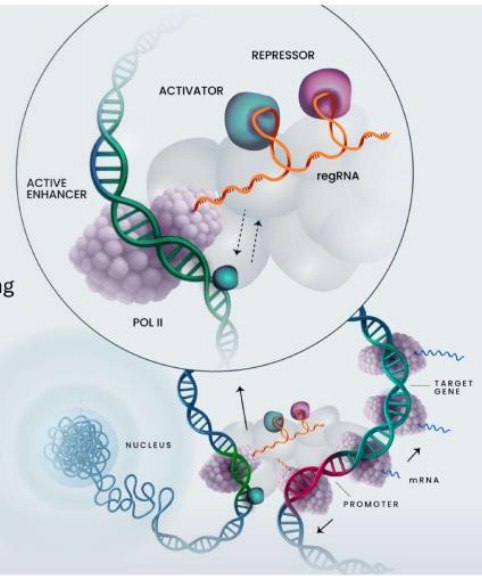
- **A**SO therapy for **S**YNGAP1 **C**linical **E**valuation in **N**euro **D**evelopment

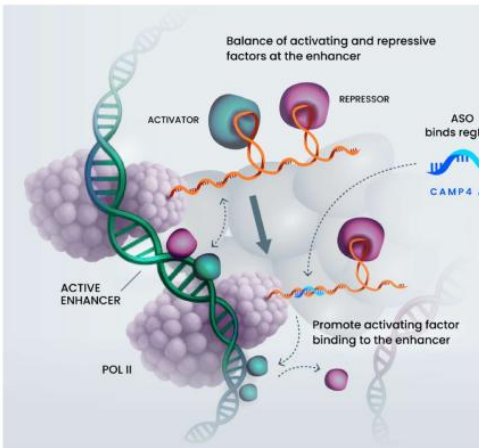
regRNAs play a central role in the regulation of every gene's expression

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

1 regRNAs originate from enhancers and promoters

2 Activators and repressors bind to regRNAs to control the expression of protein-coding genes

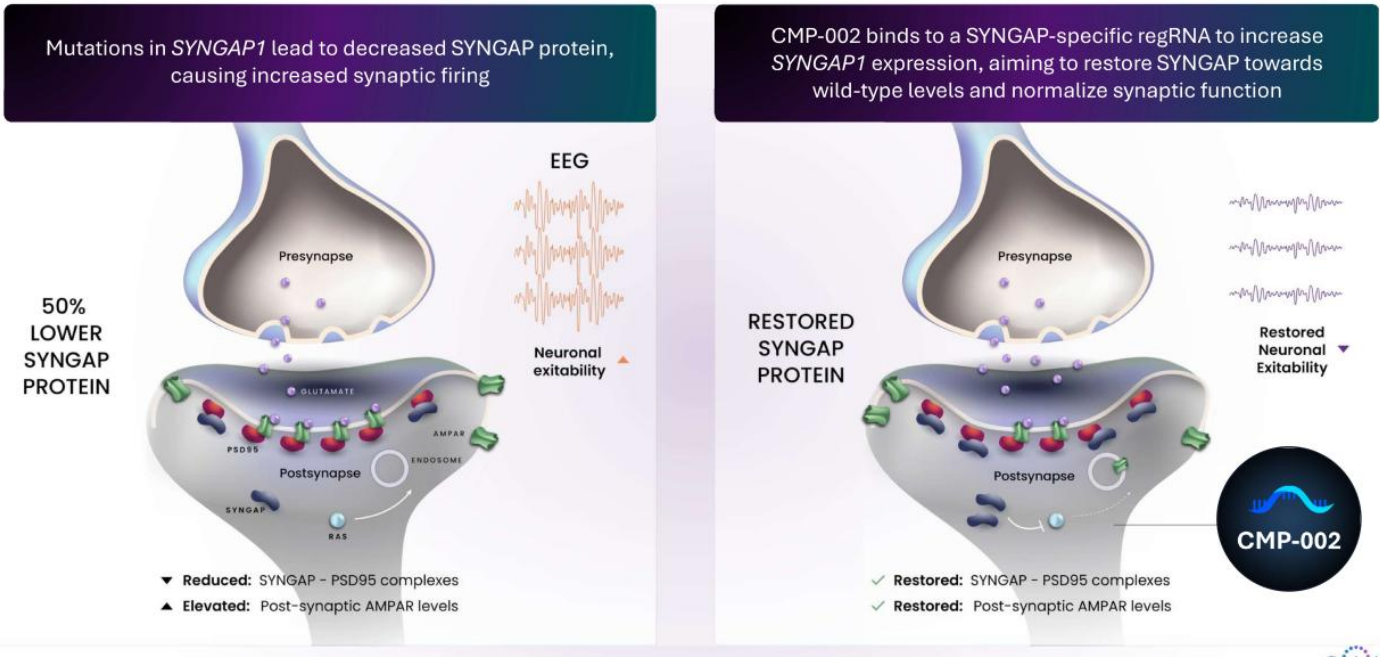




3 ASOs disrupt the interactions between transcription factors and regRNAs enabli increases in gene expression

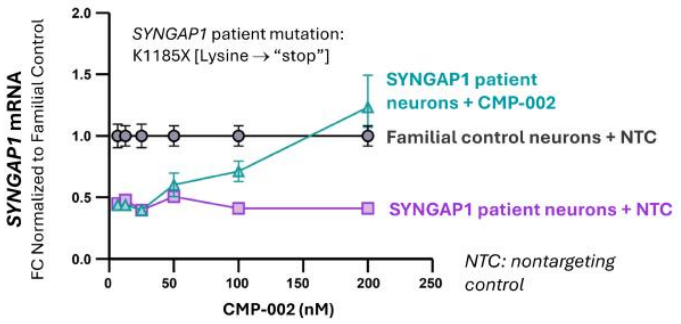
Source: Sharp et al. (2022) RNA 28: 52-57; Henninger et al. (2021) Cell 184:207-225; Sartorelli & Lauberth (2020) Nat. Struct. Mol. Biol. 27: 521-8; Oksuz et al. (2023) Molecular Cell 83: 2449-63.

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

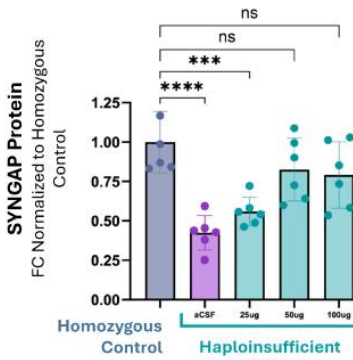


CMP-002 restored SYNGAP levels in models of haploinsufficiency

SYNGAP1 Patient iPSC-derived neurons



"Humanized" *SYNGAP1* mouse



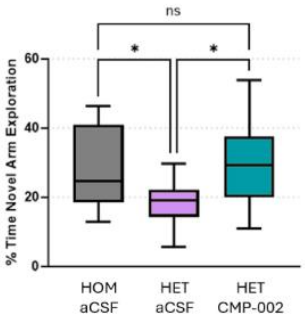
- ICV dosing in at P21
- Takedown 2-wks post-dos

Dose-dependent increase in SYNGAP approaching wild-type levels → both ASO activity and r translated from human patient-derived neurons in a dish to neurons in an intact anir

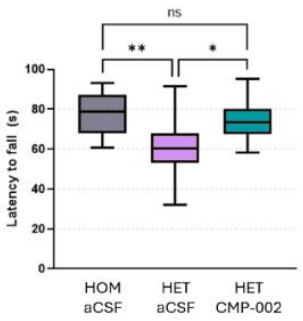
(SYNGAP-ZBTB9)[1]Bpro/Mmjax mouse strain obtained from the Mutant Mouse Resource and Research Centers (originally deposited by Benjamin Prosser, Ph.D., University of Pennsylvania).

CMP-002 broadly improved neurological phenotypes in humanized SYNGAP1 mice

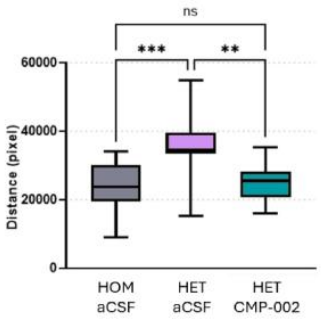
Learning & Memory



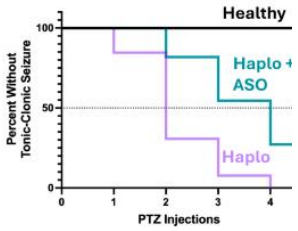
Motor Function



Hyperactivity



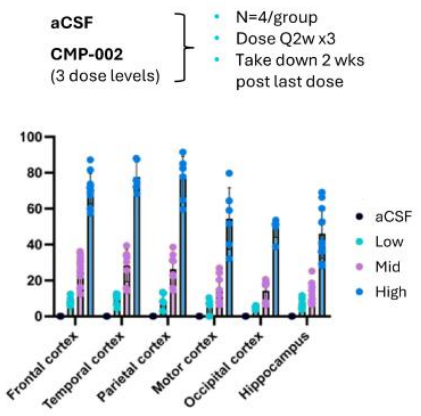
Seizures



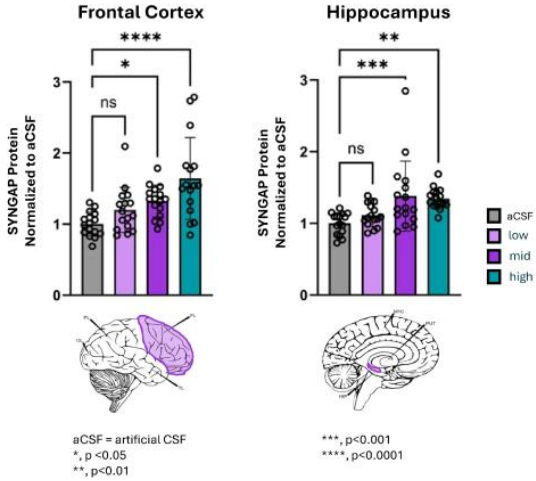
Broad benefit of CMP-002 supports potential translation across multiple p SYNGAP1 patients

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

ASO Concentrations



SYNGAP Protein Levels



aCSF = artificial CSF
*, p <0.05
**, p<0.01

***, p<0.001
****, p<0.0001

Summary

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

Broad ASO distribution throughout disease-relevant brain regions

↑ SYNGAP protein throughout brain

SYNGAP1 is a devastating disease with significant unmet need for a targeted disease modifying therapy

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 words*** [4]

No Approved Therapy

Non-specific treatments have limited impact on SYNGAP1 symptoms
- Anti-seizure medications
- Cannabinoids
- Sleep medications

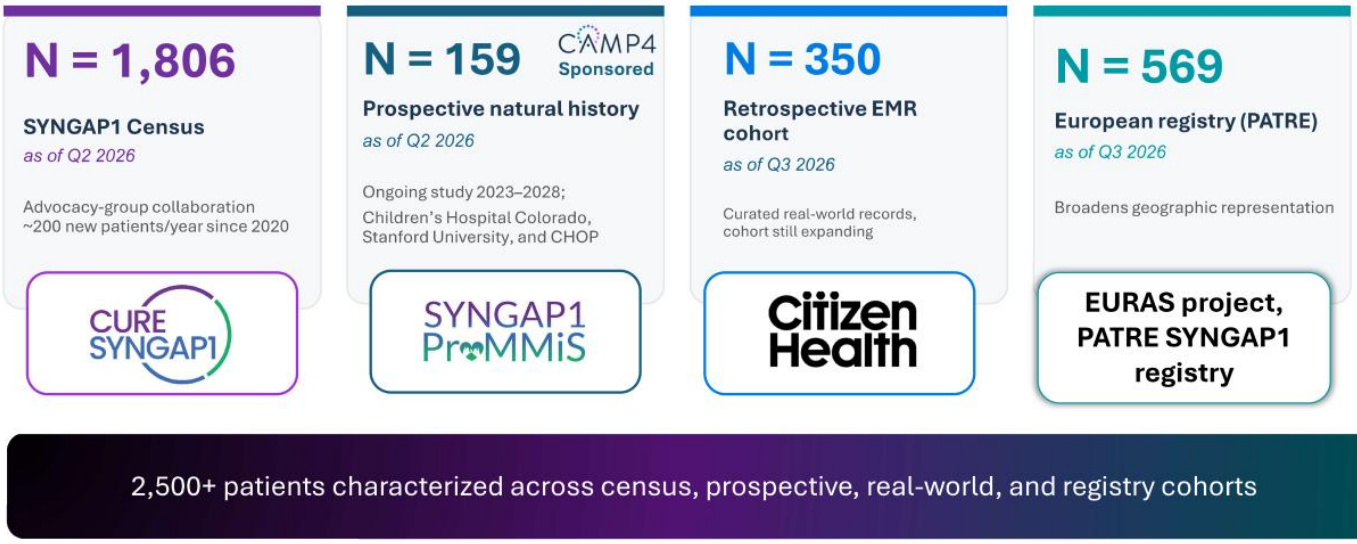
Polypharmacy is common – *Patient regimen* [6] *example:*
- *Epidiolex*
- *Ravicti*
- *Sodium bicarb*
- *Amantadine*

Constant patient care needed
- Caregivers vigilant at all times
- Significant lifelong cost of care

JAELI, 16

[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] *Vlaskamp et al. Neurology, 2019;* [6] *CURE SYNGAP1*

Natural history studies and registries inform ASCEND clinical trial



Sources: SYNGAP1 Census Q2 2026 (curesyngap1.org); McKee et al.,2026; PATRE Q3 2026 (euras-project.eu).

Majority of SYNGAP1 patients have severe protein-truncating varia

Natural history studies have replicated highest burden of disease across multiple domains in PTV 5-19 genotype

ASCEND

Ph1/2 Clinical Trial target population

MILDER

Intermediate (>50%) SYNGAP protein levels

- Verbal responses
- Milder epilepsy in some patients

MOST SEVERE

True haploinsufficiency: 50% SYNGAP protein levels

- most homogeneous
- highest seizure burden

Sources: Graglia et al, 2025: Variant types across 195 SRD patients; Vlaskamp et al, 2019: Table 1: SYNGAP1 Phenotypes in Patients with SYNGAP1 mutations and microdeletions.

SYNGAP1-related disorder impacts many aspects of neurological function

Phenotypic features of SYNGAP1-Related Disorder by prevalence

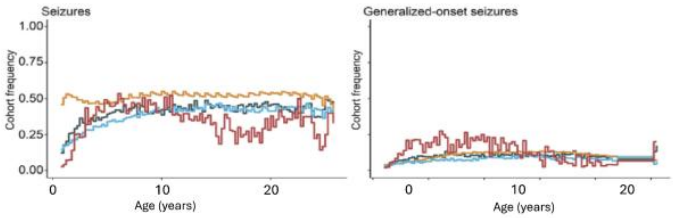
Phenotypic feature	Prevalence
Intellectual disability	100%
Epilepsy	84%
Autism	68%
Behavior	68%
Sleeping	61%
Feeding problems	47%
Gait	33%
Anxiety	24%
Sensory	22%
Ataxia	21%
Patients, n=147	

All patients display ID, majority with epilepsy, behavioral/autistic features and sleep problems

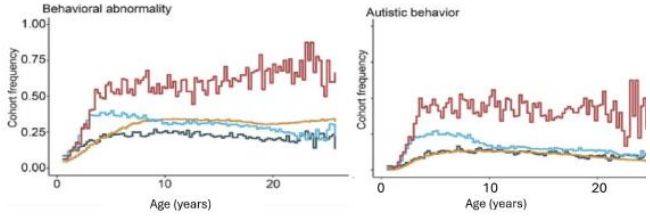
Source: Wiltrout et al., 2024

SYNGAP1 has similar or higher severity relative to analogue conditio

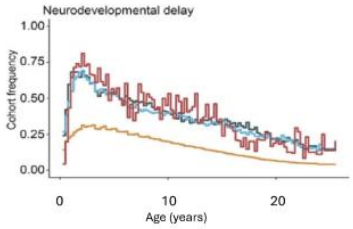
Seizures among highest frequency



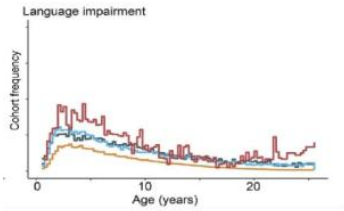
Difficult to manage autism-like behavior



Intellectual disability: neuro-arrest / developmental plateau (avg cognitive age of 2)



Communication impairment: many patients are non verbal or have few words

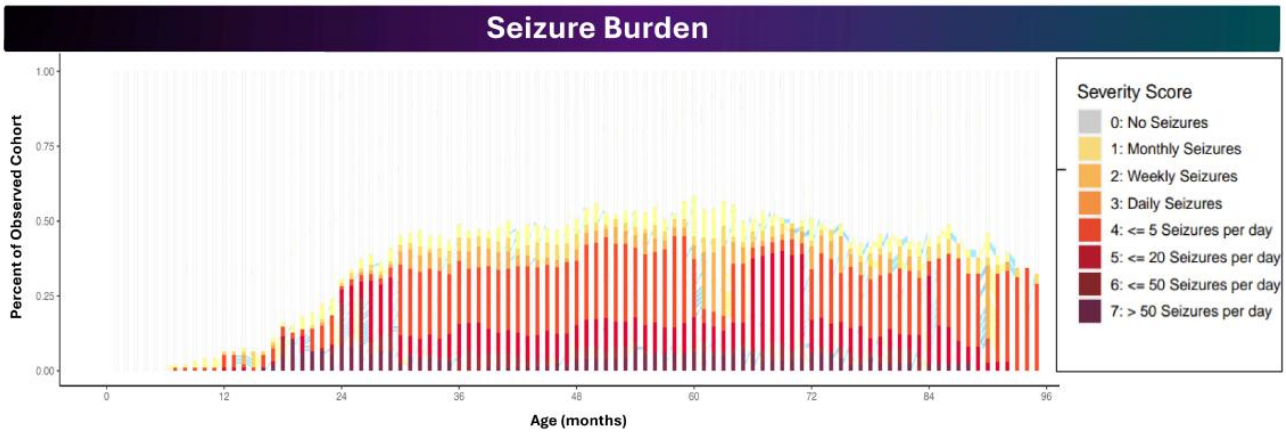


SYNGAP1 Epilepsy (G40+) Rett Syndrome Angelman Syndrome

Source: McKee et al., 2025

Majority of patients have epilepsy with high frequency of seizures

One of highest seizure burdens among genetic epilepsies, consisting of multiple types



- 85% of patients receive epilepsy diagnosis
- Compared to other DEEs, SYNGAP has some of the highest frequency of seizures
- Genotype of focus (PTV 5-19) have highest rates of daily seizures among SYNGAP1 patients
- Multiple seizure types – with non-motor seizures being common (ex. absence seizures)
- Seizure type can vary by age
- SYNGAP1-specific EEG signatures include changes in alpha-theta wave

Sources: Vlaskamp et al, 2019; Galer et al., 2025; McKee et al., 2026 (reproduced with author permission).

ASCEND clinical trial designed to assess safety & tolerability across key domains of SYNGAP1 using validated measures

CLINICAL TRIAL GOALS

- Demonstrate safety and tolerability
- Characterize pharmacokinetics and select optimal biological dose
- Identify relevant endpoints applicable to SYNGAP1 patients to chart path for regulatory approval

ASCEND exploratory endpoints assessed

Seizure | Sleep | Motor & Gait | Communication | Behavior | Development & Cognition

ASCEND Phase 1/2 Clinical Trial Summary

Overview	
Design	• Phase 1/2, randomized, double-blind, controlled, multiple ascending dose study • 3:1 randomization • IT administration or a sham pinprick • Open-Label Extension
Study population	• Minimum 8 participants per cohort • Participants ≥2 to <18 years of age with genetically confirmed SYNGAP1-Related Disorder (SRD) • Required daily seizures, impaired sleep, and inability to speak in phrases
Study periods	• Screening and baseline period • Study treatment period with 3 doses • Post-treatment follow-up
Study length	• ~10 months per participant

ASCEND eligibility criteria

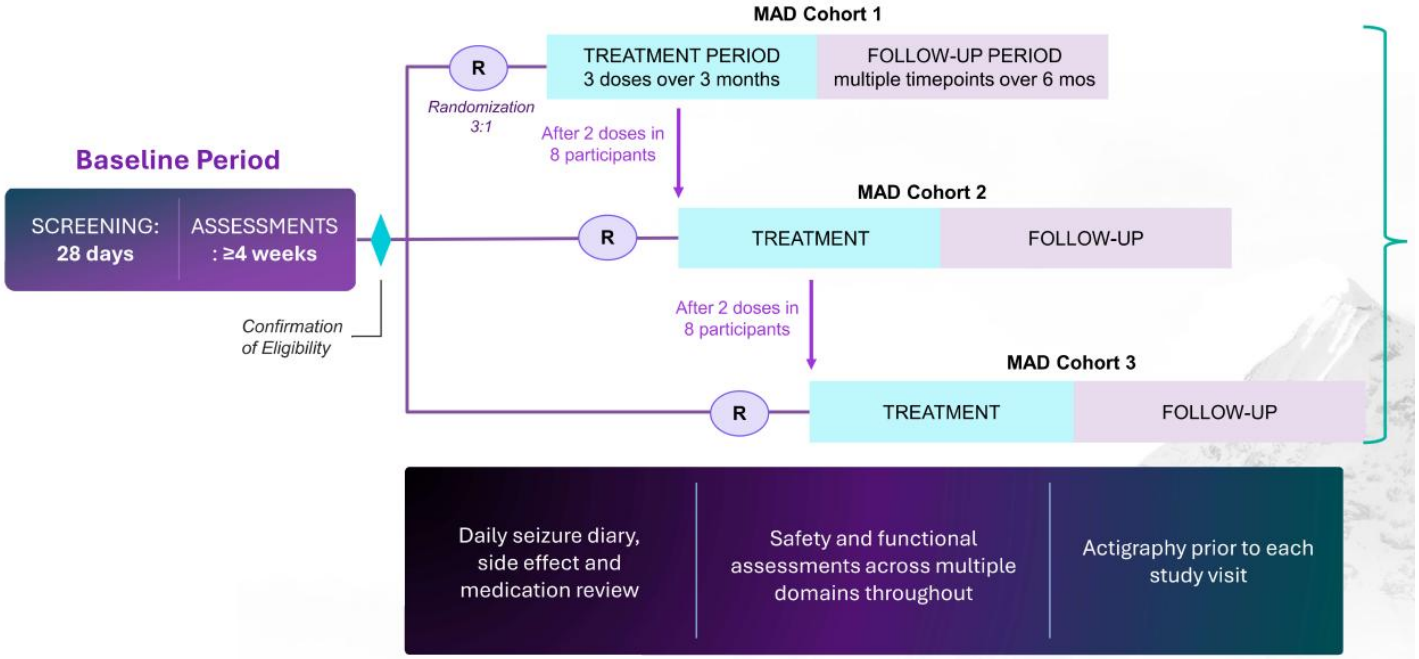
Key Inclusion Criteria

- **Age 2 to <18 years**
  Male or female participants
- **Genetically confirmed SRD**
  Clinical diagnosis of SRD with genetic confirmation of a protein-truncating mutation in SYNGAP1 exons 5–19
- **Haploinsufficiency phenotype**
  Intellectual disability, inability to speak in full phrases, and sleep disturbance consistent with haploinsufficiency
- **Refractory epilepsy**
  Daily seizures despite ≥1 ongoing anti-seizure medication and prior trial of ≥2 ASMs
- **Stable ASM regimen at baseline**
  Stable anti-seizure medication regimen prior to baseline

Key Exclusion Criteria

- **Non-truncating variants**
  SYNGAP1 variants that either are non-truncating (missense, microdeletions), located in exons 1–4, or of uncertain significance
- **Other CNS disease**
  History of central nervous system disease unrelated to SRD
- **Unsafe for intrathecal dosing**
  Spinal abnormalities, implanted CSF shunts, or intolerance to lumbar puncture
- **Clinically significant lab abnormalities**

ASCEND clinical trial schema



Five validated scales being utilized in ASCEND: Behavior, development, communication, and motor function

Scale	Communication	Motor	Behavior	Development
Bayley Scales of Infant and Toddler Development Bayley-4		✓	✓	✓
Vineland Adaptive Behavior Scales, 3rd Edition Vineland-3	✓	✓	✓	✓
Aberrant Behavior Checklist, 2nd Edition ABC-2	✓		✓	
Observer-Reported Communication Ability Measure ORCA	✓			
Gross Motor Function Measure GMFM		✓		

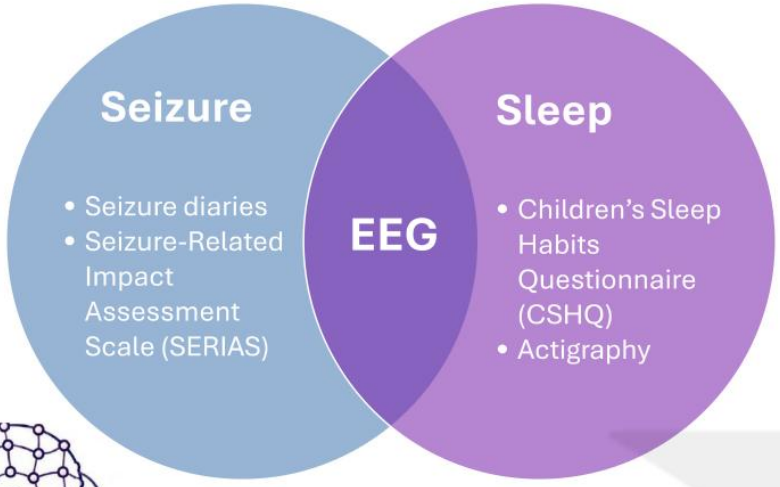
Leveraging validated scales used in DEE clinical trials and ProMMiS Natural History Study

✓ Scale to be utilized to assess domain in ASCEND trial

Assessing seizure and sleep: multiple methods to measure change, with vEEG common to both domains

24hr video-EEG for seizure and sleep detection:

- REM, SOL, WASO, TST
- EEG informs both seizure and sleep endpoints



EEG measures multiple parameters

- **Clinical signatures** *spikes, seizures, slowing*
- **Spectral features** *alpha-theta wave*
- **Spatial / topographic measures**
- **Event-related potential** *VEP / AEP*
- **Sleep / state-based measures**
- **Functional connectivity**
- **Temporal / nonlinear dynamics** *entropy, temporal correlations*

Planned global footprint of ASCEND Phase 1/2 clinical trial

11+ Total sites

9 Countries

5 Regions with sites planned

4 approved: UK, ARG, AUS

7 EU sites under review

CAMP4 is pioneering SYNGAP1 development with clearly defined Phase 1/2 study goals

- **Severe, life-long disease** with consistent **natural history data** across multiple studies to guide development
- Partnering with leading experts and patient community
- Signal-seeking across all domains of disease using validated measures
- Expect first patient dosed by year end. Topline read-out targeted first-half of 2028

Video: Co-Founder and Managing Director, CURE SYNGAP1

KOL fireside chat

Emerging pipeline & closing remarks

Early pipeline focused on haploinsufficient neurodevelopmental disorders to expand on expertise gained through SYNGAP1 program

Program	Indication	Target	Discovery & Preclinical Development	Phase 1/2	Anticipated Milestone	Commercial Rights
CNS DISEASES						
CMP-002	SYNGAP1-related disorder	*SYNGAP1*			Clinical initiation in Q4 2026. Topline data 1H 2028.	CAMP4
SHANK3	Phelan-McDermid Syndrome	*SHANK3*			Development Candidate in 2027.	CAMP4
CNS Program 1	CNS haploinsufficiency					CAMP4
CNS Program 2	CNS haploinsufficiency					CAMP4

SHANK3 – Phelan-McDermid Syndrome

- Haploinsufficiency due to a mutation in SHANK3, a central scaffolding protein in the postsynapse
- Clear unmet need with no approved treatments
- Autism, developmental delay, intellectual disability, motor impairment, milestone regression
- 45,000+ patients in the US[1] with a well-organized and sophisticated patient advocacy group
- Overlapping KOLs & physician network with SYNGAP1

Source: [1]Levy et al., Autism Research (2026)

Analyst Q&A


CAMP4
Corporate Overview
Pioneering a new class of RNA medicines to increase targeted gene expression.
SEPTEMBER 2

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 company, corporate strategy and operational and development plans; plans and expectations regarding the design of, and potential endpoints for, the company's clinical trials; the timing, progress and results of preclinical and clinical trials of CAMP4's product candidates; the timing or outcome of regulatory interactions, filings and approvals for any of its product candidates; expectations regarding the clinical and therapeutic potential of CAMP4's product candidates; estimates with respect to addressable patient populations and markets for the company's 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 June 30, 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

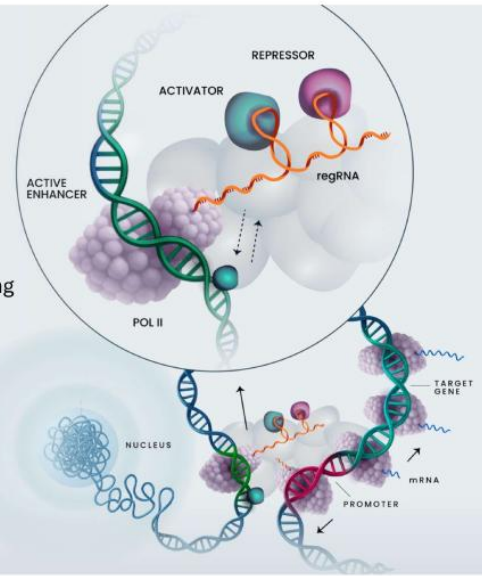
- Developing a targeted disease modifying therapy to address dire unmet need in SYNGAP1-related disorder
  - SYNGAP1 is a haploinsufficient CNS disorder, and an optimal target for CAMP4
  - CMP-002 is designed to increase SYNGAP protein levels, restore *SYNGAP1* function and improve disease symptoms
  - >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
  - Proof of concept data in humanized mice showed reversal of disease phenotype; significant protein upregulation and broad ASO distribution in primates
  - CTA approvals received in Australia, Argentina and the United Kingdom
  - Expect to dose first subject in ASCEND global Ph 1/2 clinical trial of CMP-002 in patients in the fourth quarter of 2026. Topline read-out targeted first half of 2028
- 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
  - Early pipeline focused on haploinsufficient neurodevelopmental disorders to expand on expertise gained through SYNGAP1 program. Discovery program targeting SHANK3 for the treatment of Phelan-McDermid Syndrome; DC targeted 2027
  - Strategic discovery partnership with GSK unlocks platform value beyond CNS and validates CAMP4's novel approach to gene upregulation

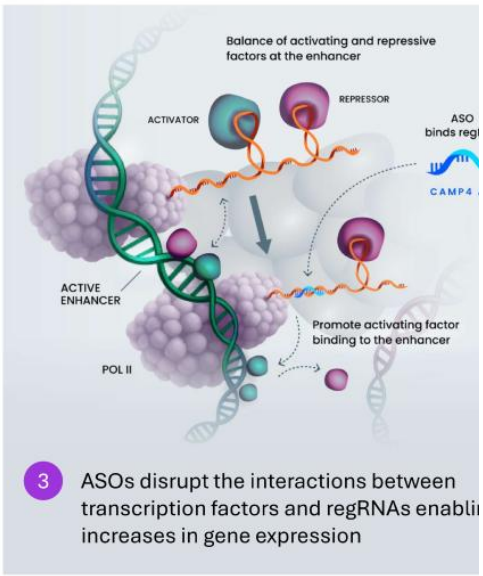
regRNAs play a central role in the regulation of every gene's expression

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

1 regRNAs originate from enhancers and promoters

2 Activators and repressors bind to regRNAs to control the expression of protein-coding genes



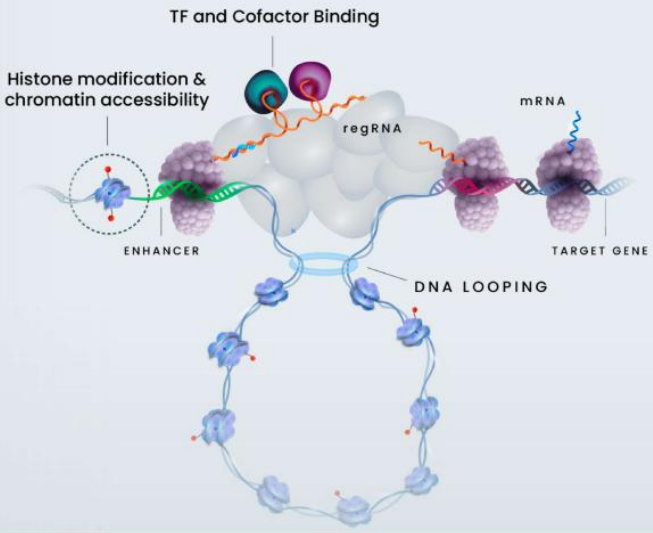


3 ASOs disrupt the interactions between transcription factors and regRNAs enabli increases in gene expression

Source: Sharp et al. (2022) RNA 28: 52-57; Henninger et al. (2021) Cell 184:207-225; Sartorelli & Lauberth (2020) Nat. Struct. Mol. Biol. 27: 521-8; Oksuz et al. (2023) Molecular Cell 83: 2449-63.

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

Genome-wide analyses of chromatin & RNA

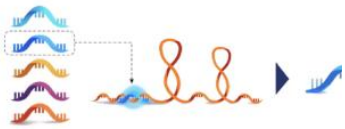


1 Map candidate regRNAs

- Generate large scale genomic datasets for cells & tissues
- Deploy proprietary ML/AI platform to identify regulatory regior
- 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 Milestone	Commercial Rights
CNS DISEASES							
CMP-002	SYNGAP1-related disorder	*SYNGAP1*				Clinical initiation in Q4 2026. Topline data 1H 2028.	CAMP4
SHANK3	Phelan-McDermid Syndrome	*SHANK3*				Development Candidate in 2027.	CAMP4
CNS Program 1	CNS haploinsufficiency						CAMP4
CNS Program 2	CNS haploinsufficiency						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 diseases 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,

"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"

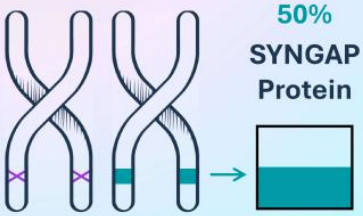
- Tony's Father

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

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

Haploinsufficiency results in 50% of normal protein levels

SYNGAP1 Haploinsufficiency



>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

[1] López-Rivera et al., Brain, 2020; [2] Marotta et al., Curr Probl Pediatr Adolesc Health Care, 2024; [3] Holder et al., GeneReviews, 2019; [4] SYNGAP-Related Epilepsy, Epilepsy Foundation (Accessed May 2025); [5] Vlaskamp et al. Neurology, 2019

SYNGAP1 is a devastating disease with significant unmet need for a targeted disease modifying therapy

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 words*** [4]

No Approved Therapy

Non-specific treatments have limited impact on SYNGAP1 symptoms
- Anti-seizure medications
- Cannabinoids
- Sleep medications

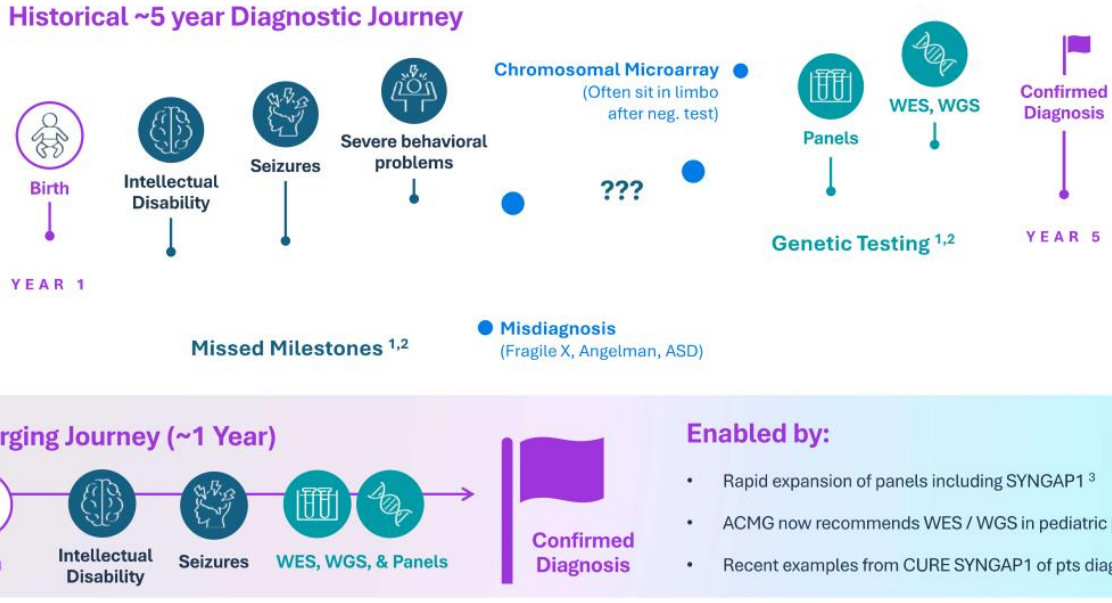
Polypharmacy is common – *Patient regimen* [6] *example:*
- *Epidiolex*
- *Ravicti*
- *Sodium bicarb*
- *Amantadine*

Constant patient care needed
- Caregivers vigilant at all times
- Significant lifelong cost of care

JAELI, 16

[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] Vlaskamp et al. Neurology, 2019; [6] CURE SYNGAP1

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



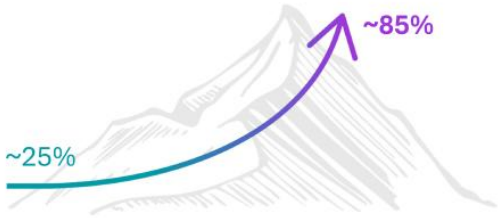
ASD = Autism Spectrum Disorders; WES = Whole Exome Sequencing, WGS = Whole Genome Sequencing, DD = Developmental Delay, ACMG = American College of Medical Genetics and Genomics
Sources: Trinity Life Sciences, CURE SYNGAP1 global census, Caregiver primary market research, [1] Vlaskamp et al. (2019), [2] Graglia et al. (2025), [3] SYNGAP1 included on gene panels provided by Invitae, Ambry Genetics, Fulgent, GeneDx, Baylor Genetics, Prevention Genetics, Revvity, [4] Manickam et al. (2021)

~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**

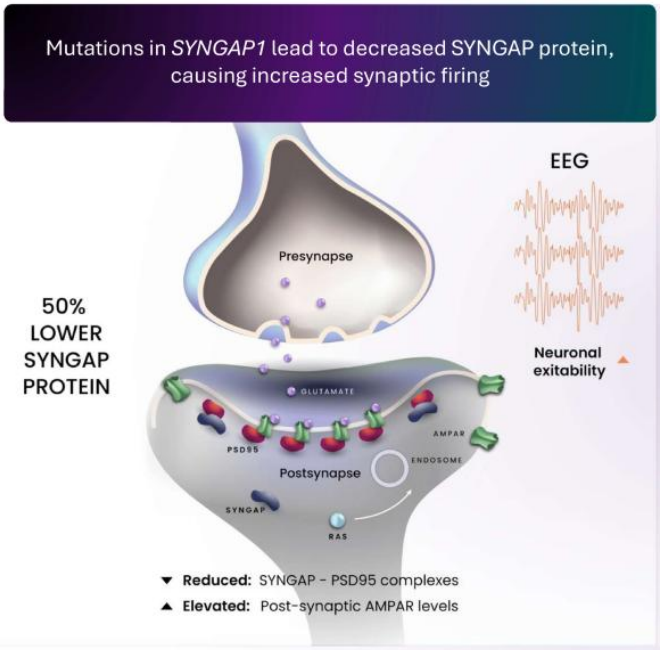
Increasing Diagnosis Rate

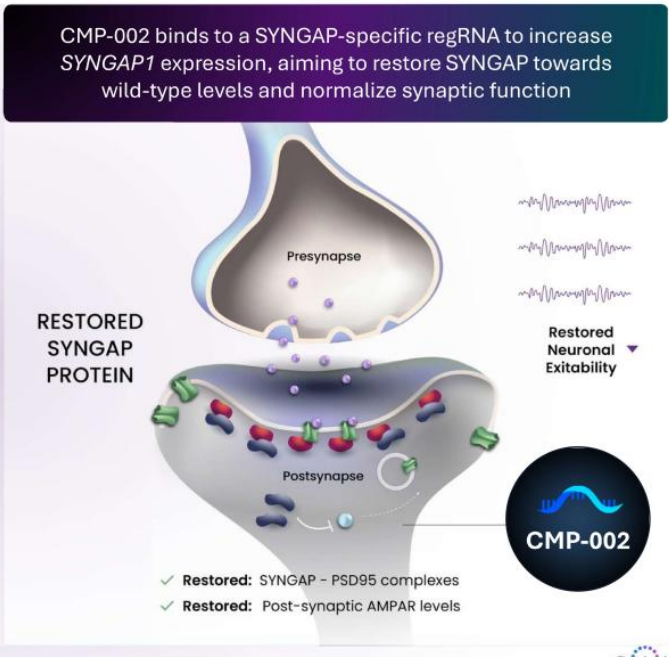


- **Predict notable increase in diagnosis:**
  - Increase in US claims, ICD-10 code added 2021 (+200 pt / yr)
  - CURE SYNGAP1 global census (+50 pt / quarter)
  - SYNGAP1 has been added to many genetic testing pane
  - Increasing use of genetic testing in ASD, ID, DEE [4,5]

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] Betancur et al. (2013), (5) Sanders et al. (2018)

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





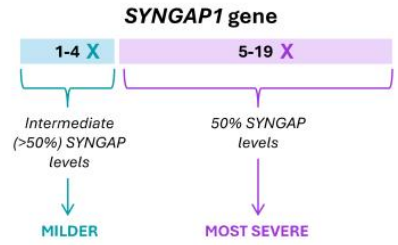
SYNGAP1 represents an ideal target for CAMP4; restoring SYNGAP protein has the potential to meaningfully improve patient outcomes

Opportunity in SYNGAP1 driven by unmet need and compelling preclinical data

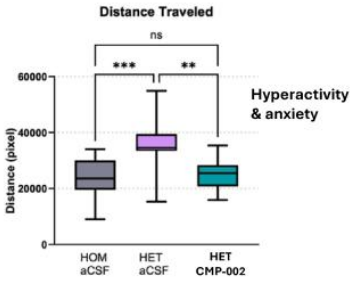
- Ultra-rare SYNGAP1 sub population with intermediate SYNGAP levels equate to milder disease state
- CMP-002 rescued functional defects in relevant human mouse model
- IT administration in NHPs well-tolerated and showed significant increase in SYNGAP in key brain regions

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

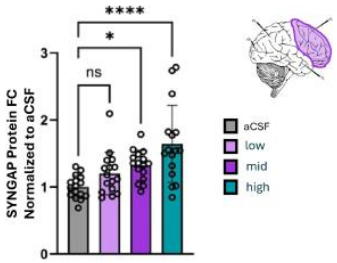
Milder disease severity (verbal responses, milder epilepsy) in minority of patients with intermediate SYNGAP levels



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



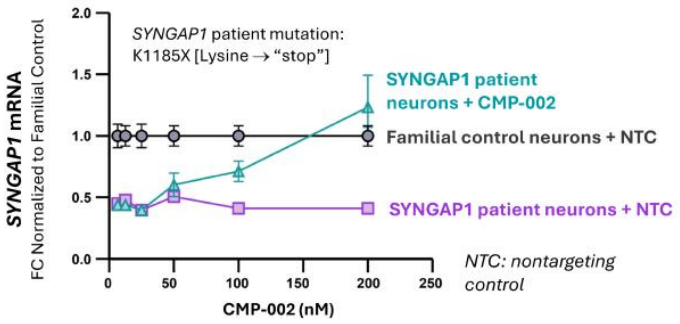
CMP-002 delivered intrathecally increased SYNGAP in disease-relevant brain regions in monkeys



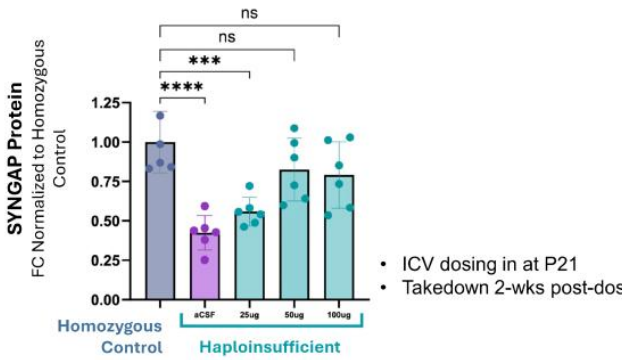
FC = Fold change
Mignot et al., 2016, Vlaskamp et al., 2019, Wiltrout et al., 2024, Kilinc et al., 2022

CMP-002 restored SYNGAP levels in models of haploinsufficiency

SYNGAP1 Patient iPSC-derived neurons



"Humanized" *SYNGAP1* mouse

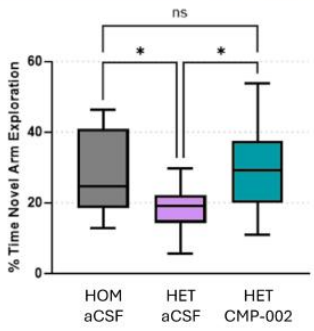


Dose-dependent increase in SYNGAP approaching wild-type levels → both ASO activity and regRNA function translated from human patient-derived neurons in a dish to neurons in an intact animal brain

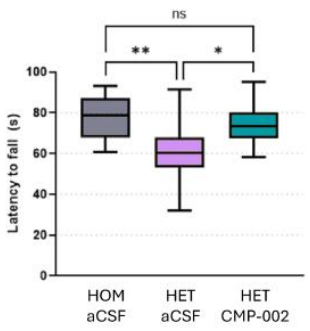
(SYNGAP-ZBTB9)1Bpro/Mmjax mouse strain obtained from the Mutant Mouse Resource and Research Centers (originally deposited by Benjamin Prosser, Ph.D., University of Pennsylvania).

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

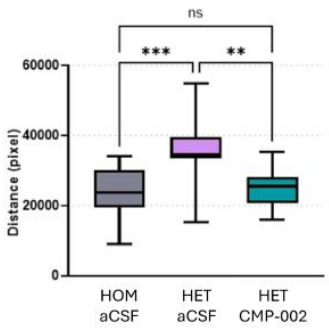
Learning & Memory



Motor Function



Hyperactivity



aCSF = artificial CSF

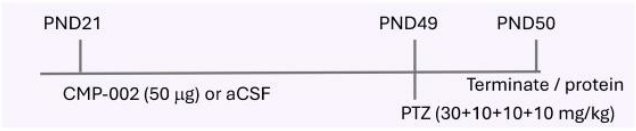
*, p <0.05
**, p<0.01
***, p<0.001

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

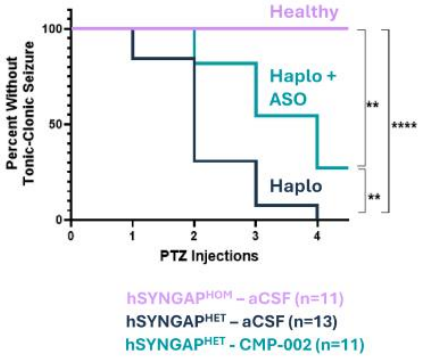
Restoring SYNGAP protein to near wild-type levels ameliorates multiple behavioral phenotypes caused by *SYNGAP1* haploinsufficiency

CMP-002 reduced 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

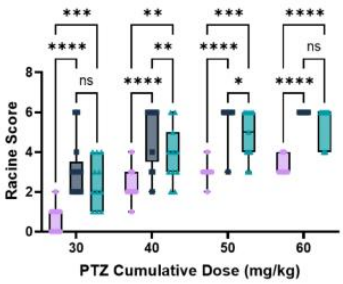


Seizure Threshold

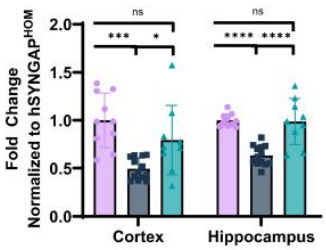


hSYNGAP^{HOM} – aCSF (n=11)
hSYNGAP^{HET} – aCSF (n=13)
hSYNGAP^{HET} - CMP-002 (n=11)

Racine Score

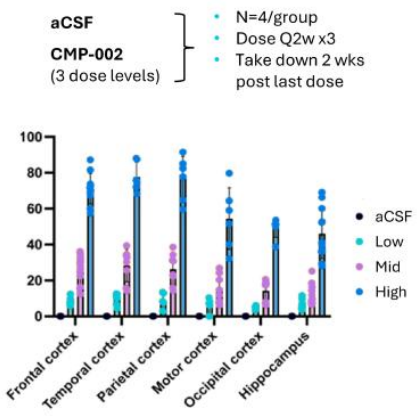


Protein Level

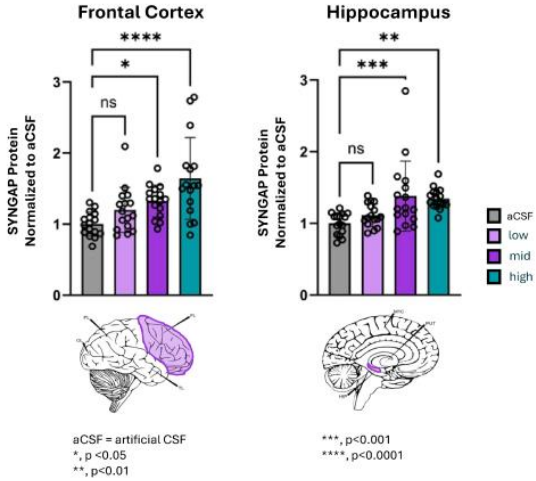


Intrathecal administration of CMP-002 to cynomolgus monkeys achieved broad brain distribution to increase SYNGAP protein levels

ASO Concentrations



SYNGAP Protein Levels



aCSF = artificial CSF
*, p <0.05
**, p<0.01
***, p<0.001
****, p<0.0001

Summary

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

Broad ASO distribution throughout disease-relevant brain regions

↑ SYNGAP protein throughout brain

ASCEND clinical trial designed to assess safety & tolerability across key domains of SYNGAP1 using validated measures

CLINICAL TRIAL GOALS

- Demonstrate safety and tolerability
- Characterize pharmacokinetics and select optimal biological dose
- Identify relevant endpoints applicable to SYNGAP1 patients to chart path for regulatory approval

ASCEND exploratory endpoints assessed

Seizure | Sleep | Motor & Gait | Communication | Behavior | Development & Cognition

ASCEND Phase 1/2 Clinical Trial Summary

Overview	
Design	• Phase 1/2, randomized, double-blind, controlled, multiple ascending dose study • 3:1 randomization • IT administration or a sham pinprick • Open-Label Extension
Study population	• Minimum 8 participants per cohort • Participants ≥2 to <18 years of age with genetically confirmed SYNGAP1-Related Disorder (SRD) • Required daily seizures, impaired sleep, and inability to speak in phrases
Study periods	• Screening and baseline period • Study treatment period with 3 doses • Post-treatment follow-up
Study length	• ~10 months per participant

ASCEND eligibility criteria

Key Inclusion Criteria

Age 2 to <18 years

Male or female participants

Genetically confirmed SRD

Clinical diagnosis of SRD with genetic confirmation of a protein-truncating mutation in SYNGAP1 exons 5–19

Haploinsufficiency phenotype

Intellectual disability, inability to speak in full phrases, and sleep disturbance consistent with haploinsufficiency

Refractory epilepsy

Daily seizures despite ≥1 ongoing anti-seizure medication and prior trial of ≥2 ASMs

Stable ASM regimen at baseline

Stable anti-seizure medication regimen prior to baseline

Key Exclusion Criteria

Non-truncating variants

SYNGAP1 variants that either are non-truncating (missense, microdeletions), located in exons 1–4, or of uncertain significance

Other CNS disease

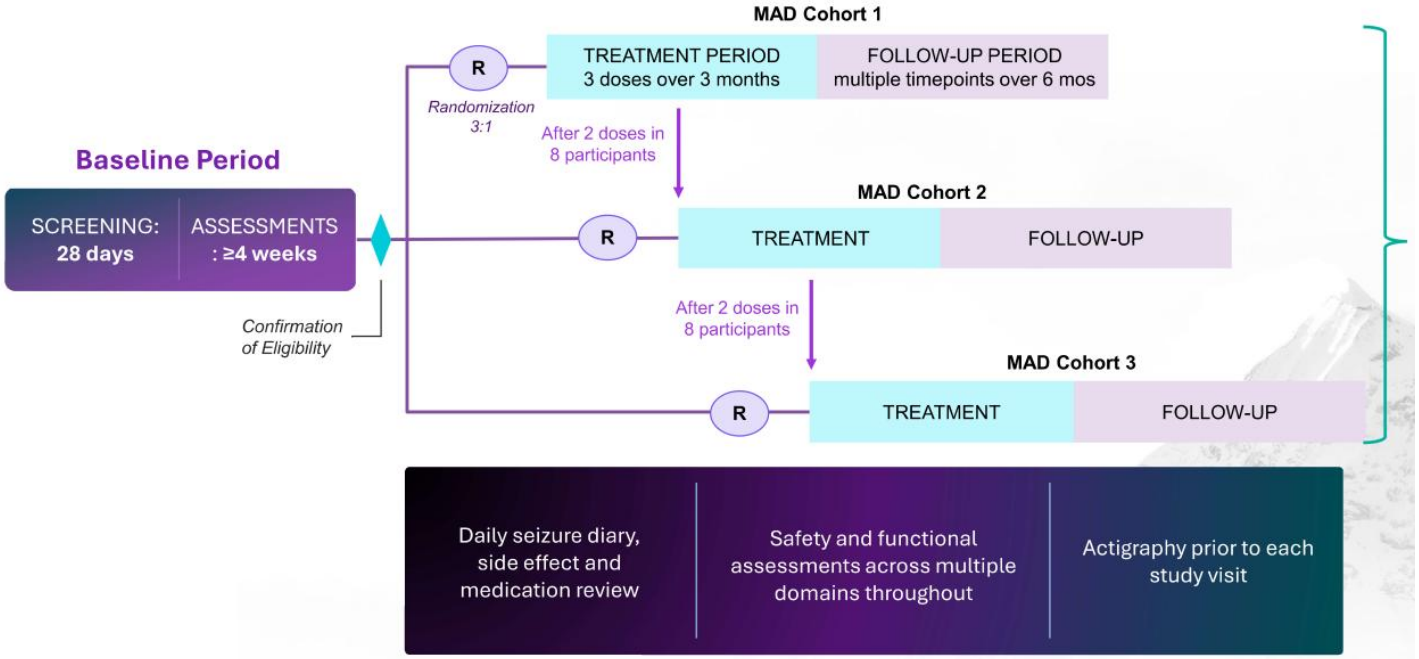
History of central nervous system disease unrelated to SRD

Unsafe for intrathecal dosing

Spinal abnormalities, implanted CSF shunts, or intolerance to lumbar puncture

Clinically significant lab abnormalities

ASCEND clinical trial schema



Five validated scales being utilized in ASCEND: Behavior, development, communication, and motor function

Scale	Communication	Motor	Behavior	Development
Bayley Scales of Infant and Toddler Development Bayley-4		✓	✓	✓
Vineland Adaptive Behavior Scales, 3rd Edition Vineland-3	✓	✓	✓	✓
Aberrant Behavior Checklist, 2nd Edition ABC-2	✓		✓	
Observer-Reported Communication Ability Measure ORCA	✓			
Gross Motor Function Measure GMFM		✓		

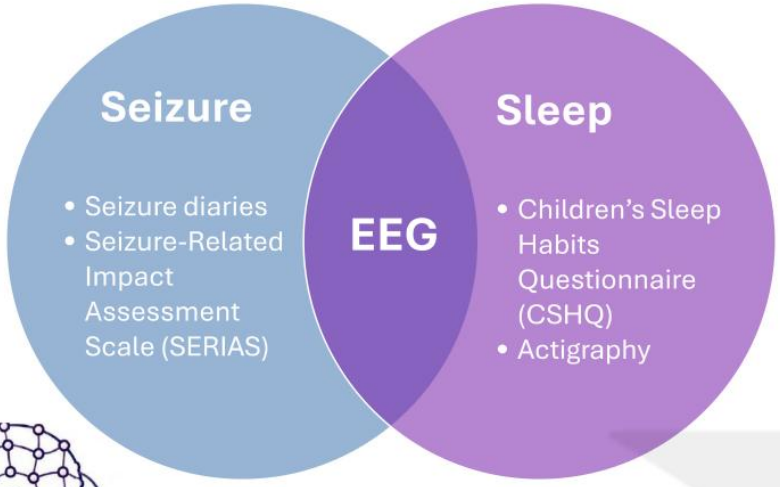
Leveraging validated scales used in DEE clinical trials and ProMMiS Natural History Study

✓ Scale to be utilized to assess domain in ASCEND trial

Assessing seizure and sleep: multiple methods to measure change, with vEEG common to both domains

24hr video-EEG for seizure and sleep detection:

- REM, SOL, WASO, TST
- EEG informs both seizure and sleep endpoints



EEG measures multiple parameters

- **Clinical signatures** *spikes, seizures, slowing*
- **Spectral features** *alpha-theta wave*
- **Spatial / topographic measures**
- **Event-related potential** *VEP / AEP*
- **Sleep / state-based measures**
- **Functional connectivity**
- **Temporal / nonlinear dynamics** *entropy, temporal correlations*

Planned global footprint of ASCEND Phase 1/2 clinical trial

11+ Total sites

9 Countries

5 Regions with sites planned

4 approved: UK, ARG, AUS

7 EU sites under review

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 Yumanity

Yuri Maricich, MD
Chief Medical Officer

corixa Cavion 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 Alnylam

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- Steven Holtzman
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* 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 Q4 '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
  - Building a pipeline focused on haploinsufficient neurodevelopmental disorders to expand on expertise gained through SYNGAP1 program, staring with SHANK3 for Phelan-McDermid Syndrome
  - 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