CAMP4®

Corporate Overview

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

OCTOBER 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.

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, the United Kingdom, and multiple EU countries
  - 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

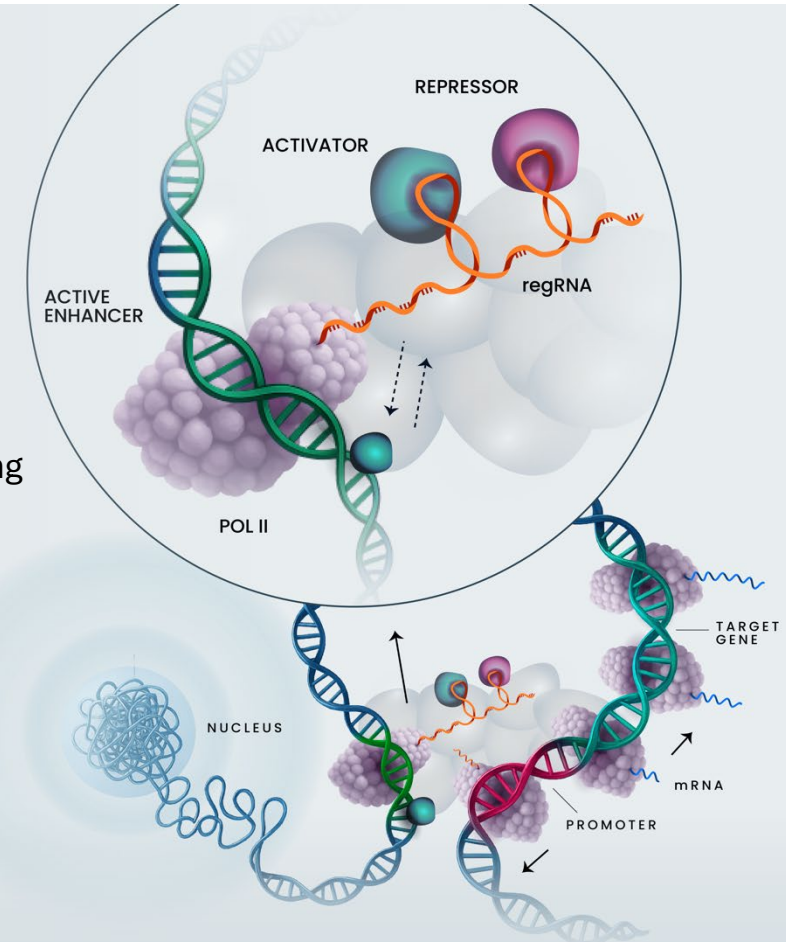
- CNS-focused pipeline, leveraging BD to derive additional value from the platform
  - Proprietary RAP Platform® was built for the discovery of novel regRNAs that regulate the expression of every protein-coding gene that can be selectively drugged using state of the art ASO chemistry
  - Early pipeline 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

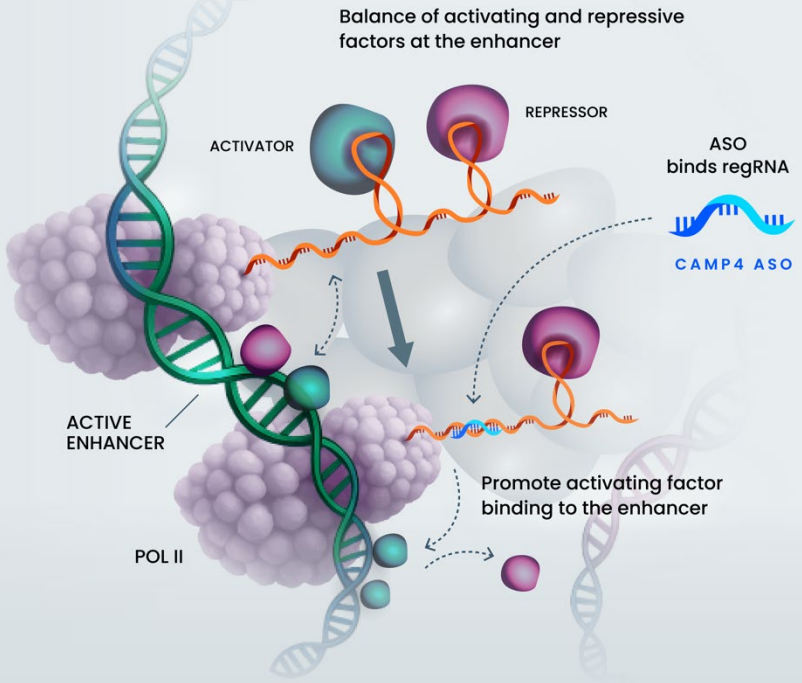
regRNAs play a central role in the regulation of every gene's expression

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



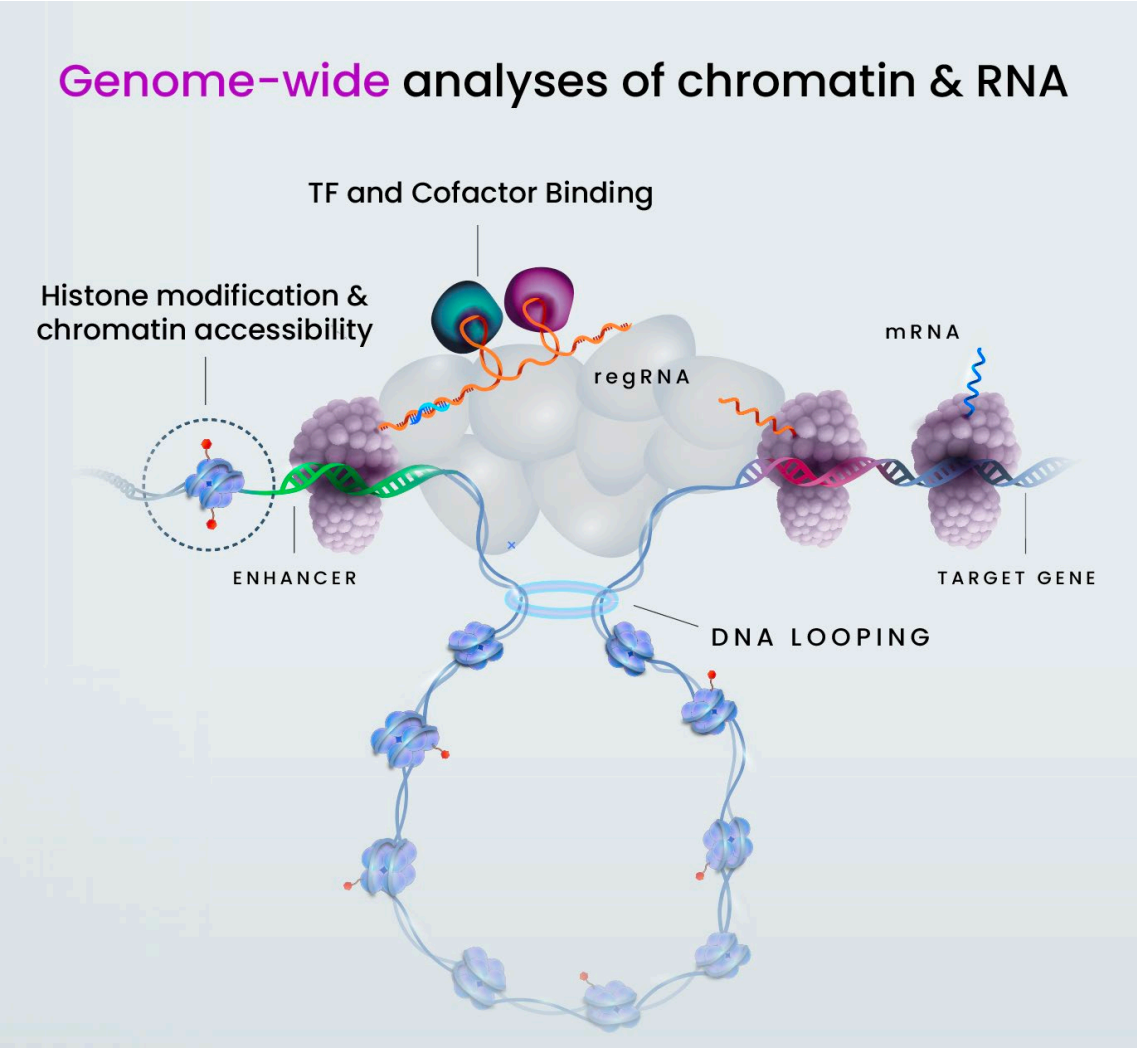


3 ASOs disrupt the interactions between transcription factors and regRNAs enabling increases in gene expression

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.

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

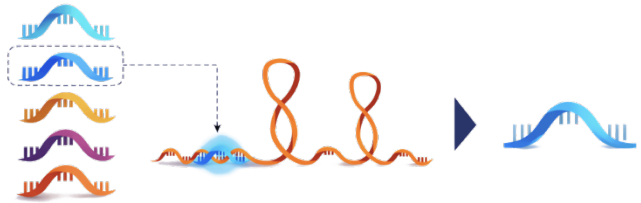


1 Map candidate regRNAs

- 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



CAMP4®

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

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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

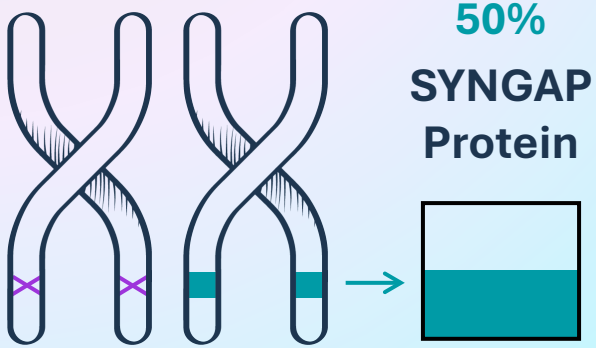
Patient story and images used with permission; 'Our Son Has a Rare Genetic Disorder, Life Is Risky for Us' Mike Graglia, Newsweek (January 25, 2023)

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

Haploinsufficiency results in 50% of normal protein levels

SYNGAP1 Haploinsufficiency



50%
SYNGAP Protein

>10,000 SYNGAP1 patients in the US



6.1–10 per 100K incidence rate [1,2]

100% have intellectual disability, **~85%** have seizures, potentially experiencing **10+ per day** [3,4,5]



High unmet need for disease-modifying therapy

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



0 approved disease modifying therapies

[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

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



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 words*** [4]

No Approved Therapy

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

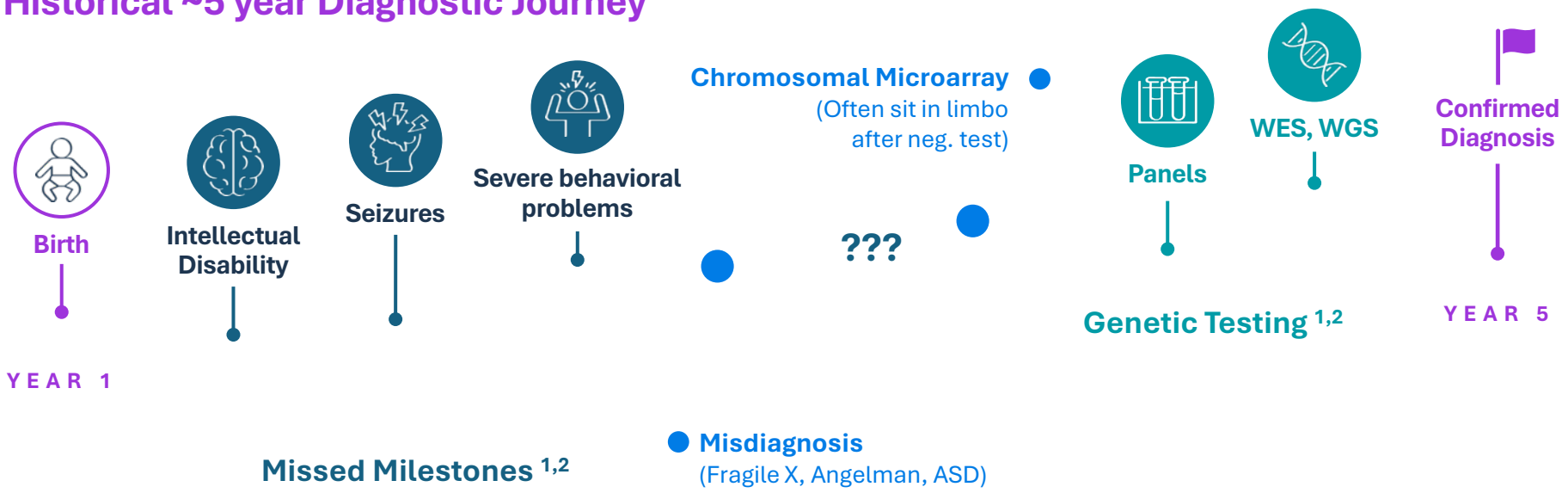
Polypharmacy is common – *Patient regimen* [6] *example:*
- *Epidiolex*
- *Ravicti*
- *Sodium bicarb*
- *Amantadine*

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

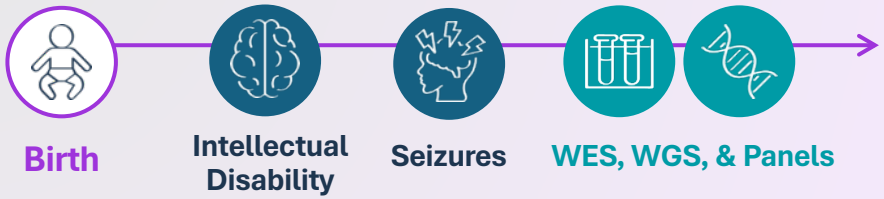
[1] *Wiltrout, et al., Epilepsia, 2024;* [2] *Jimenez-Gomez, et al. J Neurodev Disord, 2019;* [3] *Holder et al., GeneReviews, 2019;* [4] *SYNGAP-Related Epilepsy, Epilepsy Foundation (Accessed May 2025);* [5] *Vlaskamp et al. Neurology, 2019;* [6] *CURE SYNGAP1*

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

Historical ~5 year Diagnostic Journey



Emerging Journey (~1 Year)



Enabled by:

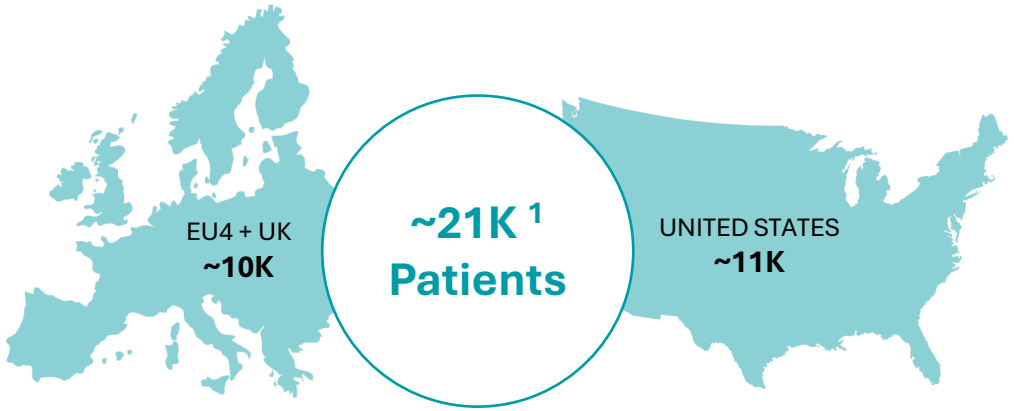
- Rapid expansion of panels including SYNGAP1 [3]
- ACMG now recommends WES / WGS in pediatric pts w DD [4]
- Recent examples from CURE SYNGAP1 of pts diagnosed <1 yr

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)

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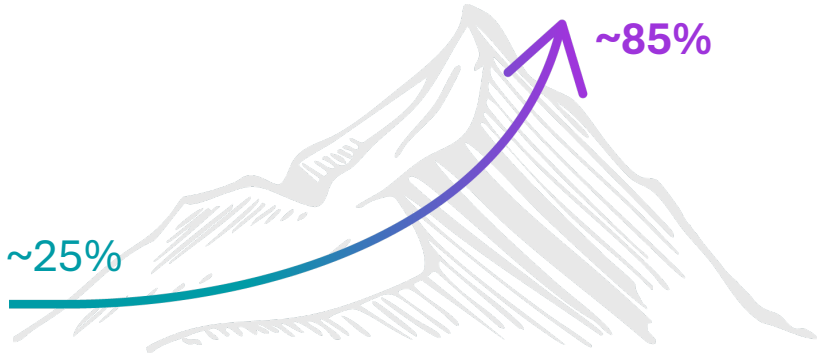
~21K patients across key global geographies (US + EU5); SYNGAP1 remains highly underdiagnosed

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

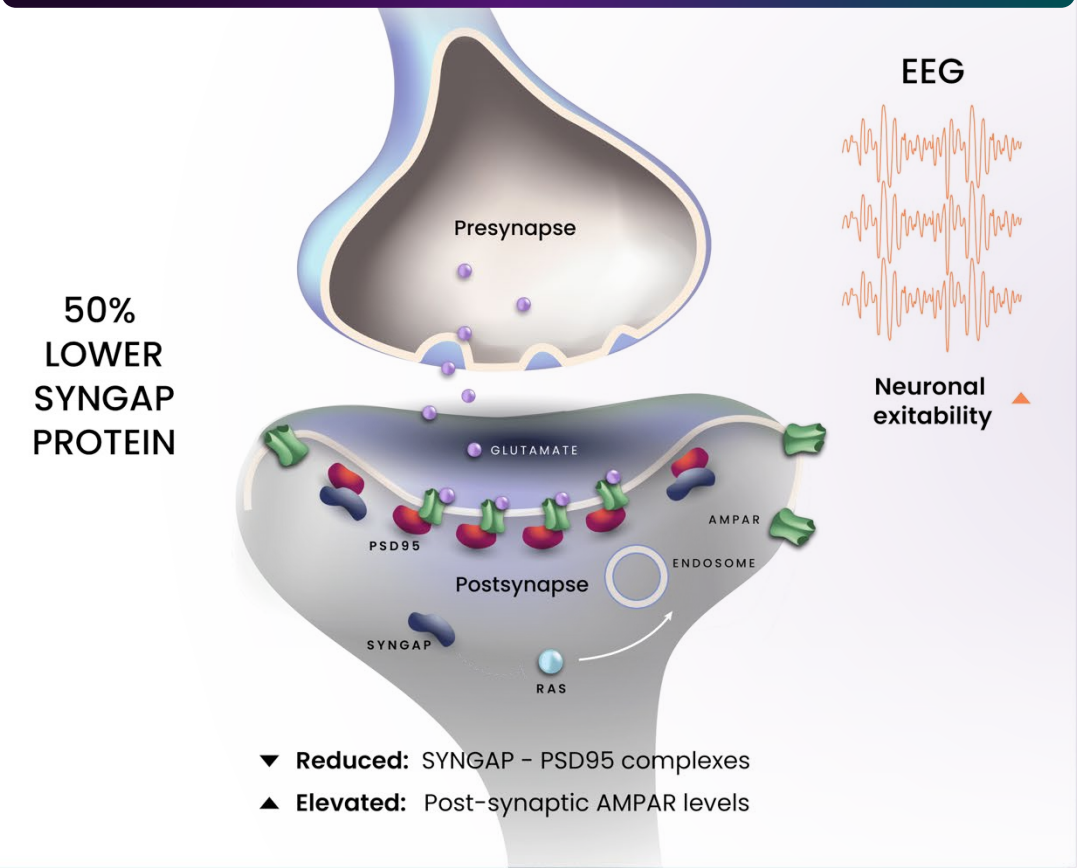


- **Predict notable increase in diagnosis:**
  - Increase in US claims, ICD-10 code added 2021 (+200 pt / yr)
  - CURE SYNGAP1 global census (+50 pt / quarter)
  - SYNGAP1 has been added to many genetic testing panels [3]
  - Increasing use of genetic testing in ASD, ID, DEE [4,5]

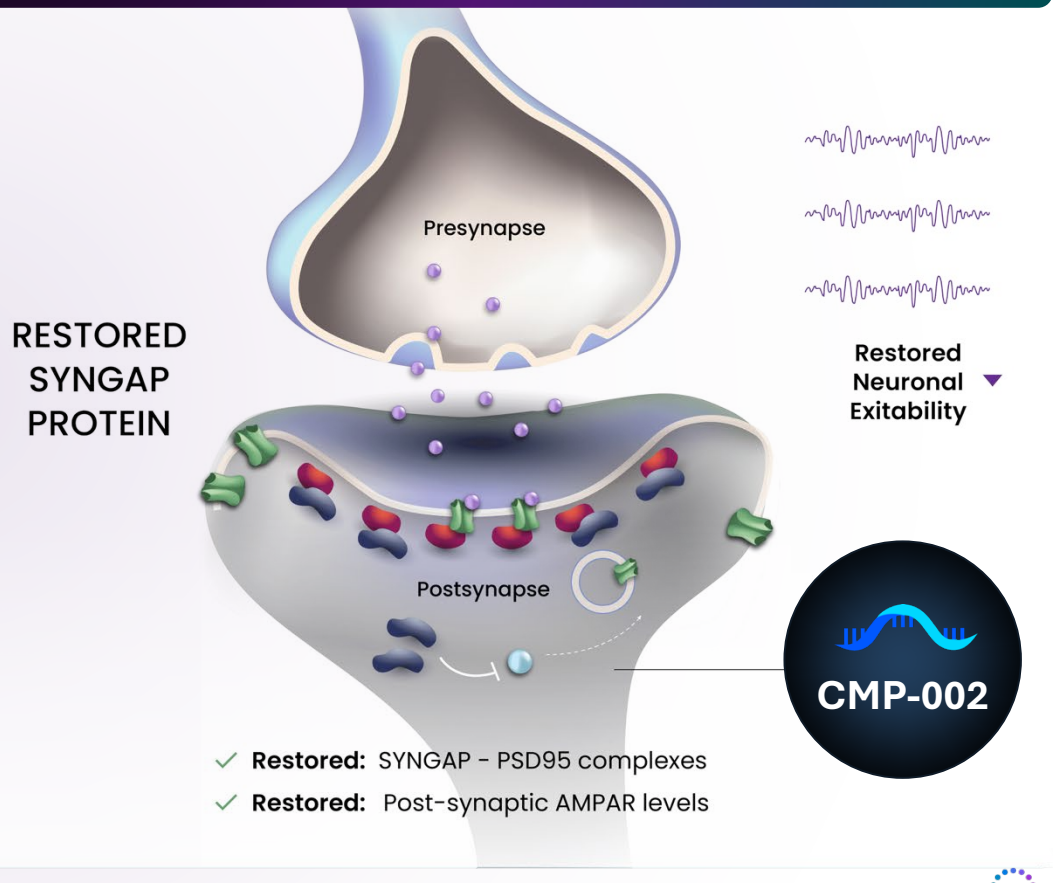
DEE = Developmental Epileptic Encephalopathies, ID = Intellectual Disability, ASD = Autism Spectrum Disorders
Sources: Trinity Life Sciences, Komodo claims data (2021-2025), CURE SYNGAP1; [1] Scaled annual incidence to prevalence using country specific live births and adjusted for mortality estimates; 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] Betancur et al. (2013), (5) Sanders et al. (2018)

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

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



CMP-002 binds to a SYNGAP-specific regRNA to increase *SYNGAP1* expression, aiming to restore SYNGAP towards wild-type levels and normalize synaptic function



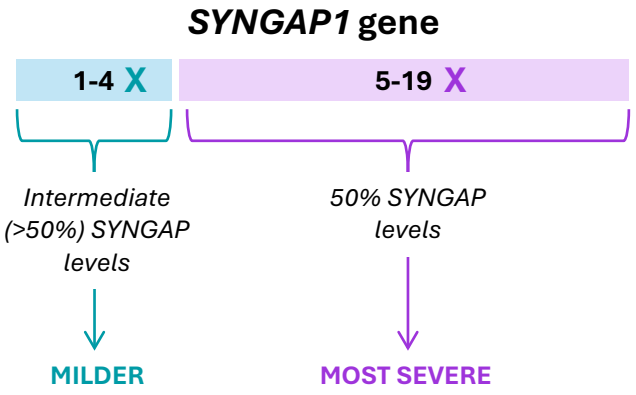
SYNGAP1 represents an ideal target for CAMP4; restoring SYNGAP protein has the potential to meaningfully improve patient outcomes

Opportunity in SYNGAP1 driven by unmet need and compelling preclinical data

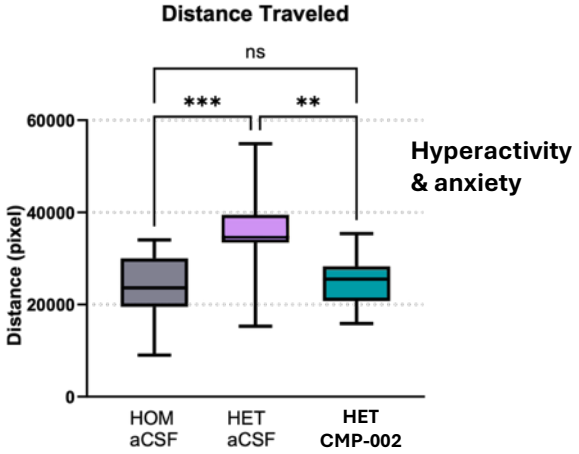
- Ultra-rare SYNGAP1 sub population with intermediate SYNGAP levels equate to milder disease state
- CMP-002 rescued functional defects in relevant human mouse model
- IT administration in NHPs well-tolerated and showed significant increase in SYNGAP in key brain regions

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

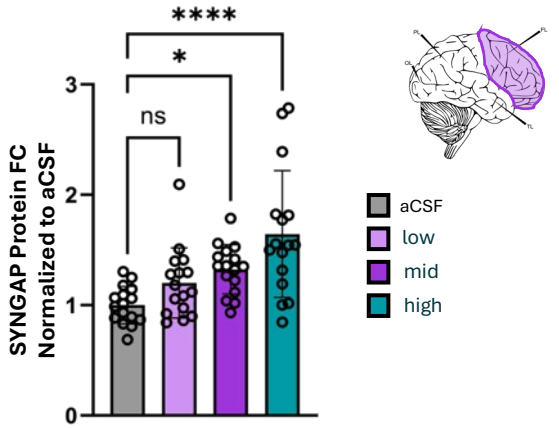
Milder disease severity (verbal responses, milder epilepsy) in minority of patients with intermediate SYNGAP levels



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



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

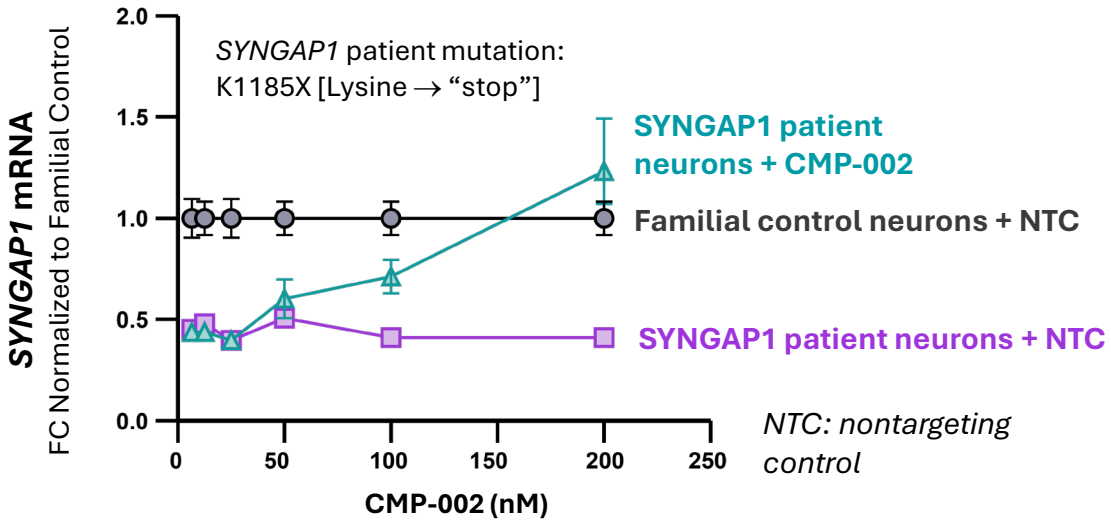


FC = Fold change

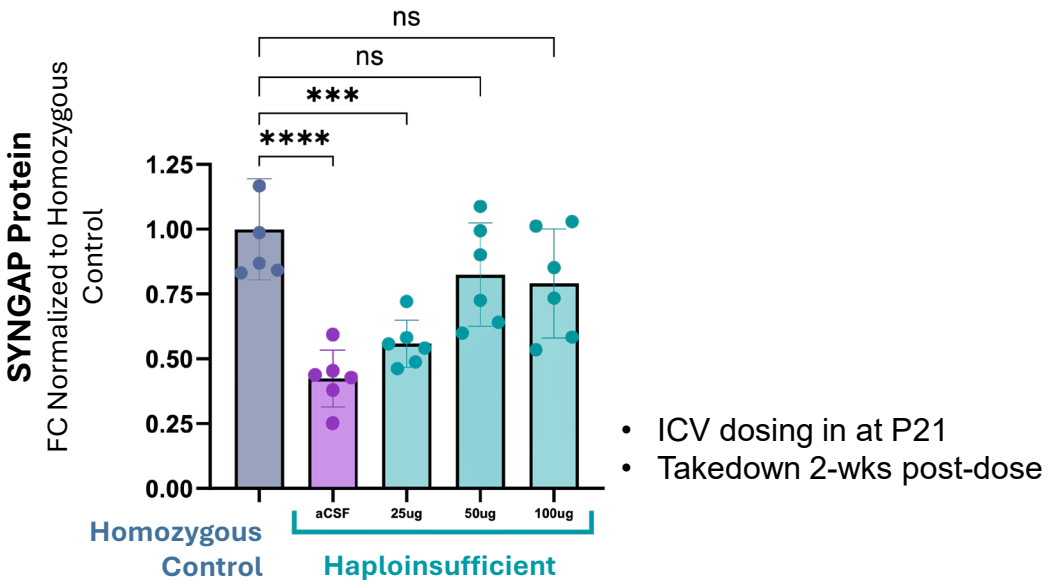
Mignot et al., 2016, Vlaskamp et al., 2019, Wiltrout et al., 2024, Kilinc et al., 2022

CMP-002 restored SYNGAP levels in models of haploinsufficiency

SYNGAP1 Patient iPSC-derived neurons



"Humanized" *SYNGAP1* mouse



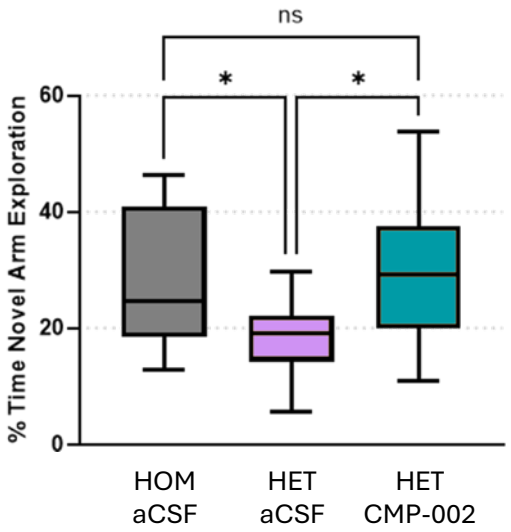
- ICV dosing in at P21
- Takedown 2-wks post-dose

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

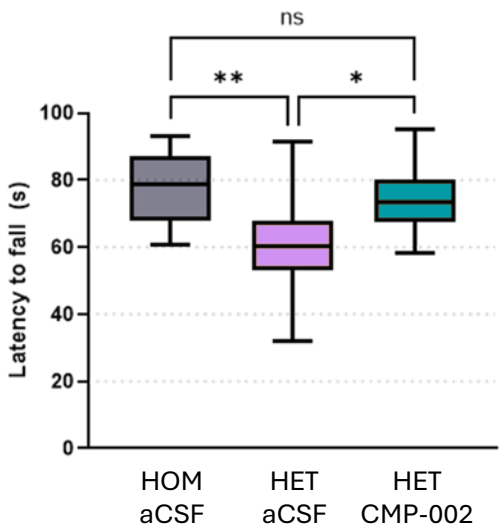
(SYNGAP-ZBTB9)[1]Bpro/Mmjax mouse strain obtained from the Mutant Mouse Resource and Research Centers (originally deposited by Benjamin Prosser, Ph.D., University of Pennsylvania).

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

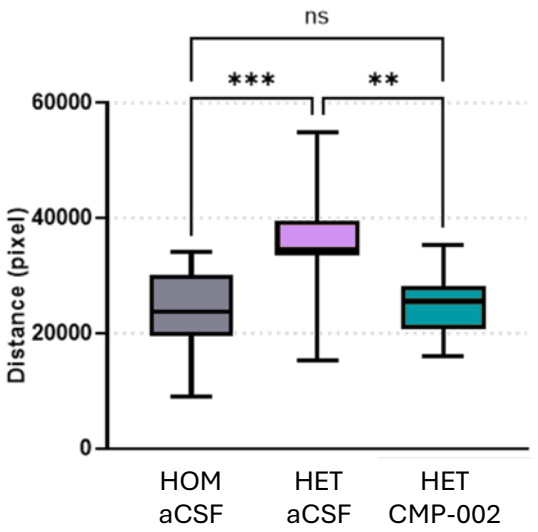
Learning & Memory



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

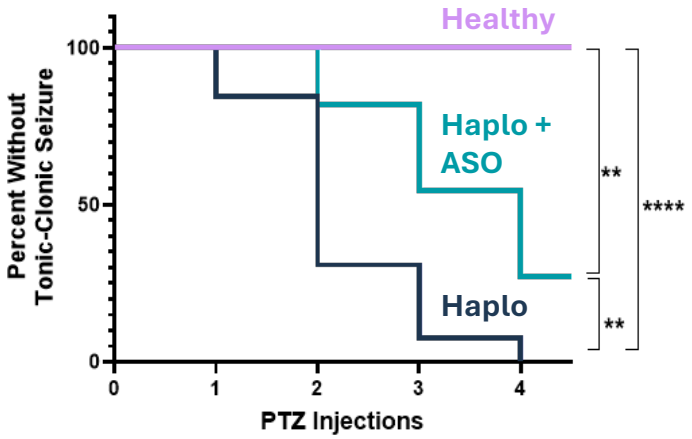
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CMP-002 reduced PTZ-induced seizures in SYNGAP1 humanized mouse

- 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

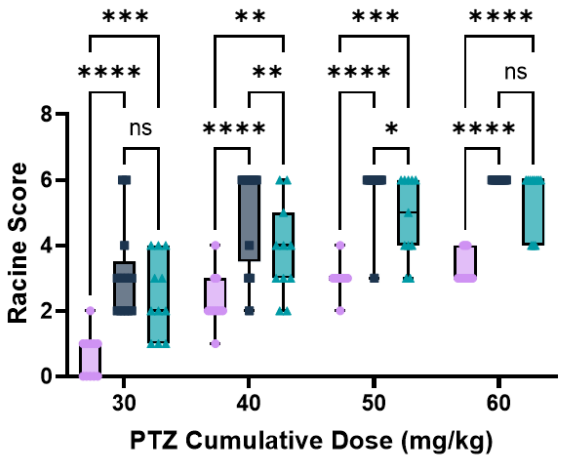


Seizure Threshold

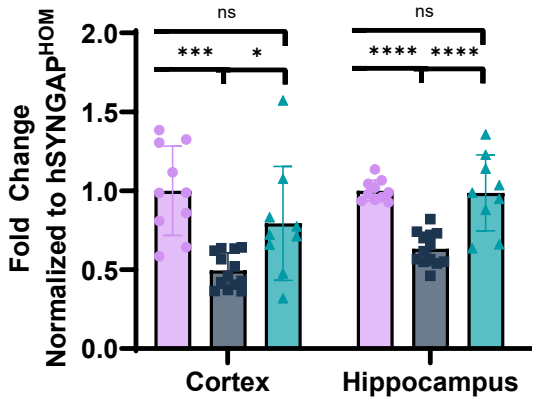


hSYNGAP^{HOM} – aCSF (n=11)
hSYNGAP^{HET} – aCSF (n=13)
hSYNGAP^{HET} - CMP-002 (n=11)

Racine Score



Protein Level



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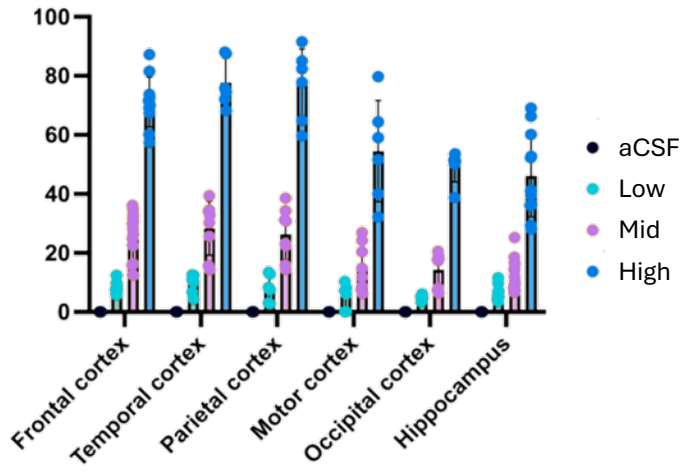
Intrathecal administration of CMP-002 to cynomolgus monkeys achieved broad brain distribution to increase SYNGAP protein levels

ASO Concentrations

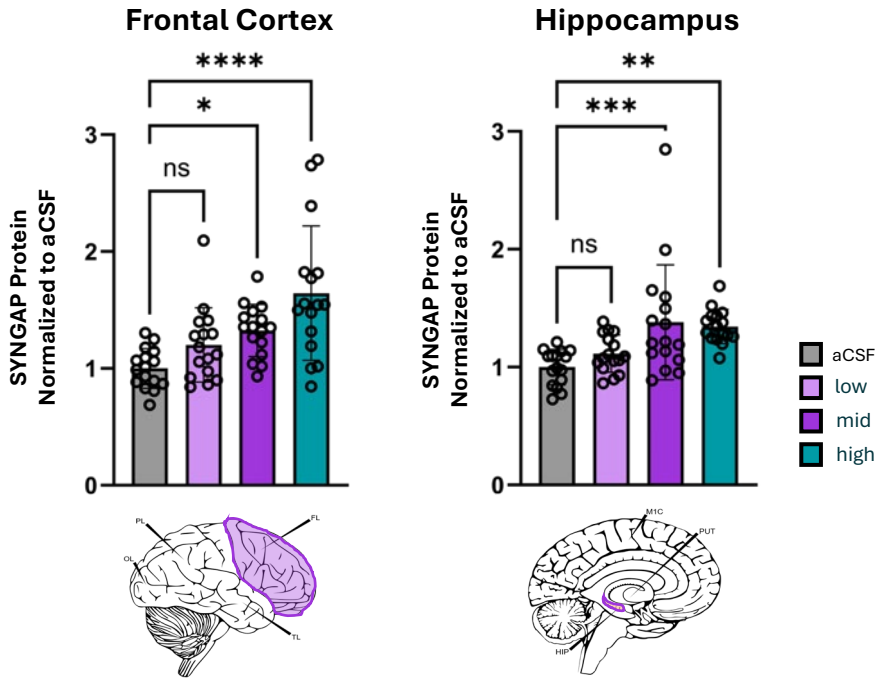
aCSF

CMP-002
(3 dose levels)

- N=4/group
- Dose Q2w x3
- Take down 2 wks post last dose



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

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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

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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

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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

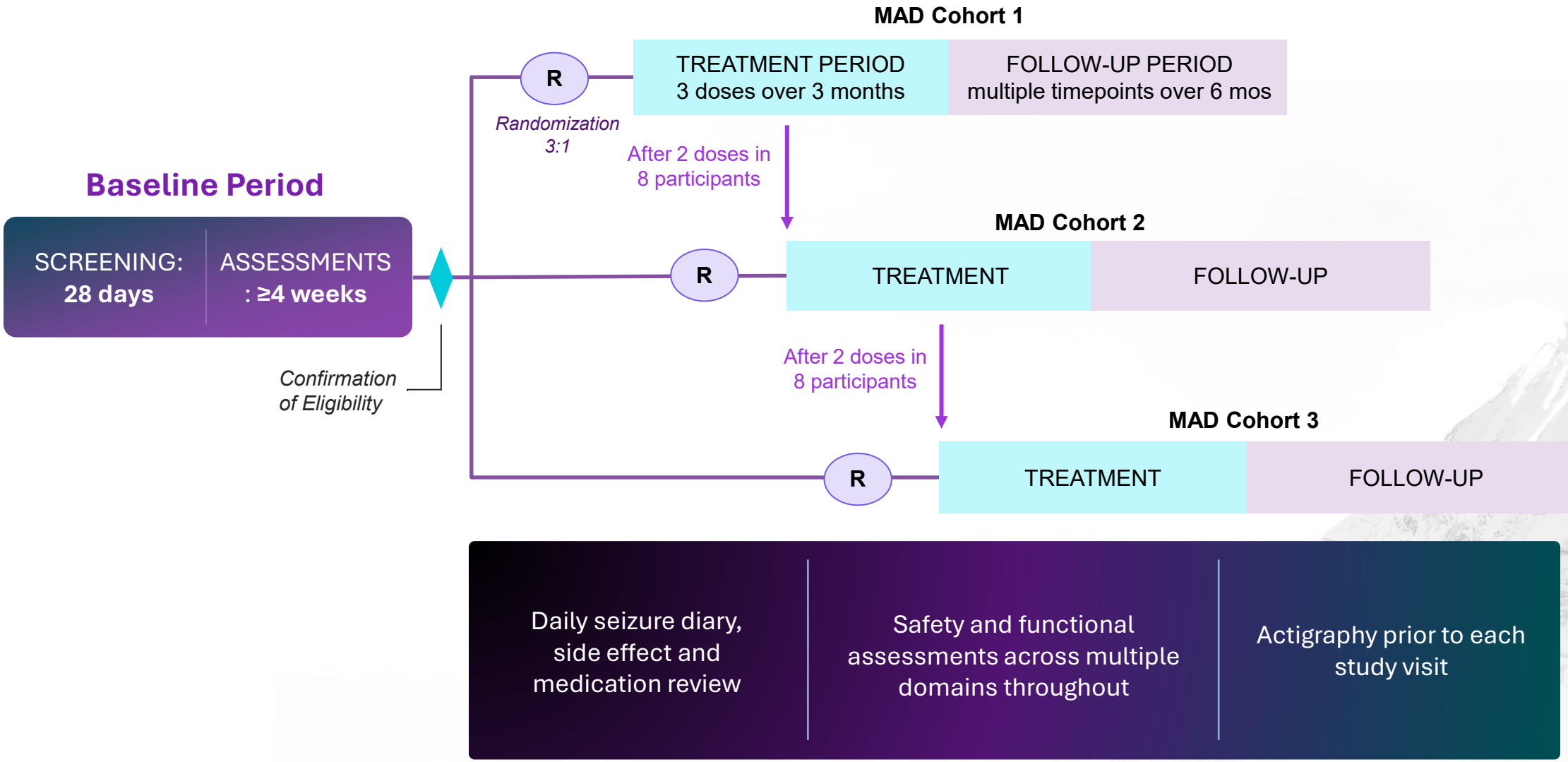
Unsafe for intrathecal dosing

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

Clinically significant lab abnormalities

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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

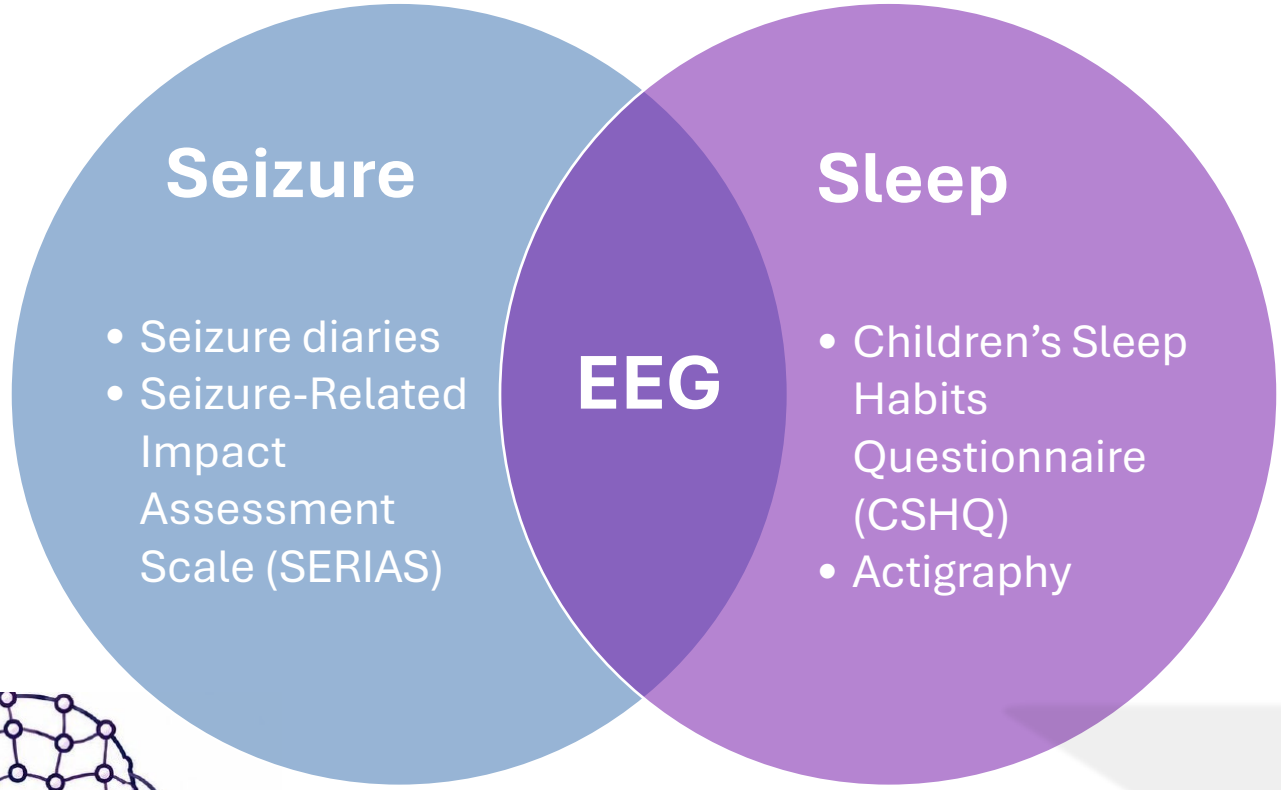
✓ Scale to be utilized to assess domain in ASCEND trial

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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 potentials**
  VEP / AEP
- **Sleep / state-based measures**
- **Functional connectivity**
- **Temporal / nonlinear dynamics**
  entropy, temporal correlations

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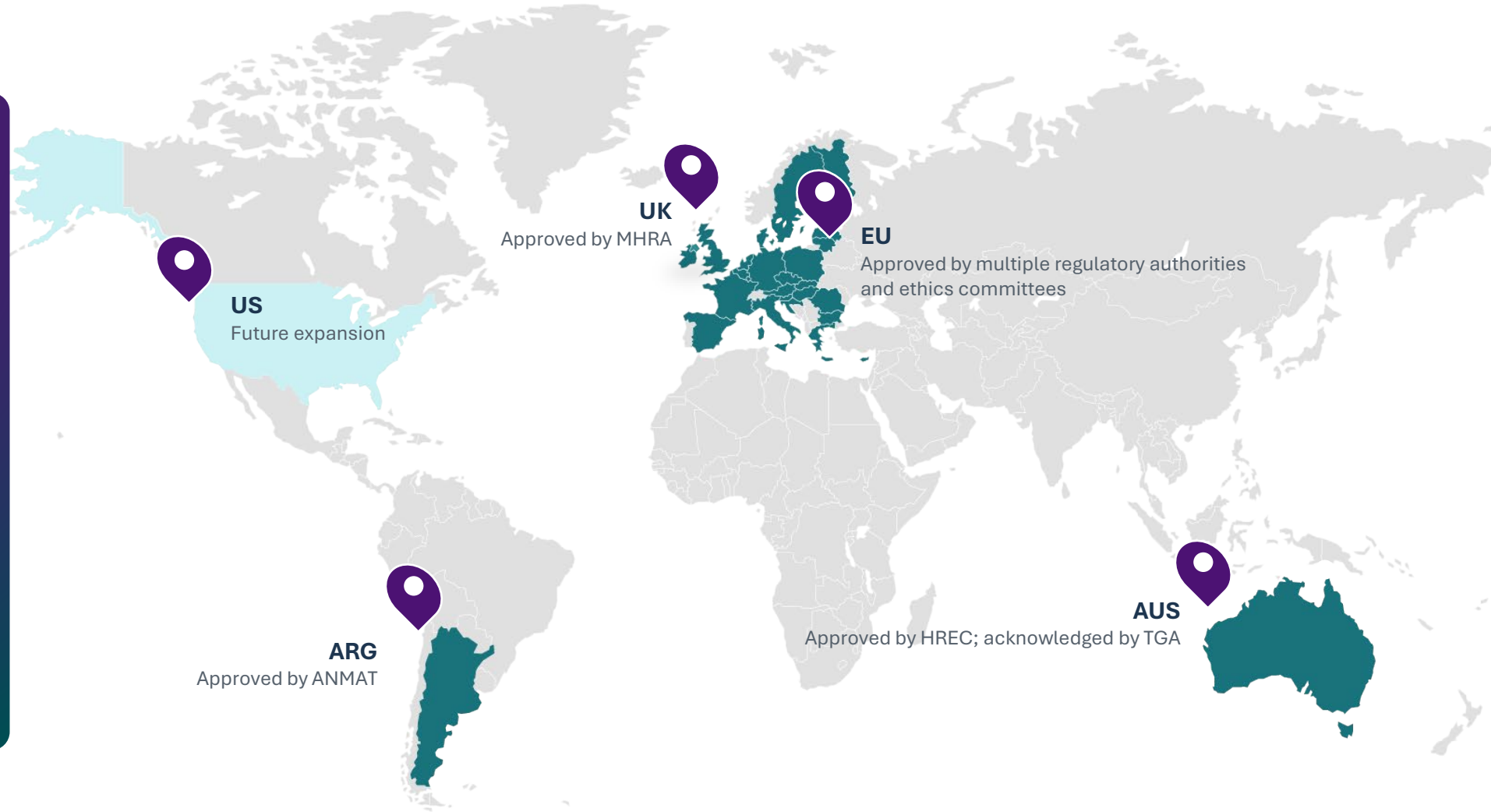
Planned global footprint of ASCEND Phase 1/2 clinical trial

11+ Total sites

9 Countries

5 Regions with sites planned

11 approved: UK, ARG, AUS, EU



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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 Therapeutics



Yuri Maricich, MD
Chief Medical Officer

corixa · Cavion · PEAR Therapeutics



Caleb Moore
Chief Business Operations Officer

genzyme · ACCELERON · CUBIST PHARMACEUTICALS



Michelle Gates
Chief People Officer

Akamai



Alla Sigova, PhD
SVP, Head of Platform

SAIL · Whitehead Institute



Satya Kuchimanchi, PhD
SVP, Technical Operations

TRIPLET · Alnylam PHARMACEUTICALS

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* Board Chair

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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