



Corporate Overview

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

AUGUST 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.

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

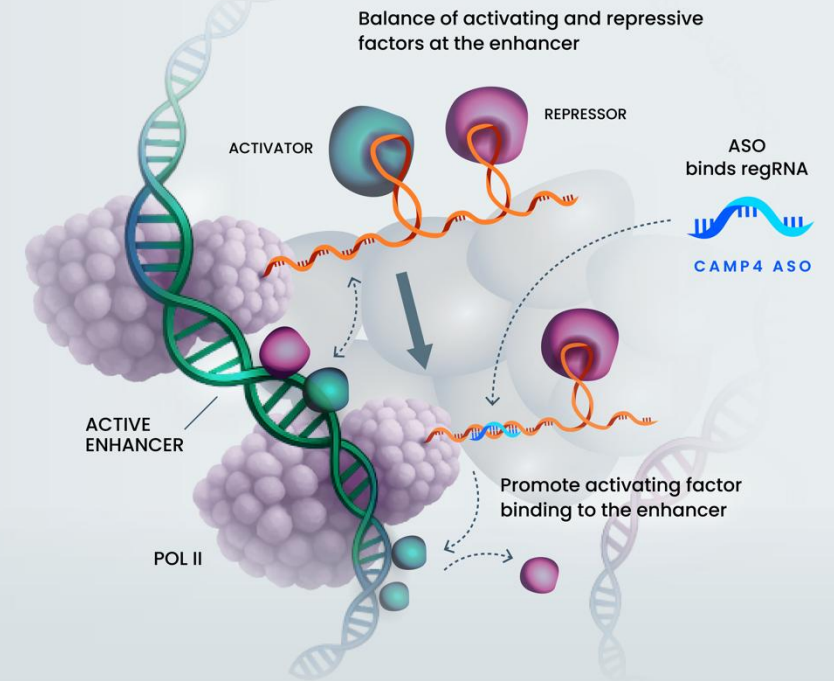
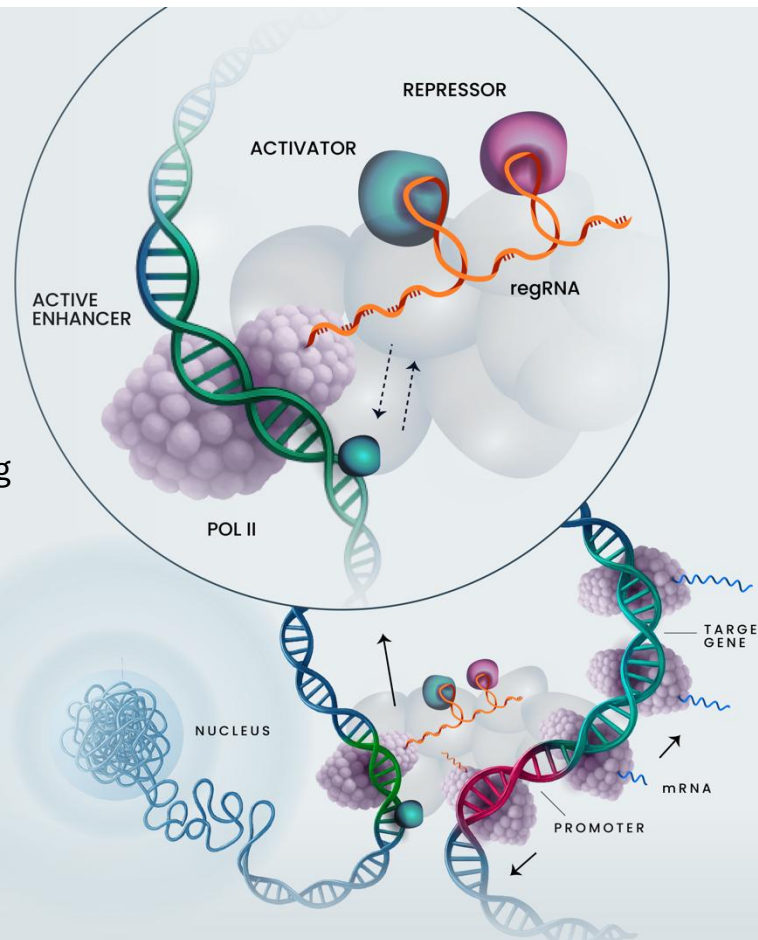
- Developing a targeted disease modifying therapy to address dire unmet need in SYNGAP1-related disorder
 - SYNGAP1 is a haploinsufficient CNS disorder, and an optimal target for CAMP4
 - CMP-002 is designed to increase SYNGAP protein levels, restore *SYNGAP1* function and improve disease symptoms
 - >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
 - CTA approvals received in Australia and Argentina
 - Expect to advance CMP-002 to a global Ph 1/2 study in patients in the fourth quarter 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 neurodevelopmental 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

1 regRNAs originate from enhancers and promoters

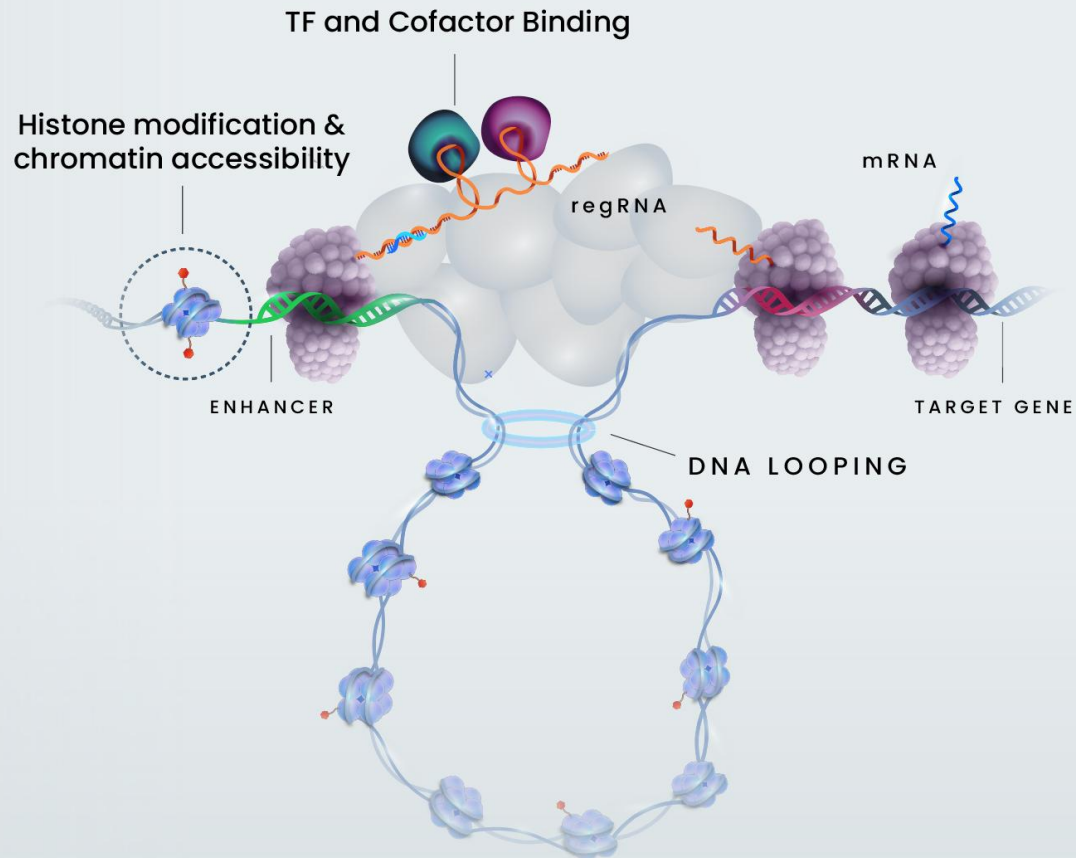
2 Activators and repressors bind to regRNAs to control the expression of protein-coding genes



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

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

Genome-wide analyses of chromatin & RNA

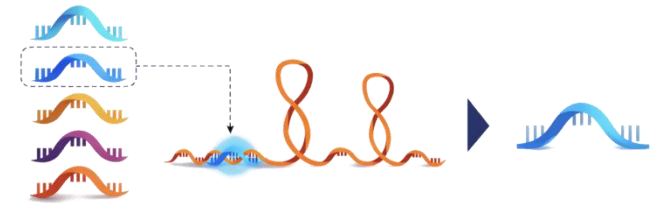


1 Map candidate regRNAs

- 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 Milestone	Commercial Rights
CNS DISEASES							
CMP-002	SYNGAP1-related disorder	SYNGAP1				Clinical initiation in Q4 2026	
New Discovery Programs	CNS	Numerous	Active discovery and development of multiple programs utilizing the RAP Platform®				
METABOLIC DISEASES							
CMP-001	Urea Cycle Disorders	CSP1		Exploring potential partnership opportunities.			
COLLABORATIONS							
Strategic research collaboration to identify and develop antisense oligonucleotide (ASO) drug candidates for multiple gene targets relevant to neurodegenerative and kidney diseases indications.							

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."

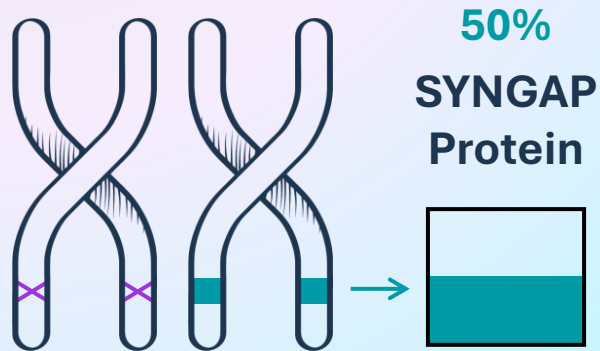
"My life is dedicated to this cause; as a parent, it's the number one thing I strive to do for my sons: alleviate Tony's suffering to help him live the best possible life"

- Tony's Father

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

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

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 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

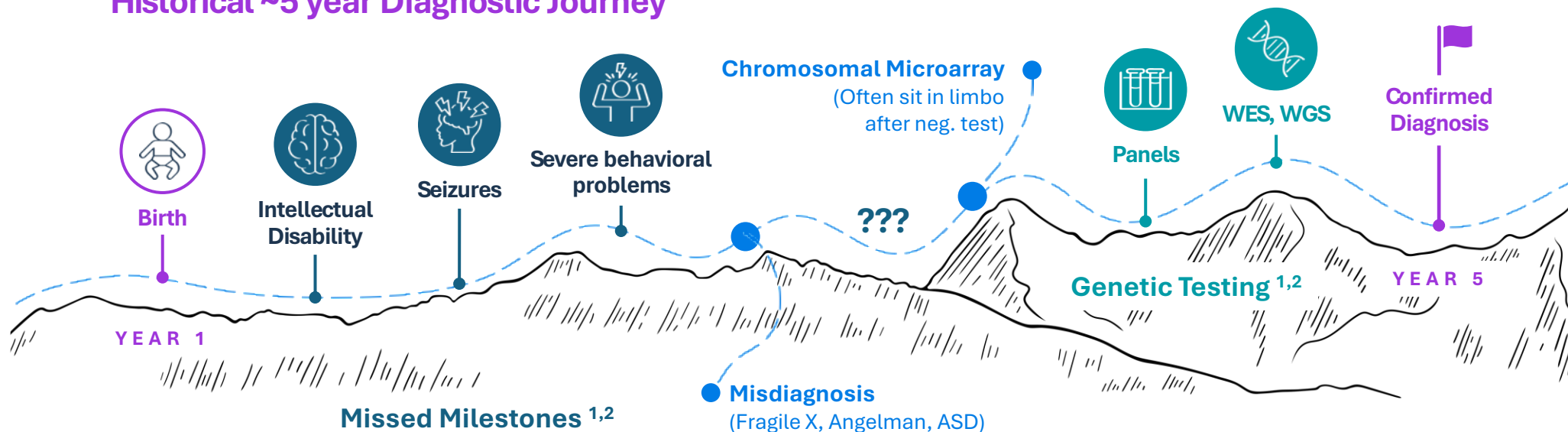
Constant patient care needed

- 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)

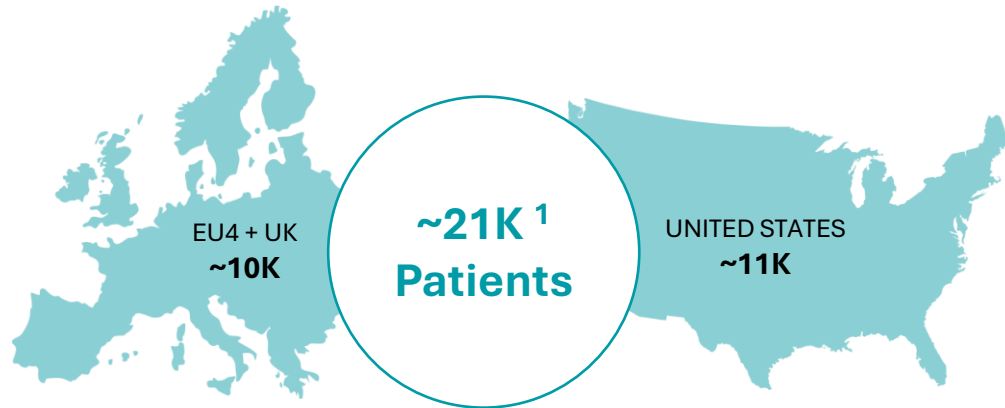


Enabled by:

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

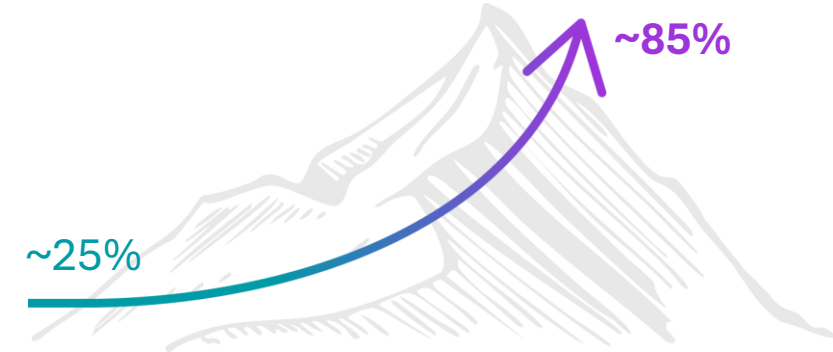
~21K patients across key global geographies (US + EU5); SYNGAP1 remains highly underdiagnosed

Major Market Prevalence



- **Scaled annual incidence to prevalence¹**
- **Prevalence may be larger;** diagnosis rates may increase significantly with genetic testing awareness and utilization²
- **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 panels³
 - Increasing use of genetic testing in ASD, ID, DEE^{4,5}

DEE = Developmental Epileptic Encephalopathies, ID = Intellectual Disability, ASD = Autism Spectrum Disorders

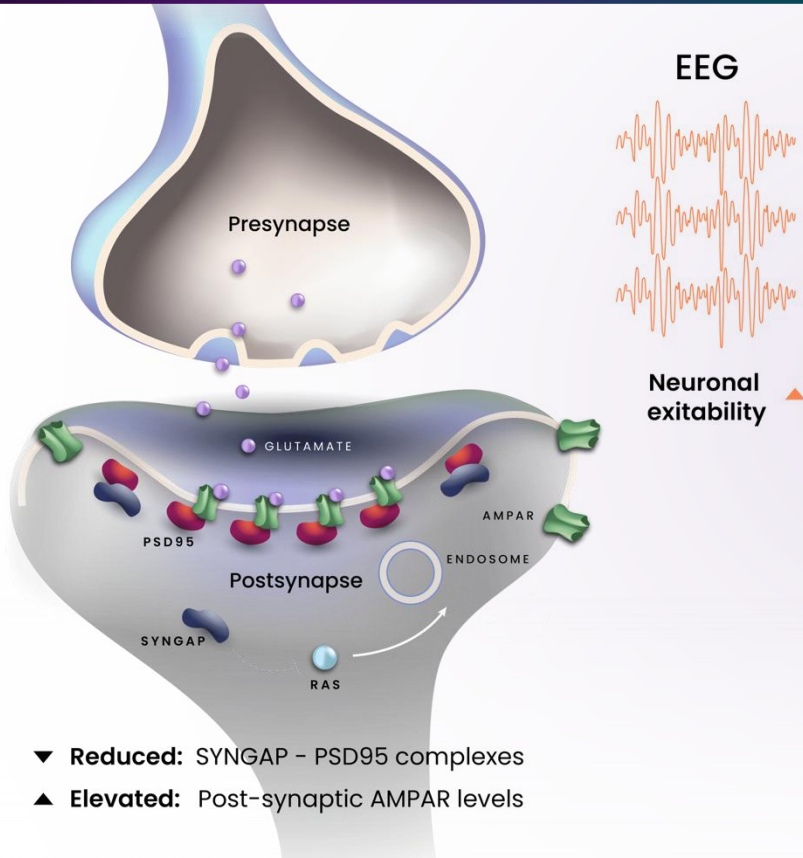
Sources: Trinity Life Sciences, Komodo claims data (2021-2025), CURE SYNGAP1; ¹ Scaled annual incidence to prevalence using country specific live births and adjusted for mortality estimates; incidence rates based on Lopez Rivera et al. (2020)

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

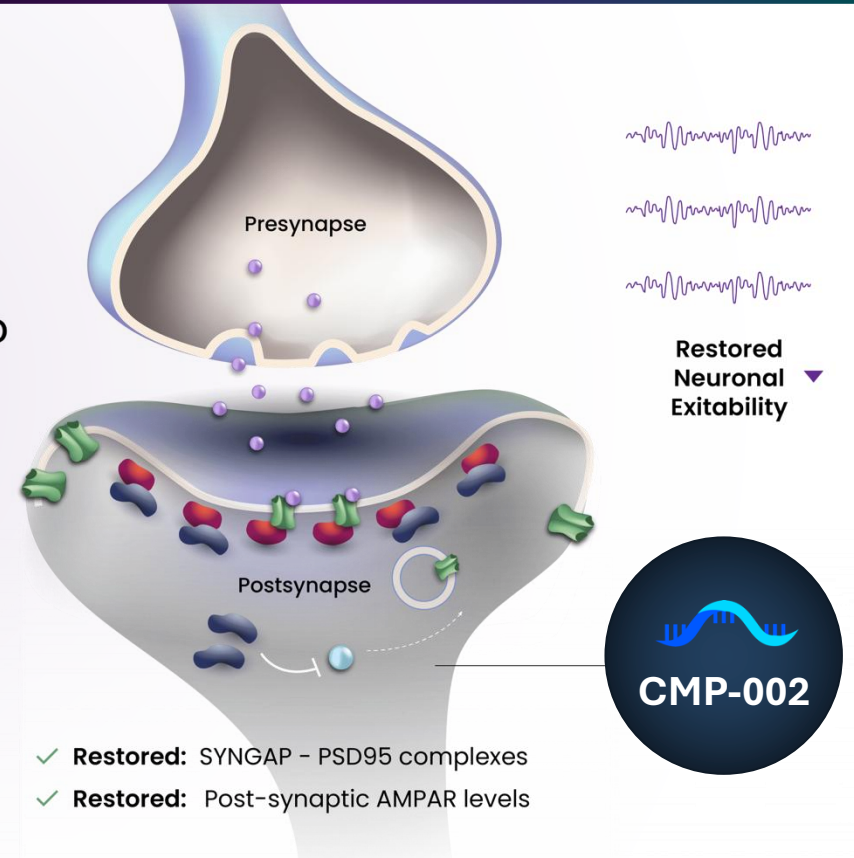
Mutations in *SYNGAP1* lead to decreased SYNGAP protein, causing increased synaptic firing

50%
LOWER
SYNGAP
PROTEIN



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

RESTORED
SYNGAP
PROTEIN



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

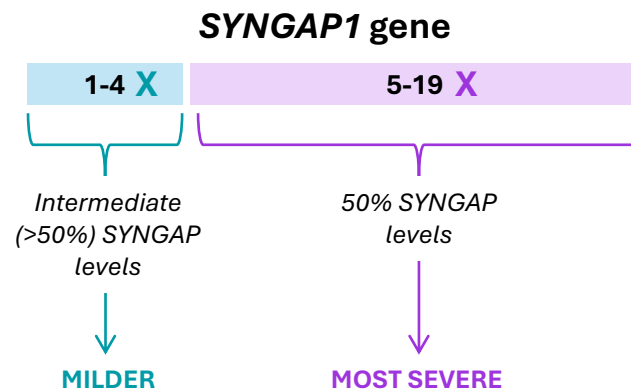


Opportunity in SYNGAP1 driven by unmet need and compelling preclinical data

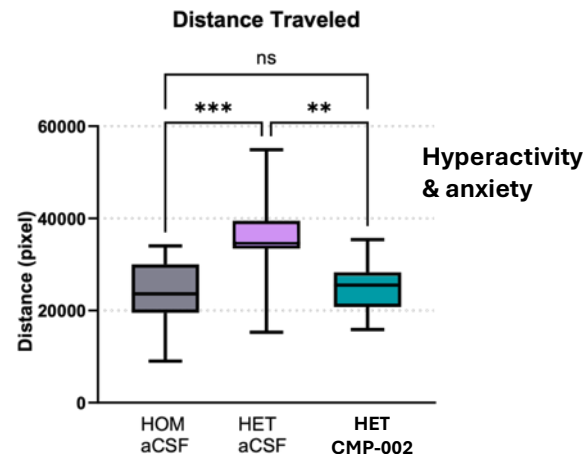
- Ultra-rare SYNGAP1 sub population with intermediate SYNGAP levels equate to milder disease state
- CMP-002 rescued functional defects in relevant human mouse model
- IT administration in NHPs well-tolerated and showed significant increase in SYNGAP in key brain regions

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

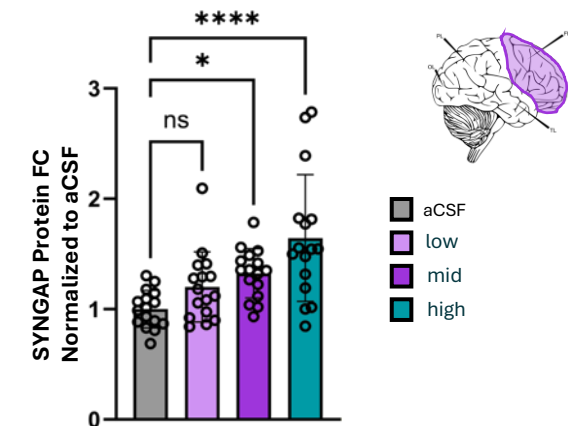
Milder disease severity (verbal responses, milder epilepsy) in minority of patients with intermediate SYNGAP levels



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

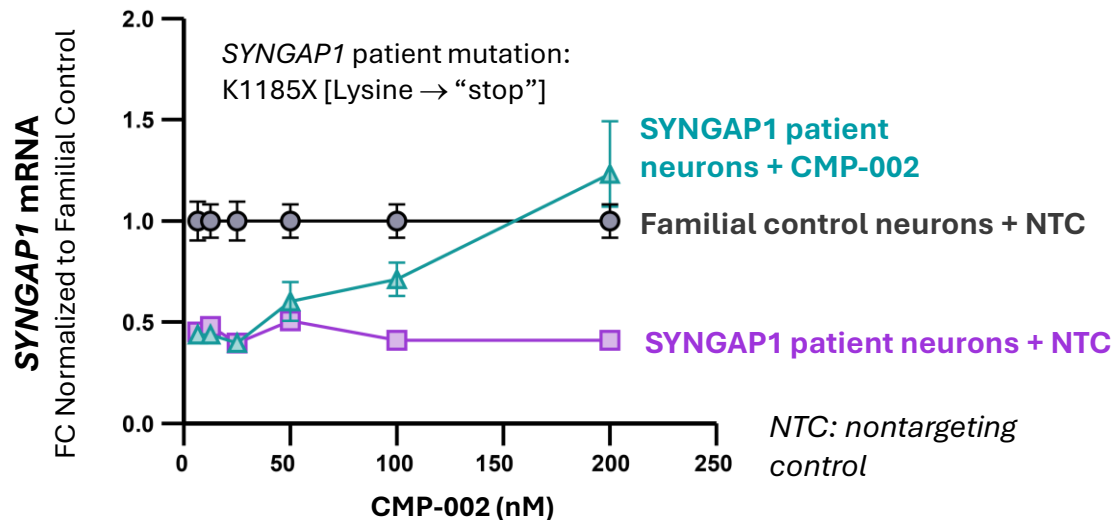


CMP-002 delivered intrathecally increased SYNGAP in disease-relevant brain regions in monkeys

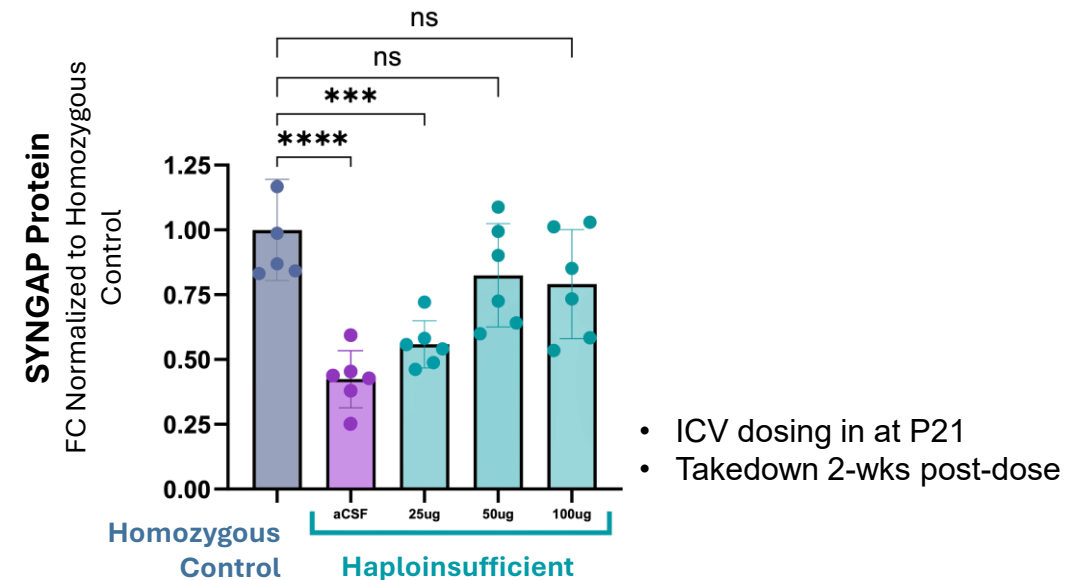


CMP-002 restores SYNGAP levels in models of haploinsufficiency

SYNGAP1 Patient iPSC-derived neurons



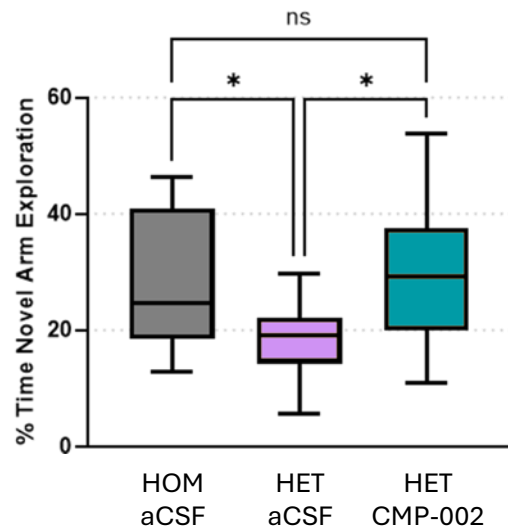
“Humanized” SYNGAP1 mouse



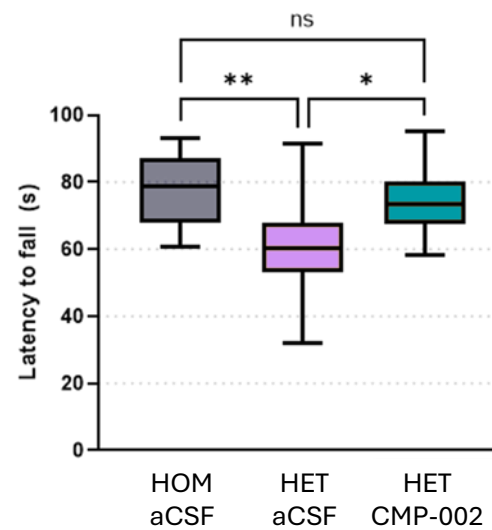
Dose-dependent increase in SYNGAP approaching wild-type levels → both ASO activity and regRNA function translates from human patient-derived neurons in a dish to neurons in an intact animal brain

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

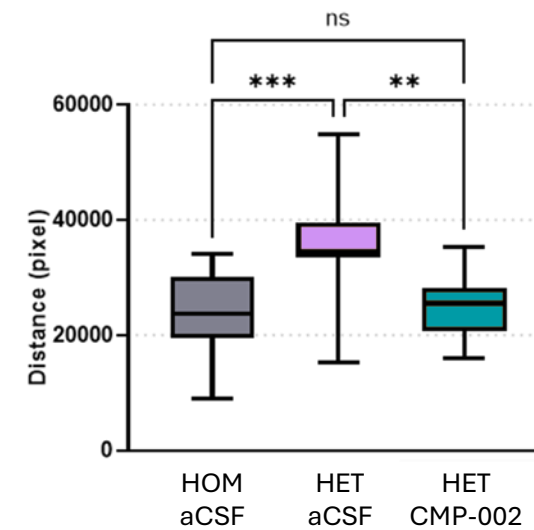
Learning & Memory



Motor Function



Hyperactivity



aCSF =
artificial CSF

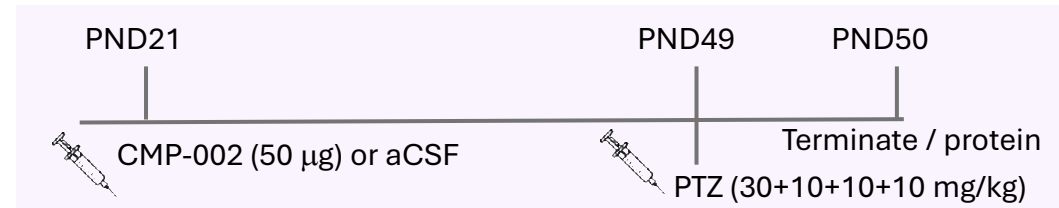
*, p < 0.05
**, p < 0.01
***, p < 0.001

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

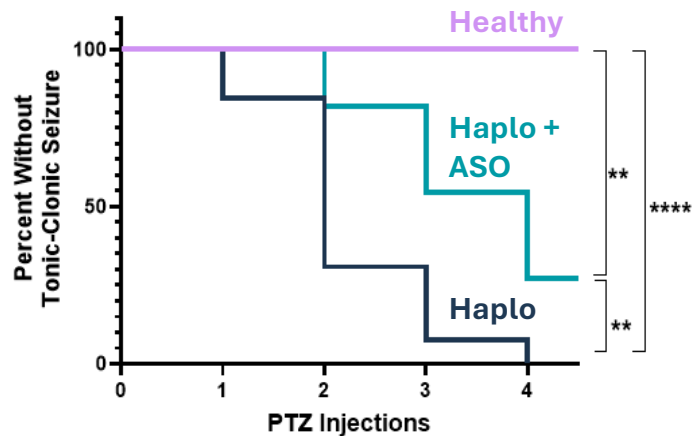
Restoring SYNGAP protein to near wild-type levels ameliorates multiple behavioral phenotypes caused by *SYNGAP1* haploinsufficiency

CMP-002 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

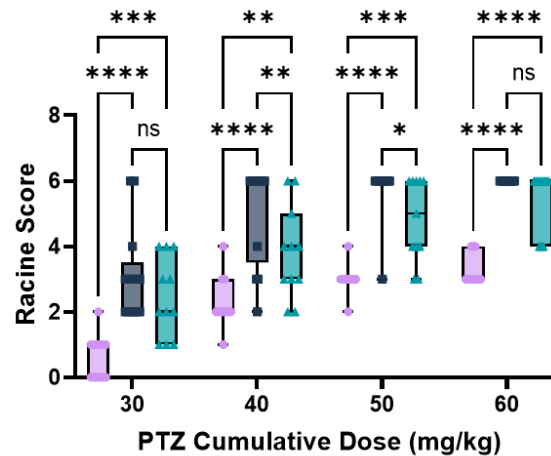


Seizure Threshold

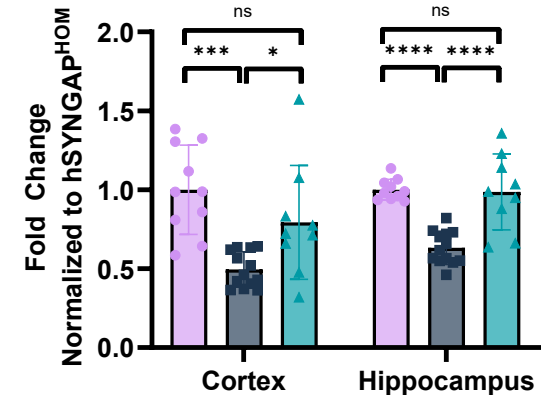


hSYNGAP^{HOM} – aCSF (n=11)
hSYNGAP^{HET} – aCSF (n=13)
hSYNGAP^{HET} – CMP-002 (n=11)

Racine Score

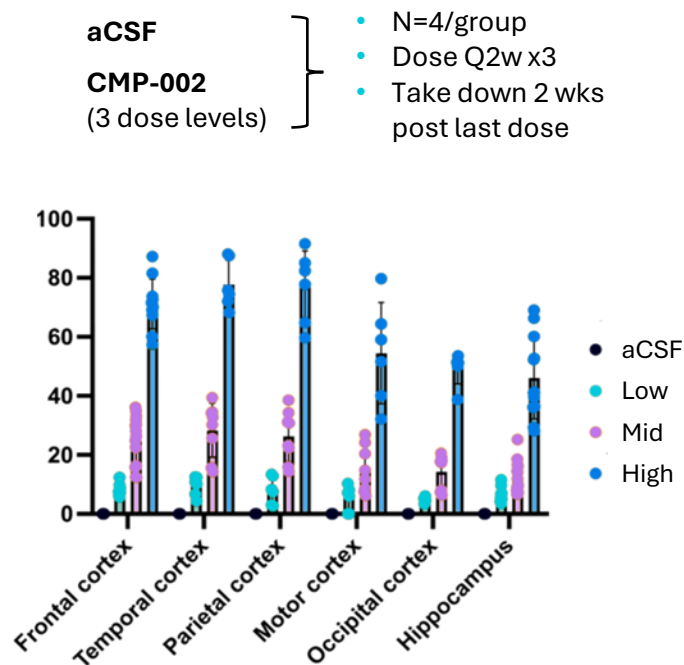


Protein Level

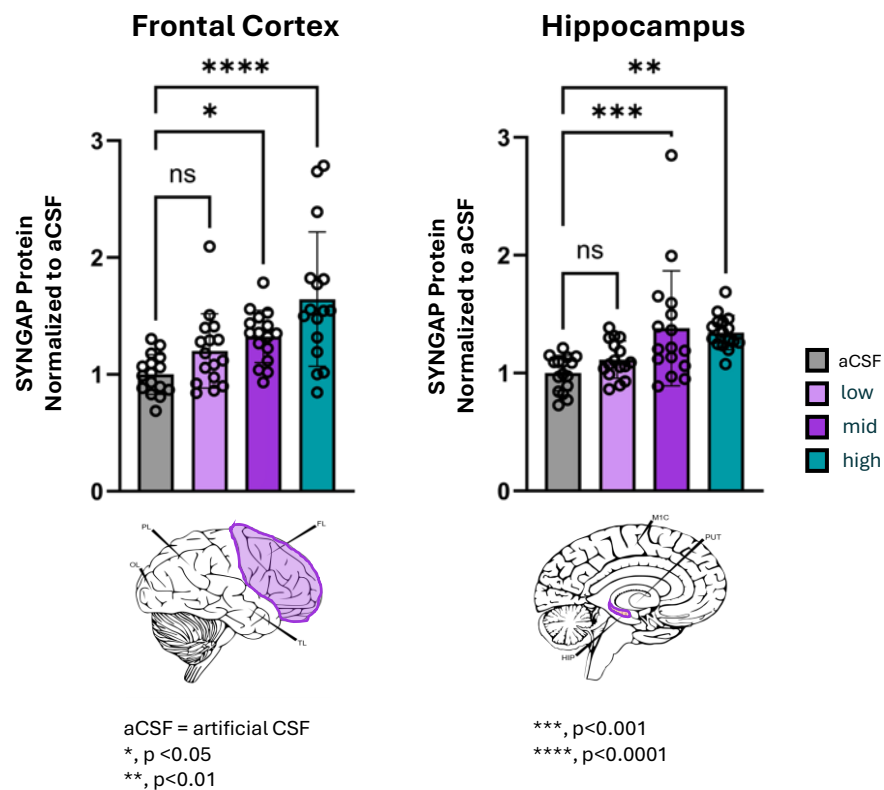


Intrathecal administration of CMP-002 to cynomolgus monkeys achieves broad brain distribution to increase SYNGAP protein levels

ASO Concentrations



SYNGAP Protein Levels



Summary

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

Broad ASO distribution throughout disease-relevant brain regions

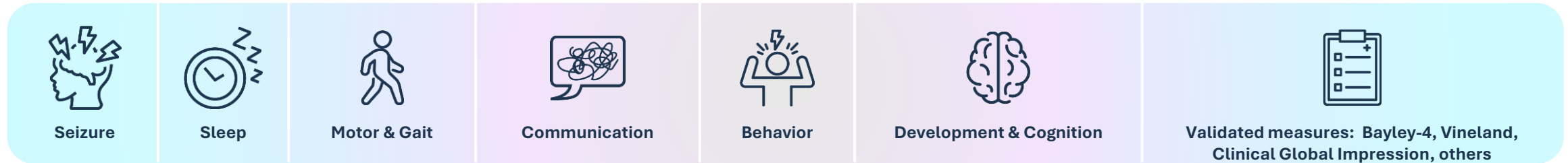
↑ SYNGAP protein throughout brain

CAMP4 positioned to be first in the clinic for SYNGAP1

Standard-of-Care	• No disease modifying therapies available • Patients currently managed symptomatically, with complications of polypharmacy
Natural History Study	• 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	• 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	• Clinical trial applications submitted to multiple global regulatory authorities; approval received in Australia and Argentina • Planning to initiate global Ph1/2 study in patients in Q4 2026
Path to Approval	• 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



Kelly Gold
Chief Financial Officer



Dan Tardiff, PhD
Chief Scientific Officer



Yuri Maricich, MD
Chief Medical Officer



Caleb Moore
Chief Business
Operations Officer



Michelle Gates
Chief People Officer



Alla Sigova, PhD
SVP, Head of Platform



Satya Kuchimanchi, PhD
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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
 - 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