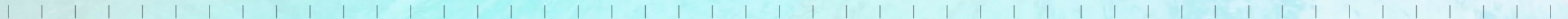




CAMP4 Analyst Event

September 28, 2026



Forward-Looking Statements

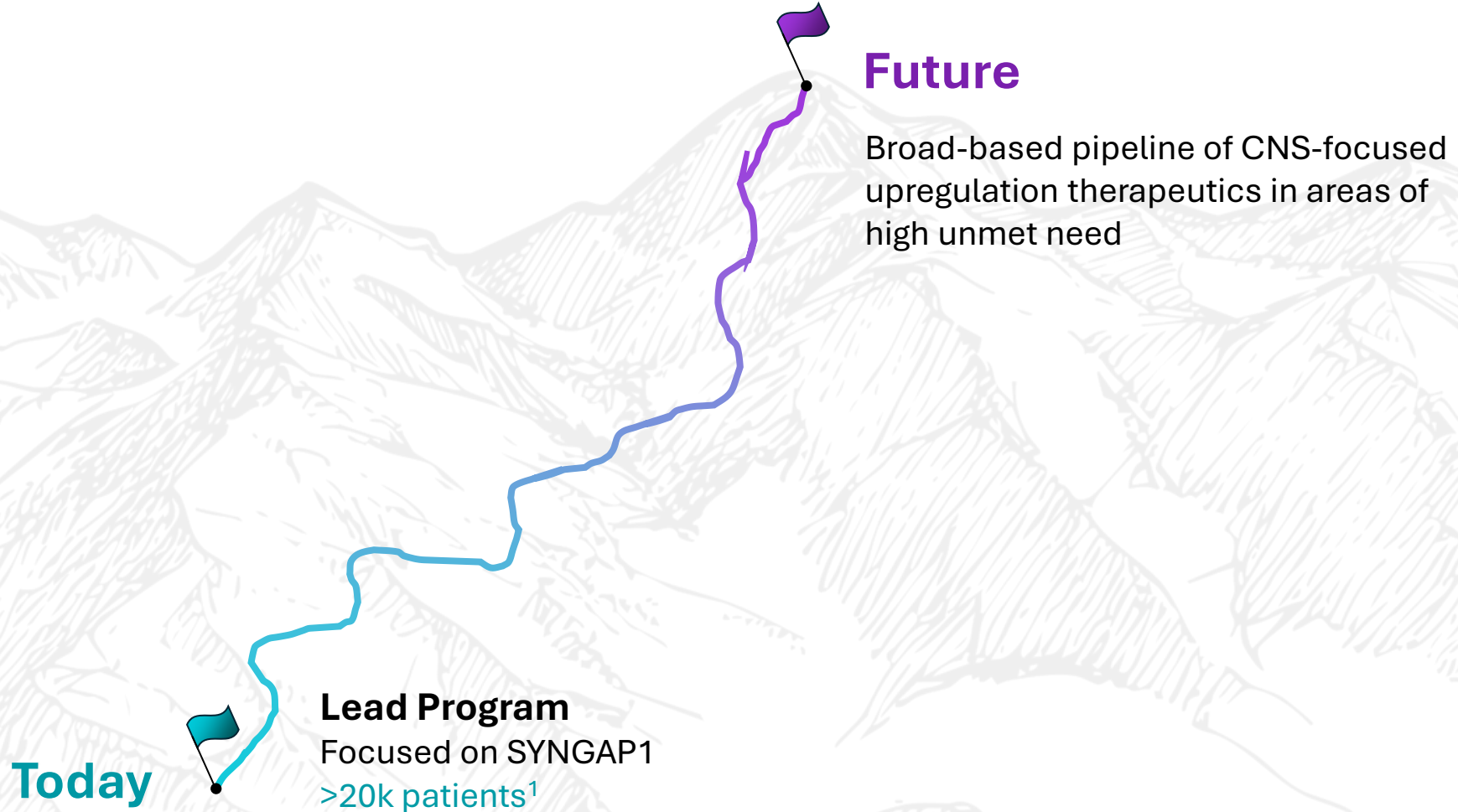
This presentation contains forward-looking statements that are based on the beliefs and assumptions and on information currently available to CAMP4's management. All statements other than statements of historical fact contained in this presentation are forward-looking statements. Forward-looking statements 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



RAP Platform®

Addressing the root cause of genetic disease by restoring healthy protein levels

Video: Patient story



ASCEND clinical trial in SYNGAP1

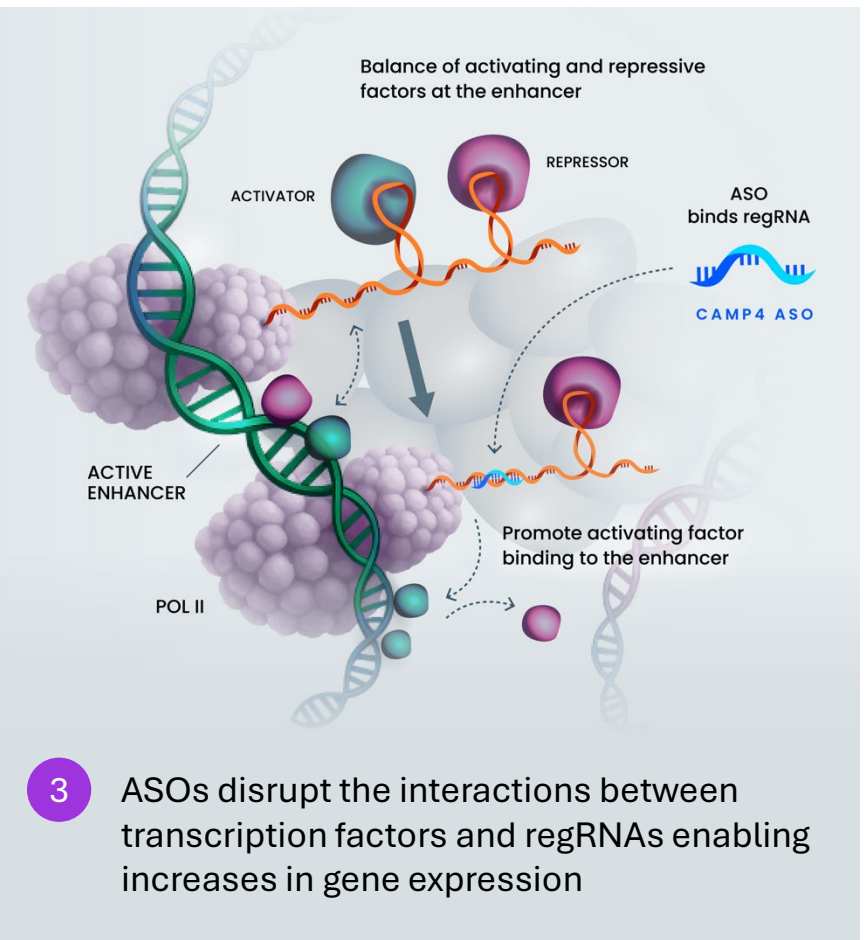
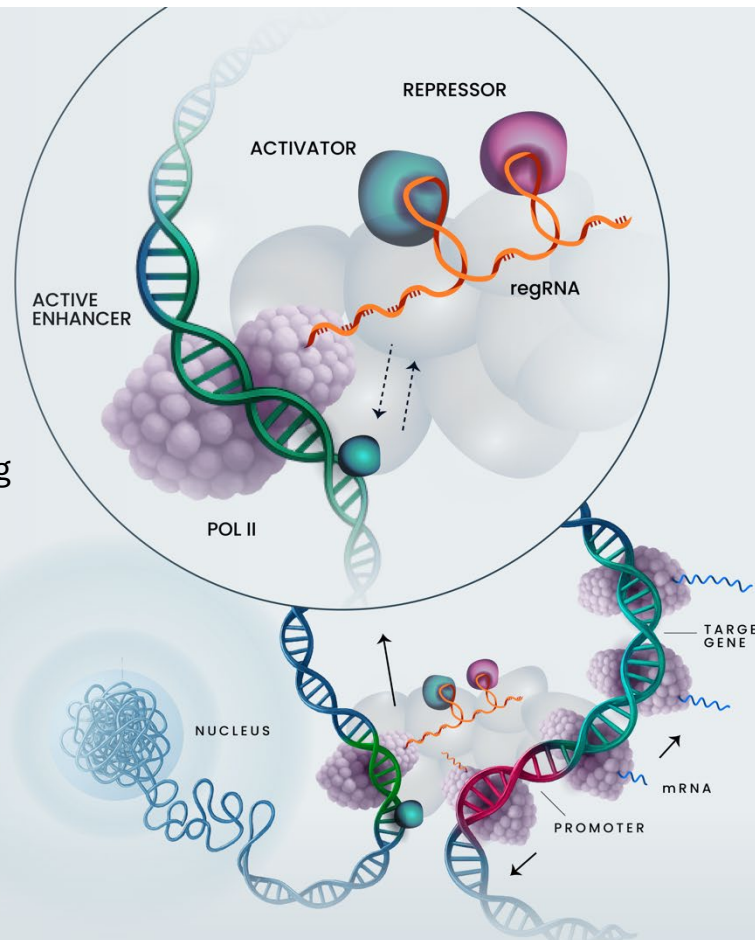
- ASO therapy for SYNGAP1 Clinical Evaluation in Neuro Development

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

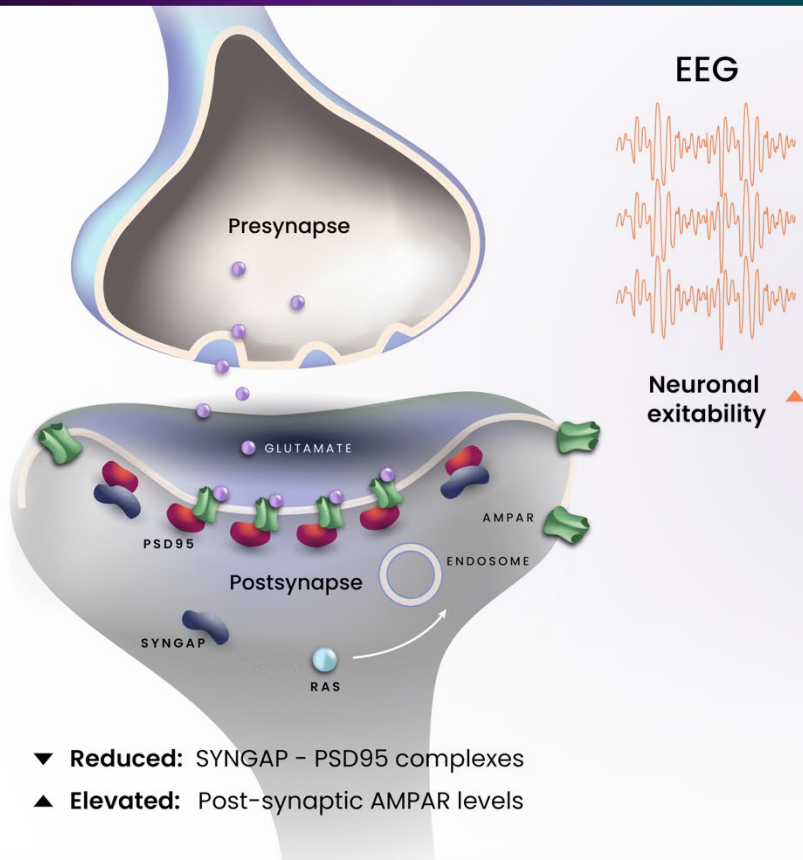


3 ASOs disrupt the interactions between transcription factors and regRNAs enabling increases in gene expression

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

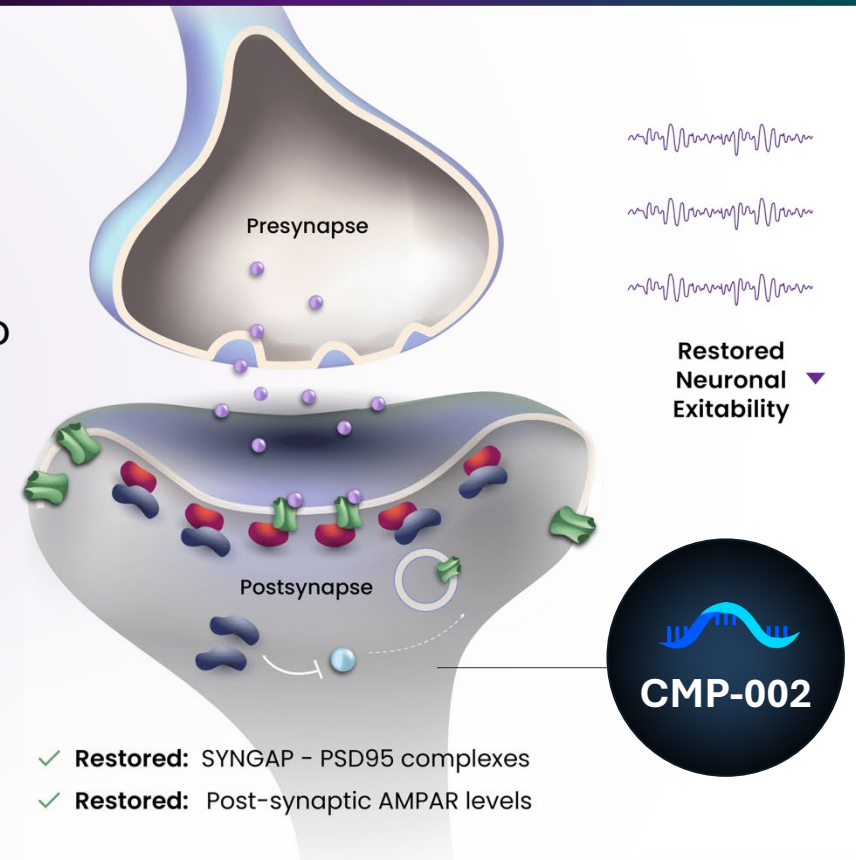
Mutations in *SYNGAP1* lead to decreased SYNGAP protein, causing increased synaptic firing

50%
LOWER
SYNGAP
PROTEIN



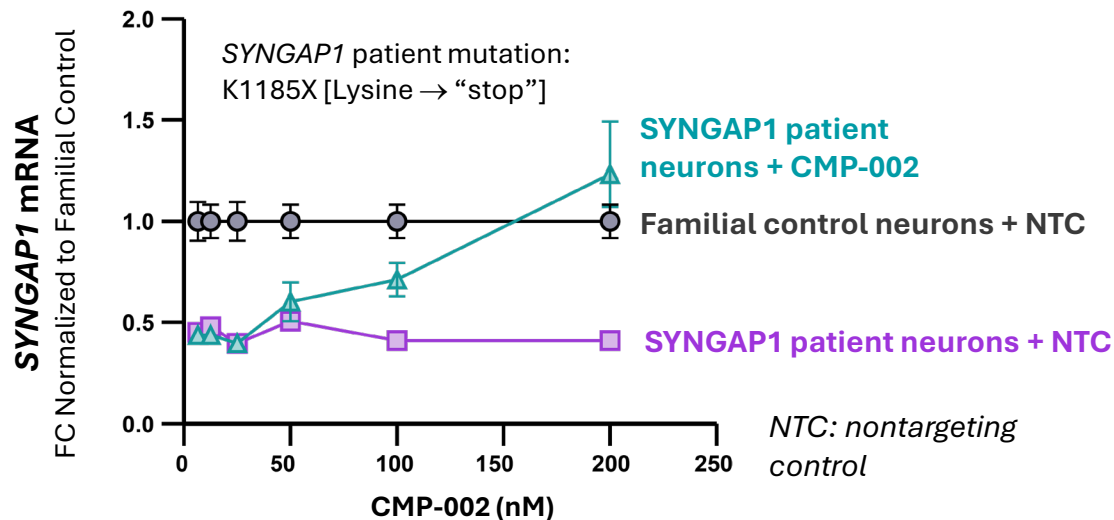
CMP-002 binds to a SYNGAP-specific regRNA to increase *SYNGAP1* expression, aiming to restore SYNGAP towards wild-type levels and normalize synaptic function

RESTORED
SYNGAP
PROTEIN

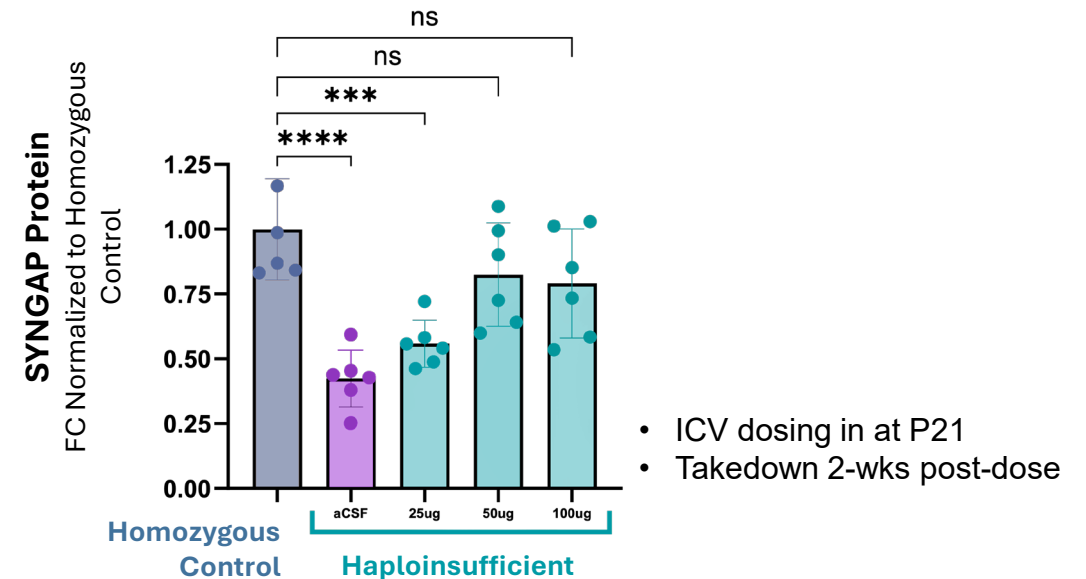


CMP-002 restored SYNGAP levels in models of haploinsufficiency

SYNGAP1 Patient iPSC-derived neurons



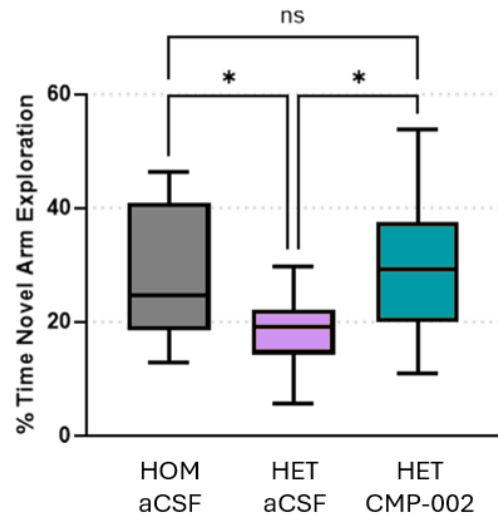
“Humanized” SYNGAP1 mouse



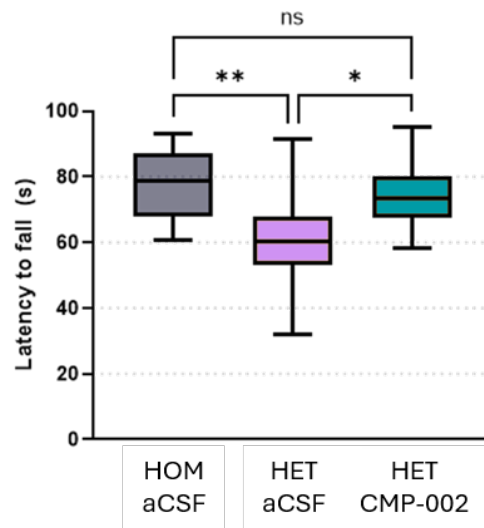
Dose-dependent increase in SYNGAP approaching wild-type levels → both ASO activity and regRNA function translated from human patient-derived neurons in a dish to neurons in an intact animal brain

CMP-002 broadly improved neurological phenotypes in humanized SYNGAP1 mice

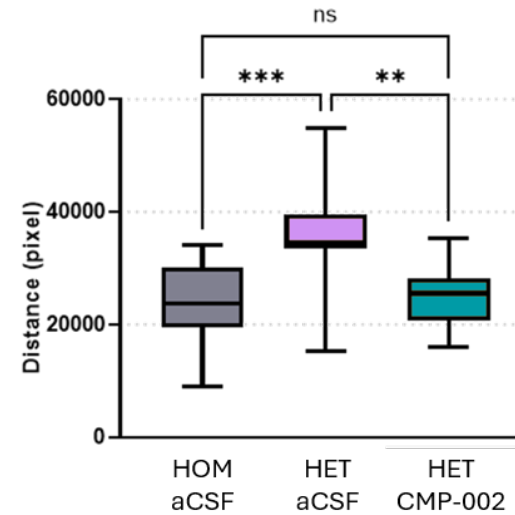
Learning & Memory



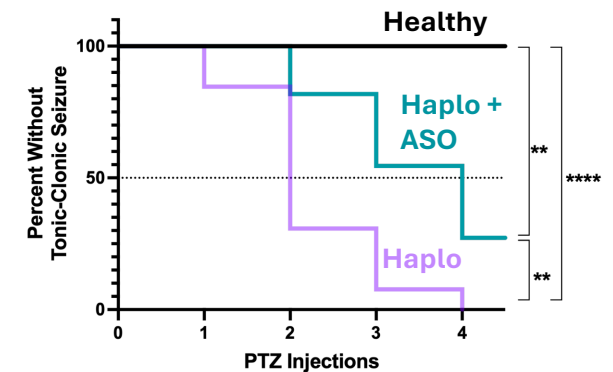
Motor Function



Hyperactivity



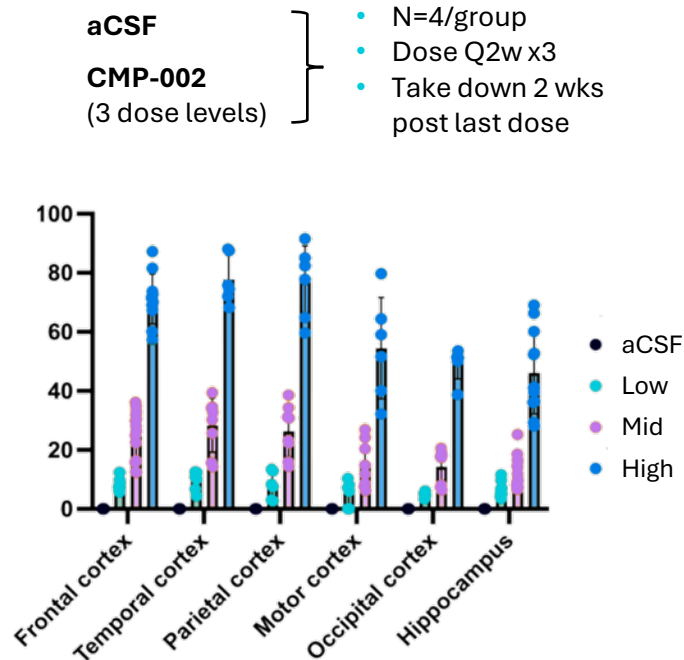
Seizures



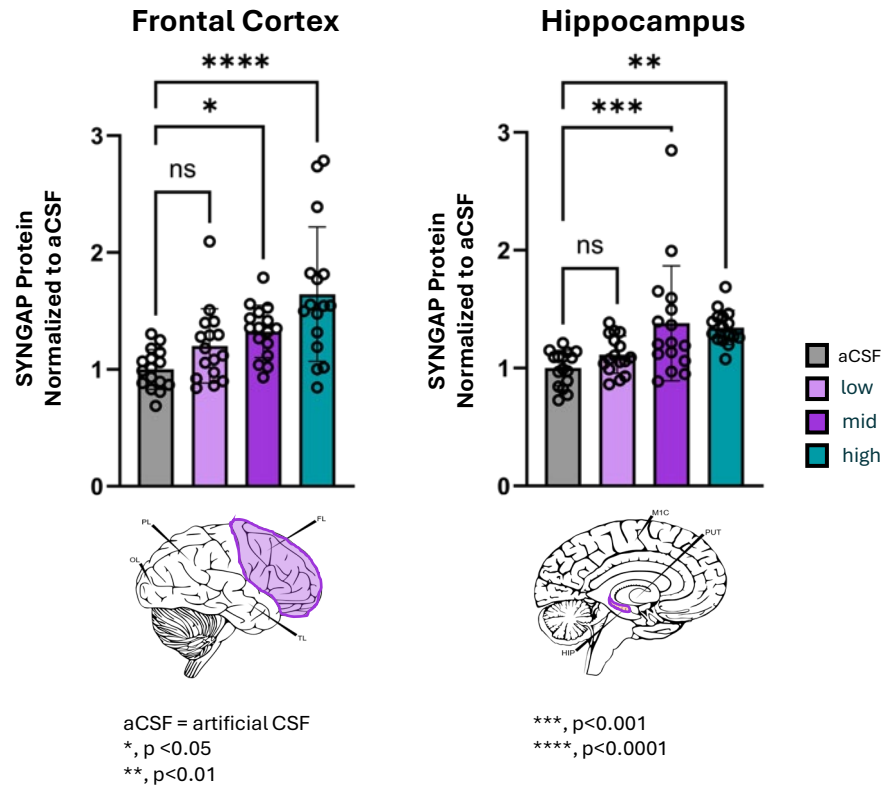
Broad benefit of CMP-002 supports potential translation across multiple phenotypes in SYNGAP1 patients

Intrathecal administration of CMP-002 to cynomolgus monkeys achieved 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

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 Wiltout, 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

N = 1,806

SYNGAP1 Census
as of Q2 2026

Advocacy-group collaboration
~200 new patients/year since 2020



N = 159

CAMP4
Sponsored

Prospective natural history
as of Q2 2026

Ongoing study 2023–2028;
Children's Hospital Colorado,
Stanford University, and CHOP

SYNGAP1
ProMMiS

N = 350

Retrospective EMR
cohort
as of Q3 2026

Curated real-world records,
cohort still expanding

Citizen
Health

N = 569

European registry (PATRE)
as of Q3 2026

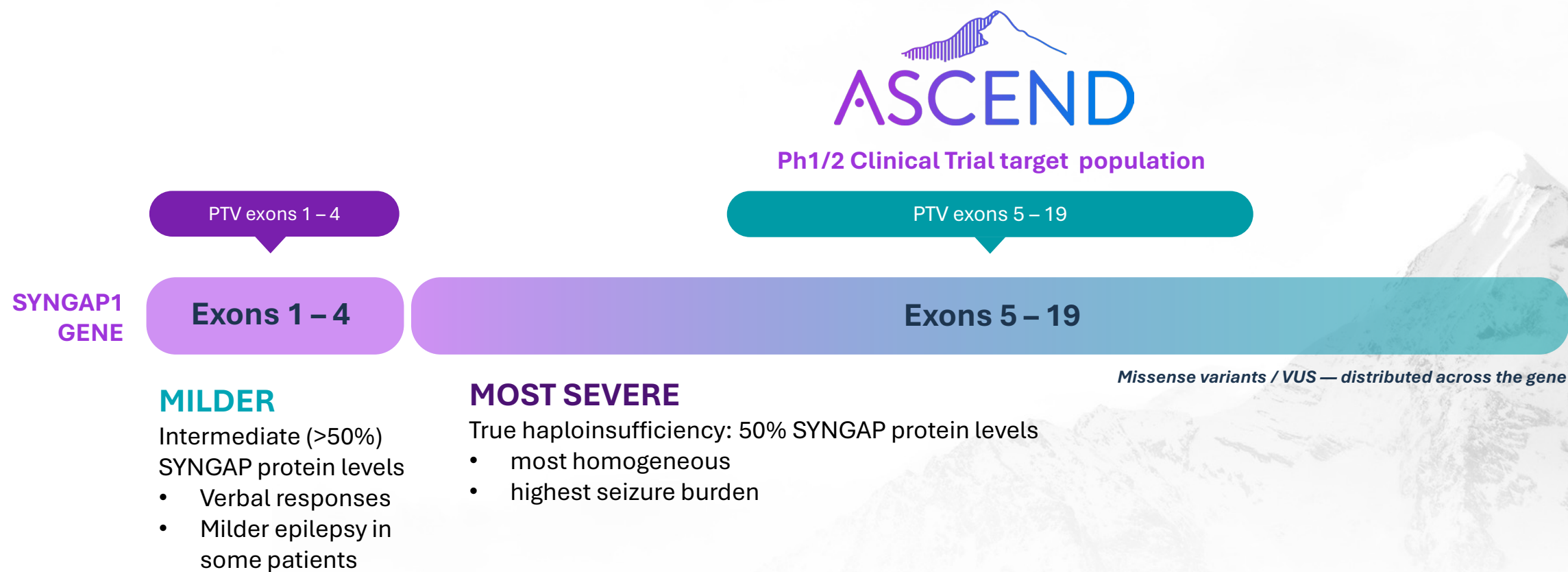
Broadens geographic representation

EURAS project,
PATRE SYNGAP1
registry

2,500+ patients characterized across census, prospective, real-world, and registry cohorts

Majority of SYNGAP1 patients have severe protein-truncating variants

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



SYNGAP1-related disorder impacts many aspects of neurological function

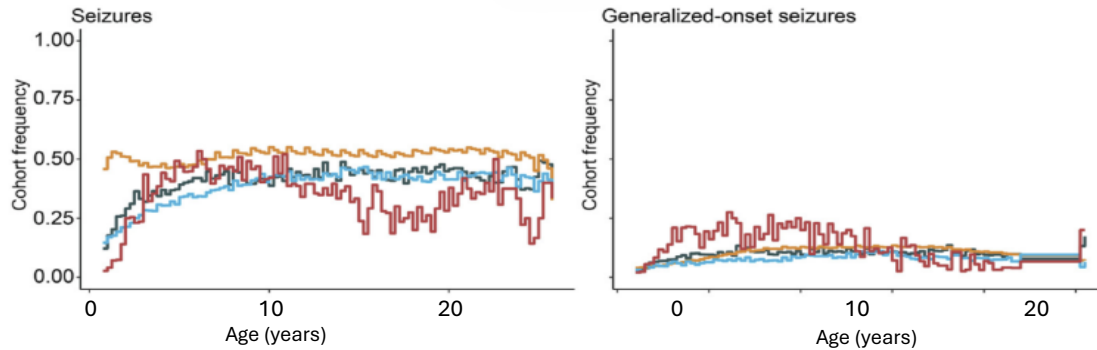
Phenotypic features of SYNGAP1-Related Disorder by prevalence

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	

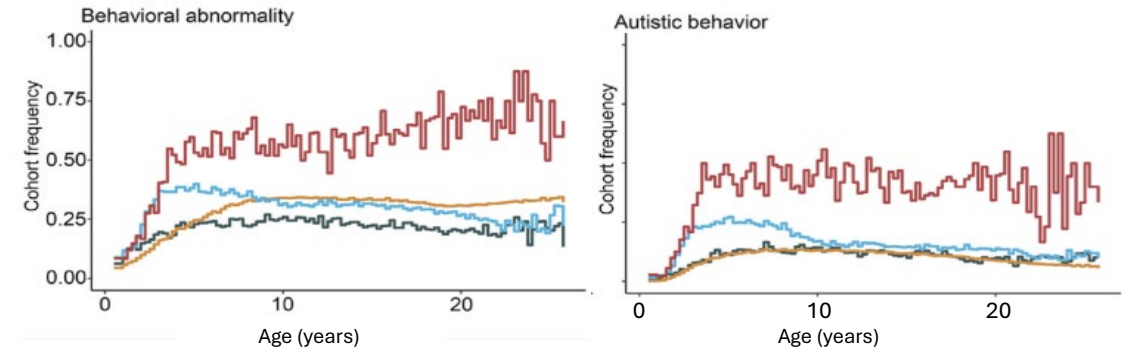
All patients display ID, majority with epilepsy, behavioral/autistic features and sleep problems

SYNGAP1 has similar or higher severity relative to analogue conditions

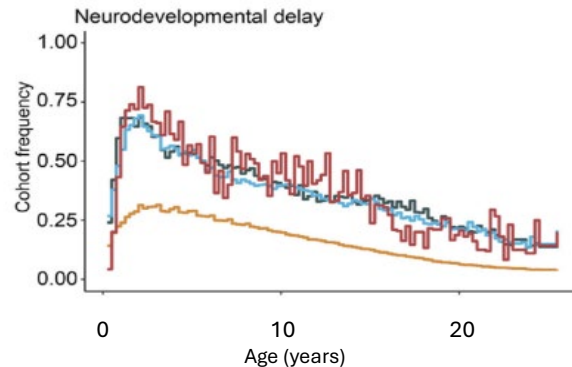
Seizures among highest frequency



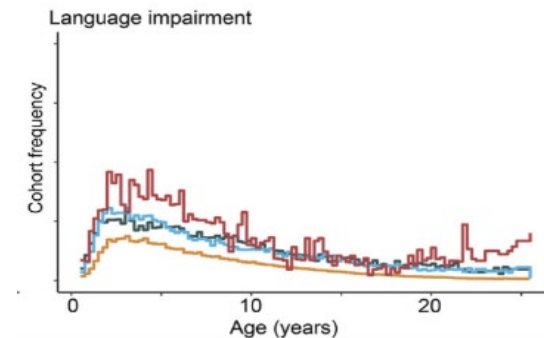
Difficult to manage autism-like behavior



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

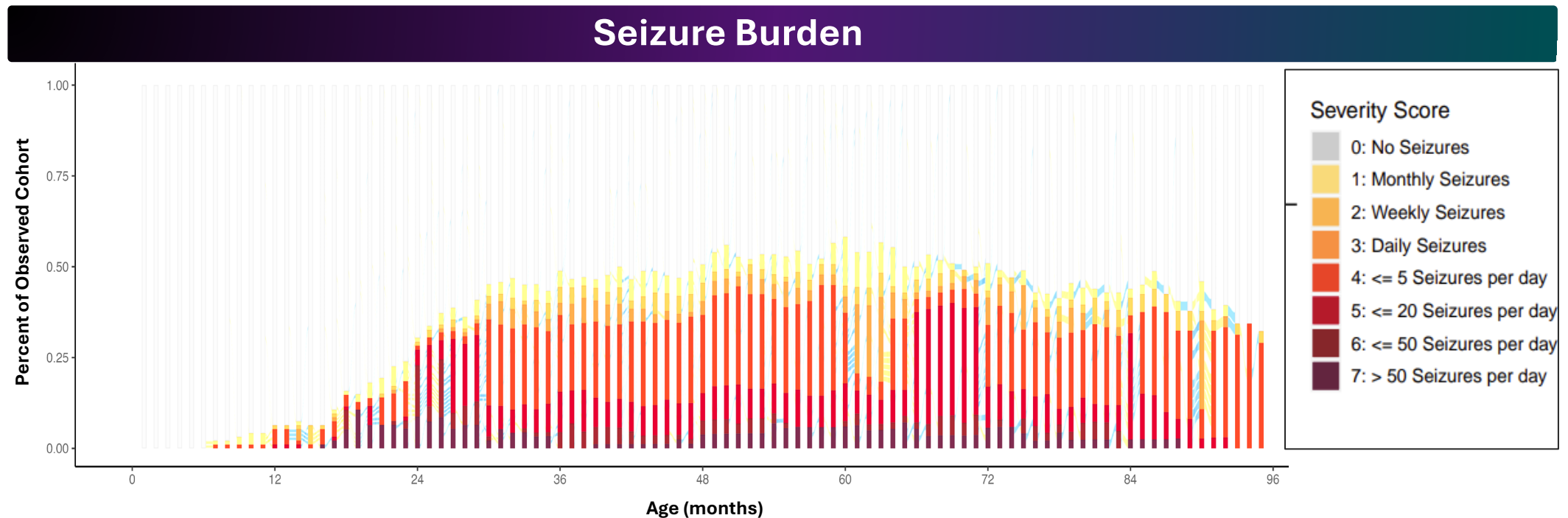


Communication impairment: many patients are non-verbal or have few words



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

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

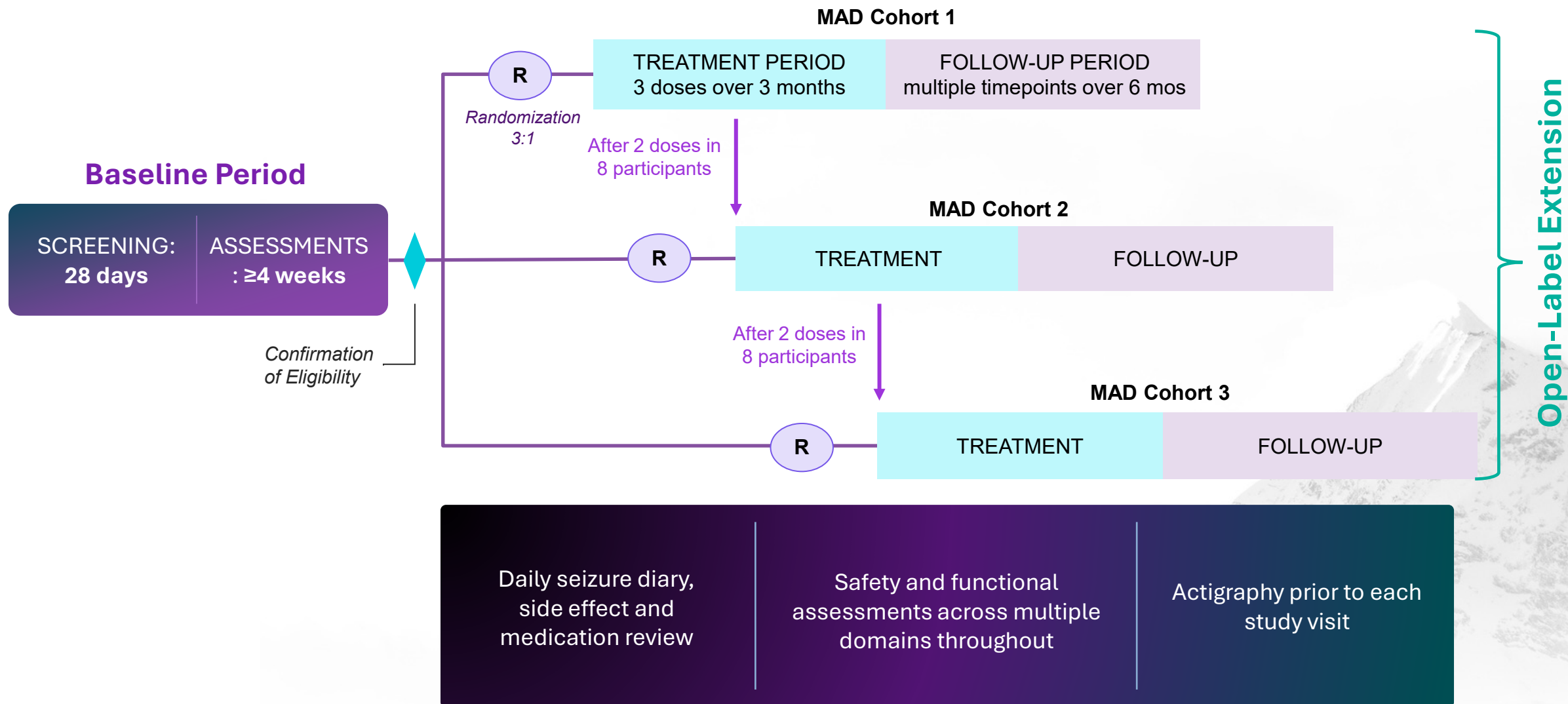
Key Inclusion Criteria

- ✓ **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

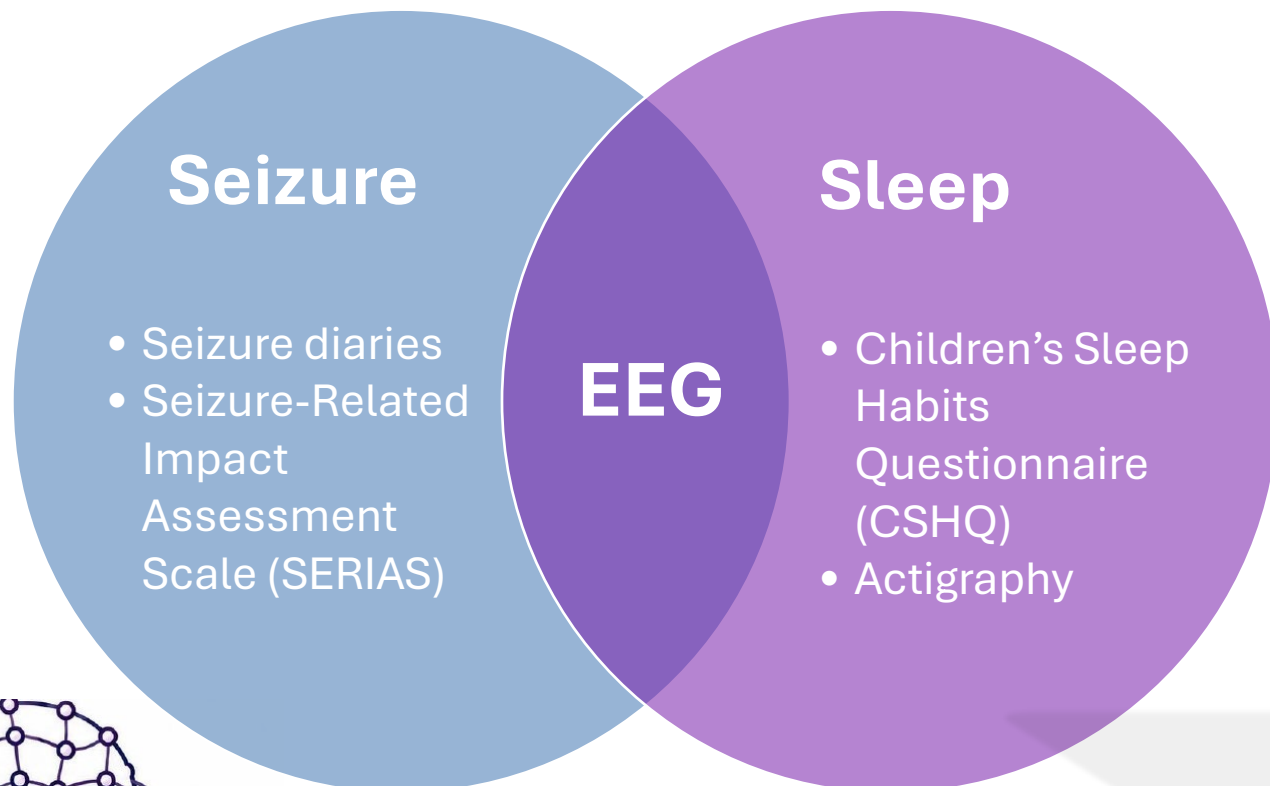
Scale	Communication	Motor	Behavior	Development
Bayley Scales of Infant and Toddler Development Bayley-4		✓	✓	✓
Vineland Adaptive Behavior Scales, 3rd Edition Vineland-3	✓	✓	✓	✓
Aberrant Behavior Checklist, 2nd Edition ABC-2	✓		✓	
Observer-Reported Communication Ability Measure ORCA	✓			
Gross Motor Function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

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

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

Planned global footprint of ASCEND Phase 1/2 clinical trial

11+

Total sites

9

Countries

5

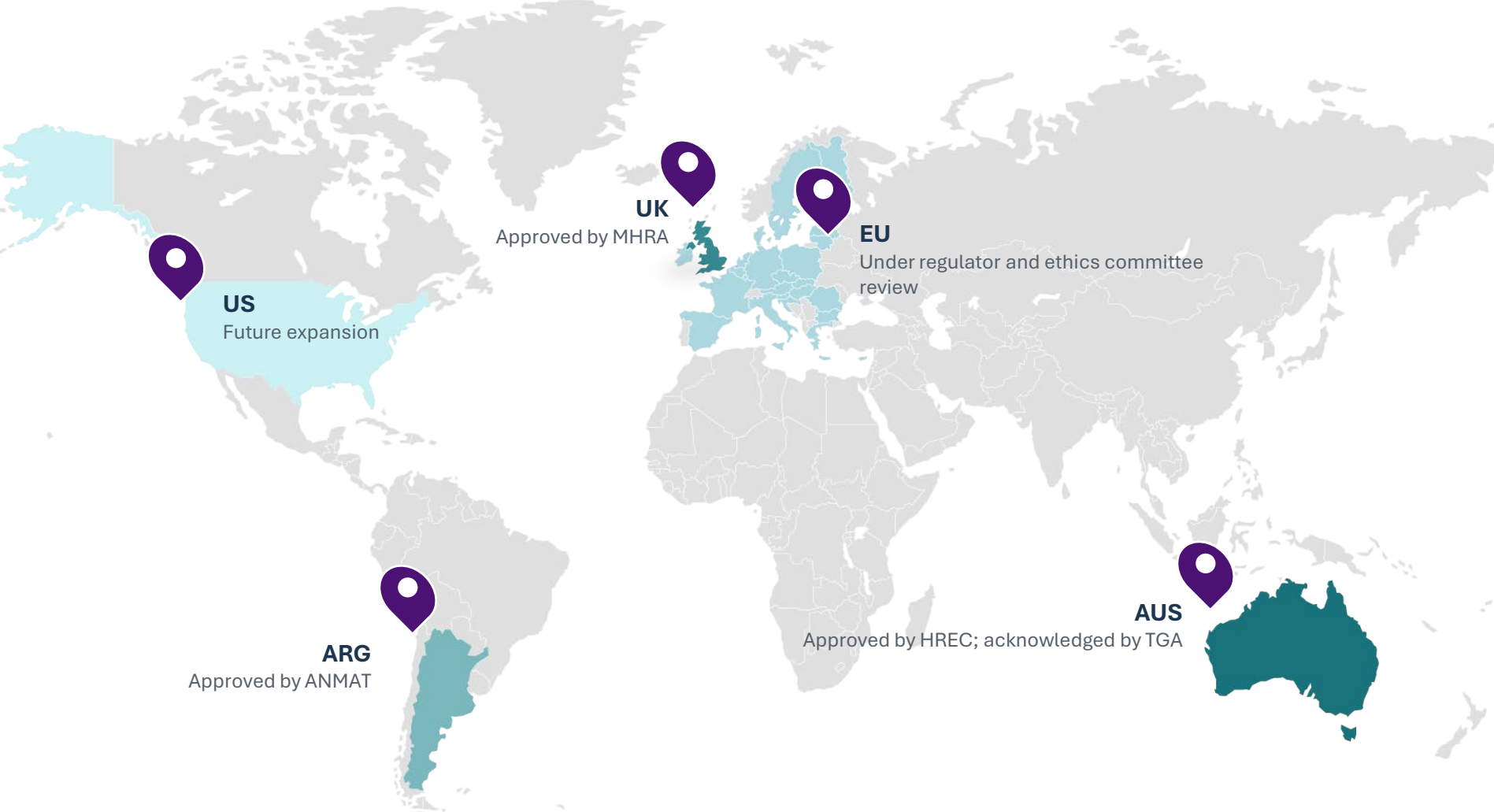
Regions with sites planned

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



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 US¹ with a well-organized and sophisticated patient advocacy group



Overlapping KOLs & physician network with SYNGAP1

SHANK3 – Phelan-McDermid Syndrome

Analyst Q&A

