Developing regulatory RNA-based therapeutics for DEEs and an ASO to increase SYNGAP gene expression

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CAMP4 Therapeutics Corp.
09/25/25

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Disclosure

Yuri Maricich, M.D. is an employee and equity holder of CAMP4 Therapeutics Corp.

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

NASDAQ: CAMP

There are prevalent and genetic diseases where gene upregulation is likely to have a meaningful clinical benefit

CAMP4 is the leader in gene regulatory RNA (regRNA) discovery and regRNA-targeting antisense oligonucleotide (ASO) therapies to upregulate gene expression to restore healthy protein levels

Our proprietary RAP Platform® was built for the discovery of novel regRNAs that regulate the expression of every protein-coding gene

Our current focus is on haploinsufficient or genetic diseases where modest increases in protein expression could have a clinically meaningful benefit

CAMP4 is rapidly advancing CMP-SYNGAP-01 into the clinic as early as 2H 2026. Safety, PK, and biomarker data from Phase 1 study of CMP-CPS-001 for Urea Cycle Disorders expected in Q4 ’25

CAMP4: Pioneering a new class of medicines to increase gene expression

- Pioneering the discovery and development of regulatory RNA-targeting antisense oligonucleotide (ASO)-based therapies to upregulate gene expression and restore healthy protein levels
- ~50 employees, headquartered in Kendall Square, Cambridge, MA
- Founders from Whitehead Institute, Boston Children's Hospital, Harvard Medical School

IPO: OCT 2024 | NASDAQ: CAMP

Advancing a pipeline in genetic diseases

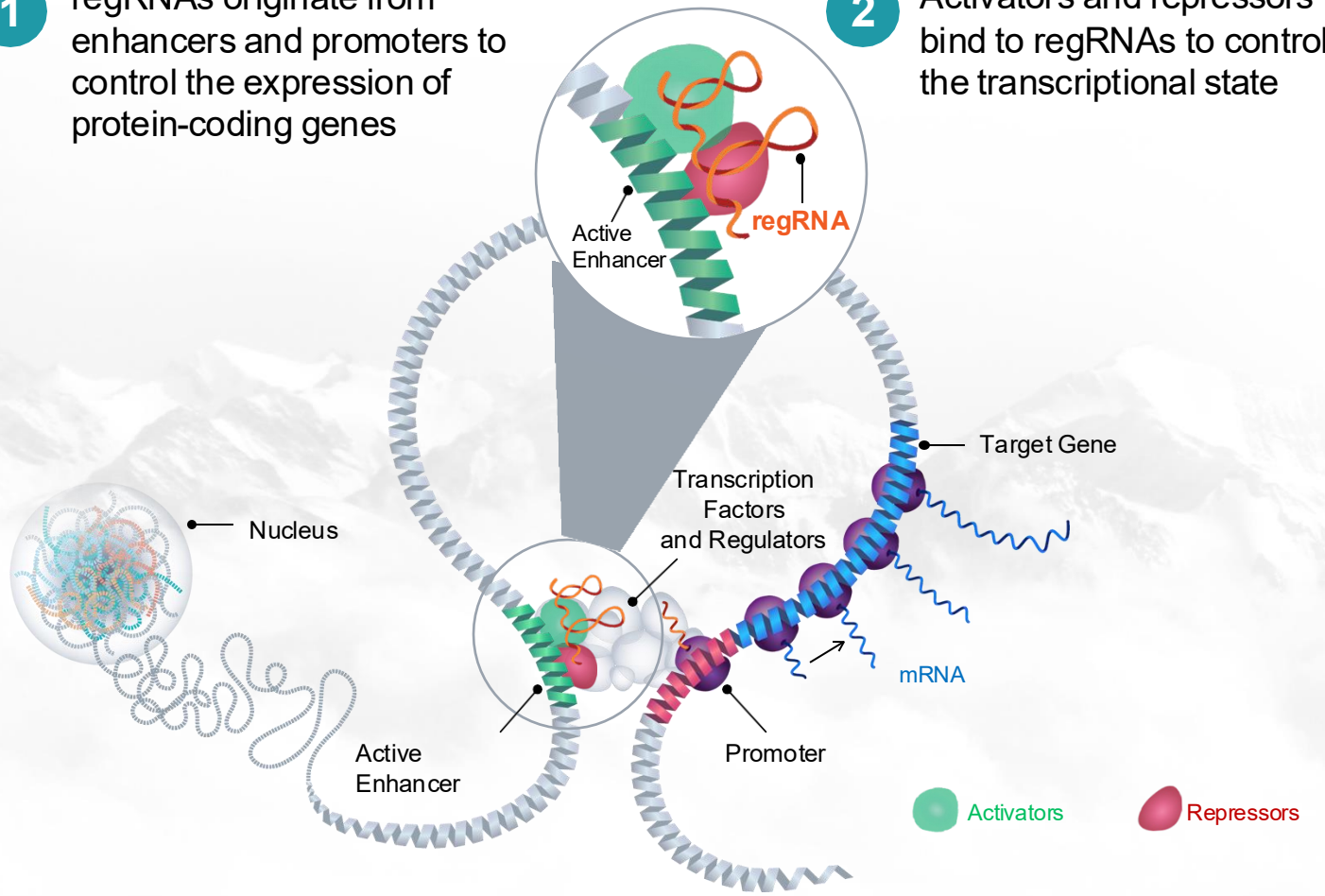
Program	Indication	Target	Discovery & Preclinical	Phase 1	Phase 2	Phase 3	Anticipated Milestones	Commercial Rights
CNS diseases								
CMP-SYNGAP-01	SYNGAP-related Disorders	SYNGAP					GLP tox studies in '25 Clinical initiation in '26	CAMP4
Discovery	Genetically defined Parkinson's disease (PD) and sporadic PD	GBA1						CAMP4
New Discovery Programs	CNS	Numerous	Active discovery and development of multiple programs utilizing RAP Platform					CAMP4
Metabolic diseases								
CMP-CPS-001*	Urea Cycle Disorders	CPS1						CAMP4
Collaborations								
Strategic research collaboration leveraging CAMP4's RAP Platform advancing novel therapeutics that increase protein levels by targeting regRNA sequences for two genetic targets.								BIOMARIN

*CAMP4 to evaluate potential next steps for the further development of CMP-CPS-001 based on data readout anticipated in Q4 2025, which may include exploring potential partnership opportunities.

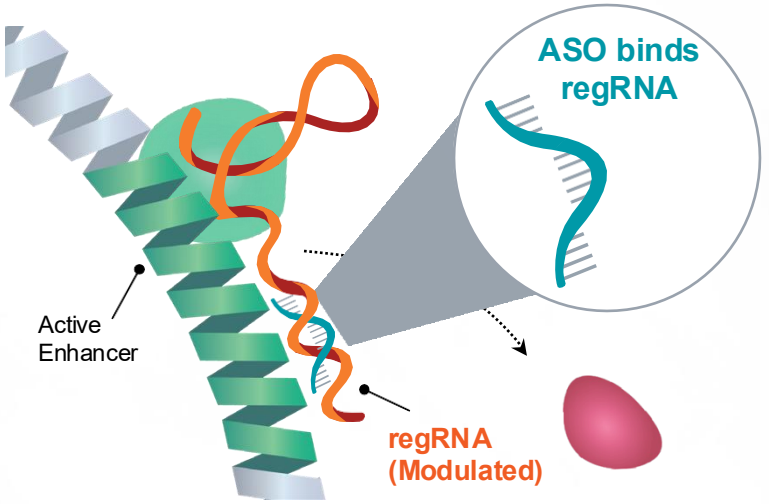
regRNAs play a central role in the regulation of every gene's expression

1 regRNAs originate from enhancers and promoters to control the expression of protein-coding genes

2 Activators and repressors bind to regRNAs to control the transcriptional state



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



Increased mRNA expression

Addresses root cause of **haploinsufficient or partial loss-of-function diseases by** returning **targeted protein levels** to within a healthy range

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.

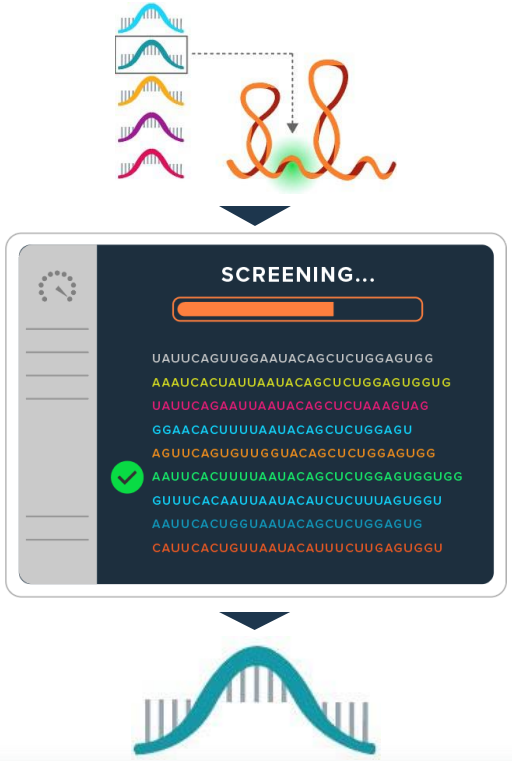
CAMP4's proprietary RAP Platform® catalogs thousands of regRNAs in any tissue and generates ASO leads to increase gene expression

1. Map candidate regRNAs

Next-gen sequencing and machine learning algorithms map regRNAs controlling every expressed gene.

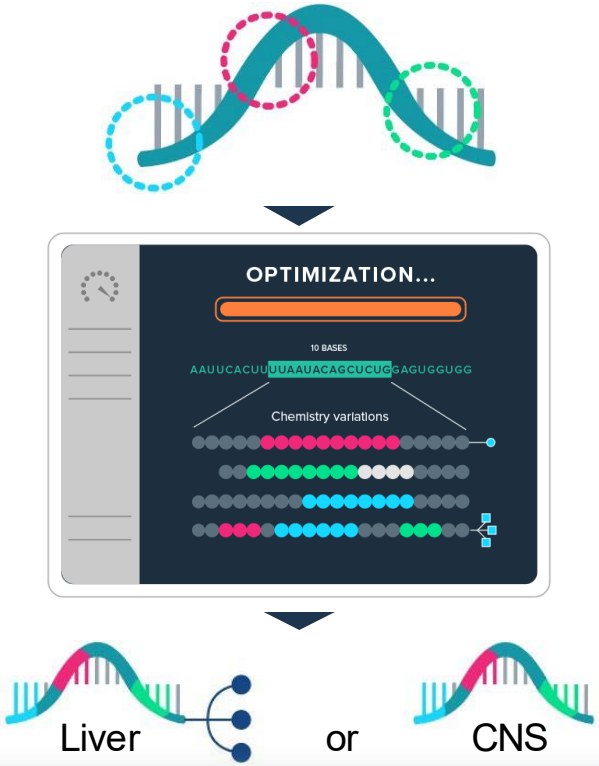
One of the most extensive, proprietary regRNA databases to date across wide range of tissue types.

2. Generate ASO leads



Rapidly pinpoint regions where ASO binding results in optimal upregulation of a target gene

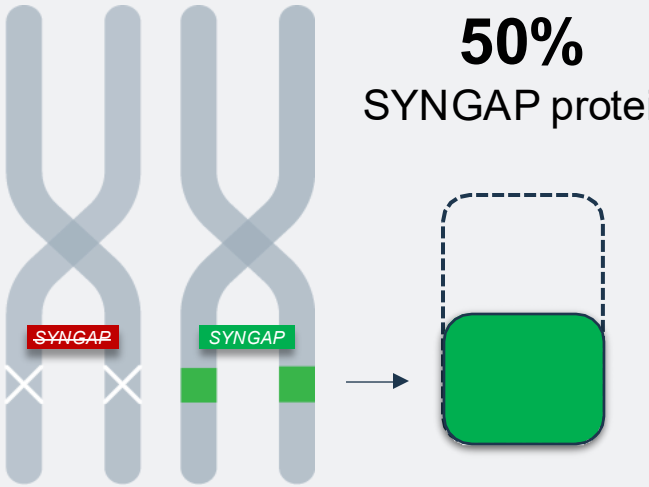
3. Optimize leads



Therapeutic candidates are designed to integrate a range of chemical modifications and tissue-targeting delivery strategies optimizing potency and safety

SYNGAP is a haploinsufficiency with 10,000+ US patients in need of therapy

SYNGAP Haploinsufficiency



50%
SYNGAP protein

6.1–10 per 100K
incidence rate [1,2]

F78.A1

ICD-10 code
assigned in 2021

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

0
approved therapies

Haploinsufficiency results in 50% of normal protein levels

10,000+
SYNGAP patients in the US

High unmet need for disease-modifying therapy

[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 SYNGAP disease course

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]

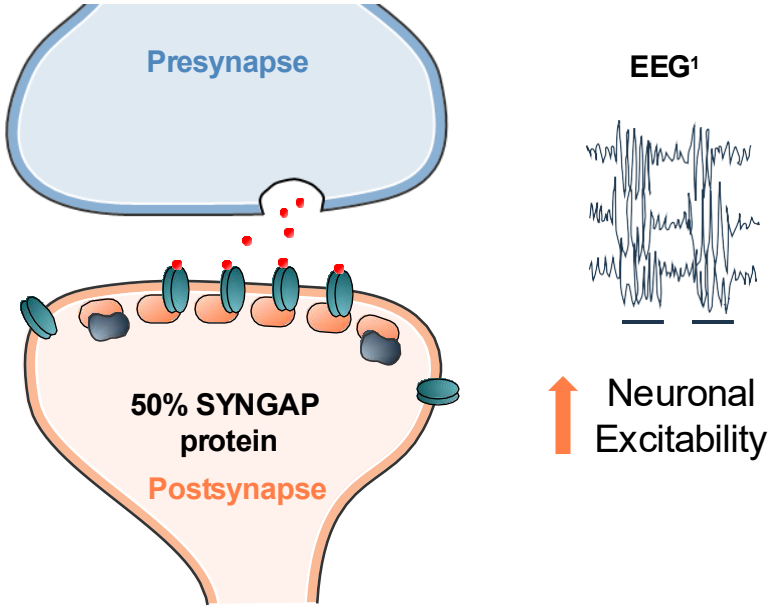
No Approved Therapy

- Non-specific treatments have limited impact on SYNGAP symptoms
  - Anti-seizure medications
  - Cannabinoids
  - Sleep medications
- Polypharmacy is common – *Patient regimen* [6] *example:*
  - *Epidiolex*
  - *Ravicti*
  - *Sodium bicarb*
  - *Amantadine*
  - *Trazodone in PM*
  - *Melatonin in PM*
  - *1:1 CBD/THC in PM*
  - *Small dose of Onfi*
- Constant patient care needed
  - Caregivers vigilant at all times

[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] *SYNGAP Research Fund (SRF)*

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

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

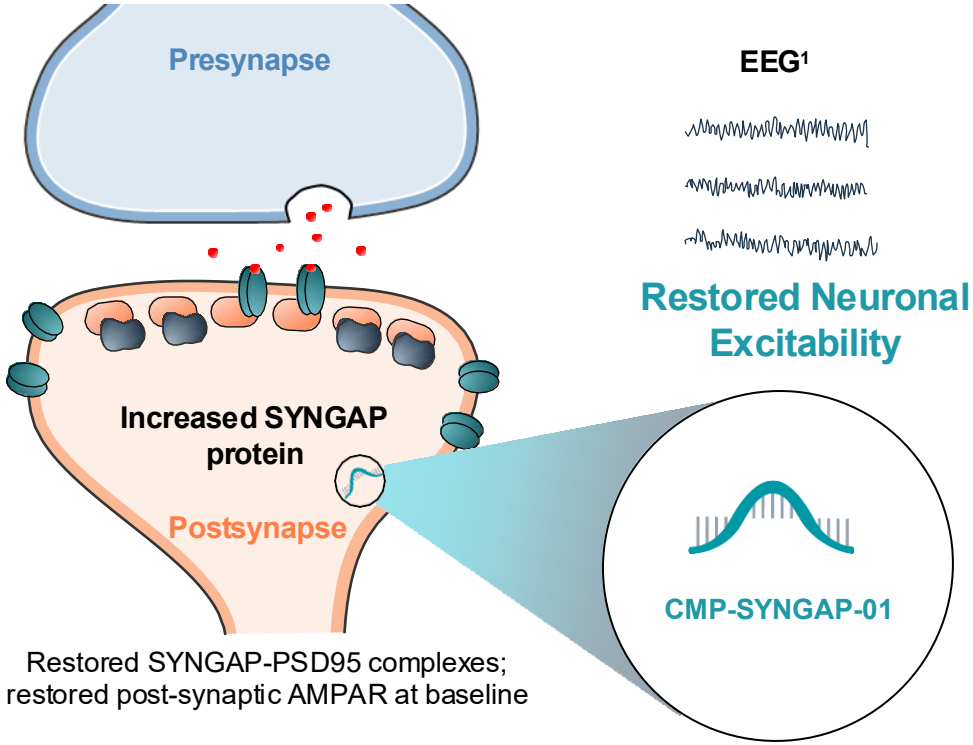


Neuronal Excitability

Reduced SYNGAP-PSD95 complexes; increased post-synaptic AMPAR at baseline

PSD95 SYNGAP AMPAR Glutamate

CMP-SYNGAP-01 binds to a *SYNGAP*-specific regRNA to increase *SYNGAP* expression, aiming to restore SYNGAP towards wild-type levels and normalize synaptic function

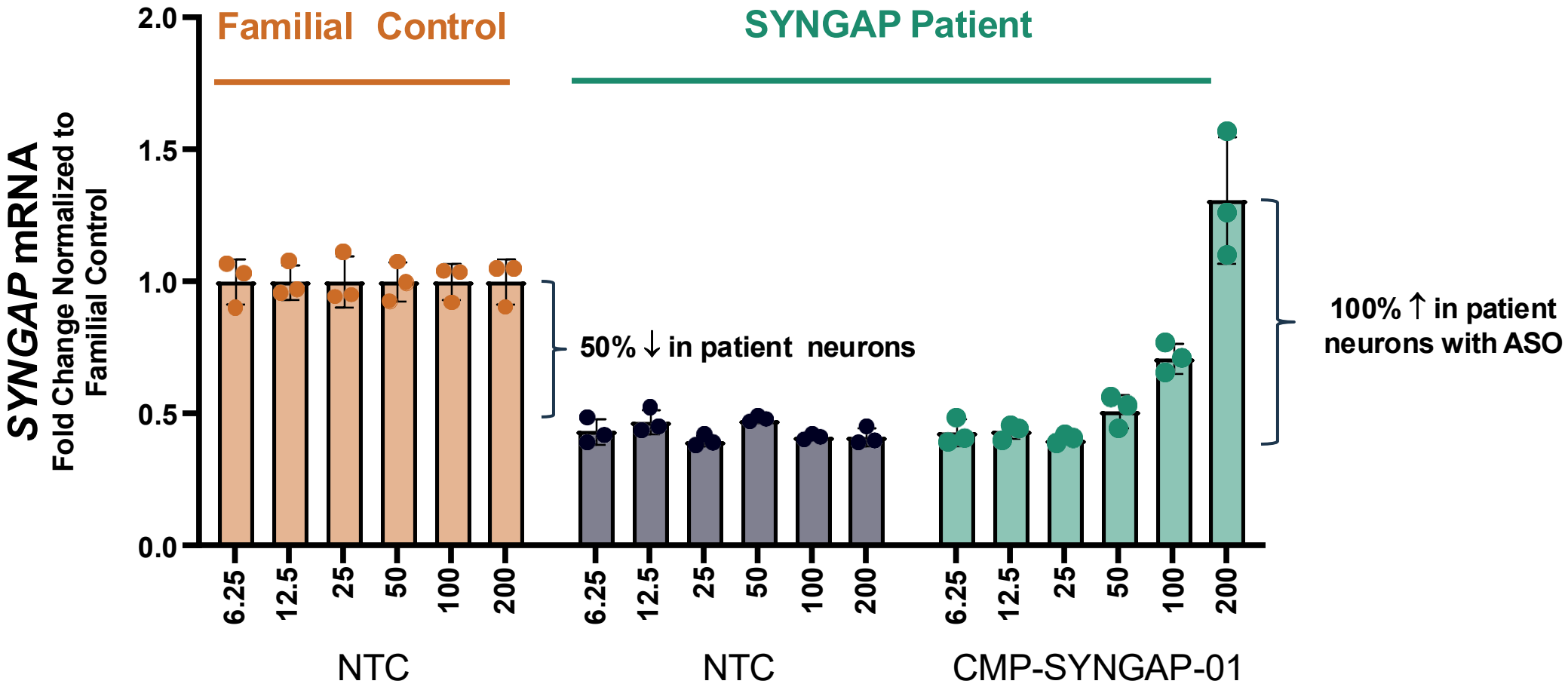


Restored SYNGAP-PSD95 complexes; restored post-synaptic AMPAR at baseline

PSD95 SYNGAP AMPAR Glutamate

[1] *Illustrative depiction of Electroencephalogram*

Targeting *SYNGAP* regRNA with CMP-SYNGAP-01 restores wild-type *SYNGAP* levels in patient iPSC-derived neurons compared to familial control



Patient with a heterozygous nonsense mutation in SYNGAP (K1185X) and their healthy familial control

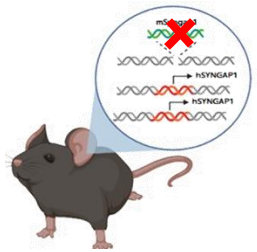
NTC – nontargeting control ASO

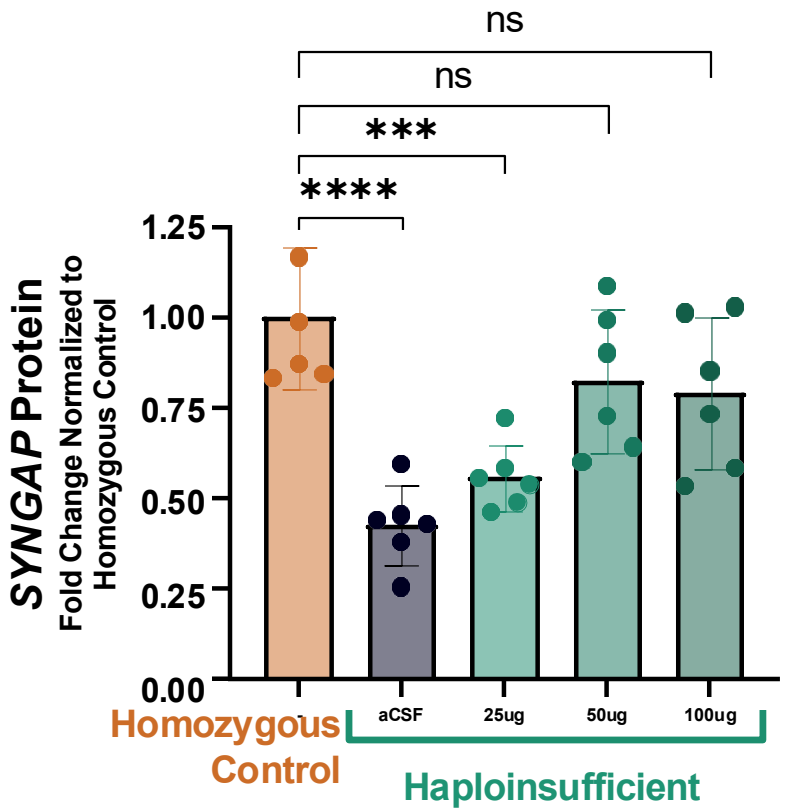
NTC = non-targeting control

CMP-SYNGAP-01 restores near-normal protein levels in Humanized *SYNGAP1* Haploinsufficient Mice

Homozygous Humanized Mouse

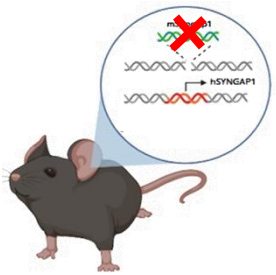
- No copies of mouse *SYNGAP*
- Two copies of Human *SYNGAP*





Haploinsufficient Humanized Mouse

- No copies of mouse *SYNGAP*
- Single copy of Human *SYNGAP*

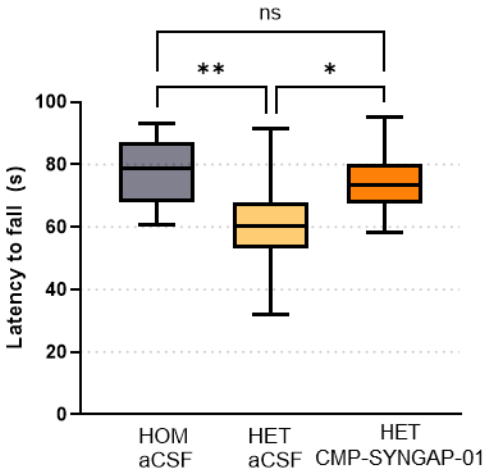


aCSF = artificial CSF
***, p<0.001
****, p<0.0001

(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).

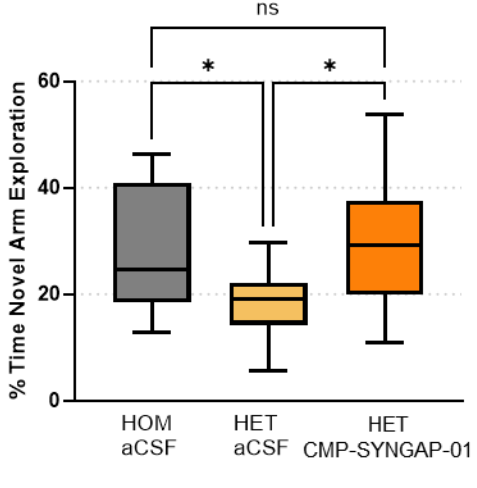
CMP-SYNGAP-01 rescues functional defects in Humanized SYNGAP1 Haploinsufficient Mice

Motor defects are rescued (Rotarod)



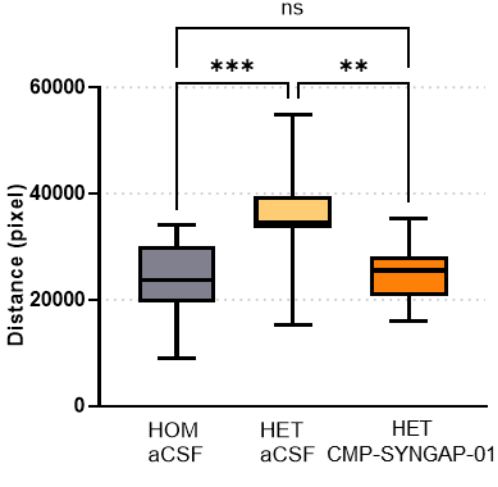
Motor function

Spatial learning defect is rescued (Y-Maze)



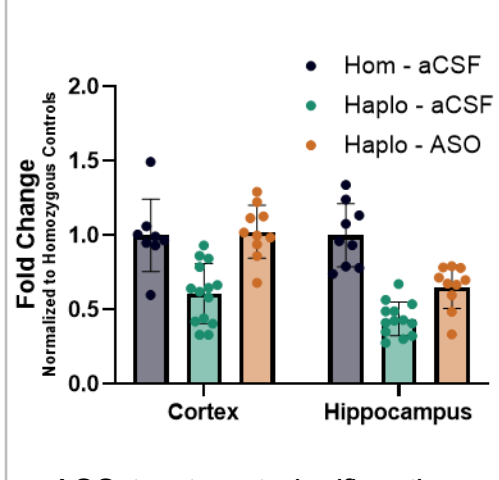
Cognition & memory

Increased activity is rescued (PalmReader)



Hyperactivity & anxiety

SYNGAP protein levels are increased



ASO treatment significantly increases SYNGAP protein levels

aCSF = artificial CSF

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

- Hom- aCSF (N=12)
- Haplo- aCSF (N=18)
- Haplo- ASO (N=10)

CMP-SYNGAP-01 was well-tolerated and significantly increased SYNGAP protein levels in NHPs

Study Design

Cynomolgus monkey

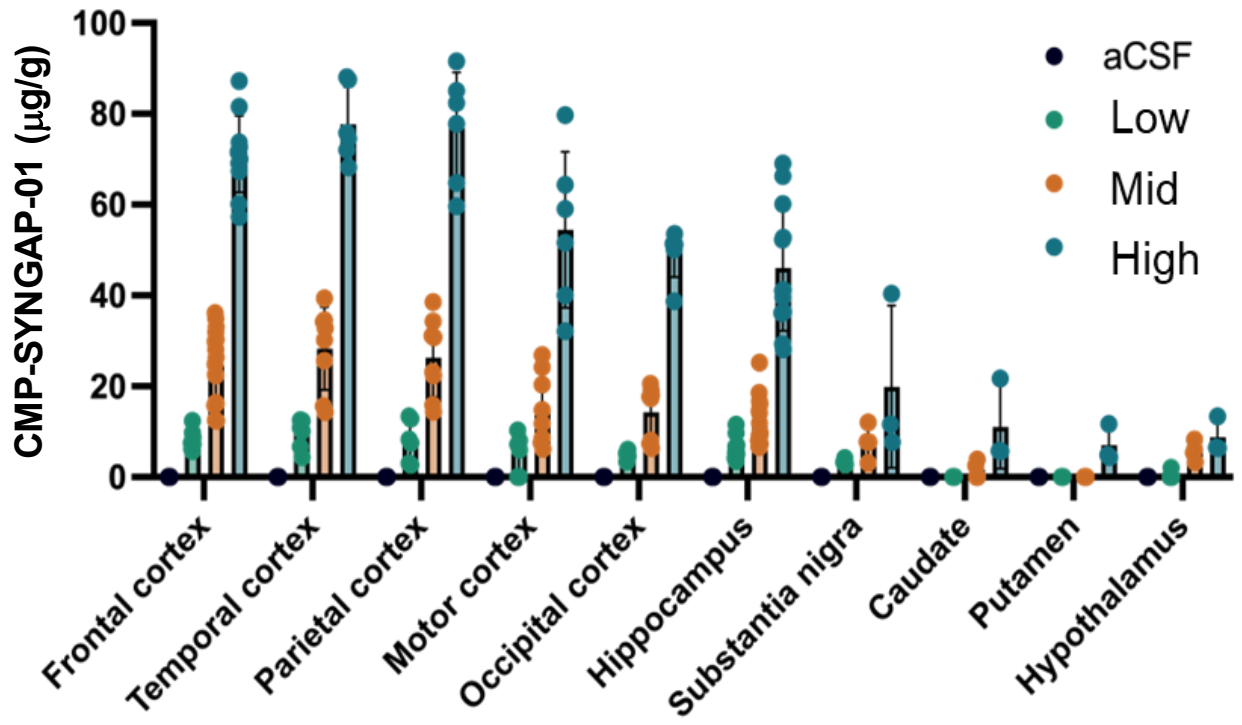
TA	Dose	Dosing frequency
aCSF		Q2w x3
CMP-SYNGAP-01	low	Q2w x3
	mid	Q2w x3
	high	Q2w x3

- Intrathecal administration
- N = 4 animals/group
- Formulated in aCSF

Key Takeaways

- CMP-SYNGAP-01 was clinically well-tolerated at all dose levels
- IT administration achieved dose-linear exposure across brain regions
- SYNGAP protein significantly increased across brain regions implicated in disease

Dose-linear increases of CMP-SYNGAP-01 levels across brain regions following repeat IT administration

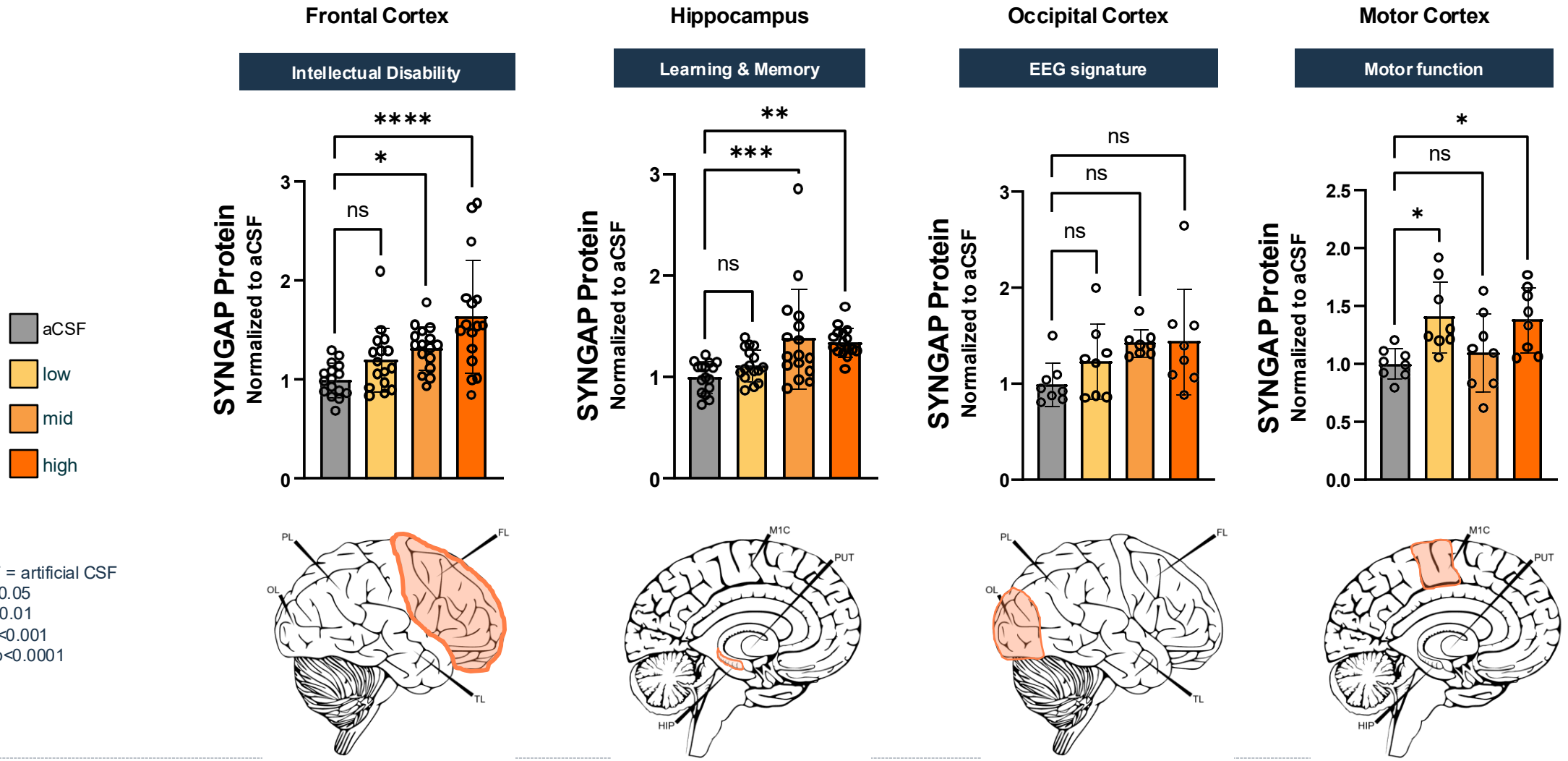


aCSF = artificial CSF

- Dose-linear CMP-SYNGAP-01 concentrations achieved with repeat IT administration
- Generally, even distribution across multiple disease-relevant brain regions

Highest ASO levels observed in brain regions primarily involved in SYNGAP-related disorders

Intrathecal administration of CMP-SYNGAP-01 to monkeys resulted in a significant increase in SYNGAP protein in key brain regions



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*, p <0.05
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***, p<0.001
****, p<0.0001

CAMP4 positioned to be first in the clinic for SYNGAP

Standard-of-Care	✓ No disease modifying therapies available ✓ Patients currently treated using a polypharmacy approach of symptomatic treatments
Natural History Study	✓ Third party study* is ongoing, advocacy / center of excellence driven ✓ > 1 yr duration, 100 patients of data obtainable from natural history study
Center-of-Excellence	✓ Existing center of excellence is central node for translational / clinical excellence
Path to Clinic	✓ GLP toxicity studies expected to initiate in Q3 2025 ✓ Potential for CMP-SYNGAP-01 to be evaluated in a global Ph1/2 study in patients initiating as early as H2 2026
Path to Approval	✓ Established path to approval for a developmental epileptic encephalopathy (DEE) ✓ Seizure quantification as primary + neurodev. scale

*sponsored by SYNGAP Research Foundation

ASO therapy for SYNGAP1 patients

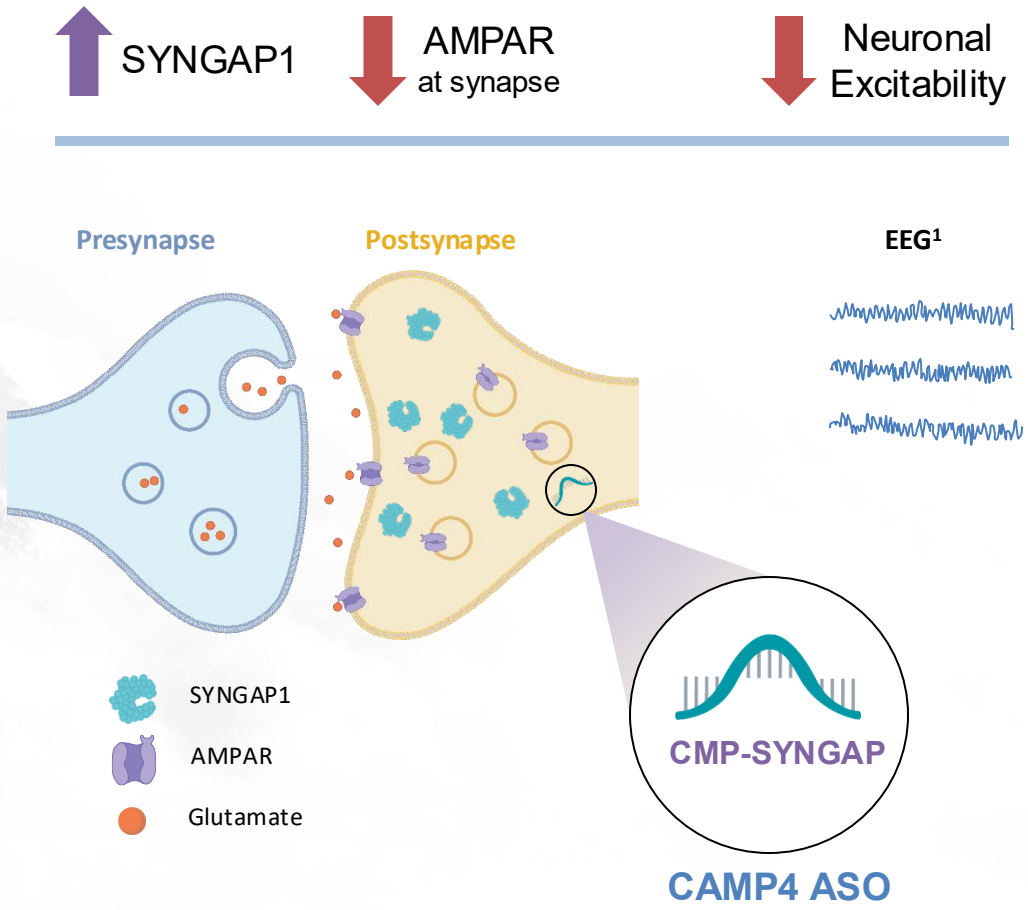
What is an ASO?

ASO stands for **A**nti**S**ense **O**ligonucleutide

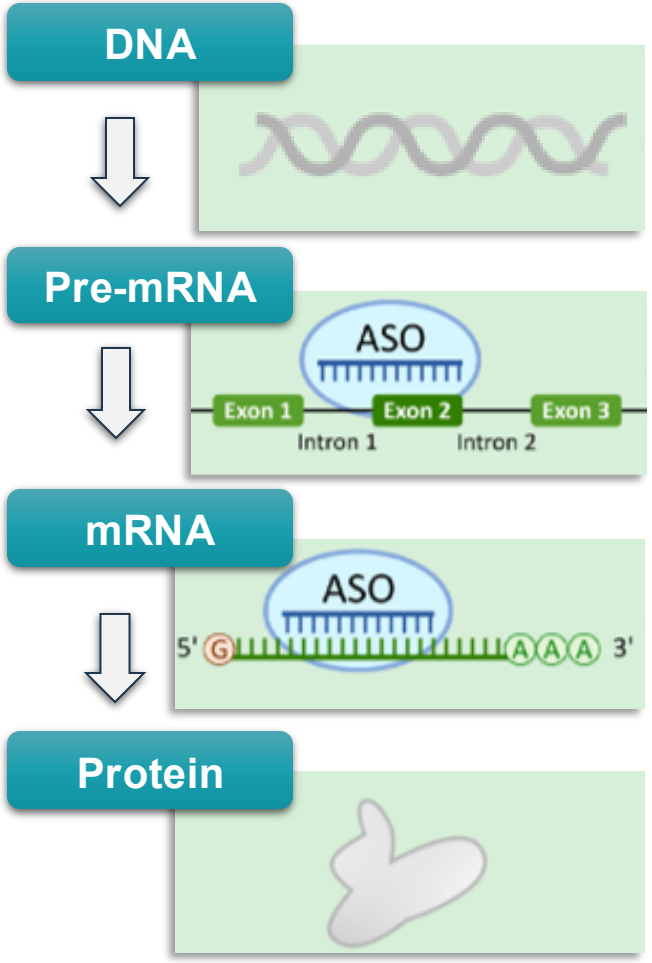
ASOs are small genetic sequences that can be engineered to target specific sequences of RNA

ASOs can fine tune genetic instructions, helping regulate production of a specific protein

CAMP4 is developing an ASO that aims to increase expression of the *SYNGAP1* gene to near-normal levels, improve how neurons transmit signals, and alleviate symptoms of SYNGAP1 patients



ASOs target RNA - benefit of modality demonstrated with multiple approved therapies



Approved Antisense Oligonucleotides (ASO) Therapies

ASO	Disease	Delivery
Tegsedi® (inotersen) injection 284 mg/1.5 mL	Hereditary Transthyretin Amyloidosis, Polyneuropathy	Subcutaneous (SC)
SPINRAZA® (nusinersen) injection 12 mg/5 mL	Spinal Muscular Atrophy (SMA)	Intrathecal (IT)
QALSODY® (tofersen) 100 mg/15 mL injection	Amyotrophic lateral sclerosis (ALS), SOD1	IT
EXONDYS 51 (eteplirsen) Injection	Duchenne muscular dystrophy (DMD), Exon 51	Intravenous (IV)
VYONDYS 53 (golodirsen) Injection	Duchenne muscular dystrophy (DMD), Exon 53	IV
Viltepso® (viltolarsen) injection	Duchenne muscular dystrophy (DMD), Exon 53	IV
AMONDYS 45 (casimersen) 100 mg/2 mL injection	Duchenne muscular dystrophy (DMD), Exon 45	IV

Figure adapted from Grabowska-Pyrzewicz et al. (2021) EBioMedicine 74: 103691, CC BY 4.0, cropped. See also Collotta et al. (2023) Frontiers in Pharmacology 14: 1304342.

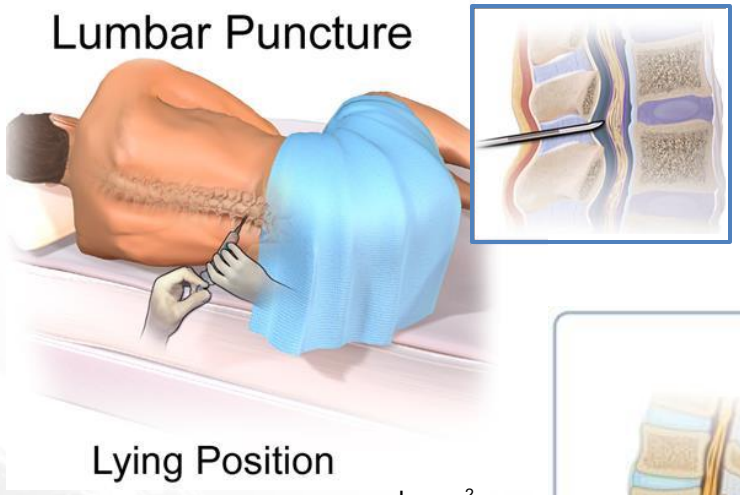
Intrathecal (IT) administration of ASOs to reach target cells in the brain

ASOs for SYNGAP1 patients would be administered by intrathecal (IT) injection, also known as lumbar puncture or spinal tap

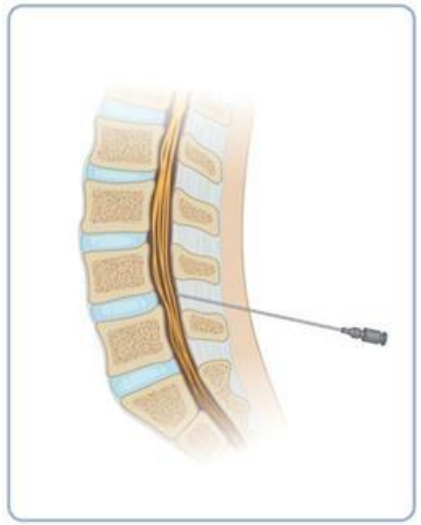
IT delivery allows the ASO to travel directly to the brain via the cerebrospinal fluid (CSF), resulting in more rapid and potent effects compared to other routes of administration

IT delivery allows the ASO to reach the target cells

If given systemically, ASOs for SYNGAP1 patients may not cross the blood-brain barrier efficiently



Image[2]



Image[1]

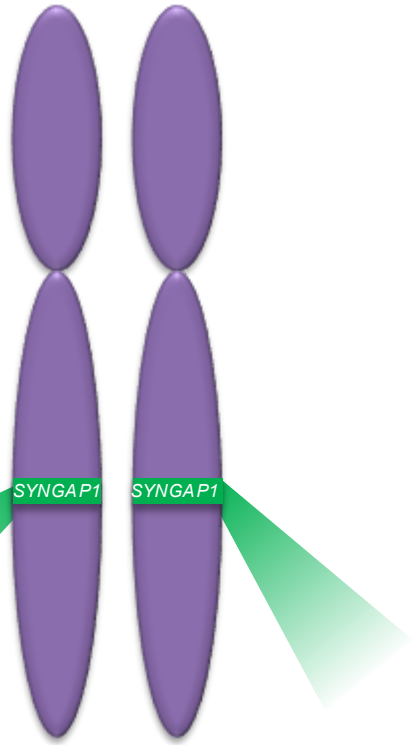
[1]Adapted from McCartan et al. (2023) Front Genet. 14: 1250276, CC BY 4.0, cropped and rearranged. [2]Adapted from "Lumbar Puncture" by Bruce Blaus, CC BY 3.0, via Wikimedia Commons, cropped.

Haploinsuffient disease = one good copy of a gene and one bad* copy

Healthy Individual

Everyone has two copies of each gene

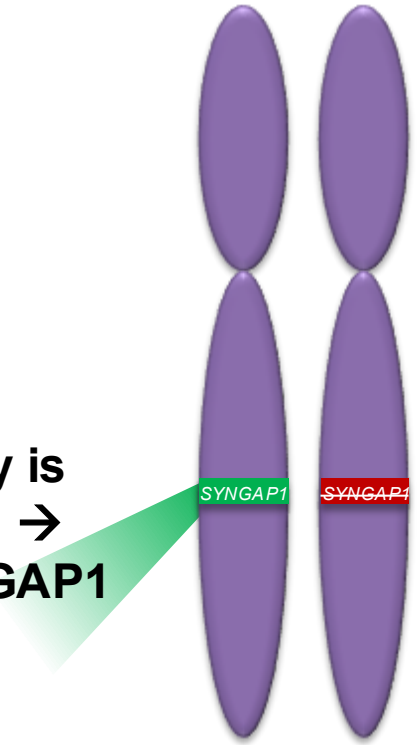
Both copies are expressed → make SYNGAP1 protein



Normal SYNGAP1 Protein

SYNGAP1 Patient

Good copy is expressed → makes SYNGAP1 protein

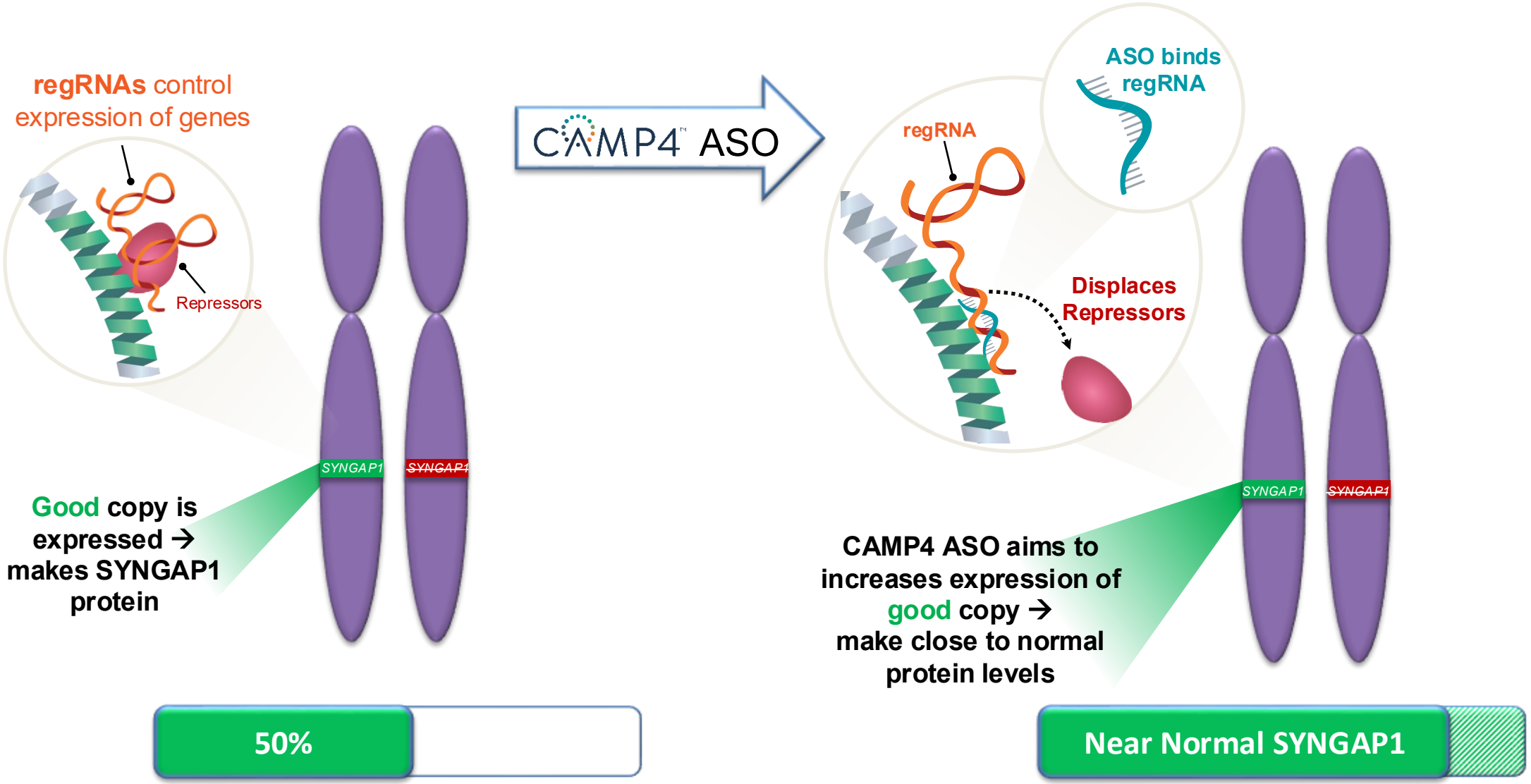


Bad copy may not be or may be expressed but degraded → makes NO or Non-functional mRNA or protein

50%

*Mutated allele may have one of multiple variants or abnormalities or mutations, including frameshift, nonsense, missense, deletion, etc.

ASO targets SYNGAP1 regRNA to make close to normal levels of protein



Works at transcriptional level rather than as splice-modulator or post mRNA splicing.

CAMP4™

Thank you