Capsida Biotherapeutics
Corporate Update

April 2025

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Solving the Challenges of Gen-1 Genetic Medicines

Starting in CNS and Ophthalmology with IP rights and applicability across all therapeutic areas

	CNS Challenges	Capsida Solutions
Crossing the BBB	Limited ability to cross BBB; < 1% neuronal transduction	>70% of neurons transduced in NHPs
Safety Concerns	Liver and dorsal root ganglia (DRG) toxicity	>16x liver & >50x DRG detargeting; lower dosing
Patient Populations	Narrow therapeutic index (TI) limits to ultra-rare/rare diseases	Broader TI = more common diseases across ages
Route of Administration	Direct injection to brain or CSF causes significant risks and inconsistent expression	IV limits risks and allows consistent expression
	IV delivery increases risk of off-target effects (esp. liver) and triggering immune response	Well-tolerated safety profile with no adverse histopathological findings

Next-generation Genetic Medicines Company

Unlocking the full potential of gene therapy for all

Wholly-owned Pipeline

Two clinical programs in 2025

CAP-002: STXBP1-DEE

CAP-003: PD-GBA
- Human POC in Q4

Third clinical program in 2026

CAP-004: FA

Fully-integrated Capabilities

- » **Capsid engineering**
- » **Cargo optimization**
- » **Clinical development**
- » **In-house manufacturing**
- » **Protected by expansive IP portfolio**

External Validation

Strategic partnerships

abbvie » 1st AbbVie opt-in ($40M) achieved

Lilly Prevail THERAPEUTICS — A Wholly Owned Subsidiary of Eli Lilly and Company

CRISPR THERAPEUTICS

Contract manufacturing

KATE THERAPEUTICS — A NOVARTIS COMPANY

>$300M funding to date, including $50M Series A

VERSANT ventures

Westlake BioPartners

Near Clinical Stage Wholly-owned Programs

First human POC by end of 2025

Disease / Target	Preclinical	IND-Enabling	Phase 1/2	Key Catalysts
STXBP1 Developmental and Epileptic Encephalopathy (STXBP1-DEE)	First-in-class CAP-002			2025 Q1 - IND filing Q3 - First patient dosed 2026 Q1 - First efficacy data
Parkinson's disease associated with GBA mutations (PD-GBA)	Best-in-class CAP-003			2025 Q2 - IND filing Q3 - First patient dosed Q4 - First biomarker data 2026 Q3 - First efficacy data (1 yr)
Friedreich's ataxia (FA)	Best-in-class CAP-004			2025 Q1 - IND-enabling studies ongoing Q3 - Traditional & self-regulating cargo results 2026 Q2/Q3 - IND filing

AbbVie, Lilly, and CRISPR Tx partnerships each include one co-development/co-commercialization option

CAP-002: STXBP1-DEE

STXBP1 Developmental and Epileptic Encephalopathy

STXBP1 Developmental and Epileptic Encephalopathy

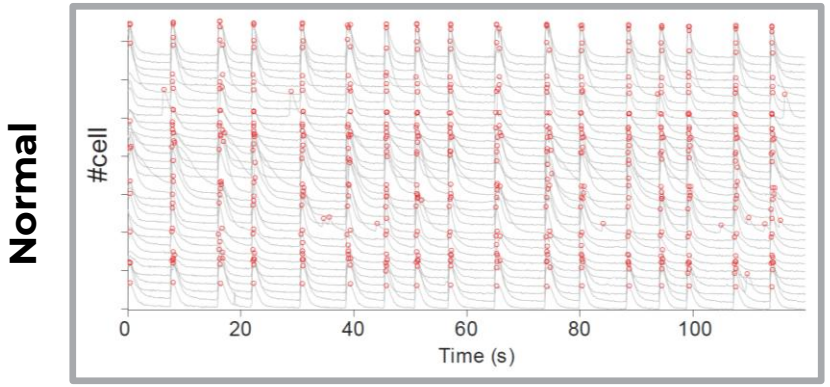
Opportunity

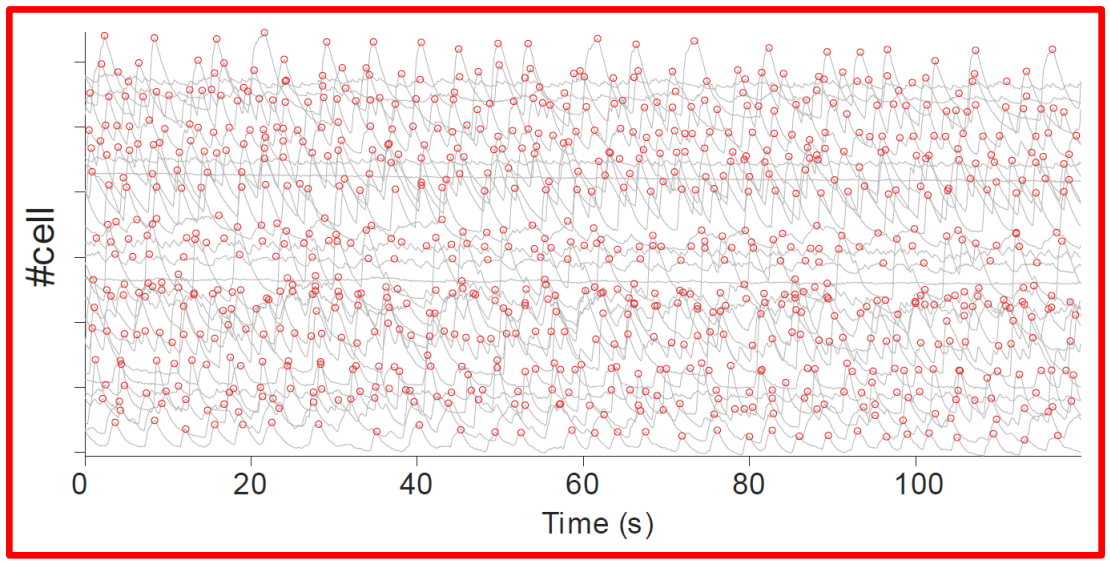
- DEE (like LGS and Dravet) with no approved treatments
- Seizures, developmental delays, and motor impairment
- ~5,000 patients in US and Europe
- Genetic validation and potential for FDA approval after Ph2
- >$1B peak year sales

CAP-002

- ✓ First and best in class IV-administered program
- ✓ Industry-leading brain wide STXBP1 protein increases
- ✓ Potential for correction of all phenotypes
- ✓ Safety demonstrated in NHPs, including liver and DRGs
- ✓ Successful pre-IND meeting, ODD granted, & GLP-tox dosed
- ○ Q1 IND and Q2 Fast Track filings

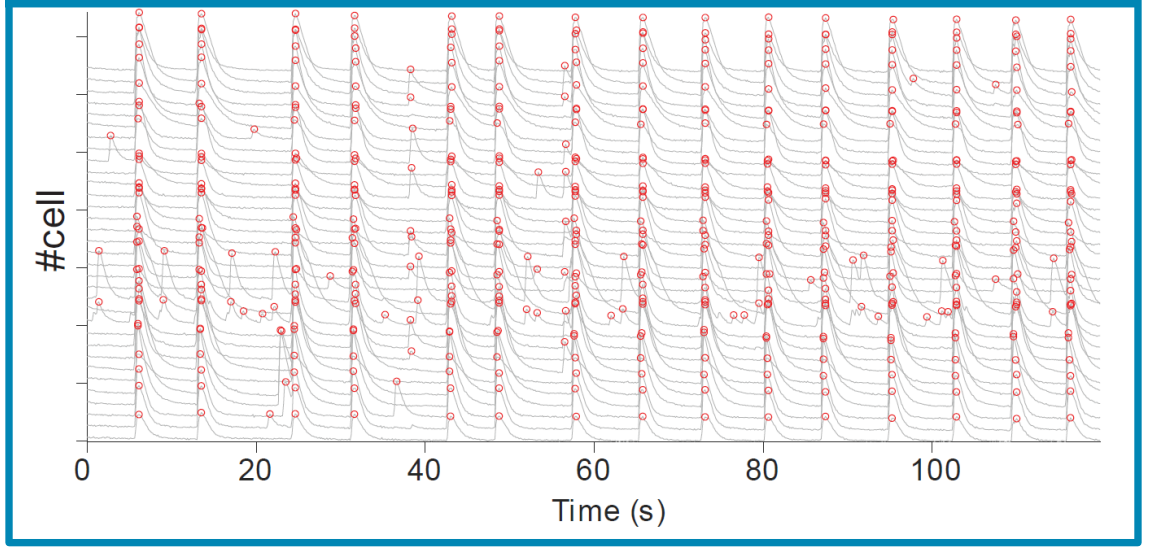
CAP-002 Restores Normal STXBP1 Expression and Neuronal Firing in Human KO Neurons





STXBP1 Knock Out

CAP-002 restores neurons to normal firing

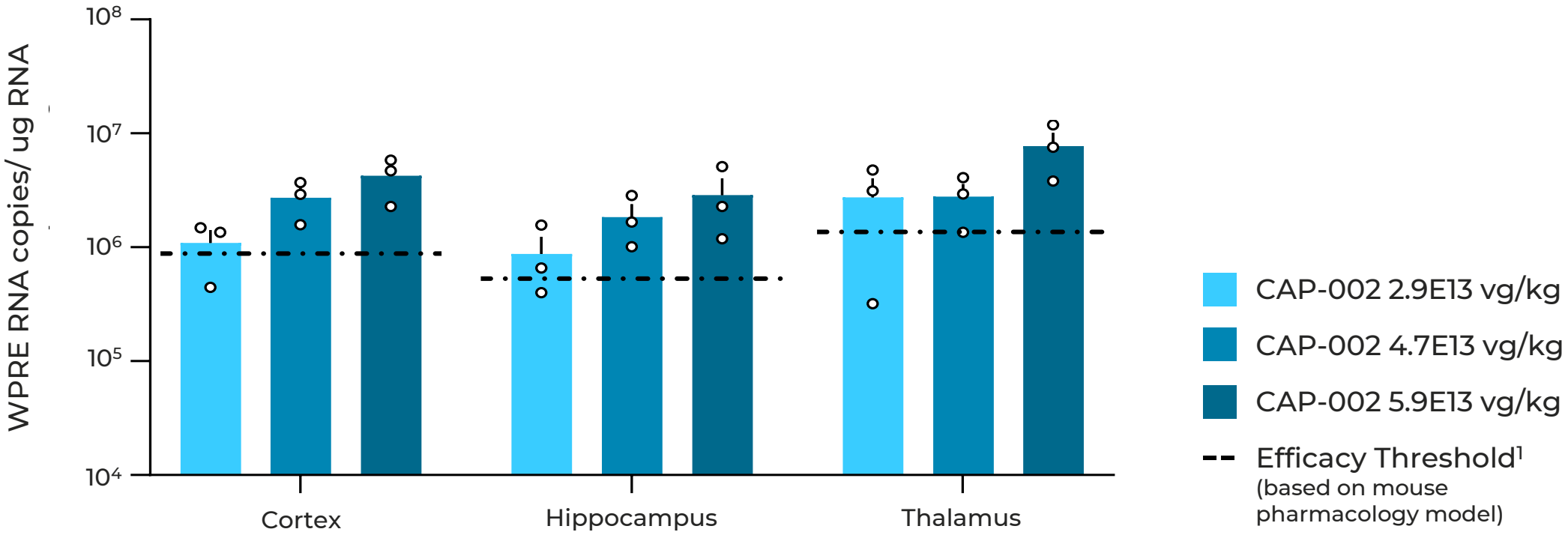


STXBP1 KO with CAP-002

Generated in collaboration with Neurospector, a CRO established by Matthijs Verhage's laboratory at VUmc Research BV, part of Foundation Stichting Amsterdam UMC

STXBP1 Expression with CAP-002 is Above Levels Required for Significant Correction of All Disease Phenotypes

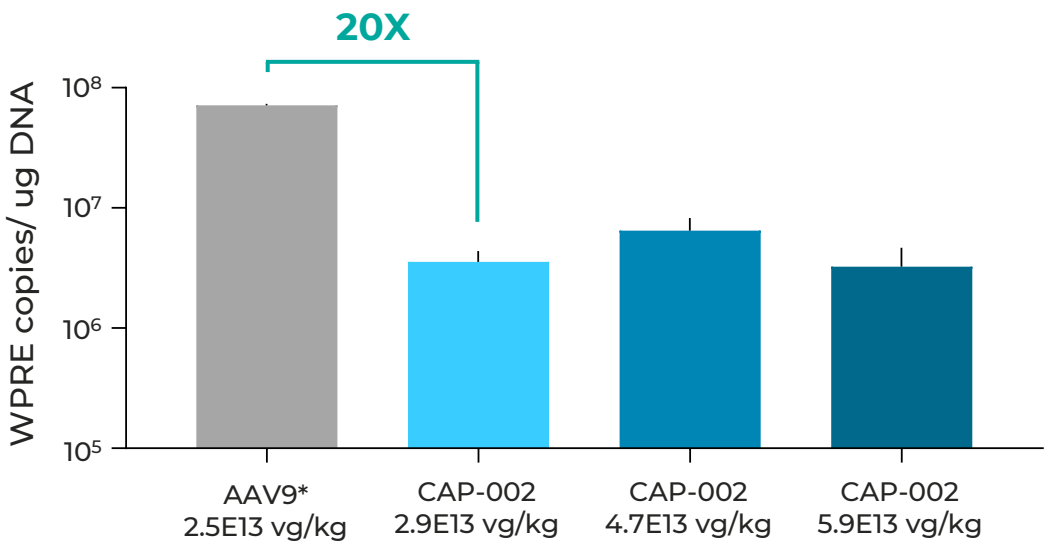
Brain Expression in NHPs



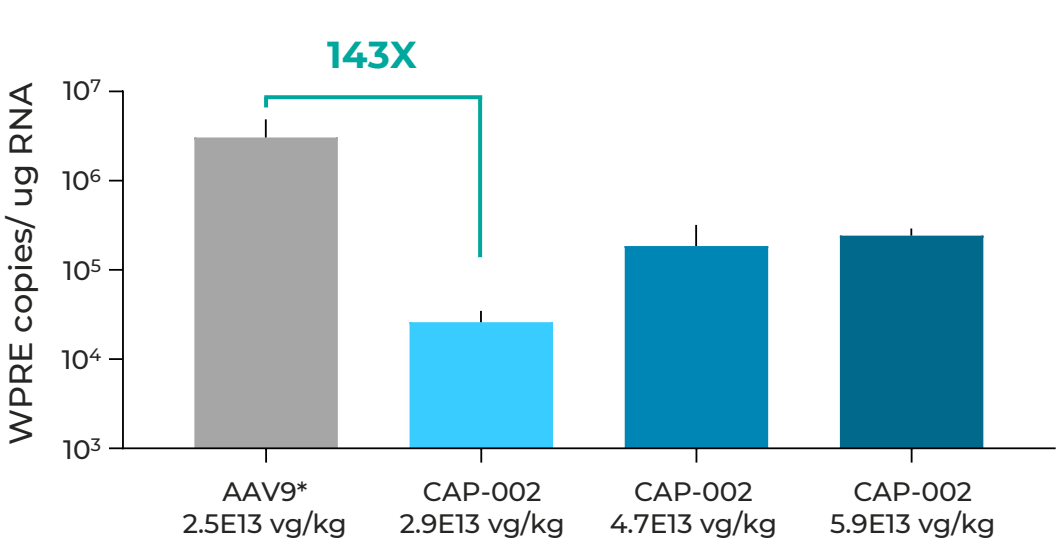
[1]Murine data generated in collaboration with lab of Mingshan Xue, Baylor College of Medicine

CAP-002 is Substantially Detargeted from Liver and DRGs in NHPs Compared to AAV9

Liver DNA



DRG RNA



Well-tolerated safety profile with no adverse histopathological findings

*Historical IV-delivered AAV9 2.5E13 vg/kg (non-STXBP1 cargo control)

CAP-002 STXBP1-DEE Clinical Plan

Potential for approval after Ph1/2 study

Clinical Ph1/2 Planning

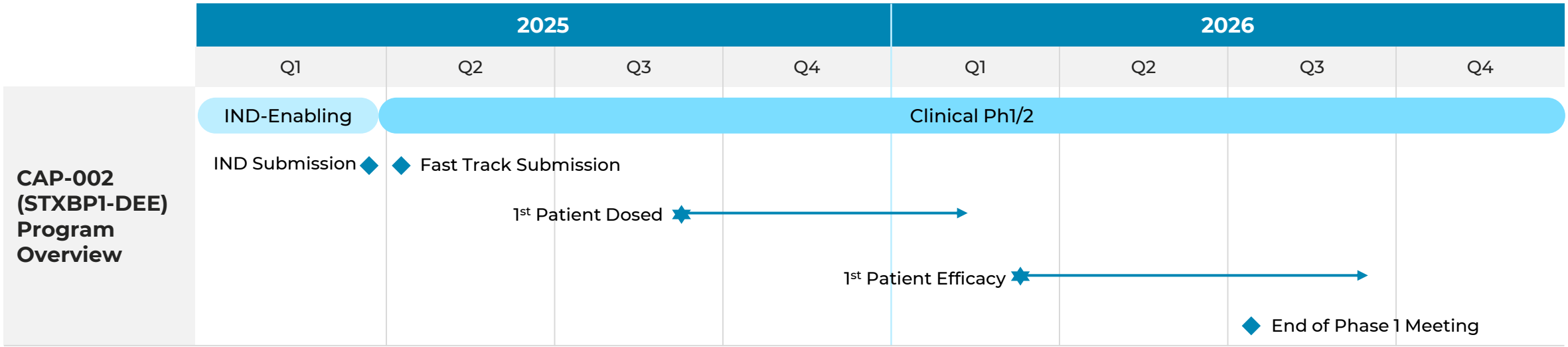
Population

- 18 months – 7 years
- Potential expansion to broader ages
- Ph1: 6 patients

Key Measurements

- Safety
- Motor, language, neurocognitive, seizure, and EEG
- Clinical scales consistent with STARR natural history study

Plan to leverage ODD and other designations to accelerate approval



EEG = electroencephalogram; STARR = STXBP1 Clinical Trial Ready

CAP-003: PD-GBA

Parkinson’s disease associated with GBA mutations

Parkinson's Disease Associated with GBA Mutations

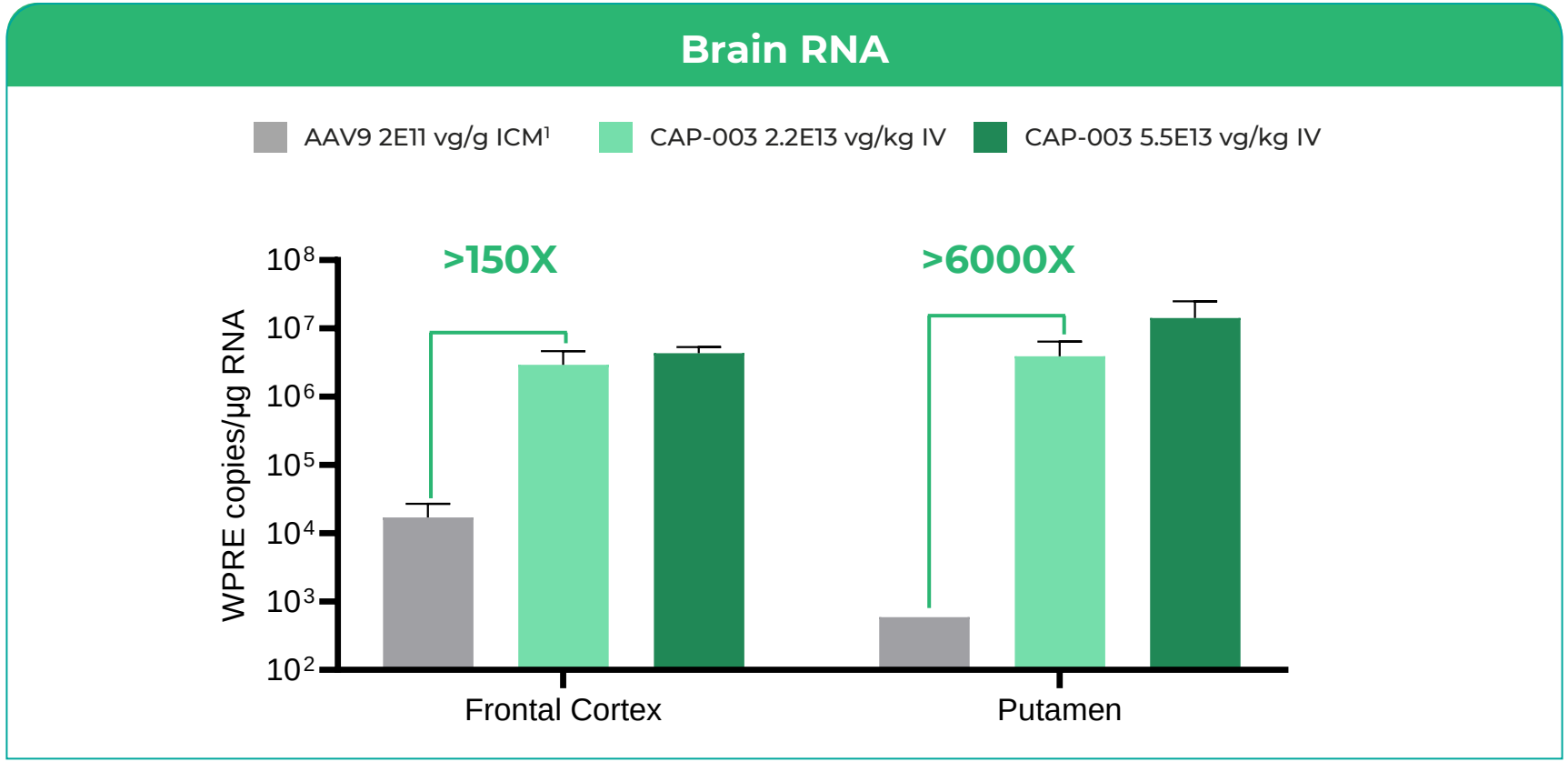
Opportunity

- Up to 15% of all PD cases = ~330k patients in US and Europe
- No approved GBA1 treatments and no PD disease modifying treatments
- >$1B peak year sales
- Potential for expansion to idiopathic PD

CAP-003

- ✓ Best in class IV-administered program
- ✓ Industry-leading brainwide GCase enzyme elevation
- ✓ Potential for significant disease modification
- ✓ Safety demonstrated in NHPs, including liver and DRGs
- ✓ Successful pre-IND meeting & GLP-tox dosed
- ○ Q2 IND and Fast Track filings

IV-delivered CAP-003 Achieves Superior RNA Expression Compared to ICM-delivered AAV9 in NHPs

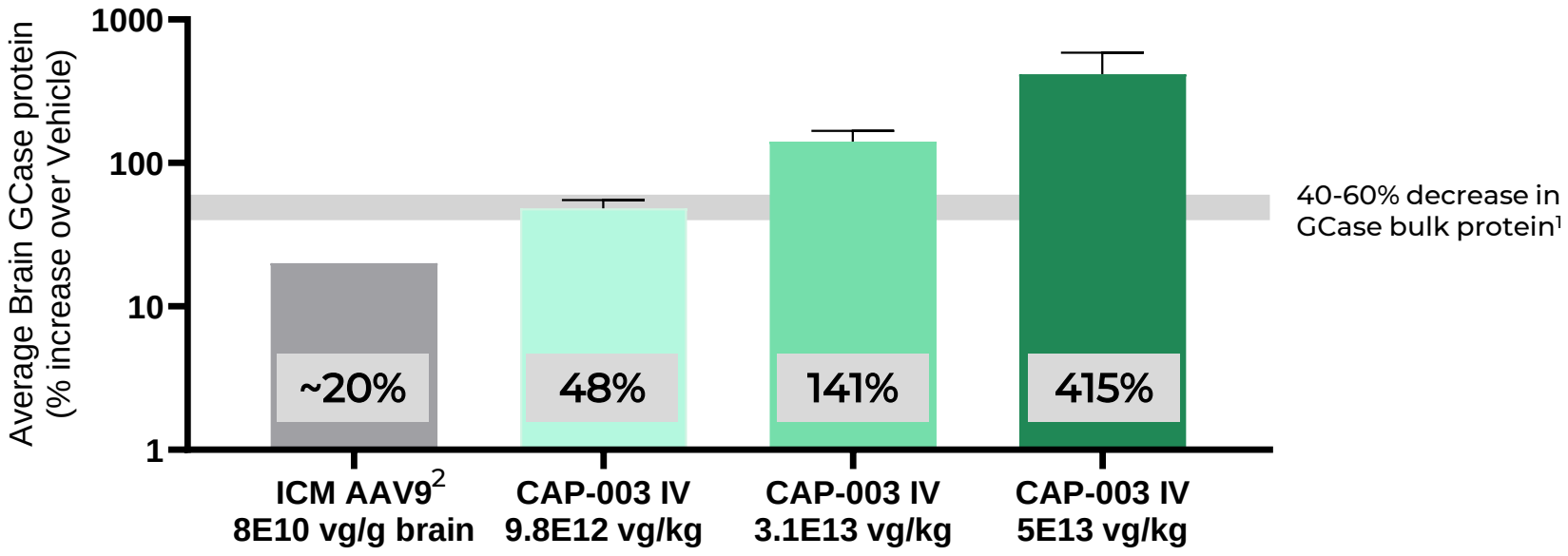


[1] AAV9 ICM data generated from past Capsida study with same expression cassette (promoter, regulatory elements, etc.) but different therapeutic cargo; no protein or %neuron comparison possible

Dose-Range Finding Study
In-life: 6 weeks
Species: Cynomolgus macaques (n=4/grp)

IV CAP-003 Achieves Superior GCase Protein Expression in GLP Tox Study Compared to ICM-delivered AAV9

Brain GCase Protein in NHP GLP Tox Study

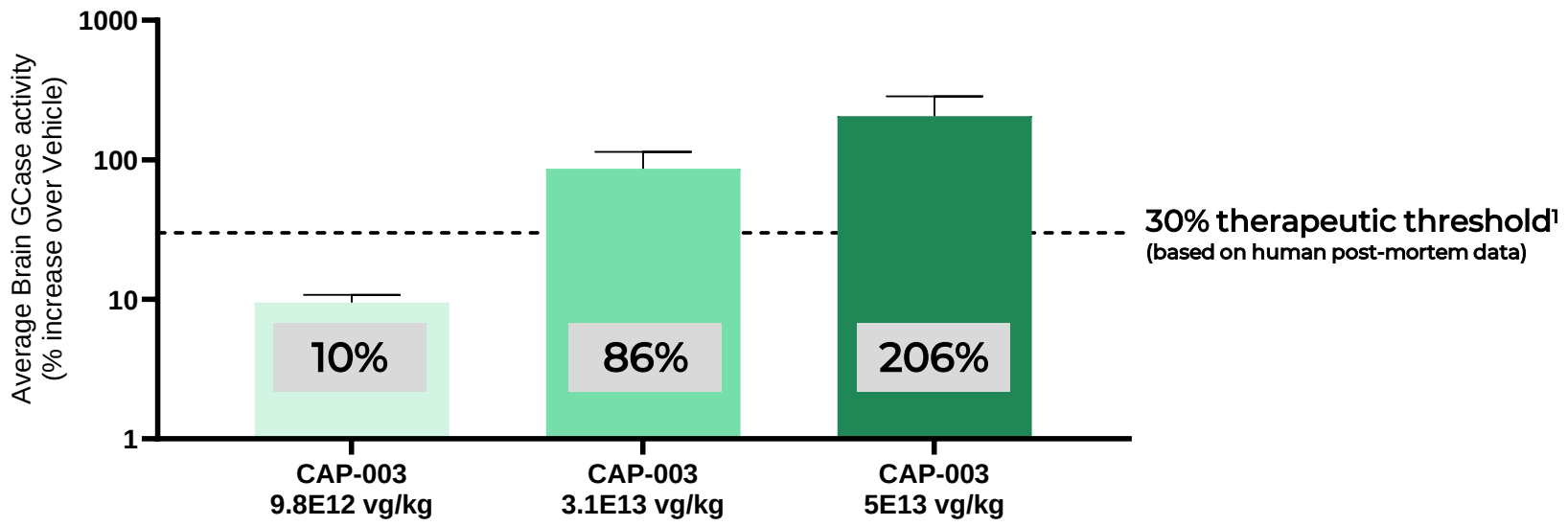


[1] Sanz Munoz et al., 2021 Decrease in GCase bulk protein in post-mortem brain tissues compared to healthy individuals
[2] ICM AAV9 reported by Prevail (Source: 2019 S1 filing) in their non-clinical NHP studies (~20% increase across cortex, hippocampus, midbrain 6 months after administration)

GLP Tox Study
In-life: 3 months
Species: Cynomolgus macaques (n=3/grp)

CAP-003 Exceeds Efficacy Threshold for Normalizing GCase Activity in Patients in NHP GLP Tox Study

Brain GCase Activity in NHP GLP Tox Study

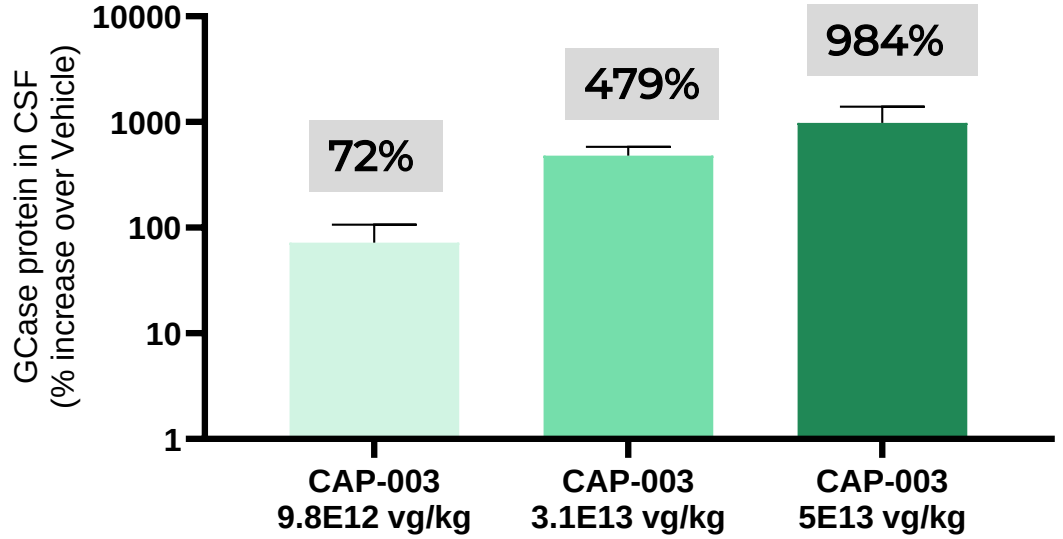


[1]Leyns et al., 2023. Post-mortem studies demonstrate an approximate 30% GCase activity deficit in patients compared to healthy individuals

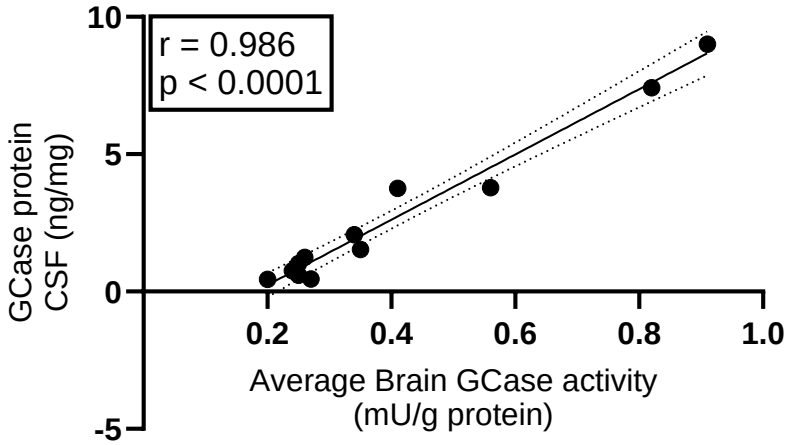
GLP Tox Study
In-life: 3 months
Species: Cynomolgus macaques (n=3/grp)

CAP-003 Significantly Increases GCase in CSF in GLP Tox Study Validating Use as Clinical Biomarker

GCase Protein in CSF in NHP GLP Tox Study

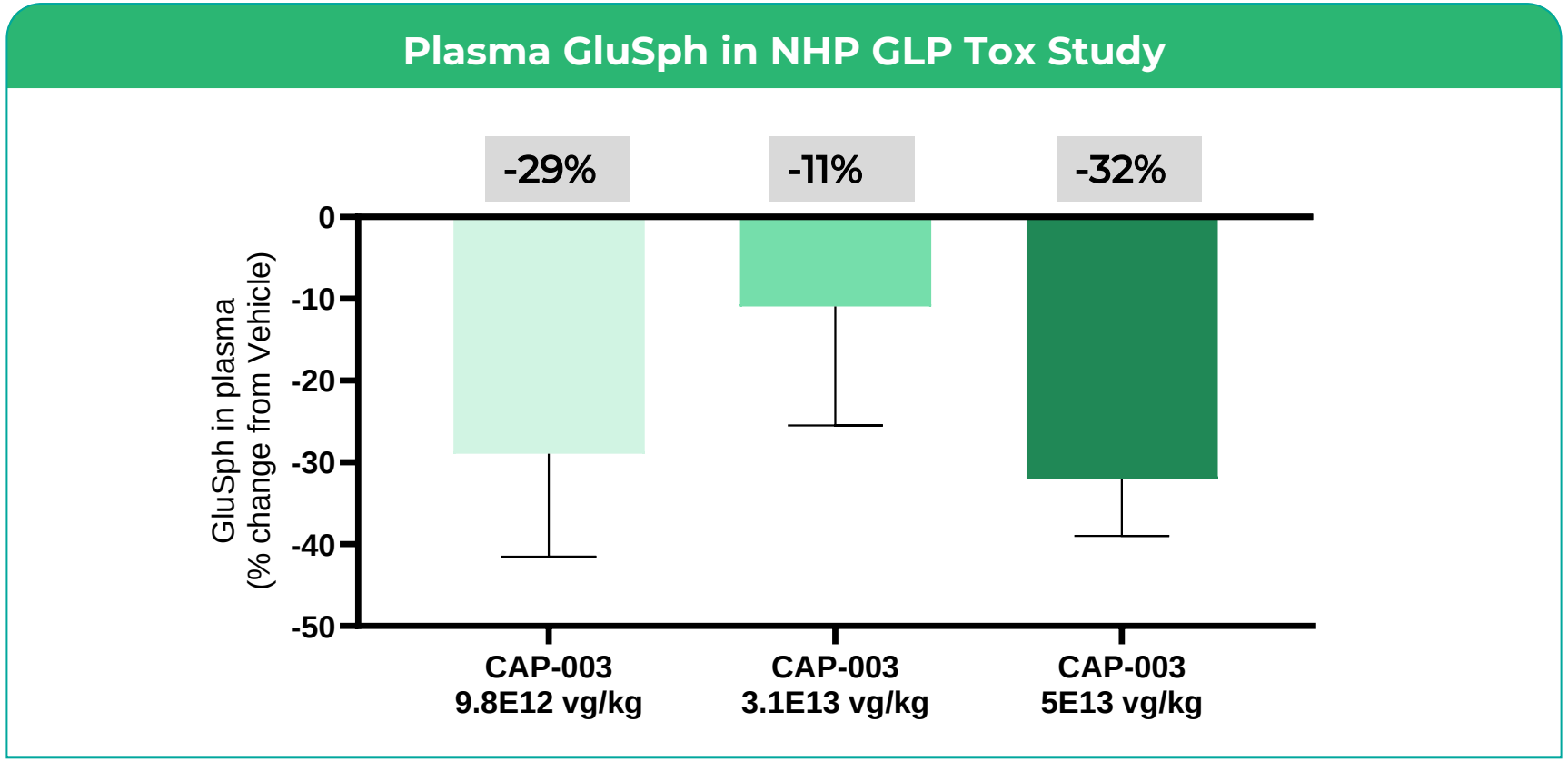


Correlation of CSF GCase Protein & Brain GCase Activity



GLP Tox Study
In-life: 3 months
Species: Cynomolgus macaques (n=3/grp)

Decreased GluSph Levels in Plasma Confirm Target Engagement in GLP Tox Study in Healthy NHPs

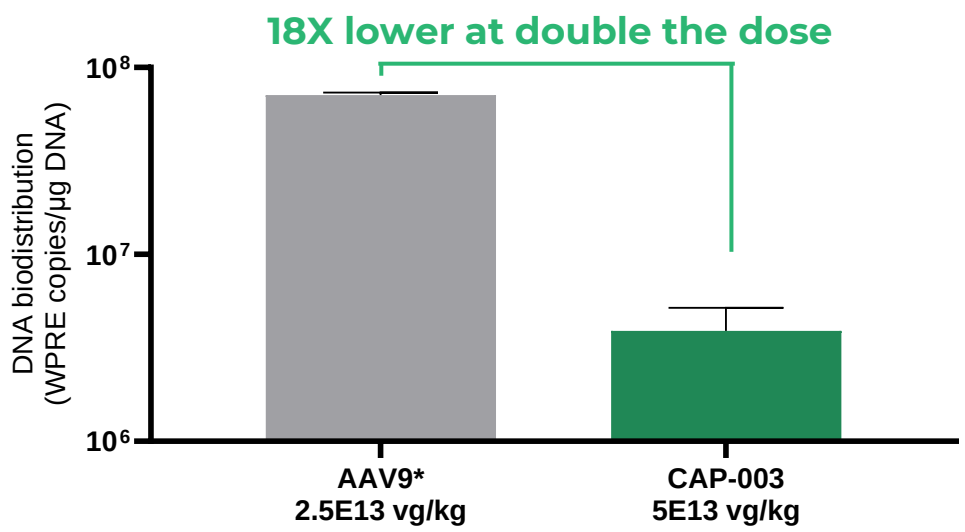


GluSph = Glucosylsphingosine

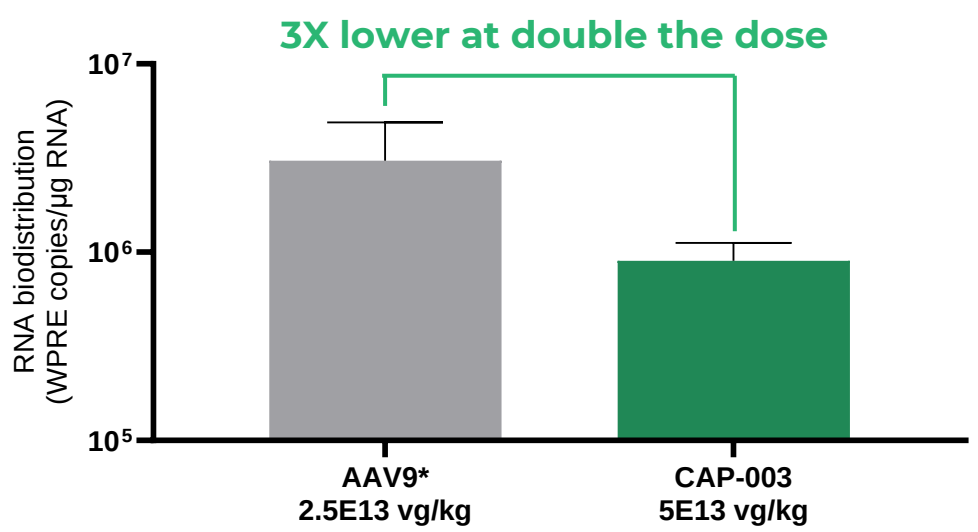
GLP Tox Study
In-life: 3 months
Species: Cynomolgus macaques (n=3/grp)

GLP Tox Data Demonstrate CAP-003 is Substantially Detargeted from Liver and DRGs in NHPs Compared to AAV9

Liver DNA



DRG RNA



Well-tolerated safety profile with no adverse histopathological findings

*Historical IV-delivered AAV9 2.5E13 vg/kg (non-GBA cargo control)

GLP Tox Study
In-life: 3 months
Species: Cynomolgus macaques (n=3/grp)

CAP-003 (PD-GBA) Phase 1/2 Clinical Plan

Clinical Ph1/2 Planning

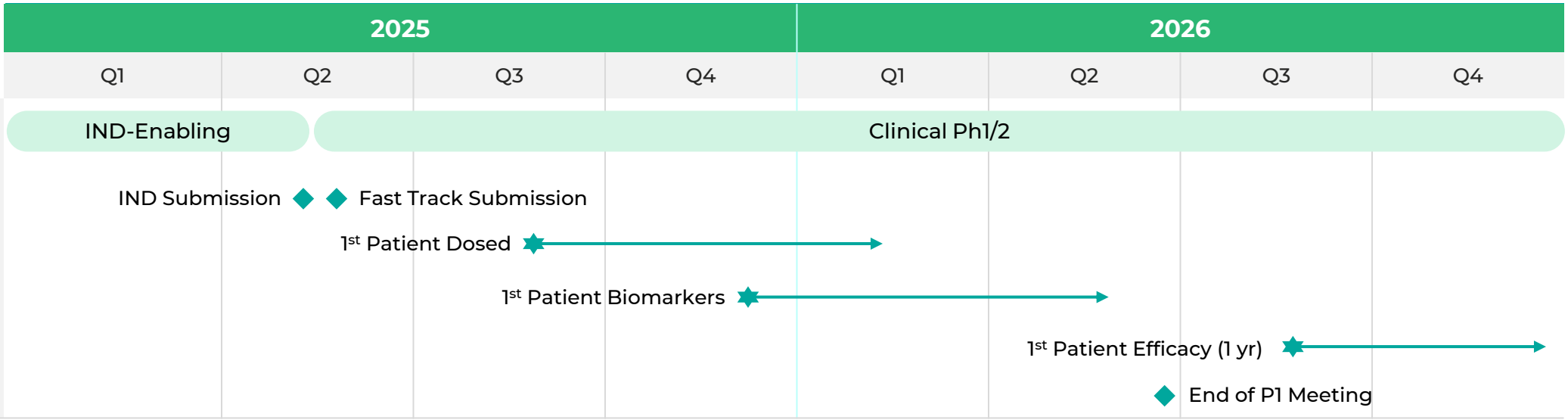
Population

- Ages 21-75 years
- PD Hoehn & Yahr Stage 1-3
- Ph1: 6 patients

Key Measurements

- Safety
- Biomarkers (e.g., GCase, GluSph, & GluCer)
- 1 & 2-year efficacy (e.g., MDS-UPDRS, cognition, etc.)

CAP-003 (PD-GBA) Program Overview



GluSph = glucosylsphingosine; GluCer = glucosylceramide;
MDS-UPDRS = Unified Parkinson's Disease Rating Scale

CAP-004: FA

Friedreich’s ataxia

Friedreich's Ataxia

Opportunity

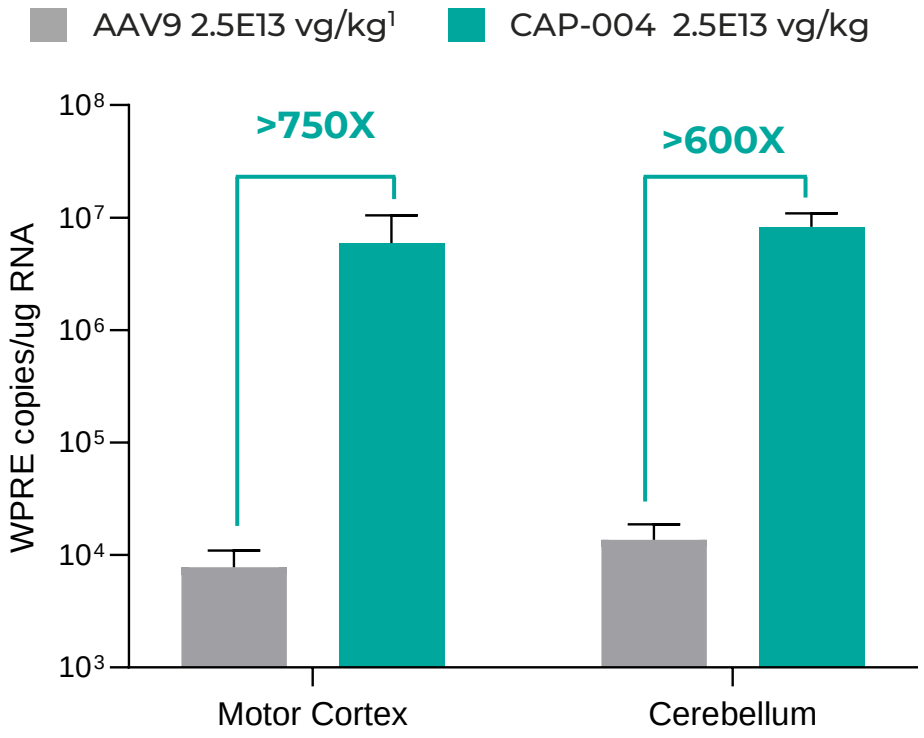
- CNS, cardiac, and sensory manifestations
- ~5,000 patients in the US and 15,000 worldwide
- Genetic validation and potential for FDA approval after Ph2
- >$1B peak year sales

CAP-004

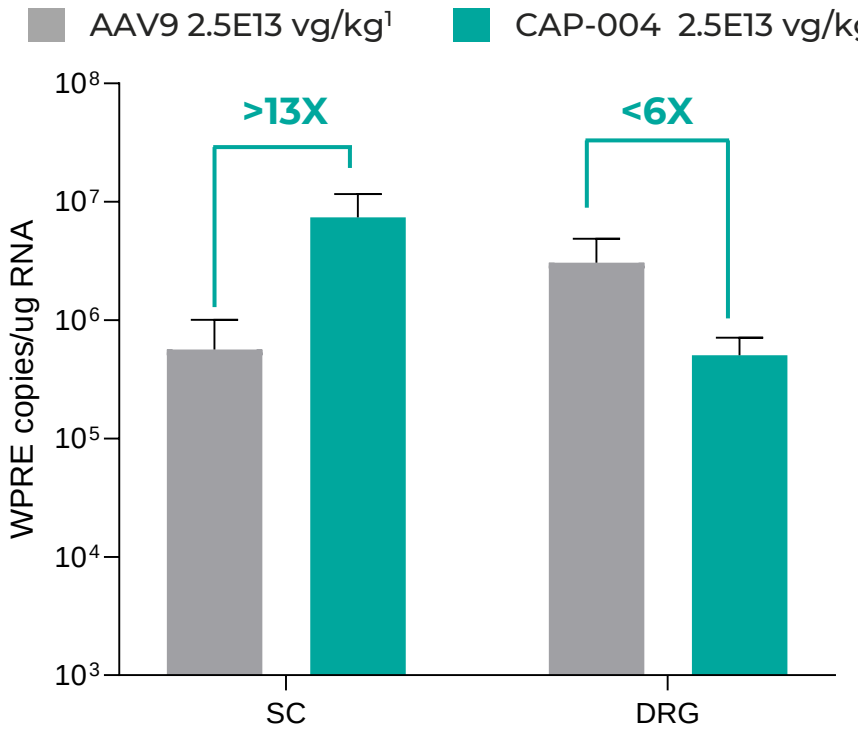
- ✓ Best in class IV-administered program
- ✓ Industry-leading frataxin protein expression in all relevant tissues
- ✓ Potential for correction of CNS, cardiac, and sensory manifestations
- ✓ Safety demonstrated in NHPs, including liver and DRGs
- ✓ IND-enabling studies ongoing, incl. self-regulating cargo
- ○ Q2/Q3 2026 IND Filing

IV CAP-004 Achieves CNS/PNS RNA Expression Levels Expected to Fully Correct FXN deficits in Patients

Brain FXN in NHPs



Spinal Cord & Sensory Neuron FXN in NHPs

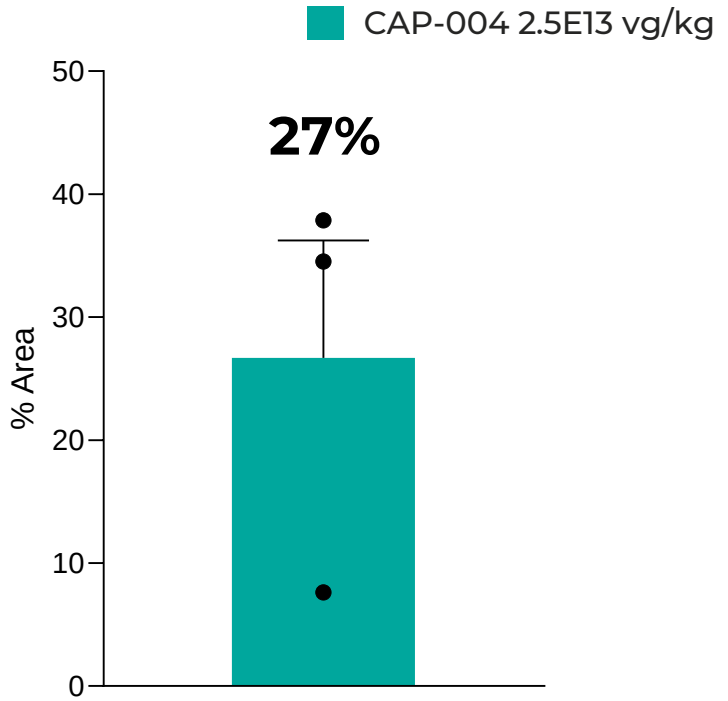


FXN = Frataxin

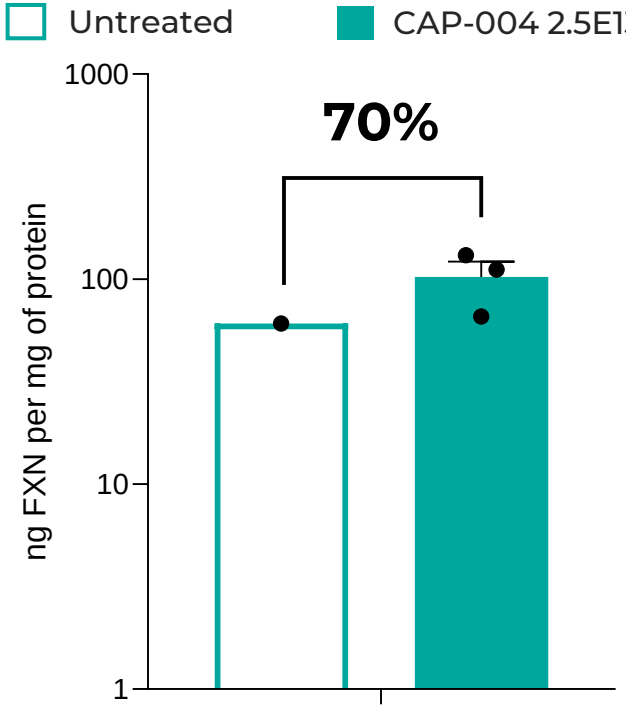
[1] AAV9 data generated from past Capsida study with same expression cassette (promoter, regulatory elements, etc.) but different therapeutic cargo

27% Average Transduction of Left Ventricle with CAP-004 Results in >70% Increase in FXN Expression in Heart

Left Ventricle %HA Positive Area in NHPs



Cardiac FXN protein levels increase in NHPs



Platform and Capabilities

Capsida – Uniquely Positioned to Lead Gene Therapy

Capsid Engineering Scale

Fully industrialized and roboticized platform

Screening capabilities across cell types in NHPs and human cells

CNS Tropism

>99% specific to neurons

>70% neurons transduced

Broad IP capsids and capsid/cargo

Peripheral Detargeting

>16x liver & >50x DRG detargeted

Superior off-target safety profile

Broad IP protecting detargeting

Therapeutic Expression

Expression in NHPs with potential for full disease correction

Industry leading expression levels across pipeline programs

Clinical Translatability

Identified/patented novel human receptor with complete homology in NHPs and humans

Validated expression in NHPs including primates >20 years old

Manufacturability

In-house process development and GMP manufacturing

Productivity surpassing AAV9

Quality specifications > FDA requirements

Only Capsida has created all the capabilities and results needed to deliver on the promise of gene therapy

In-house Process & Analytical Development and cGMP Manufacturing

Enables Capsida to control quality, timelines, and costs

Vector Production		Rapid production of engineered capsids for preclinical studies
Process & Analytical Development		Conduct manufacturability assessment in HEK293 suspension process
		Up to 50 L bioreactor scale
		Develop and optimize key analytical assays
cGMP Manufacturing		15,000 ft^2 cGMP Manufacturing Facility
		Leverages single use systems
		200L bioreactor scale with ability to significantly expand capacity
		Finish – fill operations
		Unidirectional flow
		In-house QC capabilities for product release
		Modular clean rooms

Excellent yields and quality specifications at or above FDA standards

Corporate & Finance

Leadership Team and Board of Directors

Decades of Industry Experience and Drug Development Expertise

Leadership

Peter Anastasiou
Chief Executive Officer

Nicholas Flytzanis, PhD
Founder, Chief Research and Innovation Officer

Nick Goeden, PhD
Founder, Chief Technology Officer

Julie Hakim
Chief Financial Officer

Bethany Mancilla
Chief Business Officer

Rob Murphy
Chief Manufacturing and Quality Officer

Swati Tole, MD
Chief Medical Officer

Board Members

Clare Ozawa, PhD

Beth Seidenberg, MD

Viviana Gradinaru, PhD
Founder

Rita Balice-Gordon, PhD
CEO, Muna Tx

Frank Verwiel, MD
Chairman, Intellia

Peter Anastasiou
Chief Executive Officer

>$300M Funding to Date

2019

$50M Series A

2021

CNS **$90M**

2023

CNS **$55M**

Ophthalmology **$70M**

Contract manufacturing

$12M convertible note

2025

AbbVie opt-in **($40M)**

Potential additional milestones

Series B (TBD)

Next-generation Genetic Medicines Company

Unlocking the full potential of gene therapy for all

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CAPSIDA BIOTHERAPEUTICS

Our Pipeline is Making the Impossible Possible

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