Corporate Deck

CAPSIDA BIOTHERAPEUTICS

Unlocking the Potential of Gene Therapy for All

Jan 2024

Capsida Biotherapeutics

Foundation in capsid engineering with focus on building a new class of targeted, non-invasive gene therapies

Pipeline of wholly owned and partnered programs in rare and more common Neurological and Ophthalmology diseases

Fully integrated capabilities: capsid engineering, cargo optimization, discovery, preclinical research, process development, manufacturing, and clinical development

Company History

2019

Founded
Based upon breakthrough AAV engineering technology from the laboratory of Viviana Gradinaru

Series A
$50M Series A co-led by Westlake Village BioPartners and Versant Ventures

2021

Partner
AbbVie CNS deal **$90M** upfront including equity

Partner
CRISPR Research Collaboration

2022

Hire & Partner
Hired new CEO to lead experienced management team and initiated second partnering process

2023

Partner
Prevail / Lilly CNS deal **$55M** upfront including equity

Partner
AbbVie Ophthalmology deal **$70M** upfront including equity

Data Milestones
ASGCT Industry Symposium on **Breakthrough Capsids (up to 68% neurons)** and ETDD Presentation on **genetic epilepsy** (STXBP1 preclinical data)

Partner
Kate Therapeutics Manufacturing Collaboration

Leadership Team and Board of Directors

Decades of Industry Experience and Drug Development Expertise

Leadership

Peter Anastasiou
Chief Executive Officer

Susan Catalano, PhD
Chief Scientific 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
Vice President, Technical Operations

Swati Tole, MD
Chief Medical Officer

Board Members

Clare Ozawa, PhD

Beth Seidenberg, MD

Viviana Gradinaru, PhD
Founder

Rita Balice-Gordon, PhD

Frank Verwiel, MD

Peter Anastasiou
Chief Executive Officer

Recent FDA Approvals Create Significant Momentum

Gene Therapy Approvals

Growing number of successfully developed and approved gene therapy products transformative for patients:

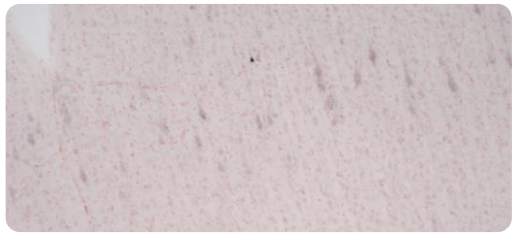
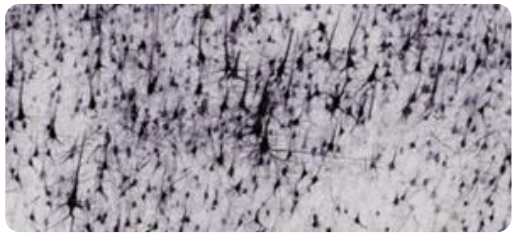
- **Hemgenix™** (Hemophilia B)
- **Zolgensma™** (SMA)
- **Roctavian™** (Hemophilia A)
- **Skysona™** (CALD)
- **Vyjuvek™** (Dystrophic epidermolysis bullosa)
- **Luxturna™** (RPE65 retinal dystrophy)
- **Zynteglo™** (ß-thalassemia)
- **Elevidys™** (DMD)
- **Casgevy™** (Sickle cell disease)
- **Lyfgenia™** (Sickle cell disease)

Breakthrough Neurological Therapies

FDA Approval for disease modifying CNS therapies based on biomarker data:

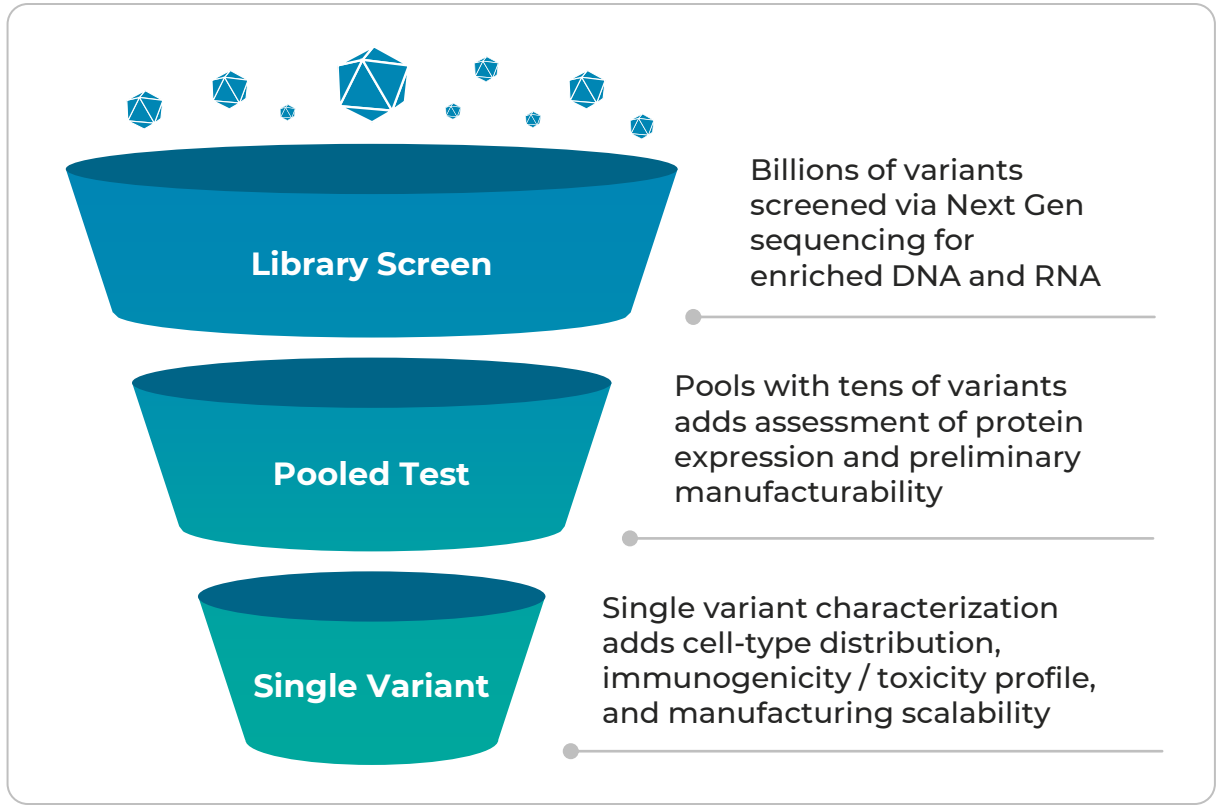
- **Qalsody™** (SOD1 ALS)
- **Leqembi™** (AD)
- **Skyclarys™** (FA)
- **Aduhelm™** (AD)

Capsida Addresses CNS Challenges Through our Engineered Gene Therapies

	CNS Challenges	Capsida Solutions
	Wild Type AAV9 (IV Delivery)	**Capsida Engineered Capsid** (IV Delivery)
	NHP Cortex 	NHP Cortex 
Neuronal Transduction	Limited ability to cross biological barriers, esp. BBB - < **1% transduction** with wild type AAV9 IV	Capsida engineered capsids cross BBB with high levels of neuronal transduction – **up to 70% neurons**
Safety Concerns	Safety concerns / **liver toxicity**	Enabling lower dosing and **~4000x difference** in CNS to liver targeting vs wild type AAV9
Patient Populations	Traditional gene therapies **primarily for ultra-rare/rare diseases**	**Access to more** common diseases across all ages
Risks	Direct injection into the brain or CSF is invasive with **significant risks**	Targeted IV admin **increases effectiveness** and **reduces risks**

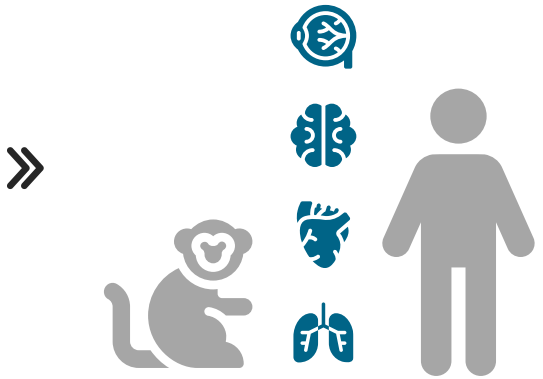
NHP Driven Targeted Gene Therapy Engineering Platform

High-throughput Process Identifies Capsids that Target Desired Tissues and Cell Types While De-targeting Undesired Tissues

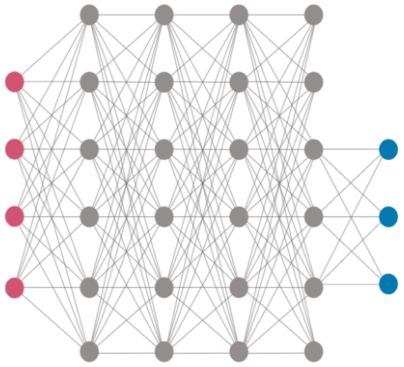


IND-enabling

Variant Optimization and Re-Diversification



Biologically Driven NHP Screening Combined with Analysis of Murine and Human Cells In Vitro



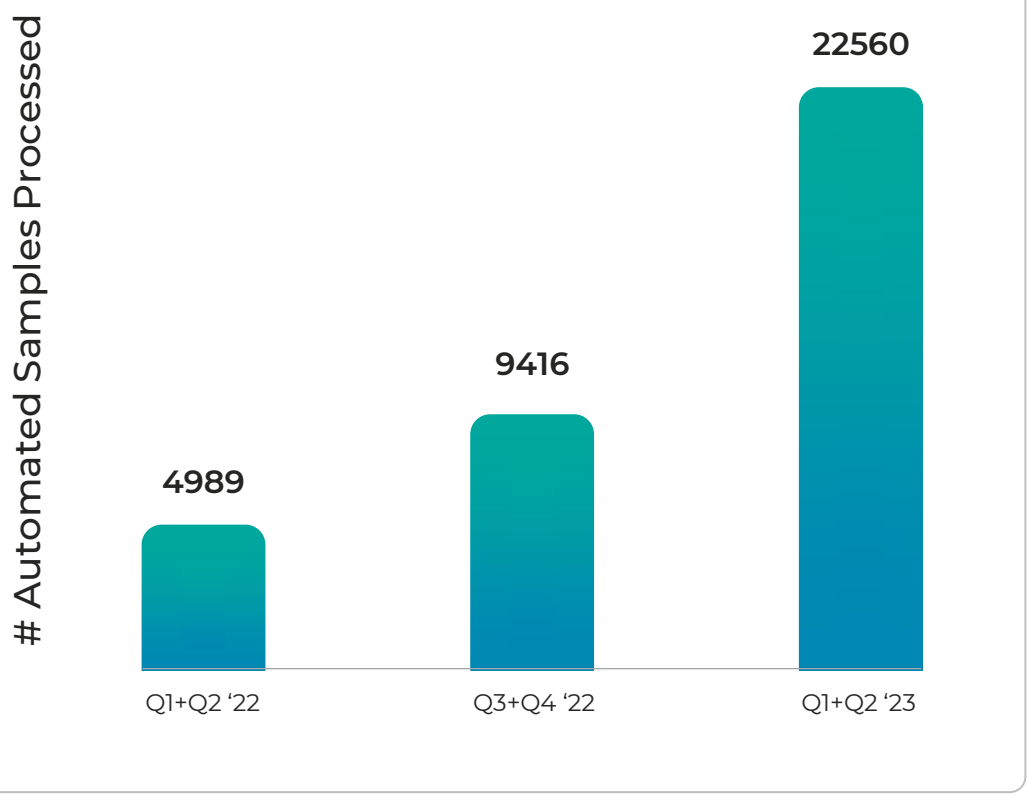
Sequence and Structure based analysis

Automated and High-Throughput NHP Screening Platform Accelerates Identification of Breakthrough Capsids

Improves data accuracy and reproducibility – highly consistent process

Increases data quantity – scale up on animal and tissue utilization

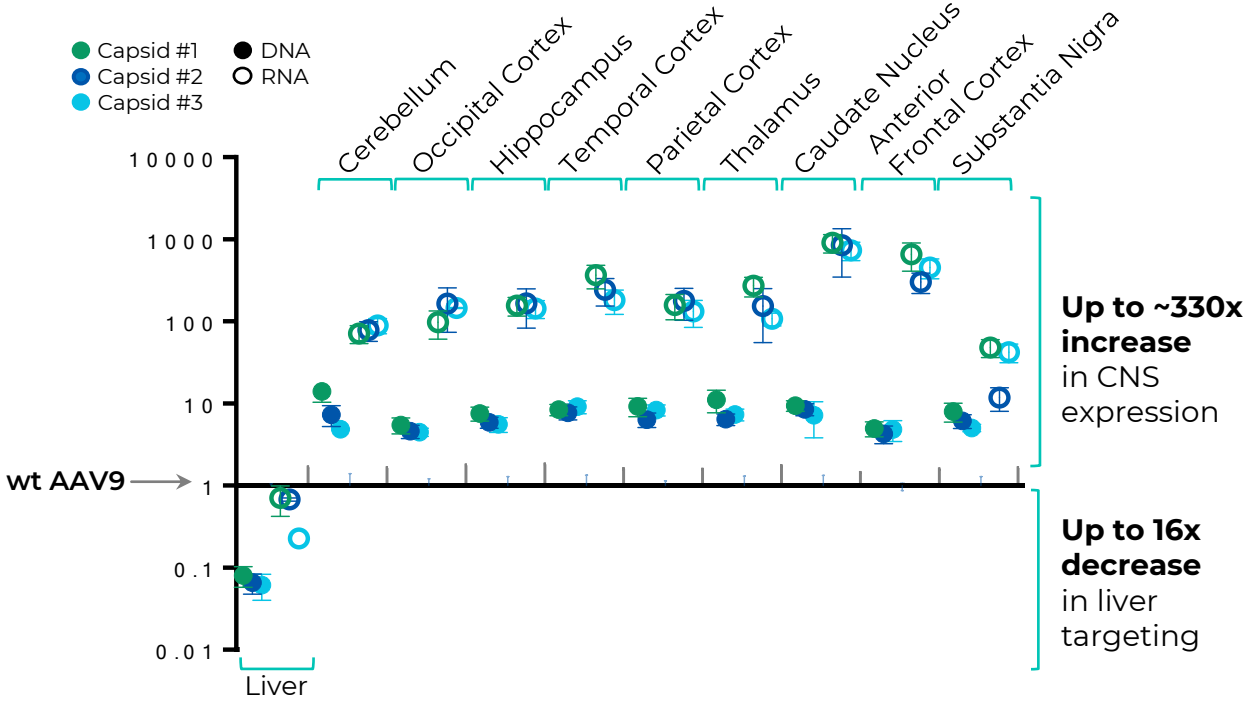
Screening capacity increases **250%**



Gen 5 Capsids Yield Breakthrough Expression Across The CNS And Significant Liver De-Targeting vs WT AAV9

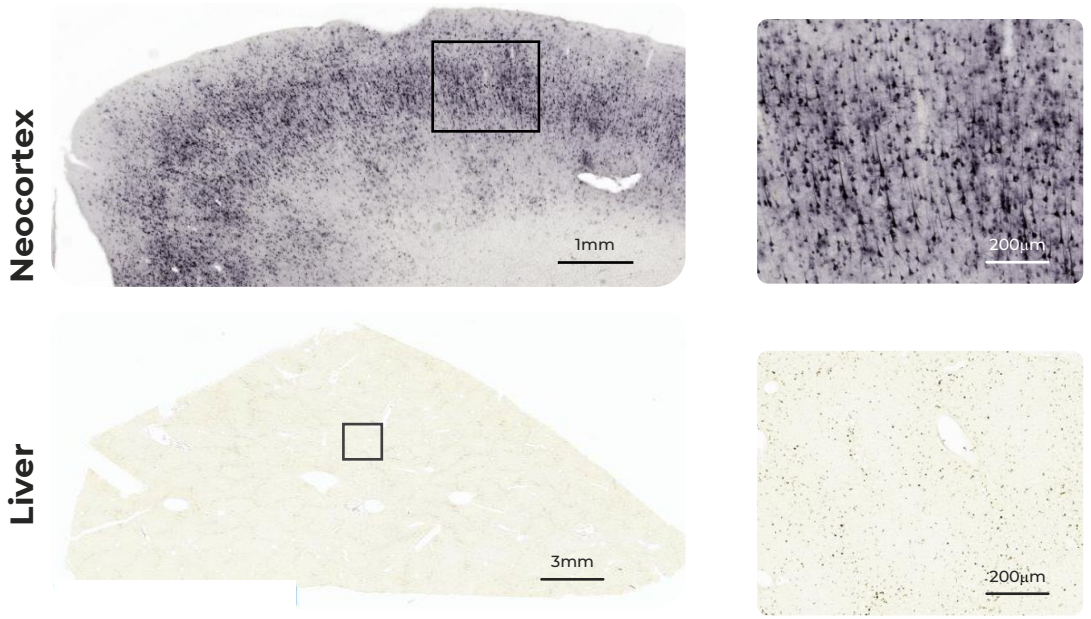
IV Delivery

DNA and RNA Enrichment over AAV9 From Individual Capsid Characterization Studies In Cynos



IV Delivery

Protein localization (HA tagged GOI)



~4000X difference in CNS expression vs liver targeting with Capsida's breakthrough capsids relative to wtAAV9

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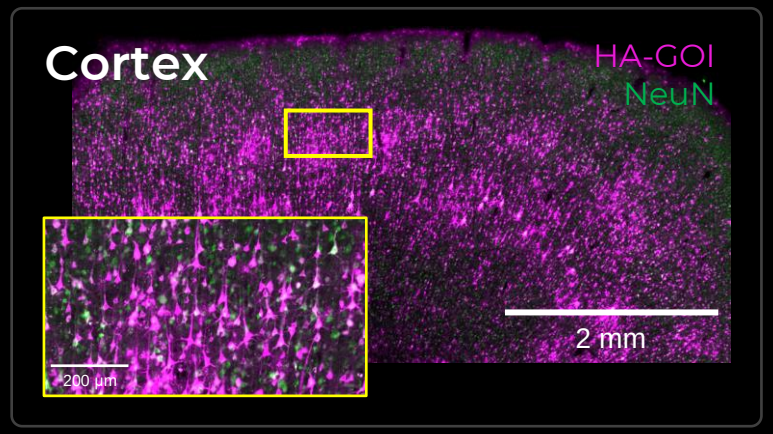
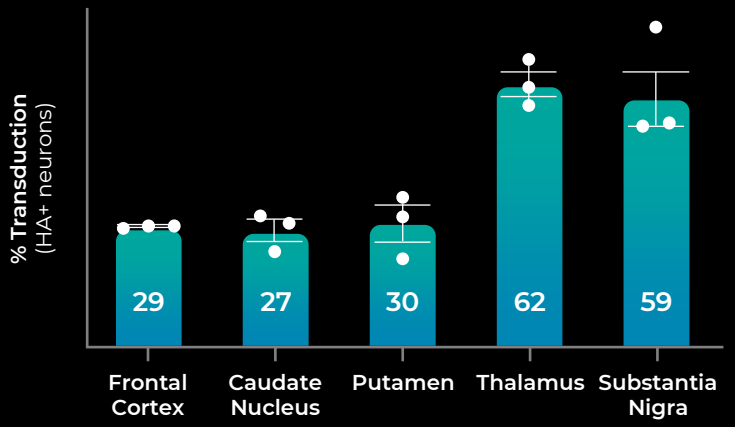
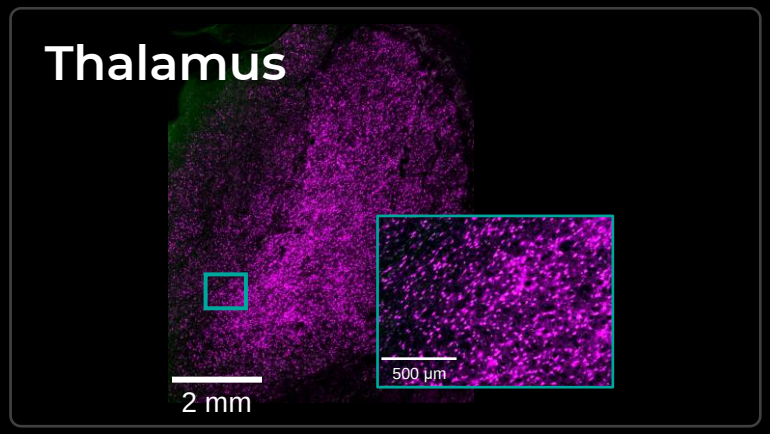
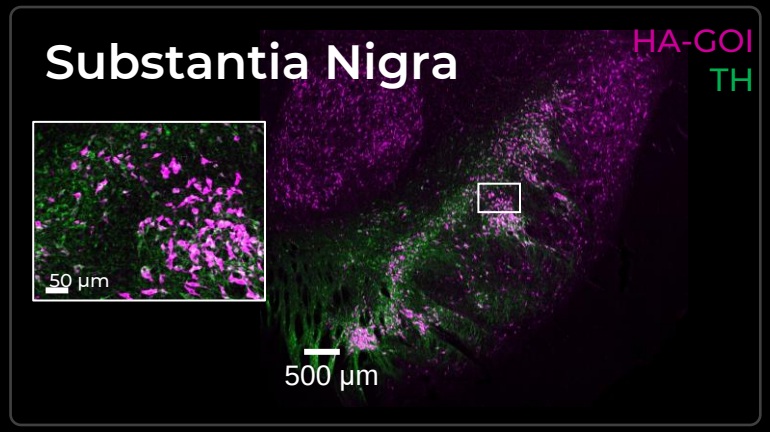
Lower efficacious doses
Wider Therapeutic Index

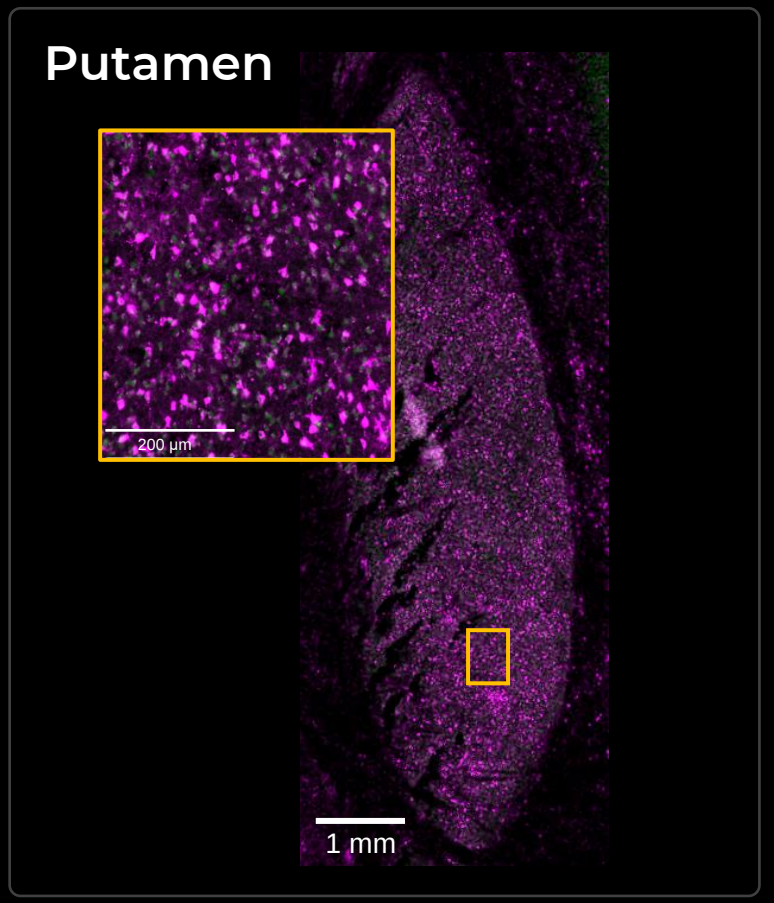
Gen 5 Capsid Results In Widespread Protein Expression Across Representative Areas Of The Brain Following IV Delivery











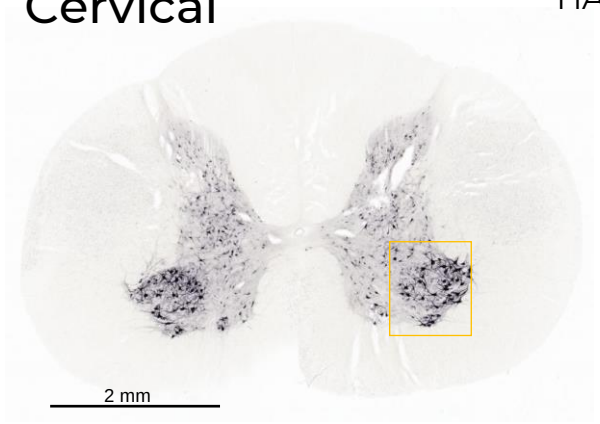
Capsid: Gen 5
Cargo: HA-GOI
Species/Age: N = 3 cynomolgus macaques, ~42mo

Gen 5 Capsid Expression Extends To Spinal Cord And Is Well Tolerated Following IV Delivery

Spinal Cord

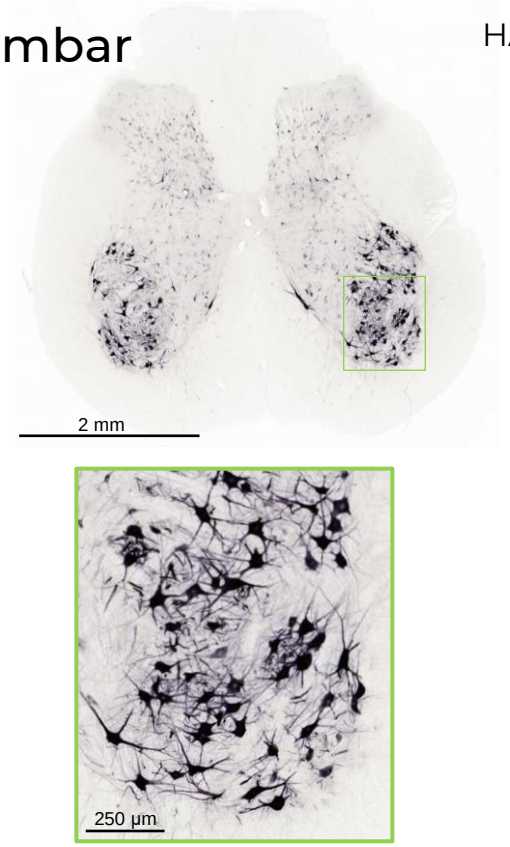
Cervical

HA



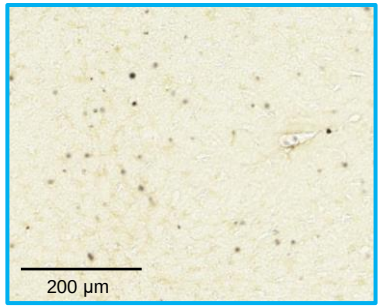
Lumbar

HA



Liver

HA



16X de-targeted compared to AAV9

Capsid: Gen 5
Cargo: HA-GOI
Species/Age: N = 3 cynomolgus macaques/ ~42mo

Gen 5 capsid is **well tolerated** with no clinical pathology or immunogenicity findings

Unremarkable histopathology across the body, including **liver** and **DRGs**

Pipeline for Rare and Common Diseases Across All Ages

Capsida Wholly Owned Programs

Disease / Target	Cargo	Discovery	IND-Enabling	Clinical
Genetic Epilepsy due to STXBP1 mutations	Gene Supplementation	CAP-002		
Undisclosed	Gene Supplementation	CAP-003		

Partnered Programs

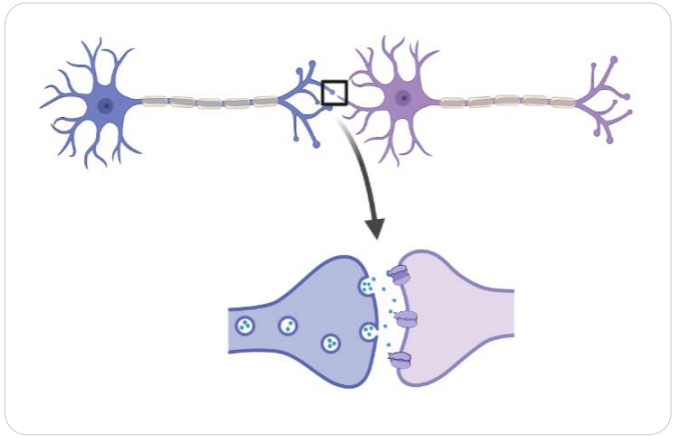
Disease / Target	Cargo	Partner	Co/Co Option
Neurological Diseases & Disorders (3)	Undisclosed	abbvie	One Program, U.S. Profit Share
Neurological Diseases & Disorders	Undisclosed	Lilly Prevail Therapeutics	One Program, U.S. Margin Share
Ophthalmology Diseases & Disorders (3)	Undisclosed	abbvie	
Friedreich's Ataxia	Editing	CRISPR Therapeutics	CRISPR owned, Capsida Co/Co Option
ALS	Editing	CRISPR Therapeutics	Capsida owned, CRISPR Co/Co Option

Syntaxin-binding Protein 1 (STXBP1) Genetic Epilepsy

STXBP1 Genetic Mutation
Autosomal dominant

STXBP1 is expressed in every neuron and is essential for neurotransmitter release

Reduction in STXBP1 levels results in impaired neurotransmission



Severe Disease Manifestations

- Refractory seizures
- Developmental delay, cognitive dysfunction, and intellectual disability
- Absent speech
- Behavioral issues
- Motor abnormalities
- Early mortality

Extreme Unmet Need

- No approved therapies
- Anti-seizure medications only partially effective

Robust Translational Evidence

- Collaboration & exclusive license with Mingshan Xue, Baylor College of Medicine
- Haploinsufficient mouse model shows **dose-dependent correction of seizures, cognitive, and motor deficits** with STXBP1 gene supplementation

Commercial Opportunity Potential >$1B

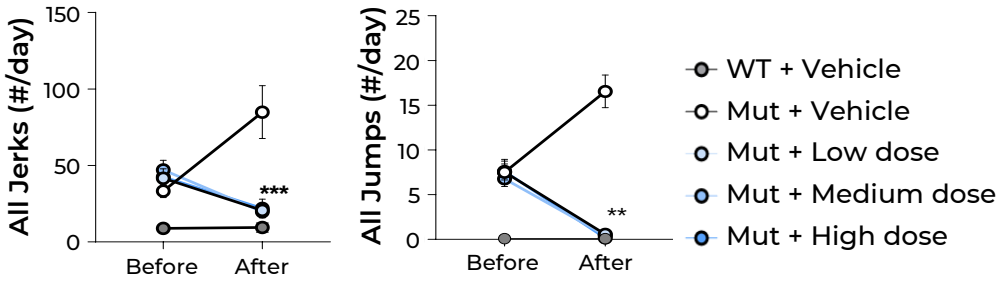
- No disease modifying programs in clinical development
- Potential to be first-in-class and first-in-disease
- 1:30,000 live births[1] (up to 4500 in US and EU) and growing

[1]Lopez-Rivera et al., Brain, 2020

Dose-dependent Therapeutic Efficacy of Gene Supplementation Enabled by Brain-wide STXBP1 Expression

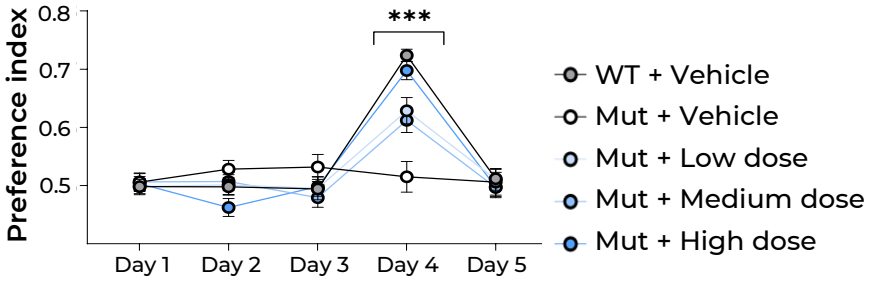
Epilepsy

Myoclonic Seizures



Cognitive Dysfunction

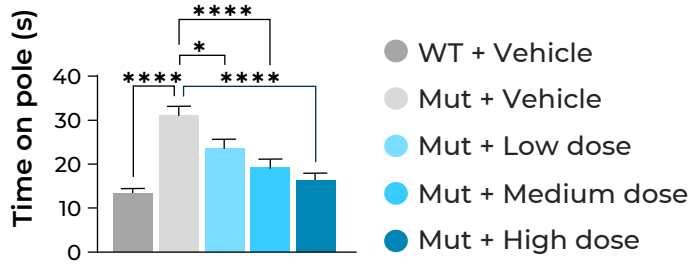
Novel Object Recognition



Brain-wide Stxbp1 expression achieves significant reduction of myoclonic seizures and novel object recognition in murine model at all doses

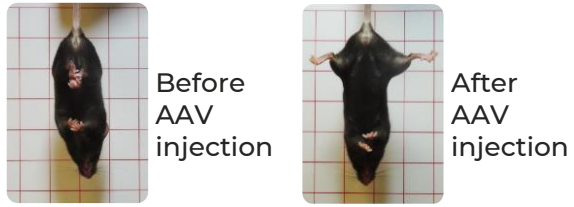
Motor Deficits

Vertical Pole



Dystonia

Mut + High Dose AAV



Dystonia[1] Correction by Gene Supplementation

WT	Mut	Low	Medium	High
0.18	2.50	1.49	0.84	0.62

[1]Medians based on ordinal data

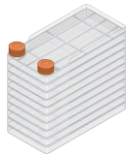
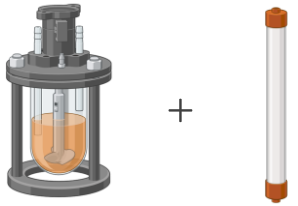
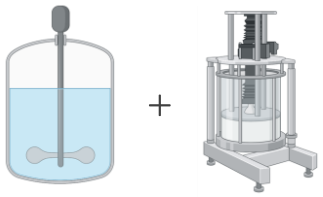
Brain-wide Stxbp1 expression achieves correction of vertical pole motor ability and dystonia (hindlimb clasping) in murine model

Difference from Mut + VEH: ns, non-significant, * $p < 0.05$, ** $p < 0.01$, *** $p < 0.001$, **** $p < 0.0001$

Data generated in collaboration with lab of Mingshan Xue, Baylor College of Medicine

Effective treatment of disease manifestations requires brain-wide supplementation of the hSTXBP1 gene **achievable with Capsida's breakthrough capsids** to correct circuits associated with seizure, cognitive and motor phenotypes

Integrated Process & Analytical Development and cGMP Capabilities Accelerates Capsid Prioritization and Clinical Supply

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

Experienced MFR and PD staff

In house capabilities reduce turn-around times and expedite process transfer to support clinical studies

Strong Capital Position Through Venture and Partnerships

$265M
Funding to Date

>$2B
In Milestones

$50M
Series A

VERSANT ventures

WESTLAKE VILLAGE BIOPARTNERS

$90M
Upfront & Equity

AbbVie CNS collaboration, up to **$530M** in development potential milestones, excluding commercial

abbvie

$55M
Upfront & Equity

Lilly CNS collaboration, up to **$685M** in potential development and commercial milestones

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

$70M
Upfront & Equity

AbbVie Ophthalmology expansion, **$595M** in potential development milestones, excluding commercial

abbvie

Looking Ahead

Advance

Advance differentiated internal development candidates to IND and through clinical proof-of-concept

- 4000x therapeutic window vs. wt AAV9
- Broad and well-tolerated neuronal expression across brain regions and spinal cord with significant liver detargeting
- Manufacturability profile inline with wtAAV9

Collaborate

Collaborate with our partners to achieve milestones and advance potential co-development programs

abbvie

CRISPR THERAPEUTICS

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

Expand

Expand opportunities to leverage breakthrough capsids for additional high unmet diseases

- Neurology
- Ophthalmology
- Other TA

Execute

Execute by managing cash and cultivating talent to grow and maximize value creating opportunities for patients and shareholders

CAPSIDA BIOTHERAPEUTICS

Our Pipeline is Making the Impossible Possible

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