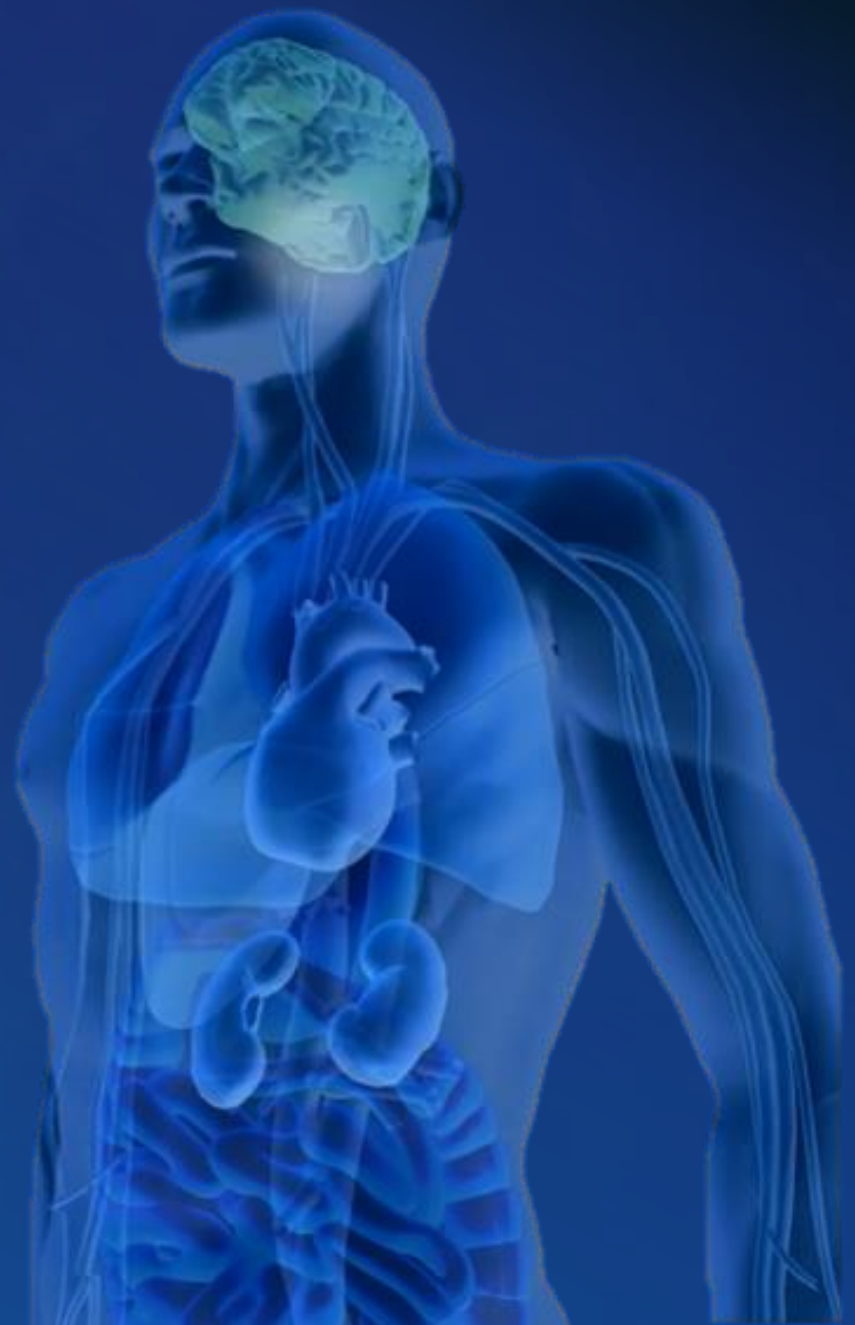
CAPSIDA BIOTHERAPEUTICS

UNLOCKING THE POTENTIAL OF GENE THERAPY FOR ALL



October 2023

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

2020

Build
Design & break ground on new Research & GMP Manufacturing facility in Thousand Oaks

2021

Team Up
CRISPR Research Collaboration

2022

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

Partner
AbbVie CNS deal **$90M** upfront incl. equity

Celebrate
Grand Opening of 50,000ft^2 research and GMP manufacturing facility

2023

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

Partner
AbbVie Ophthalmology deal **$70M** upfront incl. equity

Present
ASGCT Industry Symposium on **Breakthrough** Capsids (**up to 68%** neurons)

Present
ETDD Presentation on **genetic epilepsy** (STXBP1 preclinical data)

Partner
Kate Therapeutics Manufacturing Collaboration

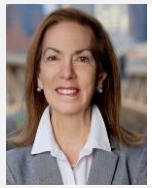
LEADERSHIP TEAM AND BOARD OF DIRECTORS

Decades of Industry Experience and Drug Development Expertise

Leadership

Peter Anastasiou
Chief Executive Officer

Julie Hakim
Chief Financial Officer



Susan Catalano
Chief Scientific Officer

Bethany Mancilla
Chief Business Officer

Nicholas Flytzanis, PhD
Founder
Chief Research and Innovation Officer

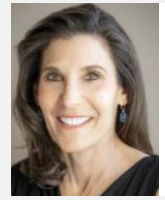
Rob Murphy
Vice President, Technical Operations

Nick Goeden, PhD
Founder
Chief Technology Officer

Swati Tole, MD
Chief Medical Officer

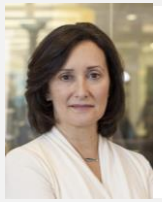
Board Members

Clare Ozawa, PhD



Beth Seidenberg, MD

Viviana Gradinaru, PhD
Founder



Rita Balice-Gordon, PhD

Frank Verwiel, MD

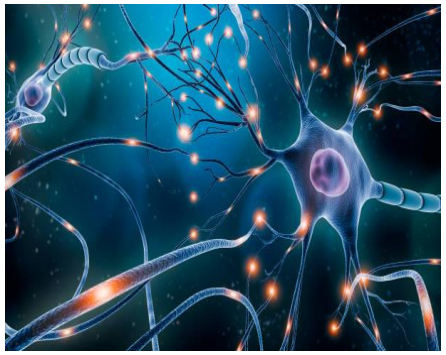
Peter Anastasiou
Chief Executive Officer

RECENT FDA APPROVALS CREATE SIGNIFICANT MOMENTUM

01 Gene Therapy Approvals

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

- **Luxturna**™ (RPE65 retinal dystrophy), **Zolgensma**™ (SMA), **Zynteglo**™ (ß-thalassemia), **Skysona**™ (CALD), **Hemgenix**™ (Hemophilia B), **VYJUVEK**™ (dystrophic epidermolysis bullosa), **Elevidys**™ (DMD), **Roctavian**™ (Hemophilia B)



02 Breakthrough Neurological Therapies

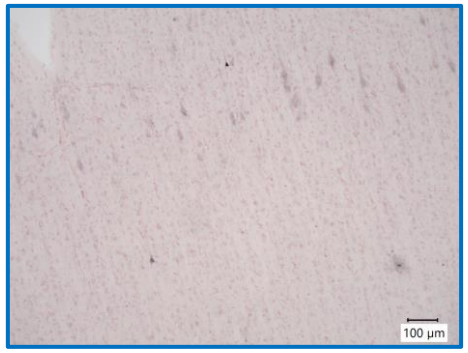
FDA Approval for disease modifying CNS therapies based on biomarker data:

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

CAPSIDA ADDRESSES CNS CHALLENGES THROUGH OUR ENGINEERED GENE THERAPIES

Challenges

AAV9 (IV)

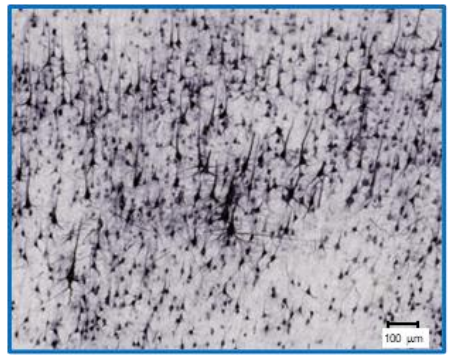


Cortex

1. **Limited ability to cross biological barriers, esp. BBB - <1% transduction with AAV9 IV**
2. **Safety concerns / liver toxicity**
3. **Traditional gene therapies primarily for ultra-rare/rare diseases**
4. **Direct injection ICM/IT invasive with significant risks**

Capsida Solutions

Capsida Engineered (IV)



Cortex

1. **Engineered capsids cross BBB with high levels of neuronal transduction – up to 68% neurons**
2. **Enabling lower dosing and up to 1000x difference in CNS to liver targeting vs. AAV9**
3. **Access to more common diseases across all ages**
4. **Targeted IV admin increases effectiveness and reduces risks**

NHP DRIVEN TARGETED GENE THERAPY ENGINEERING PLATFORM

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

Billions of variants to screen for enriched variants by DNA and RNA via NGS

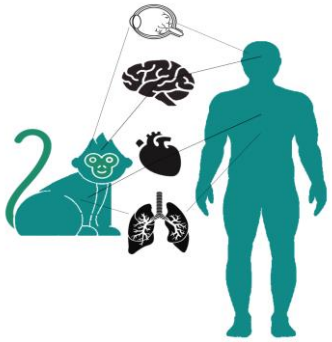
Library Screen

Pools with thousand of variants adds ability to assess protein expression and preliminary manufacturability

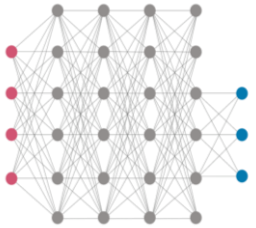
Pooled Test

Characterization adds complete assessment of cell-type distribution, immunogenicity/toxicity profile, and manufacturing scalability

Characterization



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



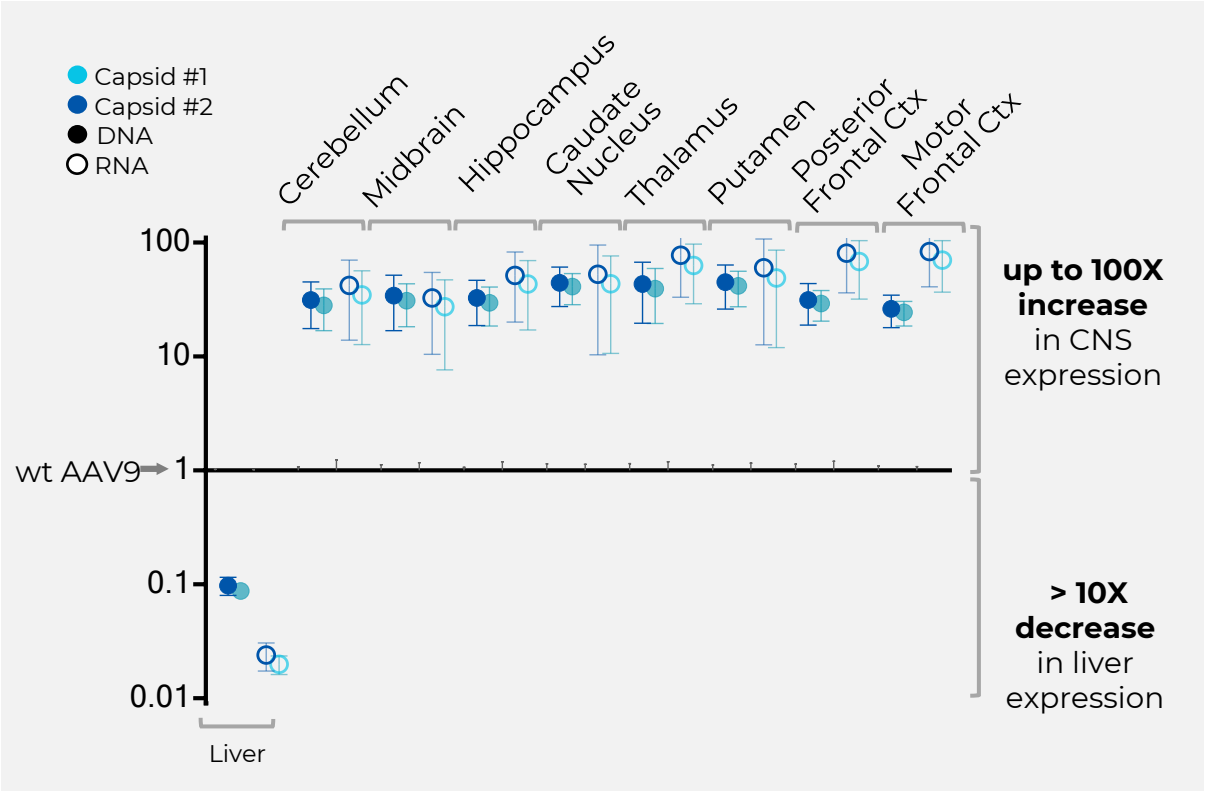
Sequence and Structure based analysis

Further Variant Optimization and Re-Diversification

IND-enabling

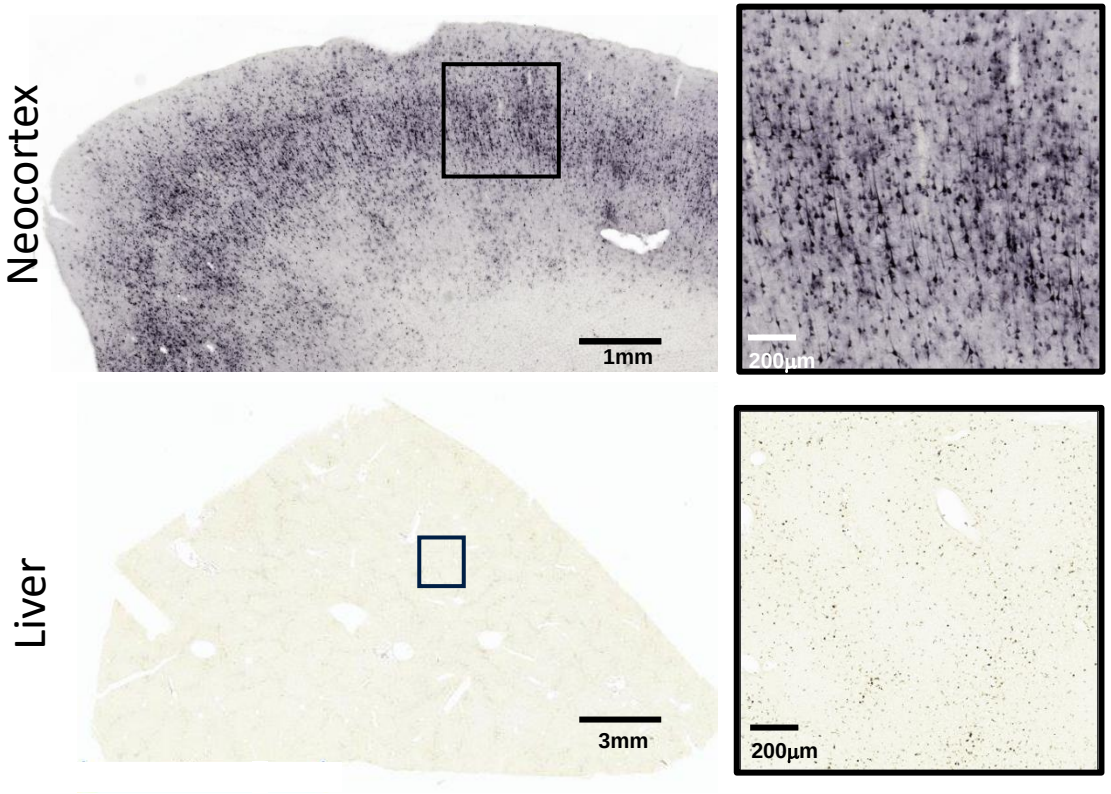
CAPSIDA'S ENGINEERED CAPSIDS YIELD ROBUST EXPRESSION ACROSS THE CNS AND SIGNIFICANT LIVER DE-TARGETING vs AAV9 IN NHPs

DNA and RNA Enrichment over AAV9



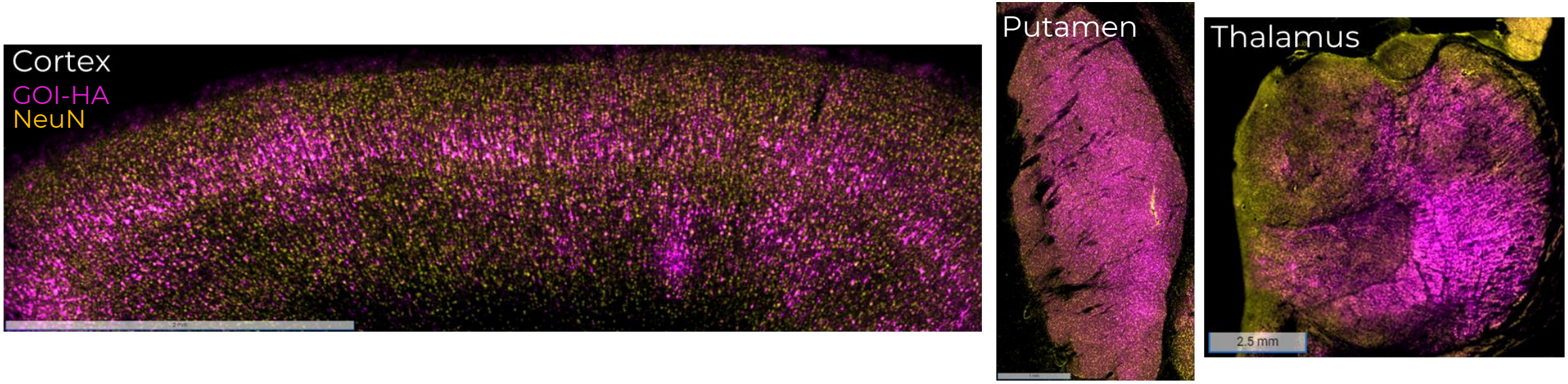
Single Variant Characterization Study

GOI-HA (single strand cargo)



Up to 1000X difference in CNS vs liver targeting with Capsida's breakthrough capsids relative to wtAAV9 = **Lower efficacious doses Wider Therapeutic Index**

NHP ENGINEERED CAPSID SHOWS WIDESPREAD TRANSDUCTION OF NHP BRAIN TISSUE



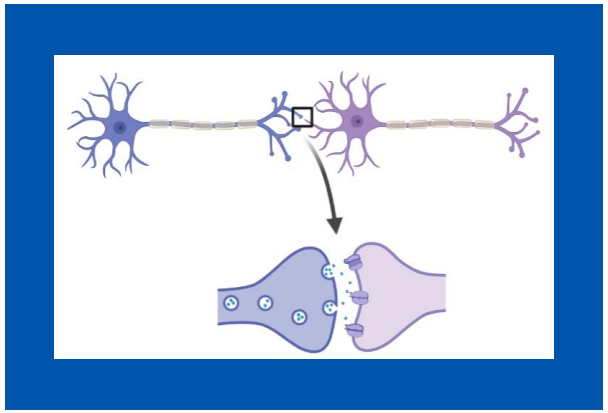
- **Systemic (i.v.) delivery** of **next generation engineered capsid** at 2.5 e13 vg/kg in 17-18 month old cynomolgus non-human primates with single strand cargo
- Preliminary data showcase **widespread transduction** across **multiple brain regions**, including **neocortex, putamen and thalamus**
- In **some brain regions**, **up to 68%** of neurons are transduced

PIPELINE FOR RARE AND COMMON DISEASES ACROSS ALL AGES

Two wholly owned programs and four co-development and co-commercialization program options

Indication	Cargo	Ownership	
Capsida Wholly Owned Programs			
Genetic Epilepsy STXBP1 mutations	Replacement	CAPSIDA BIOTHERAPEUTICS	
Undisclosed	Replacement	CAPSIDA BIOTHERAPEUTICS	
Partnered Programs		**Partner**	**Co / Co Option**
Neurological Diseases & Disorders (3)	Undisclosed	abbvie	*One Program U.S. Profit Share*
Neurological Diseases & Disorders	Undisclosed	Lilly Prevail THERAPEUTICS A Wholly Owned Subsidiary of Eli Lilly and Company	*One Program U.S. Margin Share*
Friedreich's Ataxia	Editing	CRISPR THERAPEUTICS	*CRISPR owned Capsida Co/Co Option*
ALS	Editing	CRISPR THERAPEUTICS	*Capsida owned CRISPR Co/Co Option*
Ophthalmology Diseases & Disorders (3)	Undisclosed	abbvie	

SYNTAXIN-BINDING PROTEIN 1 (STXBP1) GENETIC EPILEPSY



STXBP1 Genetic Mutation
Autosomal dominant

STXBP1 is present in every neuron in the brain and has an essential role in synaptic vesicle fusion and neurotransmitter release

Reduced STXBP1 protein results in impaired synaptic vesicle release and impaired neurotransmission

Severe Disease Manifestations

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

Robust Translational Evidence

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

Extreme Unmet Need

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

Commercial Opportunity

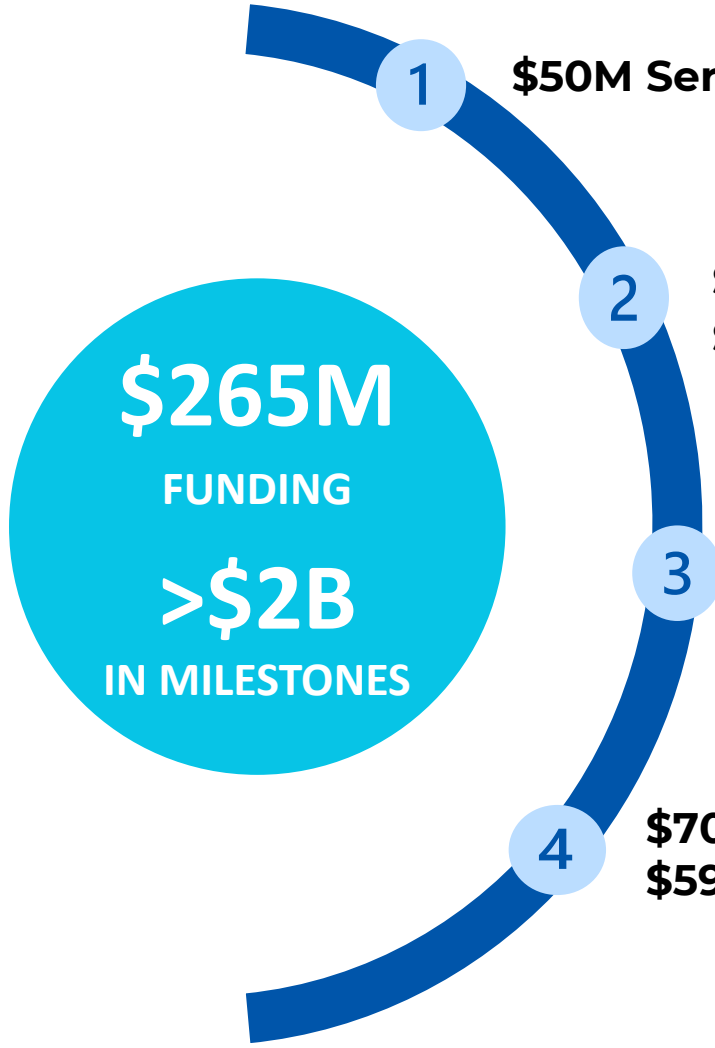
- No disease modifying programs in clinical development
- Potential to be first-in-class and first-in-disease
- >$1B opportunity, 1:30,000 live births[1] (up to 4500 in US and EU) and growing

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

15,000 FT^2 GMP MANUFACTURING FACILITY IN THOUSAND OAKS, CA

- Brand-new (2021) state-of-the-art facility
- AAV suspension process scalable up to 1000L
- Modular clean-room suites
- Finish - Fill operations
- Analytical & quality control labs
- Experienced Mfg and PD staff

STRONG CAPITAL POSITION THROUGH VENTURE AND PARTNERSHIPS



1. **$50M Series A**: Versant and Westlake Village BioVentures
2. **$90M Upfront & Equity**: AbbVie CNS collaboration, up to **$530M** in development milestones, excluding commercial
3. **$55M Upfront & Equity**: Lilly CNS collaboration, up to **$685M** in development and commercial milestones
4. **$70M Upfront & Equity**: AbbVie Ophthalmology expansion, **$595M** in development milestones, excluding commercial

LOOKING AHEAD

Advance

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

Collaborate

abbvie

Lilly

CRISPR THERAPEUTICS

Prevail THERAPEUTICS
A Wholly Owned Subsidiary of Eli Lilly and Company

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

Expand

- Neurological
- Ophthalmology
- Other TA

Expand opportunities to leverage breakthrough capsids for additional high unmet diseases

Execute

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

CAPSIDA BIOTHERAPEUTICS

THANK YOU

info@capsida.com

1300 Rancho Conejo Blvd
Thousand Oaks, California

www.capsida.com