

## UNLOCKING THE POTENTIAL OF GENE THERAPY FOR ALL

June 2023





#### **CAPSIDA BIOTHERAPEUTICS**







Fully integrated capabilities: discovery, pre-clinical, development & manufacturing under one roof



Pipeline of wholly owned and partnered programs in rare and more common CNS disorders and ophthalmology



### **COMPANY HISTORY**





Decades of Industry Experience and Drug Development Expertise





## OUR PURPOSE

Unlock The Potential of Gene Therapy for All

### **OUR CORE VALUES**





#### In It Together

We harness the power of our diverse backgrounds and thoughts to deliver on our purpose. We have a sense of collective responsibility and unity. *Traits: Collaborative. Caring.* 

#### **Driven By Curiosity**

We approach every challenge with energy and excitement. We remain resilient in the face of adversity because we understand innovative science is not easy.

Traits: Passionate. Resilient.



#### **Innovation Meets Execution**

We blend innovation with execution in the pursuit to improve patient lives. We're not limited by the way things have always been done. *Traits: Excellence. Agile.* 



## Capsida addresses the challenges with GEN-1 gene therapies in CNS

	Challenges		Capsida Solutions
1	Limited ability to cross biological barriers, esp. BBB	1	Engineered capsids cross BBB with high levels of neuronal transduction
2	Safety concerns / liver toxicity	2	Enabling lower dosing and liver de-targeting
3	Traditional gene therapies primarily for ultra-rare/rare diseases	3	Access to more common diseases across all ages
4	Direct injection ICM/IT invasive with significant risks	4	Targeted IV admin increases effectiveness and reduces risks
5	Inability to treat diseases impacting multiple organs	5	Single engineered capsid can target multiple organs simultaneously



## **EVOLUTION OF CAPSIDA'S PLATFORM FOR CNS DISEASE**

**Capsida Yesterday** 

5-fold reduction in liver burden relative to AAV9

Systemic delivery with moderate transduction

• Up to 20% of neurons depending on brain or

spinal cord regions

Engineered Capsids are Opening Doors into Previously Unreachable Indications

#### Wild-type AAV

- Local or CSF delivery
- Systemic delivery with sparse transduction
- <1% of cells in brain or spinal cord
- Liver toxicity

### AAV9 Gen3 Gen4 Gen5 GOI-HA Thalamus Cortex 100 µm 100 un

**Capsida Today** 

• Up to 68% of neurons depending on brain

region

Systemic delivery with marked transduction

5-fold reduction in liver burden relative to AAV9



#### TARGETED NHP DRIVEN GENE THERAPY ENGINEERING PLATFORM

Scalable Generation of High Complexity and High-fidelity Engineered Capsid Libraries





# Significant increase in CNS tropism & liver de-targeting vs. AAV9 in single variant characterization study



GOI-HA (single strand cargo)







Our engineered capsids yield robust expression throughout the NHP CNS with concurrent liver detargeting

Capsida Biotherapeutics



# Widespread transduction of NHP brain tissue by next-gen engineered capsid (GEN5) in single variant characterization study



- Systemic (i.v.) delivery of next generation engineered capsids 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
- Liver transduction is reduced five-fold compared to wild-type AAV



#### FULLY INTEGRATED GENE THERAPY PLATFORM COMPANY





## 15,000 FT<sup>2</sup> GMP MANUFACTURING FACILITY IN THOUSAND OAKS, CA

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





#### PIPELINE

#### CNS is initial focus, advancing into additional therapeutic areas



Current pipeline includes both rare and more common CNS diseases across ages



#### **Internal Pipeline**

Two wholly owned programs, including Genetic Epilepsy due to Syntaxin-binding protein 1 (STXBP1) mutations



#### **Partnered Pipeline**

AbbVie – 3 CNS targets, 3 Ophthalmology targets Lilly/Prevail – CNS targets CRISPR Tx – Friedreich's Ataxia and ALS



All CNS programs IVadministered, increasing effectiveness and decreasing risks associated with invasive delivery

Capsida Biotherapeutics



## **THANK YOU**

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