Capsida Biotherapeutics to Present New Data on its Wholly Owned Gene Therapy Programs in Genetic Epilepsy and Parkinson's Disease at the Annual Meeting of the American Society of Gene & Cell Therapy (ASGCT)

Capsida Biotherapeutics

Company unveils Parkinson's disease associated with GBA mutations (PD-GBA) program; two oral presentations feature new data from Capsida's wholly owned programs in PD-GBA and genetic epilepsy due to STXBP1 mutations; both programs in IND-enabling studies to support initiation of clinical trials first half of 2025

Breakthrough data in non-human primates with development candidates for STXBP1 and PD-GBA show up to 70% neuronal expression in key brain regions

Third oral presentation showcases broadly enabling IV-

delivered, highly neuronal targeted and liver-detargeted capsids from Company's proprietary engineering platform

Further advancements in capsid engineering and product manufacturing highlighted in five poster presentations

THOUSAND OAKS, Calif., April 22, 2024 /PRNewswire/ -- Capsida Biotherapeutics ("Capsida"), a leading fully integrated targeted gene therapy company, developing treatments for rare and common diseases across all ages, today announced eight presentations – three oral presentations and five poster presentations – at the American Society of Gene & Cell Therapy (ASGCT) 2024 Annual Meeting, taking place May 7-11, 2024 in Baltimore, MD and virtually.

"We are excited to unveil our PD-GBA program and new data on our STXBP1 program as part of oral presentations at ASGCT," said Peter Anastasiou, Capsida's Chief Executive Officer. "Both development candidates are IV-administered and achieve breakthrough levels of neuronal transduction throughout the brain, up to 70% in certain brain regions, while simultaneously detargeting the liver. We are focused on successfully executing IND-enabling studies for both programs to support initiation of clinical trials in the first half of 2025, so we can get these therapies to people with genetic epilepsy and Parkinson's disease who so desperately need them."

The oral presentation on Capsida's wholly owned Parkinson's disease associated with GBA mutations (PD-GBA) program showcases development candidate data demonstrating high levels of GCase supplementation throughout the brain at well-tolerated doses, while simultaneously detargeting the liver. The oral presentation on the Company's wholly owned STXBP1 program will be presented by Capsida's collaborator in the laboratory of Professor Mingshan Xue, Ph.D., Associate Professor, Department of Neuroscience, Department of Molecular and Human Genetics at the Baylor College of Medicine. Data will describe the dose-dependent rescue of key phenotypic defects in adult STXBP1 haploinsufficient mice following intravenous (IV) administration of a next-generation adeno-associated virus (AAV) gene therapy that achieves brain-wide neuronal expression. Capsida's third oral presentation highlights the power of its broadly enabling IV-delivered engineered capsids which are the backbone of the Company's differentiated central nervous system (CNS) lead programs.

In addition to the oral presentations, Capsida will have five poster presentations covering additional non-human primate (NHP) data on its lead STXBP1 program and data highlighting achievements from the Company's capsid engineering and end-to-end, fully integrated internal process development, analytical development, and

manufacturing.

The presentations are listed below. Abstracts can be found at https://annualmeeting.asgct.org/. Data from the oral presentations are embargoed until 6:00 AM ET on the presentation day.

Oral Presentations:

Preclinical
Systemic AAV Gene Therapy with CNS-Targeted Engineered Capsids Achieves Significant GCase Activity Increases in the Primate Brain to Support the Potential Treatment of GBA-PD
Date: Friday, May 10, 2024, 3:00-3:15 PM ET
Session: Neurologic Diseases III
Abstract Number: 274
Location: Room 307-308
Presenter: Nicholas Flytzanis, Ph.D., Founder, Chief Research and Innovation Officer, Capsida

AAV Gene Therapy Corrects Neurological Phenotypes with Clinically Relevant Doses in a Mouse Model of STXBP1-Related Developmental and Epileptic Encephalopathy
Date: Tuesday, May 7, 2024, 3:00-3:15 PM ET
Session: Neurologic Diseases I
Abstract Number: 38

Location: Ballroom 4
Presenter: Wu Chen, Ph.D., Instructor, Department of Neuroscience, Baylor College of Medicine

Engineering
Directed Evolution of AAV9 Libraries in Non-Human Primates Identifies a Capsid Family with Enhanced Central Nervous System Tropism and Liver De-Targeting Following Systemic Delivery
Date: Wednesday, May 8, 2024, 5:15-5:30 PM ET
Session: Breaking Barriers to the CNS via AAV Capsid Engineering
Abstract Number: 122
Location: Room 309-310
Presenter: Xiaojing Shi, Ph.D., Principal Scientist, Capsida

Poster Presentations:

Preclinical
CAP-002: Systemic AAV Gene Therapy with Next Generation Capsids for Treatment of STXBP1 Encephalopathy
Date: Wednesday, May 8, 2024, 12:00-7:00 PM ET
Session: AAV Vectors – Non-Human Primates and Large Animal Models
Abstract Number: 504
Location: Exhibit Hall
Presenter: Allison Knoll, Ph.D., Director, Preclinical Research,

Capsida

Engineering

Directed Evolution of AAV2 Libraries Yields Capsids with Improved Performance in the Central Nervous System and Cross-species Translatability

Date: Thursday, May 9, 2024, 12:00-7:00 PM ET
Session: AAV Vectors – Capsid Engineering
Abstract Number: 992
Location: Exhibit Hall
Presenter: Sean Gross, Ph.D., Principal Scientist, Capsida

Product Development, Manufacturing, and Approval

Characterization of Engineered AAV Capsids From Different HEK293 Cell Culture Fractions, Crude Lysate Versus Cell Pellet Material

Date: Wednesday, May 8, 2024, 12:00-7:00 PM ET
Session: AAV Vectors – Product Development, Manufacturing, and Approval Considerations
Abstract Number: 529
Location: Exhibit Hall
Presenter: Heidy Morales, Associate Scientist, Capsida

Alternative Plasmid Designs Including Two Plasmid Transfection Systems for Improved Production and Packaging of Engineered AAV Capsids

Date: Wednesday, May 8, 2024, 12:00-7:00 PM ET
Session: AAV Vectors – Product Development,

Manufacturing, and Approval Considerations
Abstract Number: 530
Location: Exhibit Hall
Presenter: Lysa-Anne Volpe, Principal Scientist , Capsida

Separation of Empty and Full Engineered Adeno-Associated Virus Capsids Using a Weak Anion Exchanger
Date: Thursday, May 9, 2024, 12:00-7:00 PM ET
Session: AAV Vectors – Product Development, Manufacturing, and Approval Considerations
Abstract Number: 1038
Location: Exhibit Hall
Presenter: Varun Gejji, Ph.D., Scientist II, Capsida

About Capsida Biotherapeutics
Capsida Biotherapeutics is a fully integrated gene therapy company with a central nervous system (CNS) pipeline consisting of disease modifying and potentially curative treatments for rare and more common diseases across all ages. Capsida's intravenously (IV) administered gene therapies utilize proprietary engineered capsids that enable high transduction levels to desired tissues and cells, while limiting tropism to non-target organs, such as the liver. Capsida has three wholly owned programs, including potential best-in-class treatments for genetic epilepsy due to STXBP1 mutations and Parkinson's disease associated

with GBA mutations, both of which are in IND-enabling studies. In addition to its wholly owned programs, the Company has validating CNS partnerships with AbbVie, Lilly, CRISPR Therapeutics, and the AbbVie partnership was expanded to include ophthalmology disorders. Capsida was founded in 2019 by lead investors Versant Ventures and Westlake Village BioPartners and originated from groundbreaking research in the laboratory of Viviana Gradinaru, Ph.D., a neuroscience professor at Caltech. Visit us at www.capsida.com.

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