Coave Therapeutics Launches coAAV-CSF-01, a Novel CNS-Targeted Gene Therapy Vector for Neurodegenerative and other CNS Diseases

New breakthrough data from studies with Coave's ALIGATER™-engineered AAV2-based capsid coAAV-CSF-01 (S01coAAV2) demonstrates improved brain tissue transduction and safety following intra-cerebrospinal fluid administration in non-human primates

Data selected for Late-Breaking Abstract at ASGCT 2025

Paris, France – May 9, 2025 – Coave Therapeutics ("Coave"), a company pioneering the future of genetic medicines, today announced the launch of coAAV-CSF-01, a novel CNS-targeted gene therapy capsid developed using its proprietary ALIGATER™ platform. New breakthrough data from studies evaluating the vector (research code S01coAAV2) have been selected for presentation as a Late-Breaking Abstract at the 2025 American Society of Gene & Cell Therapy (ASGCT) Annual Meeting, to be held May 13-17, in New Orleans, LA, USA.

The data highlight the therapeutic potential of coAAV-CSF-01 (S01coAAV2) to transform central nervous system (CNS) gene therapy delivery. In non-human primate (NHP) studies, coAAV-CSF-01 was administered via intra-cerebrospinal fluid (intra-CSF) routes, including intracisternal magna (ICM) and intracerebroventricular (ICV). The results demonstrated higher transgene expression in targeted brain regions and a better safety profile using coAAV-CSF-01 compared to AAV9.

Key findings with coAAV-CSF-01 include:

- 100-fold higher transgene expression in the cortex and 10,000-fold increase in the hippocampus compared to AAV9 at the same dose
- Comparable CNS biodistribution and expression at one-fifth the dose of AAV9
- Significantly reduced peripheral transduction (including strong liver de-targeting) and peripheral nerve safety, addressing key safety concerns in CNS gene therapy

"These exciting new data from coAAV-CSF-01 represent a major step forward in CNS gene therapy," said **Lolita Petit, CSO of Coave Therapeutics**. *"In non-human primates, the vector achieved robust brain transduction via intra-CSF delivery – a route long viewed as promising but historically constrained by limited efficacy and off-target effects. The enhanced biodistribution and improved safety demonstrated in these studies support its potential in developing new genetic medicines for neurodegenerative and neurodevelopmental CNS disorders.*

"Furthermore, the vector was developed using our proprietary ALIGATER™ platform, enabling modular modification of AAV2 and other capsids to optimize tissue targeting and therapeutic performance – potentially opening the door to broader therapeutic applications. Together, the data position coAAV-CSF-01 as a potentially transformative advance in gene therapy delivery."

coAAV-CSF-01 is part of Coave's growing portfolio of proprietary AAV capsids engineered to optimize gene delivery in challenging therapeutic areas. The ALIGATER™ platform enables chemical conjugation-based modification of AAV vectors (coAAVs), offering a modular and scalable approach to enhance tissue targeting and transduction efficiency.

Late-Breaking Poster Presentation Details:

- Title: *Enhanced CNS transduction and safety of S01coAAV2 (coAAV-CSF-01) following intra-cerebrospinal fluid (CSF) administration in cynomolgus macaques*
- Session: Late-Breaking Abstracts
- Date/Time: Thursday May 15, 2025, 4:30 – 6pm CDT
- Location: New Orleans
- Presenter: Julien Spatazza, Senior Director, Discovery & Preclinical Research at Coave Therapeutics

Coave's abstract (#LBA78) can be viewed on ASGCT's website (https://annualmeeting.asgct.org/).

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About ALIGATER™
Coave's proprietary ALIGATER™ (Advanced Vectors-Ligand Conjugates) platform is a breakthrough technology addressing key limitations in the delivery of genetic payloads to extra-hepatic tissues, including limited tissue specificity, delivery efficiency and safety. ALIGATER™ enables conjugation of targeting ligands, such as small molecules, peptides, or antibody fragments, on AAV or non-viral vectors, offering superior delivery efficiency, tissue specificity and safety profile for a broad range of diseases. Importantly, the platform streamlines the manufacturing process by avoiding prior AAV capsid modifications. These capabilities will enable Coave to develop best-in-class gene therapies designed for specific indications.

About Coave Therapeutics
Coave Therapeutics is a genetic medicine company pioneering the development of innovative solutions to enhance the precision, safety, efficacy and manufacturability of genetic medicines. With its proprietary ALIGATER™ platform, Coave is at the forefront of addressing challenges in gene therapy delivery to extra-hepatic tissues, creating a robust pipeline targeting CNS, neuromuscular and eye diseases.

Headquartered in Paris, France, Coave Therapeutics is backed by leading international life sciences investors. For more information about the science, pipeline, and people, please visit coavetx.com and follow us on LinkedIn.

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