



Coave Therapeutics Showcases its ALIGATER™ Platform for Generating Conjugated AAV (coAAV) Vectors Exhibiting Superior Performance in Delivering Ocular Gene Therapy through the Suprachoroidal Route

coAAV-mediated gene transfer holds promise for expanding the scope of ocular diseases treatable via suprachoroidal administration

Data to be presented in oral session at ASGCT 2024

Paris, France, April 29, 2024 – Coave Therapeutics ('Coave'), a genetic medicine company focused on developing life-changing therapies, announces today that preclinical studies on its novel Conjugated AAV (coAAV) gene therapy vectors demonstrate promising results for ocular gene therapy. Engineered coAAV vectors, generated using Coave's ALIGATER™ platform, show the ability to increase both the distribution and expression patterns of transgenes delivered via suprachoroidal administration, effectively improving the targeting of key tissues at the back of the eye. This advancement suggests a new, potentially impactful approach for treating acquired retinal diseases. These data, generated in collaboration with REGENXBIO, will be presented at the 2024 American Society of Gene & Cell Therapy (ASGCT) meeting, which will be held virtually and in person in Baltimore, MD, US May 7-11.

Oral presentation details are as follows:

- **Abstract Title:** *Identification of Novel Ligand-Conjugated AAV Vectors with Enhanced Properties for Suprachoroidal Gene Delivery*
- **Abstract Number:** 86
- **Date & Time:** Wednesday May 8, 2024; 1:30 p.m. – 3:15 p.m. ET
- **Poster Session:** Ophthalmic & Auditory: Delivery Innovations
- **Room:** 318 - 323
- **Presenter:** Gaelle Lefevre, VP Alliance & Portfolio Strategy at Coave Therapeutics

Using its ALIGATER™ platform, Coave has developed a range of new coAAV vectors, each specifically engineered with a distinct set of small molecule ligands at its surface through bioconjugation. These coAAVs have been screened for administration to the suprachoroidal space (SCS) of the eye, an attractive alternative to invasive subretinal administration to deliver AAV-based gene therapies to the back of the eye. A library of coAAV candidates was injected into the SCS of non-human primates (NHPs) and evaluated in comparison to an unconjugated, benchmark AAV capsid. Three weeks post injection, the distribution of vector genome copies and the levels of transgene-specific expression were analysed in ocular and peripheral tissues.

Several of Coave's coAAVs showed more widespread and enhanced expression in key target tissues of the eye, notably the retina, versus the control vector, and lower off-target systemic distribution to tissues such as the liver and kidney.

Coave's coAAVs represent some of the first engineered AAV vectors optimised for SCS delivery. These vectors open new avenues for next-generation gene therapy vectors for ocular administration, potentially eliminating the need for invasive subretinal injections. The superior properties of coAAVs



for ocular gene transfer via SCS administration have the potential to enable the development of more efficient, targeted, and well-tolerated gene therapies for the treatment of common retinal disorders.

Dr Lolita Petit, Coave's Chief Scientific Officer, commented: *"Delivering gene therapies to the posterior segment of the eye, notably the retina, poses a key challenge: achieving broader distribution through a less invasive procedure compared to the subretinal injection route, while still ensuring sustained transgene expression in target cells. This is an exciting time as our new data emphasize, for the first time, the potential of improving the delivery of gene therapy through the suprachoroidal space using Coave's chemically engineered coAAVs. This holds the potential to enable broader applications in terms of scope of ocular diseases."*

Coave's abstract (number 86) can be viewed on ASGCT's website (<https://annualmeeting.asgct.org/>).

About Coave Therapeutics

At Coave Therapeutics, we are leading the transition of genetic medicine from rare to prevalent conditions, starting with neurodegenerative and eye diseases. Our proprietary ALIGATER™ (Advanced Vectors-Ligand Conjugates) platform introduces chemical modifications onto AAV capsids or Lipid Nanoparticles (LNPs) to overcome the limitations of current vectors on efficacy, safety, and manufacturability.

With low doses and optimized routes of administration, our conjugated vectors have demonstrated markedly improved transduction and biodistribution in the central nervous system and the eye across different species. Our diverse pipeline of novel genetic medicines can potentially transform the lives of people afflicted by rare and prevalent neurodegenerative and ocular diseases – including genetically and non-genetically defined indications.

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 <https://coavetx.com/> and follow us on [LinkedIn](#).

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