AAVantgarde presents positive preclinical data in large animal models from its Stargardt disease program in an oral presentation at the ASGCT 2024 annual meeting

April 23, 2024 — AAVantgarde Bio (AAVantgarde), a clinical-stage, Italian-based international biotechnology company with two proprietary Adeno-Associated Viral (AAV) vector platforms for large gene delivery, today announces the publication of an abstract accepted as an oral presentation submitted to The American Society of Gene & Cell Therapy 27th Annual Meeting (ASGCT), to be held May 7-11 in Baltimore.

The data to be presented confirms that AAV intein-mediated retinal gene therapy for Stargardt disease is effective and safe in large animal models (pig and NHP). In pigs, AAVantgarde demonstrate that lipofuscin accumulation in the retinal pigmented epithelium was reduced upon subretinal delivery of AAV-ABCA4 intein vectors. Similarly, in NHP, BaseScope analysis showed nearly total photoreceptor co-expression of mRNAs encoding both ABCA4-intein halves across an extended NHP retinal region.

Prof. Alberto Auricchio, CSO of AAVantgarde stated "We are very excited to be presenting positive Pig and NHP safety and efficacy data from our Stargardt program as an oral presentation, supporting both the efficacy and safety of AAV-ABCA4-intein vectors in relevant large animal models, and providing important insights towards the clinical translation of this platform for gene therapy of STGD1."

Dr. Natalia Misciattelli, CEO of AAVantgarde added that "This preclinical positive data in large animal models is promising and provides hope for underserved Stargardt patients that don't have any therapeutic options to prevent them from losing their sight."

Oral Presentation details:

Presentation Date/Time: Friday May 10, 2024, 4:00pm - 4:15pm

Session title: AAV Vectors - Non-Human Primates and Large Animal Models

Session Room: Ballroom 4

Final abstract number: 297

About AAVantgarde Bio
AAVantgarde Bio is a clinical stage, Italian headquartered, international biotechnology company that has developed two proprietary Adeno-Associated Viral (AAV) vector platforms to address the gene therapy cargo capacity limitations of AAV vectors. The AAVantgarde platforms could be used to deliver large genes to ocular and non-ocular tissues. Co-founded by Professor Alberto Auricchio at TIGEM (Telethon Institute of Genetics and Medicine) in Naples, Italy, and Telethon Foundation, AAVantgarde will initially validate the platform in the clinic in two inherited retinal diseases with clear unmet need. For more information, please visit: www.aavantgarde.com

Contact:
Magda Blanco – Head of Corporate Development AAVantgarde
Phone: +44 (0) 7522 128285
Email: m.blanco@aavantgarde.com