AAVantgarde announces its innovative clinical study design for its lead program in Usher 1B

May 3, 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 announced the presentation of the clinical trial design of the Company's lead program in Usher 1B by the program's Principal Investigator, Prof. Francesca Simonelli, at the 9th Annual Retinal Cell and Gene Therapy Innovation Summit, being held today in Seattle.

The LUCE-1 clinical study is a first-in-human Phase 1/2 clinical study designed in collaboration with Prof. Simonelli and other leading experts in the field. Leveraging AAVantgarde's proprietary Dual Hybrid platform, this study aims to provide robust evidence supporting the effectiveness and safety profile of the Company's lead program, AAVB-081, that addresses the retinitis pigmentosa derived from MYO7A-related Usher syndrome (USH1B).

"I am delighted to be presenting the pre-clinical and clinical activities that paved the way to the design of this first-in-human Phase 1/2 clinical study at the Summit. Through this innovative design, we aim to revolutionize our approach to understanding and treating Usher 1B patients. We are poised to generate robust evidence that will not only advance scientific knowledge, but also directly impact patient care," **said Prof. Francesca Simonelli, Head of Ophthalmology at the University of Campania Luigi Vanvitelli (Naples).**

Dr. Natalia Misciattelli, CEO of AAVantgarde added "We are honoured to have Prof. Simonelli as Principal Investigator for this first in human Phase 1/2 clinical study aimed at providing hope for underserved USH1B patients that have no therapeutic options to prevent them from losing their sight. Prof. Simonelli is a pioneer in gene therapy in ophthalmology and her valuable experience in this space will greatly help us to the successful development of this novel therapy."

Presentation details:

Title: Design of a Phase 1/2 clinical trial using a dual vector strategy for the treatment of MYOA7-related Usher syndrome (USH1B)

Session Title: Session 2 – Pre-clinical Gene Therapy

Date/Time: May 3, 2024 from 9:35 AM to 9.50 AM PDT

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

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