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ReiThera delivers Drug Substance & Drug Product in AAVantgarde's Groundbreaking Large-Gene Therapy Trial for Usher Syndrome Type 1B

- **AAVantgarde Bio (AAVantgarde) launched the LUCE-1 trial to test AAVB-081 gene therapy for retinitis pigmentosa related to Usher Syndrome Type 1B. This is a First-in-Human trial to deliver a large gene (MYO7A) to patients suffering from retinitis pigmentosa related to Usher syndrome type 1B.**
- **ReiThera was responsible for the manufacture and release of the Drug Substance & Drug Product.**
- **The collaboration between ReiThera and AAVantgarde has been key in advancing the dual-AAV technology for this groundbreaking gene therapy that involves the delivery of a large gene.**

Rome, October 29 2024 - **ReiThera** has recently completed all manufacturing and quality operations leading to the release of the gene therapy product named AAVB-081, originally developed at TIGEM by the research team of Prof. Alberto Auricchio and now into clinical development by AAVantgarde. This therapy uses the company's proprietary dual hybrid gene therapy platform to deliver the MYO7A protein in subjects with retinitis pigmentosa related to Usher Syndrome Type 1B (USH1B). LUCE-1 is a Phase 1/2 multicenter, open-label, dose-escalation study evaluating the safety, tolerability, and preliminary efficacy of up to three dose levels of AAVB-081 (a dual AAV vector) administered via subretinal injection in subjects with retinitis pigmentosa linked to Usher Syndrome Type 1B. In this context, ReiThera has manufactured and released the Drug Substance and the Drug Product to enable this gene therapy product to enter clinical trials. The clinical development is co-sponsored by AAVantgarde and Telethon Foundation. AAVantgarde, a spin-off of Telethon Institute for Genetics and Medicine (TIGEM) lead by Natalia Misciattelli as CEO, has recently announced that the first subject has been dosed in the LUCE-1 trial.

"We are proud to have played a crucial role in advancing this groundbreaking gene therapy trial by ensuring the successful and compliant GMP manufacture of the Drug Substance and the Drug Product. ReiThera's expertise in producing and controlling the quality of complex gene therapy products has been instrumental in bringing this innovative treatment closer to patients affected by Usher Syndrome Type 1B. Collaborating with AAVantgarde Bio and other leading institutions to push the boundaries of medical science aligns with our mission to drive advancements in biotechnology for the benefit of those in need." Said Stefano Colloca, Chief Executive Officer at Reithera.

'We are very happy and proud to have dosed our first subject in our LUCE-1 trial and would like to thank our partner Reithera for their support and expertise as our CDMO partner", said Dr. Nina Kotsopoulou, CTO of AAVantgarde.

About Usher syndrome type 1B
Usher syndrome type 1B (Usher1B) is a genetic disorder affecting both the retina and inner ear, caused by mutations in the MYO7A gene. The therapeutic gene needed to treat Usher1B is 6.7 kb in length, making it too large to fit into a conventional AAV vector. Around 20,000 individuals in the U.S. and E.U. are affected by Usher1B. Children with this condition are born deaf, experience vestibular dysfunction, and begin to lose their vision progressively during their first decade of life. While surgical options exist to address deafness, there are currently no treatments available for the progressive vision loss and eventual blindness associated with the disease.

About Reithera
ReiThera Srl is a CDMO company dedicated to technology and process development and GMP manufacturing, providing support for the clinical translation of genetic vaccines and medicinal products for advanced therapies.

The company has extensive expertise in developing scalable processes for viral-vector manufacturing and a consolidated experience in GMP production of Adeno-Associated Vector (AAV), Lentivirus, Adeno Viral vector (AdV), Modified Vaccinia Ankara and Herpes Simplex Vector.

ReiThera's core manufacturing capacity is based in a state-of-the-art facility, which includes stirred-tank bioreactors at scales of 50L, 200L, 1000L, and 2000L, as well as fixed-bed bioreactors for cell growth in adherence. The GMP facility also comprises a filling suite and quality control laboratories.

ReiThera's headquarters, R&D laboratories, and GMP facilities are located in Rome, Italy. For more information, visit www.reithera.com

About AAVantgarde
AAVantgarde is a clinical stage, 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. AAVantgarde is initially validating its platforms in two inherited retinal diseases with clear unmet need, with its lead program in Usher syndrome type 1B already in the clinic. For more information, please visit: www.aavantgarde.com