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AAVantgarde Completes Enrollment in LUCE-1 Phase 1/2 Clinical Trial of AAVB-081 for Usher Syndrome Type B-associated Retinitis Pigmentosa

This milestone marks significant progress in the first-in-human evaluation of AAVantgarde's dual-AAV gene therapy designed to address the underlying cause of vision loss in patients with USH1B

LONDON and MILAN, Jan. 15, 2026 (GLOBE NEWSWIRE) -- AAVantgarde Bio (AAVantgarde), a clinical-stage biotechnology company pioneering therapies for inherited retinal diseases (IRDs), has announced the completion of enrollment in LUCE-1, their Phase 1/2 first-in-human clinical trial evaluating AAVB-081 for the treatment of retinitis pigmentosa associated with Usher syndrome type 1B (USH1B).

LUCE-1 is a multicenter, open-label, dose-escalation and expansion study evaluating the safety, tolerability, and preliminary efficacy of a single subretinal administration of AAVB-081, AAVantgarde's dual-AAV gene therapy program, targeting mutations in the MYO7A gene, and addressing the root cause of USH1B. The study enrolled 15 adult participants between 18 to 60 years of age.

"Completing enrollment in the LUCE-1 study represents an important milestone in the clinical development of AAVB-081," **said Dr. Natalia Misciattelli, CEO of AAVantgarde**. "We are grateful to the patients, investigators and clinical site staff whose commitment has enabled the rapid advancement of this program toward generating meaningful clinical data for individuals living with Usher syndrome type 1B."

“Dosing the final patient in the LUCE Phase 1/2 study marks a significant milestone for this programme and for the patients and families affected by this devastating inherited retinal condition. Early-phase clinical research is where scientific innovation begins to translate into real-world impact, and LUCE represents an important step toward the development of a potential disease-modifying therapy. We are grateful to the sites, patients and families who have participated and now look forward to the data that will guide the next stage of development.”

stated Prof. Michaelides, from Moorfields Eye Hospital

AAVantgarde is making rapid progress on both of its clinical programs, and is recruiting in the CELESTE study for Stargardt disease. Usher 1B and Stargardt Disease are both debilitating inherited retinal conditions with no approved treatments currently available. Further updates for both programs to come in 2026.

About the AAVB-081 program
AAVB-081 is an intra-retinal AAV8-based dual hybrid product targeting MYO7A-associated Usher syndrome (USH1B). AAVantgarde’s dual hybrid platform uses two AAV8 vectors, each containing one half of an expression cassette encoding for the Myo7A gene and works at the cell nucleus level, recombining the two halves of the transgene back into a single one within the cell. This technology translates into an efficient recombination that generates therapeutically meaningful protein levels in animal models.

About the LUCE-1 Trial
LUCE-1 is a Phase 1/2 multicenter, open-label, dose escalation study investigating safety, tolerability and preliminary efficacy of 3 dose levels of dual AAV8.MYO7A (AAVB-081) administered subretinally in subjects with retinitis pigmentosa associated with Usher Syndrome Type 1B.

About Usher syndrome type 1B
Usher syndrome type 1B (Usher1B) is an inherited disease that affects the retina and the inner ear. Usher1B is caused by mutations in the *MYO7A* gene. The therapeutic gene to treat Usher1B is 6.7 kb long and is therefore too large to fit inside a standard AAV vector. Approximately 20,000 patients in the U.S. and E.U. have Usher1B. These children are born deaf, have vestibular dysfunction, and begin to progressively lose vision in their first decade of life. Although there are surgical treatments available to treat deafness in these patients, there are no treatments available to treat progressive vision loss and blindness in these patients.

About the AAVB-039 program
AAVB-039 is an intra-retinal AAV8-based dual AAV intein product targeting ABCA4-associated Stargardt disease. AAVantgarde’s dual AAV intein platform uses two AAV8 vectors, each containing one half of an expression cassette encoding for the ABCA4 gene and works at the cell protein level, splicing the two protein halves expressed back into a fully functional full-length protein. This technology translates into a very efficient splicing that generates therapeutically meaningful protein levels in animal models.

About the CELESTE Trial
CELESTE is a Phase 1/2/3 multicenter, open-label, dose escalation study investigating safety, tolerability and preliminary efficacy of 3 dose levels of dual AAV8.ABCA4 (AAVB-039) administered subretinally in subjects with Stargardt disease. For more information on the trial, visit ClinicalTrials.gov [NCT07161544]

About Stargardt disease
Stargardt disease is the most common form of inherited macular degeneration. Inherited in most cases, as autosomal recessive, Stargardt disease is caused by mutations in the ABCA4 gene, leading to the accumulation of toxic retinoid byproducts in the retina and progressive vision loss. The ABCA4 gene is 6.8 kilobases in length, too large to be packaged within a standard, single AAV vector. Stargardt disease affects an estimated 60,000 to 75,000 individuals across the U.S. and E.U and currently there are no approved treatments.

About AAVantgarde
AAVantgarde is a clinical stage, biotechnology company advancing best-in-class therapies for patients with inherited retinal diseases. The company’s lead programs target Stargardt disease and retinitis pigmentosa due to Usher syndrome type 1B, two severe, inherited retinal diseases with no approved treatments. With a strong foundation in translational science and a commitment to

clinical excellence, AAVantgarde is working to bring transformative therapies to patients. For more information, please visit: www.aavantgarde.com.

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