AAVantgarde Bio Announces FDA Fast Track Designation for AAVB-039 for the Treatment of Stargardt Disease

MILAN, August 12, 2025 — AAVantgarde Bio (AAVantgarde), a clinical-stage biotechnology company developing next-generation gene therapies for inherited retinal diseases, today announced that the U.S. Food and Drug Administration (FDA) has granted Fast Track Designation for AAVB-039, the company's gene therapy program for Stargardt disease secondary to biallelic mutation in ABCA4. The Investigational New Drug (IND) application for AAVB-039 was cleared to proceed by the FDA. Stargardt's disease is the most common inherited form of macular degeneration and a leading cause of vision loss in children and young adults. AAVB-039 addresses the underlying genetic cause of the disease by providing the full-length ABCA4 protein and has the potential to benefit all patients with ABCA4 mutations.

"We are thrilled that the FDA has granted Fast Track Designation for AAVB-039, as it underscores the urgent unmet need and important potential of our program due to the serious nature of challenges faced by patients living with Stargardt disease," said Dr. Natalia Misciattelli, Chief Executive Officer of AAVantgarde. "This designation represents an important milestone for our program and enables us to accelerate development efforts as we work to bring a potentially transformative therapy to patients as quickly as possible."

The FDA's Fast Track program is designed to facilitate the development and expedite the review of drugs and biologics intended to treat serious or life-threatening conditions and that demonstrate the potential to address unmet medical needs. AAVB-039 is currently being evaluated in the Phase 1/2 CELESTE clinical trial, which is assessing safety, tolerability, and preliminary efficacy in patients with Stargardt disease.

About AAVantgarde Bio

AAVantgarde Bio is a clinical stage, biotechnology company advancing best-in-class therapies for patients with inherited retinal diseases (IRDs). The company's lead programs target Stargardt disease and retinitis pigmentosa due to Usher syndrome type 1B, two severe, IRDs with no approved treatments. AAVB-039 and AAVB-081 are investigational, dual AAV gene therapies designed to address the root genetic causes of these diseases. 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

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.

Contact:

Magda Blanco – Head of Corporate Development

Email: info@aavantgarde.com