



AAVantgarde Closes €61 Million Series A Financing to Advance Two Therapeutic Programs into the Clinic

- Financing led by Atlas Venture and Forbion

- Company created, incubated and seed financed by Sofinnova Partners

MILAN — June 6, 2023 — 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 closing of a €61 million Series A financing. The financing was co-led by US biotechnology venture creation firm Atlas Venture and European life sciences venture capital investment firm Forbion, with participation from Longwood Fund and the Company's founding investor Sofinnova Partners through its Sofinnova Telethon Fund. In connection with the Series A financing Jason Rhodes from Atlas Venture, Dmitriy Hristodorov from Forbion, and David Steinberg from Longwood Fund will join the AAVantgarde Board.

AAVantgarde has two proprietary, AAV-based large gene delivery platforms: one leveraging DNA recombination, named dual hybrid; and one protein trans-splicing, named AAV intein. The company is validating the platforms in two lead programs: Usher Syndrome Type 1 B associated retinitis pigmentosa (Usher1B), using dual hybrid; and Stargardt disease, using AAV intein. This financing will fund completion of a first-in-man proof of concept (POC) study in subjects with Usher1B and further development of AAV intein program and entry into the clinic for Stargardt disease. The company also intends to pursue programs beyond ophthalmology.

"This financing represents a hallmark moment in our mission to help patients and we are excited to work with both our new and founding investors to advance our platforms and pipeline," **said Dr. Natalia Misciattelli, Chief Executive Officer of AAVantgarde.** "The completion of this round with such a high-caliber group of global life science investors is a recognition of the significance of AAVantgarde's unique technology, which will initially be utilized to help patients suffering with the debilitating impact of blindness associated with Usher1B syndrome and Stargardt disease."



“AAV gene therapy has been limited by transgene capacity, and AAVantgarde’s proprietary platforms enable delivery of large genes to tissue and cells in vivo; something that could extend into many disease areas,” **said Jason Rhodes, partner at Atlas Venture.**

“AAVantgarde has a clear mission to bring next generation genetic medicines to underserved patients with debilitating diseases, bringing hope to inherited retinal disease patients with Usher1B syndrome and Stargardt disease to improve vision,” **said Dmitrij Hristodorov of Forbion.**

“We are excited by the opportunity to partner with, and support, such a dedicated team with a proven track record of success.”

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.aavantgardebio.com

About Atlas Venture

Atlas Venture is a leading biotech venture capital firm. With the goal of doing well by doing good, we have been building breakthrough biotech startups for over 25 years. We work side by side with exceptional scientists and entrepreneurs to translate high impact science into medicines for patients. Our seed-led venture creation strategy rigorously selects and focuses investment on the most compelling opportunities to build scalable businesses and realize value. For more information, please visit www.atlasventure.com

About Forbion

Forbion is a dedicated life sciences venture capital firm with offices in The Netherlands, Germany and Singapore. Forbion invests in life sciences companies that are active in the (bio-) pharmaceutical space. Forbion manages €3 billion across multiple fund strategies that cover all stages of (bio-) pharmaceutical drug development. Forbion’s current team consists of over 30 life sciences investment professionals that have built an impressive performance track record since the late nineties with investments in 95 companies across 8 funds. Forbion’s record of sourcing, building and guiding life sciences companies has resulted in many breakthrough therapies and valuable exits. Portfolio company successes include NewAmsterdam Pharma, Gyroscope



(acquired by Novartis) and Replimune. Besides financial objectives, Forbion selects investments that will positively affect the health and well-being of patients. The firm is a signatory to the United Nations Principles for Responsible Investment. Forbion operates a joint venture with BGV, the manager of seed and early-stage funds, especially focused on Benelux and Germany. For more information, please visit: www.forbion.com

About Sofinnova Partners

Sofinnova Partners is a leading European venture capital firm in life sciences, specializing in healthcare and sustainability. Based in Paris, London and Milan, the firm brings together a team of professionals from all over the world with strong scientific, medical and business expertise. Sofinnova Partners is a hands-on company builder across the entire value chain of life sciences investments, from seed to later-stage. The firm actively partners with ambitious entrepreneurs as a lead or cornerstone investor to develop transformative innovations that have the potential to positively impact our collective future.

Founded in 1972, Sofinnova Partners is a deeply established venture capital firm in Europe, with 50 years of experience backing over 500 companies and creating market leaders around the globe. Today, Sofinnova Partners has over €2.5 billion under management. For more information, please visit: www.sofinnovapartners.com

About Longwood Fund

Longwood Fund is a venture capital firm dedicated to creating and investing in novel healthcare companies that develop important treatments to help patients while generating significant value for investors. The Longwood team has a long history of successfully launching and building important life science companies while providing operational leadership and strategic guidance. Collectively, the Partners at Longwood Fund have co-founded 24 companies with over 20 launched or marketed drugs and therapies, as well as over two dozen clinical stage assets, all focused on helping patients in need. Companies founded by Longwood Fund, or its principals prior to the founding of the Firm, as lead investor and CEO/CBO include Acceleron, Momenta, Alnylam, Vertex, Sirtris, Vor, TScan, Pyxis Oncology, Immunitas, Be Biopharma, Tome Bio, Photys, Carbon Biosciences, and DEM BioPharma. For more information, please visit www.longwoodfund.com.

About Fondazione Telethon and TIGEM

Fondazione Telethon (the Telethon Foundation) is a major biomedical charity in Italy whose mission is to advance biomedical research to cure rare genetic diseases. Throughout its 32 years of activity, Fondazione Telethon has invested almost €600 million in funding over 2,700 projects to study 580 diseases, involving more than 1,600 researchers in Italy. As part of its goal to foster



development and maximize the therapeutic impact of research, Fondazione Telethon further pursues cooperation with academia, industry, and venture capital through the creation of partnerships and start-ups. TIGEM is a Telethon Foundation research centre in Pozzuoli, near Naples, and comprises several research groups and over 200 staff members, all dedicated to understanding the molecular mechanisms behind rare genetic diseases and developing novel treatments. For more information, please visit www.telethon.it/en

About Usher syndrome

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 Stargardt Disease

Stargardt disease is the most common inherited macular degeneration. Inherited in most of the cases as autosomal recessive, Stargardt disease is caused by mutations in the *ABCA4* gene. The therapeutic gene to treat Stargardt disease (*ABCA4*) is 6.8 kb long which is too large to fit inside a standard AAV vector. Stargardt disease affects approximately 60,000-75,000 patients in the U.S. and E.U. Currently there are no treatments for the blindness caused by Stargardt disease.

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