SpliceBio Announces First Patient Dosed in Phase 1/2 ASTRA Study of SB-007, a Dual-AAV Gene Therapy for Stargardt Disease

- *SB-007 addresses the root cause of Stargardt disease with the potential to treat all patients across all ABCA4 mutations*
- *SB-007 is the first dual AAV gene therapy in clinical development for Stargardt disease*

BARCELONA, SPAIN, 13 March 2025 – SpliceBio, a clinical-stage genetic medicines company pioneering Protein Splicing to address diseases caused by mutations in large genes, today announced that it has dosed the first patient in the Phase 1/2 ASTRA study of SB-007, a dual adeno-associated viral (AAV) vector, for the treatment of Stargardt disease. Stargardt disease is an inherited retinal disorder caused by mutations in the ABCA4 gene. It is the most common form of juvenile macular degeneration, leading to progressive central vision loss and ultimately resulting in blindness, with no approved treatments available.

"Stargardt disease has remained elusive to gene therapies due to the large size of the ABCA4 gene. SB-007 is the first gene therapy in clinical development designed to restore expression of the full-length ABCA4 protein across all Stargardt disease patients, regardless of their mutations," **said Miquel Vila-Perelló, Ph.D., Chief Executive Officer and Co-Founder of SpliceBio**. "Treating the first patient in the ASTRA study with SB-007 is an important milestone for SpliceBio and the Stargardt disease patient community, who are in desperate need for a therapy. I am immensely proud of our outstanding team at SpliceBio whose depth of experience in leading numerous ophthalmology gene therapy trials in the last 15 years is unparalleled."

Paul Yang, M.D., Ph.D., Chief of the Paul H. Casey Ophthalmic Genetics Division at Oregon Health & Science University Casey Eye Institute and Principal Investigator of Phase 1/2 ASTRA Study, commented: "Stargardt disease is a devastating inherited retinal disorder with no approved treatments available. The initiation of this study with the dual AAV vector gene therapy, SB-007, represents a critical advancement in finding a potential treatment option for patients with this disease. It is a privilege to be able to collaborate with SpliceBio on this groundbreaking clinical study, and I look forward to advancing research that could bring hope to patients affected by Stargardt disease."

Aniz Girach, MD., Chief Medical Officer of SpliceBio, remarked: "We are excited to have treated the first patient in the US with our lead gene therapy. A single dose of SB-007 has the potential to address the root cause of Stargardt disease across all ABCA4 mutations. The Phase 1/2 ASTRA study will assess the safety and efficacy of SB-007 in patients with Stargardt disease. We look forward to continuing enrolment in our Phase 1/2 ASTRA study, alongside our foundational POLARIS trial, with the ultimate purpose of providing an effective and safe therapy for patients. As the study progresses, we aim to present data at upcoming scientific conferences."

Alongside the ASTRA study, SpliceBio continues to actively enrol patients through its POLARIS trial, a natural history study monitoring disease progression and endpoints in Stargardt disease patients. For more information or to enquire about participation in the studies, please visit https://splice.bio/clinical/.

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About SpliceBio

SpliceBio is a clinical-stage genetic medicines company pioneering Protein Splicing to address diseases caused by mutations in large genes. The Company's lead program, SB-007, targets the root cause of Stargardt disease, a genetic eye disease that causes blindness in children and adults. SpliceBio's pipeline comprises additional gene therapy programs across therapeutic areas, including ophthalmology and neurology. SpliceBio's platform is based on technology developed in the Muir Lab at Princeton University after more than 20 years of pioneering intein, Protein Splicing, and protein engineering research. For additional information, please visit www.splice.bio.

About SB-007

SB-007 is an investigational Protein Splicing dual AAV gene therapy in development for the treatment of Stargardt disease. It is designed to restore expression of the native full-length ABCA4 protein in the retina. SB-007 has been granted Orphan Drug Designation from both the FDA in the US and the European Commission in Europe. In December 2024, SB-007 received FDA IND clearance, marking the first-ever clearance for a dual AAV gene therapy in Stargardt disease. Alongside initiation of the Phase 1/2 ASTRA study, SpliceBio continues to advance POLARIS, a natural history study of the disease. Both studies are actively recruiting. For more information or to enquire about participation in the studies, please visit https://splice.bio/clinical/.

About Stargardt disease

Stargardt disease is the most common form of inherited juvenile macular degeneration, affecting approximately 1 in 8,000 to 10,000 individuals. Caused by mutations in the ABCA4 gene, Stargardt disease leads to the progressive loss of central vision due to damage to the central region of the retina known as the macula. The disease is variable in the age of onset, including early onset in children and adolescents and late-onset forms in adulthood. There are no approved treatments, and patients face significant challenges in daily life as the disease progresses. Stargardt disease has remained elusive to genetic medicines due to the large size of the ABCA4 gene.