SpliceBio Initiates Dose-Expansion Portion of Phase 1/2 ASTRA Clinical Trial for SB-007, a Dual-AAV Gene Therapy for Stargardt Disease

First dose-expansion patient successfully received SB-007

SB-007 addresses the root cause of Stargardt disease with the potential to treat patients across all ABCA4 mutations

SB-007 granted FDA Fast Track designation, following previously received Orphan Drug designation in US and Europe

BARCELONA, January 8, 2026 – SpliceBio, a clinical-stage genetic medicines company pioneering protein splicing to address diseases caused by mutations in large genes, today announced that the first patient has been dosed in the Part B dose-expansion portion of the Phase 1/2 ASTRA clinical trial of SB-007, a dual adeno-associated viral (AAV) vector gene therapy for the treatment of Stargardt disease.

Part B will evaluate two dose levels of SB-007 versus an untreated control in patients with the rare inherited retinal disease. Stargardt disease, which has no approved treatments, is caused by biallelic mutations in the ABCA4 gene leading to a progressive deterioration of central vision and ultimately blindness. SB-007 is designed to address the underlying genetic cause by restoring expression of a functional, full-length ABCA4 protein in the retina, with the potential to treat patients across all ABCA4 mutations.

“Gene therapy has transformative promise in ophthalmology, but its application has been limited by the inability of single-AAV vectors to accommodate large, complex genes such as ABCA4. SB-007 is a dual-AAV vector that can harness our protein splicing platform and is designed to reconstitute the full-length therapeutic ABCA4 protein,” said Aniz Girach, M.D., Chief Medical Officer of SpliceBio. “Starting the dose-expansion phase of the ASTRA trial is a key milestone for our program, and we look forward to continuing this progress with the support of the trial’s participants and investigators.”

“The use of two viral vectors that recombine once inside retinal cells is a unique approach to restoring the large gene needed in Stargardt disease, and dual vectors might have implications for treating other retinal degenerations,” commented Robert MacLaren, M.D., Ph.D., Professor of Ophthalmology, University of Oxford and National Health Service Gene and Cell Therapy Research Lead, Oxford University Hospitals NHS Foundation Trust. “This unique gene therapy modality has the potential to slow or even halt progression of this debilitating disease, which is the most common cause of inherited blindness in children. We are delighted to have treated the first patient here in Oxford, in the critical second phase of the trial.”

SpliceBio’s proprietary platform technology leverages engineered inteins to enable protein trans-splicing. This allows the ABCA4 gene to be split into two transgenes that are delivered using dual AAV vectors. Once inside the target cells in the retina, the transgenes are expressed and undergo protein trans-splicing to reconstitute the full-length native ABCA4 protein.

“The ASTRA study is a critical step toward expanding our understanding of Stargardt disease and evaluating the potential of SB-007,” added Mariya Moosajee, M.B.B.S., Ph.D., Professor of Molecular Ophthalmology, University College London Institute of Ophthalmology, and Consultant

Ophthalmologist and Head of the Genetics Service, Moorfields Eye Hospital in London. “The clinical insights generated through this study have the potential to advance medicine and, critically, bring new hope to patients and families living with this condition. We are proud to have included the first participant in this groundbreaking trial.”

ASTRA is a multicenter, global clinical trial designed to evaluate the safety, tolerability and efficacy of SB-007. SpliceBio expects to enroll approximately 57 patients aged 12 to 65 with Stargardt disease into Part B. Part A of the study evaluated three dose levels of subretinal SB-007 in an open-label, dose-escalation design. Part B is randomized, controlled and masked, and will evaluate two dose levels of subretinal SB-007 compared to an untreated control group, with a follow-up of 96 weeks. The primary endpoint is safety and tolerability, assessed by the incidence and severity of ocular and non-ocular adverse events. Secondary endpoints comprise multiple efficacy measures. For more information on the trial, visit ClinicalTrials.gov [NCT06942572].

The U.S. Food and Drug Administration (FDA) recently granted Fast Track designation to SB-007, a process designed to facilitate the development and expedite the review of drugs that treat serious conditions and fill an unmet medical need. With this designation, SpliceBio is eligible for more frequent communication with the FDA, as well as the potential for expedited regulatory review. SpliceBio is also running the POLARIS study, a natural history study in patients with Stargardt disease.

Dr. MacLaren’s work to develop gene therapies for retinal conditions is supported by the National Institute for Health and Care Research (NIHR) Oxford Biomedical Research Centre.

About Stargardt Disease
Stargardt disease is a rare inherited retinal disease (IRD) that causes progressive vision loss and blindness. It affects an estimated 1 in 8,000 to 10,000 children and adults worldwide. The disease is caused by mutations in the ABCA4 gene, which result in the accumulation of toxic vitamin A byproducts that damage photoreceptor cells in the central region of the retina, known as the macula. Central vision loss is a hallmark of Stargardt disease, and the age of onset is variable, including early-onset forms in children and adolescents as well as late-onset forms in older adults. The large size of the ABCA4 gene exceeds the packaging capacity of standard single AAV vectors and has historically limited traditional gene therapy approaches. There are currently no approved treatments.

About SB-007
SB-007 is an investigational dual adeno-associated virus (AAV) gene therapy for Stargardt disease. It is designed to restore expression of a functional, full-length ABCA4 protein in the retina, with the potential to treat patients with all ABCA4 mutations. SpliceBio’s proprietary protein splicing intein platform uses two AAV serotype 8 (AAV8) vectors to overcome the size limitations of conventional AAVs, reconstituting biologically active ABCA4 through protein trans-splicing in target photoreceptor cells. In preclinical models, SB-007 demonstrated robust pharmacological activity, durable retinal expression and a favorable safety profile. SpliceBio has received Orphan Drug designation for SB-007 from the FDA and the European Commission.

About SpliceBio
SpliceBio is a clinical-stage genetic medicines company pioneering protein splicing to address diseases caused by mutations in large genes. Our lead program, SB-007, is a gene therapy designed to target the root cause of Stargardt disease, an inherited retinal disorder that causes progressive vision loss and blindness. SpliceBio is currently enrolling participants in ASTRA, a Phase 1/2 clinical trial of SB-007, and POLARIS, a natural history study in patients with the disease. The SpliceBio platform combines advanced intein, protein splicing and protein

engineering technologies, and supports a pipeline of gene therapy programs in ophthalmology and neurology. For additional information, visit www.splice.bio and follow us on LinkedIn and X.

Media Contact:

Amanda Lazaro, 1AB
amanda@1abmedia.com