SpliceBio Announces U.S. FDA IND Clearance of SB-007 to Commence Phase 1/2 Clinical Study in Patients with Stargardt Disease

- *SB-007 is the only IND-cleared, clinical-stage therapeutic addressing the root cause of Stargardt disease with the potential to treat all patients across all ABCA4 mutations*
- *Phase 1/2 ASTRA study set to begin in the first half of 2025, supported by POLARIS, a pioneering company-sponsored natural history study in Stargardt disease*
- *First ever IND clearance for a Protein Splicing gene therapy*

BARCELONA, SPAIN, 12 December 2024 – SpliceBio, a genetic medicines company pioneering Protein Splicing to address diseases caused by mutations in large genes, today announced that the U.S. Food & Drug Administration (FDA) has cleared its investigational new drug (IND) application for lead program SB-007. SB-007 is the only clinical-stage therapeutic addressing the root genetic cause of Stargardt disease with the potential to treat all patients across all ABCA4 mutations.

Miquel Vila-Perelló, Ph.D., Chief Executive Officer, and Co-Founder of SpliceBio, said: "The FDA IND clearance of SB-007 is a significant achievement for SpliceBio and Stargardt disease patients. As the first-ever IND for a Protein Splicing gene therapy, it is a huge step forward to demonstrate the potential of this new therapeutic modality to address diseases caused by mutations in large genes such as *ABCA4*. SB-007 is an adeno-associated viral (AAV) vector gene therapy aimed at restoring expression of the full-length *ABCA4* protein, and the only clinical-stage therapy with the potential to help all Stargardt patients. We look forward to accelerating the clinical development of SB-007, building on the Orphan Drug Designation granted by the FDA in 2024, and advancing this potentially life-changing therapeutic for patients with Stargardt disease."

"Stargardt disease has been a challenge for the development of gene therapies due to the large size of the *ABCA4* gene, and currently has no approved therapies available," **said Professor Paul Yang, M.D., Ph.D., Chief of the Paul H. Casey Ophthalmic Genetics Division at Casey Eye Institute at Oregon Health & Science University**. "This new therapy utilizes a unique approach to replace the full-size, normal *ABCA4* protein at high efficiency, which addresses the root cause of Stargardt disease across any pathogenic variant in the *ABCA4* gene. This IND clearance represents a major milestone in the field and I am thrilled to be part of the clinical studies exploring this promising approach that could transform the lives of Stargardt disease patients."

SpliceBio plans to initiate enrolment in the Phase 1/2 ASTRA study in the first half of 2025. ASTRA will evaluate the safety and efficacy of a single dose of SB-007 administered subretinally in patients with Stargardt disease. In March 2024, SpliceBio launched the POLARIS trial, a pioneering company-sponsored natural history study of Stargardt disease designed to evaluate disease progression, refine endpoints, and streamline eligibility criteria

for accelerated enrolment into the Phase 1/2 ASTRA study. This study will enable Stargardt disease patients to benefit from more precise diagnoses, more rigorous disease monitoring, and potentially faster access to innovative therapies.

The SpliceBio Management team will be attending J.P. Morgan's 43rd Annual Healthcare Conference 2025, being held in San Francisco from 13-16 January 2025. Please get in contact to schedule a meeting at bd@splice.bio.

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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.

About SB-007
SB-007 is a Protein Splicing dual AAV gene therapy that delivers the full-length ABCA4 gene and aims to restore expression of the native ABCA4 protein in the retina. It has demonstrated robust pharmacological activity in animal models of Stargardt disease and durable expression and safety in non-human primates. SB-007 has been granted Orphan Drug Designation by the FDA in the US and the European Commission in Europe.

About SpliceBio
SpliceBio is a 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.