

SpliceBio Secures \$135 Million Series B Financing to Advance Lead Program SB-007 in Stargardt Disease and Expand Pipeline of Genetic Medicines

- *Financing co-led by new investors EQT Life Sciences and Sanofi Ventures, with participation from Roche Venture Fund, as well as all existing investors*
- *Proceeds will support clinical development of lead program SB-007 in Stargardt disease*
- *Funding will also advance a broader pipeline of genetic medicines targeting indications in ophthalmology, neurology, and other undisclosed therapeutic areas*

BARCELONA, SPAIN, 11 June 2025 – SpliceBio, a clinical-stage genetic medicines company pioneering Protein Splicing to address diseases caused by mutations in large genes, today announced the close of a \$135 million Series B financing co-led by new investors [EQT Life Sciences](#) and [Sanofi Ventures](#), with participation from [Roche Venture Fund](#), as well as all existing investors: New Enterprise Associates, UCB Ventures, Ysios Capital, Gilde Healthcare, Novartis Venture Fund, and Asabys Partners.

The funding will be used to advance the clinical development of SpliceBio's lead gene therapy candidate, SB-007 for Stargardt disease, including the ongoing interventional Phase 1/2 ASTRA study and the observational POLARIS study. SB-007 is the first dual adeno-associated viral (AAV) gene therapy cleared by the Food and Drug Administration (FDA) to enter clinical development for Stargardt disease. SB-007 has also received regulatory clearance for clinical development from the UK Medicines and Healthcare products Regulatory Agency (MHRA).

Stargardt disease is an inherited retinal disorder caused by mutations in the ABCA4 gene that leads to progressive vision loss and blindness, with no approved treatments available. SB-007 is designed to address the underlying genetic cause of the disease by producing a functional copy of the full-length ABCA4 protein with the potential to treat all patients, regardless of their specific ABCA4 mutation. The proceeds will also be used to accelerate SpliceBio's pipeline of AAV gene therapy programs in ophthalmology, neurology, and other undisclosed indications that utilise the company's proprietary Protein Splicing platform.

"This financing marks a pivotal milestone for SpliceBio as we advance the clinical development of SB-007 for Stargardt disease and continue to expand our pipeline across ophthalmology, neurology and beyond," **said Miquel Vila-Perelló, Ph.D., Chief Executive Officer and Co-Founder of SpliceBio.** "The support from such high-quality investors underscores the strength of our programs and our unique Protein Splicing platform and its potential to unlock gene therapies for diseases that remain untreatable today. We are building a company positioned to lead the next wave of genetic medicines."

SpliceBio is redefining and expanding the scope of diseases that can be tackled with gene therapies by addressing a fundamental limitation of AAV vectors in their inability to deliver genes that exceed their limited packaging capacity of 4.7 kilobases. Many genetic disorders



remain untreatable because the necessary gene is too large to fit into the AAV vectors. SpliceBio's unique Protein Splicing platform leverages the use of a family of proprietary, engineered proteins called inteins, originally developed at Princeton University. The company's technology enables the splitting of the gene into two (or more) transgenes that are then delivered using dual AAV vectors. Once inside the cell, the DNA of each transgene is transcribed into messenger RNA and translated into protein. SpliceBio's engineered inteins are designed to then assemble the full-length protein that is needed to treat the disease.

Daniela Begolo, Managing Director at EQT Life Sciences, commented: "We are proud to support SpliceBio, a pioneer among the next-generation of genetic medicine companies. Its Protein Splicing platform is designed to offer a novel solution to deliver large genes with AAV, one of the field's most pressing challenges, and exemplifies our commitment to backing transformational science that can meaningfully benefit patients' lives."

Laia Crespo, Partner at Sanofi Ventures, remarked: "With compelling data for its lead program, SB-007, and a highly differentiated platform, we are excited to support SpliceBio as it tackles a fundamental challenge for genetic medicines. By enabling the delivery of large and complex genes through its novel AAV vector Protein Splicing technology, SpliceBio has the potential to make a significant impact on the field of gene therapy and to deliver best-in-class therapies to patients."

Carole Nuechterlein, Head of Roche Venture Fund, added: "We are impressed by the team's strong execution, the momentum behind SB-007 in Stargardt disease, and the platform's potential to unlock a new class of genetic medicines. We are proud to support SpliceBio at this pivotal stage of growth as they advance their lead program through clinical development and explore additional high-impact indications."

In connection with the financing, Daniela Begolo, Managing Director at EQT Life Sciences, Laia Crespo, Partner at Sanofi Ventures, and Carole Nuechterlein, Head of Roche Venture Fund, will join the SpliceBio Board of Directors.

-Ends-

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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, with the announcement of the first patient dosed in March 2025, 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 EQT Life Sciences

EQT Life Sciences was formed in 2022 following an integration of LSP, a leading European life sciences and healthcare venture capital firm, into the EQT platform. As LSP, the firm raised over EUR 3.0 billion (USD 3.5 billion) and supported the growth of more than 150 companies since it started to invest over 30 years ago. With a dedicated team of highly experienced investment professionals, coming from backgrounds in medicine, science, business, and finance, EQT Life Sciences backs the smartest inventors who have ideas that could truly make a difference for patients. More info: www.eqtgroup.com. Follow EQT on LinkedIn, Twitter, YouTube and Instagram.

About Sanofi Ventures

Sanofi Ventures is the corporate venture capital arm of Sanofi, focused on investing in promising early-stage healthcare companies. The firm supports pioneering innovations in biotechnology, digital health, and life sciences aligning with Sanofi's mission to bring life-changing treatments to patients worldwide. For more information visit: www.sanofiventures.com

About Roche Venture Fund

The Roche Venture Fund is the corporate venture fund of Roche and invests in innovative life science companies. Over the past 20 years, the Roche Venture Fund has invested in over 60 companies globally and currently has a portfolio of around 30 companies located in 10 countries. As part of a multinational healthcare company, the Roche Venture Fund has access to considerable expertise both internally and externally and co-invests with leading venture funds, including other corporate venture funds, on a regular basis.

<https://www.roche.com/venturefund>