



## **SpliceBio Raises EUR 50M in Oversubscribed Series A financing to Advance Protein Splicing Platform and Expand Gene Therapy Pipeline**

- *Financing co-led by UCB Ventures and Ysios Capital with participation by New Enterprise Associates, Gilde Healthcare, Novartis Venture Fund and Asabys Partners*
- *Unique Protein Splicing platform enables efficient delivery of large genes with adeno-associated vectors (AAV)*
- *Proceeds will be used to advance the lead program in Stargardt disease into the clinic and expand pipeline to other currently untreatable genetic diseases*

**BARCELONA, February 16th, 2022** – SpliceBio, a biotechnology company exploiting protein splicing to develop next generation gene therapies, today announced the completion of an oversubscribed €50 million series A financing. The financing was co-led by UCB Ventures and existing shareholder Ysios Capital and joined by new investors New Enterprise Associates (NEA), Gilde Healthcare, Novartis Venture Fund, and existing shareholder Asabys Partners. The Company was seeded in 2020 by Ysios Capital and Asabys Partners.

Adeno-associated viruses (AAV) are the gene therapy vector of choice for the treatment of genetic diseases. However, their small packaging capacity is a major challenge for the development of novel gene therapies. SpliceBio's Protein Splicing platform aims to address this major limitation to enable the efficient delivery of large genes using AAV vectors. The platform is based on technology developed in the Muir Lab at Princeton University after more than 20 years of pioneering intein and protein engineering research. In this novel approach, engineered inteins catalyze highly efficient protein trans-splicing to reconstitute the desired full-length therapeutic protein in vivo.

The proceeds from the financing, the largest Series A round for a Spanish biotech company, will enable SpliceBio to build a pipeline of Protein Splicing gene therapy programs, while advancing the lead program in Stargardt disease to the clinic. Stargardt disease is the most common form of juvenile macular dystrophy affecting more than 80,000 people in US and EU. The disease is caused by a loss of function mutation in the ABCA4 gene, which at 6.8 kb is too large for single AAV vectors. The Company will focus its efforts on ophthalmology as well as other disease areas of significant unmet patient need. The platform has been validated in several other organs beyond the retina.

**Miquel Vila-Perelló, PhD, Co-Founder and Chief Executive Officer of SpliceBio, said:** "We are very pleased to attract this outstanding syndicate of institutional and corporate investors which validates our approach to developing next generation gene therapies. I am excited to lead an exceptional team as we continue to build our platform and advance our pipeline of gene therapy programs into the clinic."

Following the closing of the financing, the Board of SpliceBio chaired by Jean Philippe Combal will include: Erica Whittaker, UCB Ventures; Joël Jean-Mairet, Ysios Capital; Ed Mathers, NEA; Arthur Franken, Gilde Healthcare; Beat Steffen, Novartis Venture Fund; and Miquel Vila-Perelló, CEO.

**Erica Whittaker, Vice President and Head of UCB Ventures, stated:** "We are delighted to support SpliceBio in the development of its innovative platform to create treatments for patients suffering from genetic diseases not currently addressable by existing gene therapy approaches."

**Joël Jean-Mairet, Managing Partner at Ysios Capital, added:** "We are proud to have been involved with the Company since its early days and are very impressed with the progress



achieved to date. SpliceBio's platform represents an unprecedented opportunity to expand the universe of diseases that can be addressed with gene therapy. This financing is also a testament to the growing potential of the biotech hub in Barcelona."

**Ed Mathers, General Partner at NEA, commented:** "We are very pleased to back this team, building on the founders' early-stage research at Princeton's Muir Laboratory to develop SpliceBio into a world leading gene therapy player. We believe SpliceBio's innovative approach to maximizing the capacity of AAV vectors has the potential to make a meaningful impact in the delivery of much needed gene therapies, and we look forward to supporting the Company through its next stages of growth."

**ENDS**

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**Notes to Editors**

**About SpliceBio**

SpliceBio is a biotechnology company exploiting Protein Splicing to develop the next generation of gene therapies. The Company's proprietary platform enables efficient delivery of large genes with adeno-associated vectors (AAV), overcoming the most fundamental challenge in the quest to curing a broad range of genetic diseases. SpliceBio's platform is based on technology developed in the Muir Lab at Princeton University after more than 20 years of pioneering intein and protein engineering research. For additional information, please visit [www.splice.bio](http://www.splice.bio).

**About Split Inteins**

Inteins are auto-processing domains found in organisms from all domains of life. These proteins carry out a process known as protein splicing, which is a multi-step biochemical reaction comprised of both the cleavage and formation of peptide bonds. While the endogenous substrates of protein splicing are specific essential proteins found in intein-containing host organisms, inteins are also functional in exogenous contexts and can be used to chemically manipulate virtually any polypeptide backbone. After more than 20 years of pioneering intein research characterizing the structure-activity relationship of inteins and optimizing their properties, SpliceBio's co-founders have developed a new generation of engineered split inteins designed for therapeutic use. The Company has developed additional proprietary technologies that altogether conform its Protein Splicing platform.

**About Stargardt disease**

Stargardt disease is a genetic eye disorder that causes retinal degeneration and vision loss. Stargardt disease is the most common form of inherited macular degeneration, affecting 1 in 8,000 people in the world, including children. There are no treatments currently available for Stargardt patients.

**About UCB Ventures**

UCB Ventures is a strategic corporate venture fund established in 2017 to further strengthen UCB's ability to create value from novel insights and technologies that can transform the lives



of patients suffering from severe diseases. UCB Ventures invests in innovative therapeutics and technology platforms that are early stage and high risk, in areas adjacent to or even beyond UCB's therapeutic focus on neurology/neurodegenerative diseases, immunology and muscular skeletal/bone health. UCB Ventures takes an active role in its portfolio companies, contributing expertise in drug discovery, development, and operations. Visit [www.UCBVentures.com](http://www.UCBVentures.com) to learn more.

### **About Ysios Capital**

Ysios Capital is a leading Spanish venture capital firm that provides private equity financing to early- and mid-stage, highly innovative life science companies bringing life-changing treatments to patients, with a focus on indications with high unmet need. Our diverse international team in San Sebastián and Barcelona is driven by science, with the ambition to transform capital into medical breakthroughs. Ysios Capital was founded in 2008 and has over \$450 million in assets under management through its three funds. For more information, please visit [www.ysioscapital.com](http://www.ysioscapital.com).

### **About New Enterprise Associates**

New Enterprise Associates, Inc. (NEA) is a global venture capital firm focused on helping entrepreneurs build transformational businesses across multiple stages, sectors, and geographies. With nearly \$24 billion in cumulative committed capital since the firm's founding in 1977, NEA invests in technology and healthcare companies at all stages in a company's lifecycle, from seed stage through IPO. The firm's long track record of successful investing includes more than 230 portfolio company IPOs and more than 390 mergers and acquisitions. [www.nea.com](http://www.nea.com).

### **About Gilde Healthcare**

Gilde Healthcare is a specialized healthcare investor with two fund strategies: Venture&Growth and Private Equity. The firm operates out of offices in Utrecht (The Netherlands), Frankfurt (Germany) and Cambridge (United States). Gilde Healthcare Venture&Growth invests in fast growing, innovative companies active in (bio)pharmaceuticals, healthtech and medtech that are based in Europe and North America. For more information, please visit: [www.gildehealthcare.com](http://www.gildehealthcare.com).

### **About Novartis Venture Fund**

Novartis Venture Fund is a financially driven corporate life science venture fund whose purpose is to foster innovation, drive significant patient benefit and generate superior returns by creating and investing in innovative life science companies at various stages of their development. For more information, go to [www.nvfund.com](http://www.nvfund.com).

### **About Asabys Partners**

Asabys Partners is a venture capital manager firm specialized in the healthcare sector. With close to 120 million euros in AUM and 12 portfolio companies (including 1 exit), Asabys invests in healthcare companies that have highly innovative and disruptive technologies. The investment in SpliceBio is partly financed by its first investment vehicle, Sabadell Asabys Health Innovation Investments SCR, SA, whose anchor investor is Banc Sabadell. The fund's investment in the company benefits from the financial backing of the European Union under the European Fund for Strategic Investments ("EFSI") set up under the Investment Plan for Europe. The purpose of EFSI is to help support financing and implementing productive investments in the European Union and to ensure increased access to financing. For more information, visit: [www.asabys.com](http://www.asabys.com)