



## SpliceBio Enters Collaboration with Spark Therapeutics to Develop a Gene Therapy Targeting an Inherited Retinal Disease

- *SpliceBio is eligible to receive upfront, opt-in and milestone payments up to \$216 million plus royalties*
- *Spark secures exclusive worldwide rights to SpliceBio's proprietary Protein Splicing platform to develop, manufacture, and commercialize a gene therapy targeting an undisclosed inherited retinal disease*

**BARCELONA, 17 October, 2023** – SpliceBio, a genetic medicines company harnessing Protein Splicing to develop the next generation of gene therapies, is pleased to announce the signing of an exclusive collaboration and licensing agreement with [Spark Therapeutics](#) to utilize SpliceBio's proprietary Protein Splicing platform to develop a gene therapy for an undisclosed inherited retinal disease.

Under the terms of the agreement, SpliceBio and Spark will conduct a research collaboration utilizing SpliceBio's proprietary Protein Splicing platform, which offers the potential to address diseases that currently cannot be treated with gene therapies because the necessary gene is too large to be delivered by adeno-associated virus (AAV) vectors. Spark will have exclusive worldwide rights to develop, manufacture, and commercialize a gene therapy arising from this research collaboration targeting an undisclosed inherited retinal disease. SpliceBio will be eligible to receive upfront, opt-in and milestone payments up to \$216 million and royalties on net sales.

"This research collaboration and license agreement is an exciting opportunity to develop a novel gene therapy in an area of high unmet medical need. We are proud that Spark Therapeutics recognizes the potential of our pioneering Protein Splicing platform and the profound impact it could have in the treatment of inherited retinal diseases that are unable to be effectively addressed by other gene therapy approaches," **said, Miquel Vila-Perelló, Ph.D., Chief Executive Officer and co-founder of SpliceBio.** "In addition to the Spark collaboration, we continue to develop our lead program in Stargardt disease and further build our capabilities and pipeline of wholly-owned gene therapy programs to develop life-changing therapies for patients in need."

"This exclusive partnership builds on Spark's innovative leadership programs in gene therapies for inherited retinal diseases. Our breakthrough gene therapy LUXTURNA® demonstrates the ability to transform the lives of patients with biallelic mutations in the *RPE65* gene whose physicians have determined their eligibility for treatment, while providing potential alternative treatment options for patients with other inherited retinal diseases," **commented, Federico Mingozzi, PhD, Chief Science and Technology Officer of Spark Therapeutics.** "With our complementary capabilities, combined deep technical knowledge and SpliceBio's revolutionary platform capabilities, we aim to further advance progress in the treatment of inherited retinal diseases, bringing new transformational gene therapies into the clinic and eventually to the global market."

**-Ends-**



## **About SpliceBio**

SpliceBio is a genetic medicines company harnessing its proprietary Protein Splicing platform to develop the next generation of gene therapies. The Company's platform offers the potential to address diseases that currently cannot be treated with gene therapies because the necessary gene is too large to be delivered by adeno-associated virus (AAV) vectors. The company's lead program targets Stargardt disease, a genetic eye disease that causes vision loss in children and adults. 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 Spark Therapeutics**

Spark Therapeutics is a fully integrated, commercial company dedicated to unlocking the power of gene therapy to accelerate healthcare transformation. At Spark, a member of the Roche Group, we see the path to a world where no life is limited by disease. For more information, visit [www.sparktx.com](http://www.sparktx.com), and follow us on [Twitter](#) and [LinkedIn](#).

### **For further information:**

#### **Optimum Strategic Communications**

Mary Clark, Zoe Bolt, Eleanor Cooper

Email: [splicebio@optimumcomms.com](mailto:splicebio@optimumcomms.com)

Tel: +44 (0) 20 3882 9621

#### **Spark Therapeutics**

Denise Bradley

Email: [communications@sparktx.com](mailto:communications@sparktx.com)