Spark Therapeutics Announces Gene Therapy Collaboration in Hemophilia B with Pfizer Inc.

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Leading gene therapy Company will partner with established market leader to develop a potential new treatment paradigm for hemophilia B

PHILADELPHIA, Penn., December 8, 2014—Spark Therapeutics, a late-stage gene therapy company developing treatments for debilitating genetic diseases, announced today that it has entered into a global collaboration with Pfizer Inc. for the development and potential commercialization of SPK-FIX, a development program advancing proprietary, bio-engineered adeno-associated virus (AAV) vectors for the potential treatment of hemophilia B. The companies will work together on a worldwide basis with the aim of bringing an important investigational therapy to patients.

“We are excited to announce our collaboration with Pfizer, as we believe it marks an important step towards bringing a potentially life-altering therapeutic to patients with hemophilia B,” said Jeffrey D. Marrazzo, co-founder and chief executive officer of Spark. “The collaboration also marks another milestone for Spark, following our recent clinical and regulatory progress and key leadership hires.”

Hemophilia B is a rare genetic blood disorder that affects approximately 4,000 males in the U.S. and 26,000 males worldwide. Current treatment requires recurrent intravenous infusions of either plasmaderived or recombinant Factor IX to control bleeding episodes. Spark’s proprietary, bioengineered vectors are designed to deliver a high-activity Factor IX gene to patients, enabling endogenous production of Factor IX, with the potential to be effective for a number of years. This program leverages a long track record of hemophilia B gene therapy research and clinical development conducted by Spark and its founding scientific team over the past two decades.

“We believe Pfizer is the ideal partner for our hemophilia B program. Pfizer is a leader in hemophilia, developing the first recombinant Factor IX product,” said Dr. Katherine High, a hematologist and cofounder, president and chief scientific officer of Spark. Dr. High, who pioneered the development of AAV-mediated gene therapy for hemophilia, noted, “Pfizer’s longtime experience in hemophilia, including strong relationships with physicians, patients and payors, as well as clinical, regulatory and commercial capabilities, will complement our team’s deep knowledge of AAV-mediated gene transfer for the disease. We look forward to working with Pfizer with the goal of making gene therapy for hemophilia B a reality for patients.”

Under the terms of the agreement, Spark will receive an upfront payment of $20 million and will be eligible for additional development and commercialization milestone payments of up to $260 million for multiple hemophilia B product candidates that may be developed under the collaboration. Under the collaboration, Spark will be responsible for conducting all Phase 1/2 studies while Pfizer will assume responsibility for pivotal studies, any regulatory approvals and potential global commercialization of the product. Spark is entitled to receive double-digit royalties based on global product sales.

“With their experience in the field of gene therapy, as well as in the research and development of potential novel treatments for hemophilia, we believe that Spark’s team of scientists and clinicians will complement Pfizer’s expertise in helping to bring a new therapy to patients,” said Kevin Lee, Senior Vice President and Chief Scientific Officer, Rare Disease Research Unit, Pfizer. “This agreement reinforces Pfizer’s longstanding commitment to the hemophilia community. Spark’s hemophilia B program has the potential to build on our leading hemophilia portfolio and could offer patients with this bleeding disorder a potential new treatment option.”

For more information on Spark Therapeutics and its clinical pipeline, including SPK-FIX, please visit www.sparktx.com.

About Spark Therapeutics Spark is a gene therapy leader seeking to transform the lives of patients suffering from debilitating genetic diseases by developing one-time, life-altering treatments. Spark’s initial focus is on treating orphan diseases where no, or only palliative therapies, exist. Spark’s most advanced product candidate, SPK-RPE65, is in a fully-enrolled pivotal Phase 3 clinical trial for the treatment of a rare blinding condition. Spark is leveraging SPK-RPE65 to address a broad spectrum of blinding conditions, and also has established a pipeline of gene therapy candidates to treat hematologic and neurodegenerative disorders. Spark’s integrated gene therapy platform builds on two decades of research, development and manufacturing at The Children's Hospital of

Philadelphia, including human trials conducted across diverse therapeutic areas and routes of administration. To learn more, visit www.sparktx.com.

About the SPK-FIX Program Hemophilia B is a serious and rare inherited hematologic disorder, characterized by a mutation in the Factor IX, or FIX, gene, which leads to deficient blood coagulation and an increased risk of bleeding or hemorrhaging. Spark's Development program for hemophilia B, SPK-FIX, is advancing proprietary, bioengineered adeno-associated virus (AAV) vectors that deliver a high-activity Factor IX gene to the liver. SPK-FIX leverages a long track record of hemophilia B gene therapy research conducted by Spark and its founding scientific team which, in prior clinical trials, has demonstrated safety and proof-of-concept in expressing Factor IX in the liver. To learn more please visit the Spark website at http://www.sparktx.com/pipeline/hematologic-disorders.

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