Spark Therapeutics and Pfizer Amend License Agreement for Investigational SPK-9001 in Hemophilia B

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Spark Therapeutics to receive up to an additional $25 million per terms of amendment

Spark Therapeutics (NASDAQ:ONCE) and Pfizer Inc. (NYSE:PFE) today announced they have entered into an amendment to their license agreement for SPK-9001, an investigational gene therapy for hemophilia B. Spark Therapeutics will enroll up to five additional participants in the current Phase 1/2 clinical trial who will receive SPK-9001 manufactured using an enhanced process to test its comparability to the SPK-9001 received by the first 10 participants enrolled in the ongoing trial.

One of the up to five participants has already received SPK-9001 manufactured using the enhanced process. As of Oct. 23, this participant has been followed for 32 weeks post-infusion. The participant's factor IX activity level has plateaued within the range that is considered comparable to the first 10 participants, and this participant's number of prophylactic intravenous factor IX infusions has been reduced to zero.

"Early data on the 11th participant suggest potential clinical comparability with the preliminary safety and efficacy results seen with the initial 10 trial participants, who all have discontinued routine infusions of factor IX concentrates," said Katherine A. High, M.D., president and head of Research & Development at Spark Therapeutics. "We look forward to transitioning this program to Pfizer and potentially bringing to market this one-time investigational gene therapy for patients with hemophilia B, who otherwise rely on frequent infusions of factor IX to control and prevent bleeding episodes."

Updated data on all 11 participants in the Phase 1/2 clinical trial will be presented by Lindsey A. George, M.D., attending physician in the Division of Hematology at Children's Hospital of Philadelphia, at the 59thAmerican Society of Hematology (ASH) Annual Meeting & Exposition, at the Georgia World Congress Center in Atlanta on Monday, Dec. 11, at 7:00 AM ET.

Spark Therapeutics is transferring the enhanced SPK-9001 manufacturing process to Pfizer, which intends to utilize material generated with this process in the Phase 3 clinical trial of SPK-9001. The activities outlined in the license agreement as amended will occur in parallel to Pfizer's ongoing preparation to assume responsibility for SPK-9001 after the transfer of the Investigational New Drug application to Pfizer.

Subject to the terms of the amendment, Spark Therapeutics will receive from Pfizer an initial $10 million cash payment and up to an additional $15 million in potential milestone payments upon completion of certain transition activities.

About Hemophilia B

Hemophilia, a rare genetic bleeding disorder that causes the blood to take a long time to clot because of a deficiency in one of several blood clotting factors, is almost exclusively found in males. People with hemophilia are at risk for excessive and recurrent bleeding from modest injuries, which have the potential to be life threatening. People with severe hemophilia often bleed spontaneously into their muscles or joints. The incidence of hemophilia B is one in 25,000 male births. People with hemophilia B have a deficiency in clotting factor IX, a specific protein in the blood. Hemophilia B also is called congenital factor IX deficiency or Christmas disease. The current standard of care requires recurrent intravenous infusions of either plasma-derived or recombinant factor IX to control and prevent bleeding episodes. There exists a significant need for novel therapeutics to treat people living with hemophilia.

About the SPK-FIX Program and SPK-9001

SPK-9001 is a novel, investigational bio-engineered adeno-associated virus (AAV) capsid expressing a codon-optimized, high-activity human factor IX variant enabling endogenous production of factor IX.

Spark Therapeutics and Pfizer entered into a collaboration in December 2014 for the SPK-FIX program, including SPK-9001, under which Spark Therapeutics is responsible for conducting all Phase 1/2 studies for any product candidates, while Pfizer will assume responsibility for pivotal studies, any regulatory activities and potential global

commercialization of any products that may result from the collaboration.

About Spark Therapeutics

At Spark Therapeutics, a fully integrated company committed to discovering, developing and delivering gene therapies, we challenge the inevitability of genetic diseases, including blindness, hemophilia and neurodegenerative diseases. We have successfully applied our technology directed to the retina and liver, and currently have four programs in clinical trials or under regulatory review, including the first potential gene therapy for a genetic disease in the United States and product candidates that have shown promising early results in patients with hemophilia. At Spark, we see the path to a world where no life is limited by genetic disease. For more information, visit www.sparktx.com, and follow us on Twitter and LinkedIn.

Spark Cautionary Note on Forward-looking Statements

This release contains "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995, including statements regarding the company's SPK-FIX program. The words ‘‘anticipate,'' ‘‘believe,'' ‘‘expect,'' ‘‘intend,'' ‘‘may,'' ‘‘plan,'' ‘‘predict,'' ‘‘will,'' ‘‘would,'' ‘‘could,'' ‘‘should,'' ‘‘continue'' and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. We may not actually achieve the plans, intentions or expectations disclosed in our forward-looking statements, and you should not place undue reliance on our forward-looking statements. Any forward-looking statements are based on management's current expectations of future events and are subject to a number of risks and uncertainties that could cause actual results to differ materially and adversely from those set forth in, or implied by, such forward-looking statements. These risks and uncertainties include, but are not limited to, the risk that: (i) our lead SPK-FIX product candidate, SPK-9001, may not produce sufficient data in our Phase 1/2 clinical trial to warrant further development; (ii) our overall collaboration with Pfizer may not be successful; (iii) we may not receive any additional milestone payments from Pfizer; and (iv) any one or more of our product candidates in preclinical or clinical development will not successfully be developed and commercialized. For a discussion of other risks and uncertainties, and other important factors, any of which could cause our actual results to differ from those contained in the forward-looking statements, see the "Risk Factors" section, as well as discussions of potential risks, uncertainties and other important factors, in our Annual Report on Form 10-K, our Quarterly Reports on Form 10-Q and other filings we make with the Securities and Exchange Commission. All information in this press release is as of the date of the release, and Spark undertakes no

duty to update this information unless required by law.

About Pfizer: Working together for a healthier world®

At Pfizer, we apply science and our global resources to bring therapies to people that extend and significantly improve their lives. We strive to set the standard for quality, safety and value in the discovery, development and manufacture of health care products. Our global portfolio includes medicines and vaccines as well as many of the world's best-known consumer health care products. Every day, Pfizer colleagues work across developed and emerging markets to advance wellness, prevention, treatments and cures that challenge the most feared diseases of our time. Consistent with our responsibility as one of the world's premier innovative biopharmaceutical companies, we collaborate with health care providers, governments and local communities to support and expand access to reliable, affordable health care around the world. For more than 150 years, we have worked to make a difference for all who rely on us. We routinely post information that may be important to investors on our website at www.pfizer.com. In addition, to learn more, please visit us on www.pfizer.com and follow us on Twitter at @Pfizer and @Pfizer_News, LinkedIn, YouTube and like us on Facebook at Facebook.com/Pfizer.

Pfizer Disclosure Notice: The information contained in this release is as of November 7, 2017. Pfizer assumes no obligation to update forward-looking statements contained in this release as the result of new information or future events or developments.

This release contains forward-looking information about SPK-9001 and the SPK-FIX program, including their potential benefits, that involves substantial risks and uncertainties that could cause actual results to differ materially from those expressed or implied by such statements. Risks and uncertainties include, among other things, the uncertainties inherent in research and development, including the ability to meet anticipated clinical study commencement and completion dates as well as the possibility of unfavorable study results, including unfavorable new clinical data and additional analyses of existing clinical data; risks associated with initial data, including the risk that the final results of the Phase 1/2 study for SPK-9001 and/or additional clinical trials may be different from (including less favorable than) the initial data results and may not support further clinical development; whether and when any applications may be filed with regulatory authorities for SPK-9001; whether and when regulatory authorities may approve any such applications, which will depend on the assessment by such regulatory authorities of the benefit-risk profile suggested by the totality of the efficacy and safety information submitted; decisions by regulatory authorities regarding labeling and other matters that could affect the availability or commercial potential of SPK-9001; and

competitive developments.

A further description of risks and uncertainties can be found in Pfizer's Annual Report on Form 10-K for the fiscal year ended December 31, 2016 and in its subsequent reports on Form 10-Q, including in the sections thereof captioned "Risk Factors" and "Forward-Looking Information and Factors That May Affect Future Results", as well as in its subsequent reports on Form 8-K, all of which are filed with the U.S. Securities and Exchange Commission and available at www.sec.gov and www.pfizer.com.

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