# Spark Therapeutics and Pfizer Announce Receipt of FDA Breakthrough Therapy Designation for SPK-9001 for the Treatment of Hemophilia B

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**PHILADELPHIA and NEW YORK CITY, July 21, 2016** -- Spark Therapeutics (NASDAQ:ONCE) and Pfizer Inc. (NYSE:PFE) announced today that the U.S. Food and Drug Administration (FDA) has granted breakthrough therapy designation to *SPK-9001,* the lead investigational candidate in the companies' *SPK-FIX* program, in development for the treatment of hemophilia B. *SPK-9001,* a novel bio-engineered adeno-associated virus (AAV) capsid expressing a codon-optimized, high-activity human factor IX variant, is being investigated in an ongoing Phase 1/2 trial as a potential one-time therapy.

Breakthrough therapy designation is intended to expedite the development and FDA review of drugs to treat a serious or life-threatening disease or condition. The designation requires preliminary clinical evidence that the investigational therapy may offer substantial improvement over existing therapies on at least one clinically significant endpoint.

In addition to *SPK-9001,* Spark Therapeutics previously received breakthrough therapy designation for *voretigene neparvovec,* which is being developed for the potential treatment of inherited retinal disease (IRD) caused by mutations in the *RPE65* gene.

"We are extremely pleased to have been granted breakthrough therapy designation for *SPK-9001*, which has shown early promise in achieving our goal of eliminating the need

for regular infusions to control and prevent bleeding episodes in patients with hemophilia B through a potentially one-time, intravenous administration of a highly optimized gene therapy," said Jeffrey D. Marrazzo, chief executive officer of Spark Therapeutics. "Together with Pfizer, we look forward to working closely with the FDA to bring *SPK-9001* to patients as quickly and responsibly as possible."

**About Hemophilia B**

Hemophilia is a rare genetic bleeding disorder that causes the blood to take a long time to clot as a result of a deficiency in one of several blood clotting factors, and occurs almost exclusively in males. People with hemophilia face specific risks as they are not able to form blood clots efficiently and 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 is also called congenital factor IX deficiency or Christmas disease. 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**

Spark Therapeutics' proprietary technology platform for selecting, designing, manufacturing and formulating highly optimized gene therapies was applied to developing compounds in the *SPK-FIX* program. The *SPK-FIX* program leverages a long history of hemophilia gene therapy research and clinical development conducted by Spark Therapeutics and its founding scientific team over nearly three decades. *SPK-9001* is a novel bio-engineered adeno-associated virus (AAV) capsid expressing a codon-optimized, high-activity human factor IX variant enabling endogenous production of factor IX. *SPK-9001* is being developed under a collaboration with Pfizer. Spark Therapeutics and Pfizer entered into a collaboration in 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

Spark Therapeutics, a fully integrated gene therapy company, is seeking to transform the lives of patients with debilitating genetic diseases by developing one-time, life-altering treatments. Spark Therapeutics' validated gene therapy platform is being applied to a range of clinical and preclinical programs addressing serious genetic diseases, including inherited retinal diseases, liver-associated diseases, such as hemophilia, and neurodegenerative diseases. Spark Therapeutics' validated and proprietary technology platform for selecting, designing, manufacturing and formulating highly optimized gene therapies has successfully delivered gene therapies with proof-of-concept data in the eye and liver. Spark Therapeutics' most advanced product candidate, voretigene neparvovec (formerly referred to as *SPK-RPE65*), which has received both breakthrough therapy and orphan product designation, reported positive top-line results from a pivotal Phase 3 clinical trial for the treatment of rare blinding conditions. Spark Therapeutics' hemophilia franchise has two lead assets: *SPK-9001,* in a Phase 1/2 trial for hemophilia B and *SPK-8011*, a preclinical candidate for hemophilia A. To learn more, please visit www.sparktx.com.

## 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. 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; and (ii) our overall collaboration with Pfizer may not be successful. 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.

**Pfizer and Rare Diseases**

Rare diseases are among the most serious of all illnesses and impact millions of patients worldwide, representing an opportunity to apply our knowledge and expertise to help make a significant impact in addressing unmet medical needs. The Pfizer focus on rare diseases builds on more than two decades of experience, a dedicated research unit focusing on rare diseases, and a global portfolio of more than 20 medicines approved worldwide that treat rare diseases in the areas of hematology, neuroscience, inherited metabolic disorders, pulmonology, and oncology.

**Pfizer Inc: 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, Pfizer has worked to make a difference for all who rely on us. For more information, please visit us at www.pfizer.com. In addition, to learn more, 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 July 21, 2016. 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 I/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, 2015 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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