Pfizer Logo

Spark Therapeutics and Pfizer Announce that SPK-9001, an Investigational Hemophilia B Medicine, has been Granted Access to the PRIority MEdicines (PRIME) Program by the European Medicines Agency

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PHILADELPHIA and NEW YORK, March 1, 2017 (GLOBE NEWSWIRE)— Spark Therapeutics (NASDAQ:ONCE) and Pfizer Inc. (NYSE:PFE) announced today that SPK-9001, the lead investigational candidate in the companies' SPK-FIX program has been granted support through the European Medicines Agency (EMA) PRIority MEdicines (PRIME) program. According to the EMA, PRIME is intended to enhance support for new treatments in development "that may offer a major therapeutic advantage over existing treatments, or benefit patients without treatment options."

"We are pleased that investigational SPK-9001 has been granted access to the PRIME program, which potentially will help accelerate the delivery of this potential gene therapy to European patients living with hemophilia B," said Jeffrey D. Marrazzo, chief executive officer of Spark Therapeutics. "We appreciate the EMA's dedication to moving treatments forward for patients who are underserved with current options, and we look forward to our continued collaboration on SPK-9001."

SPK-9001 is an experimental treatment for hemophilia B that is currently being investigated in an ongoing Phase 1/2 trial as a potential single-dose therapy. The PRIME program provides enhanced interaction and early dialogue with developers of promising medicines, which is intended to optimize development plans and speed up evaluation so these medicines can reach patients earlier.

SPK-9001 received orphan product designation from the U.S. Food and Drug Administration (FDA) in September 2015, and later received breakthrough therapy designation from the FDA in July 2016.

About Hemophilia B
Hemophilia, 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, is almost exclusively 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. 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
Spark Therapeutics' proprietary technology platform for selecting, designing, manufacturing and formulating 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, investigational 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 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
Spark Therapeutics, a fully integrated company, strives to challenge the inevitability of genetic disease by discovering, developing, and delivering gene therapies that address inherited retinal diseases (IRDs), neurodegenerative diseases, as well as diseases that can be addressed by targeting the liver. Our validated platform successfully has delivered proof-of-concept data with investigational gene therapies in the retina and liver. Our most advanced investigational candidate, voretigene neparvovec, in development for the treatment of biallelic RPE65-mediated IRD, has received orphan designations in the U.S. and European Union, and breakthrough therapy designation in the U.S. The pipeline also includes SPK-7001 in a Phase 1/2 trial for choroideremia, and two hemophilia development programs: SPK-9001 (which also has received both breakthrough therapy and orphan product designations) in a Phase 1/2 trial for hemophilia B being developed in collaboration with Pfizer, and SPK-8011, in a Phase 1/2 trial for hemophilia A to which Spark Therapeutics retains global commercialization rights. To learn more about us and our growing pipeline, 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 Disease
Rare disease includes some of the most serious of all illnesses and impacts millions of patients worldwide,i representing an opportunity to apply our knowledge and expertise to help make a significant impact on addressing unmet medical needs. The Pfizer focus on rare disease builds on more than two decades of experience, a dedicated research unit focusing on rare disease, and a global portfolio of multiple medicines within a number of disease areas of focus, including hematology, neuroscience, and inherited metabolic disorders.ii

Pfizer Rare Disease combines pioneering science and deep understanding of how diseases work with insights from innovative strategic collaborations with academic researchers, patients, and other companies to deliver transformative treatments and solutions. We innovate every day leveraging our global footprint to accelerate the development and delivery of groundbreaking medicines and the hope of cures.

Click here to learn more about our Rare Disease portfolio and how we empower patients, engage communities in our clinical development programs, and support programs that heighten disease awareness and meet the needs of patient families.

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 Feb. 28, 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 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, 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.

Investor Contact:
Ryan Asay
(215) 282-7470

Media Contact:
Monique da Silva Ryan.asay@sparktx.com
Monique.dasilva@sparktx.com (215) 239-6424

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i Rare Disease: Facts and Statistics. http://globalgenes.org/rare-diseases-facts-statistics. Accessed September 7, 2016.
ii Pfizer Inc. Rare Disease. http://www.pfizer.com/health-and-wellness/health-topics/rare-diseases/areas-of-focus. Accessed December 20, 2016.