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Spark Therapeutics and Pfizer Announce Updated Data from Hemophilia B Phase 1/2 Trial Suggesting Sustained Therapeutic Levels of Factor IX Activity

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First seven participants, through a combined 103 weeks of observation as of Aug. 4, 2016, did not need infusions of factor IX concentrates to prevent bleeding Updated SPK-9001 data in hemophilia B to be presented at the Plenary Scientific Session at 58th American Society of Hematology Annual Meeting

PHILADELPHIA and NEW YORK, Nov. 03, 2016 (GLOBE NEWSWIRE) -- Spark Therapeutics (NASDAQ:ONCE) and Pfizer Inc. (NYSE:PFE) announced today that Lindsey A. George, M.D., a hematologist and attending physician at the Children's Hospital of Philadelphia and lead investigator in the Phase 1/2 clinical trial of SPK-9001 for treatment of hemophilia B, will present an updated dataset from the ongoing Phase 1/2 clinical trial of SPK-9001 at the 58th American Society of Hematology (ASH) Annual Meeting, to be held Dec. 3-6, 2016, in San Diego. The conference abstract, including a plot of factor IX activity levels (as a % of normal) expressed over time by the first seven participants as of Aug. 4, 2016, was made available today: ASH Abstract.

The results are from the first seven participants who received a single administration of investigational SPK-9001 at a dose of 5 x 1011 vector genomes (vg)/kg body weight. Four of the seven participants had reached greater than 12 weeks post-vector administration at the time of abstract submission; those four participants experienced consistent and sustained factor IX activity levels, with a mean greater than 30% of normal, with no sustained elevation in liver enzyme levels.

Spark Therapeutics and Pfizer report that one participant, who had not reached 12 weeks post-vector administration, manifested an immune response to the adeno-associated virus (AAV) capsid, accompanied by a drop in factor IX activity level, and was put on a tapering course of corticosteroids. Despite the immune response and decline in factor IX activity level, this participant has not had any bleeds or required replacement factor. No other participant has required the use of corticosteroids. The companies expect to announce updated trial results at the upcoming ASH Annual Meeting in December.

None of the participants received infusions of factor IX concentrates to prevent bleeding events. One precautionary infusion took place in one participant two days after administration of SPK-9001 due to a suspected ankle bleed.

"These initial observations are encouraging, underscoring the potential of investigational SPK-9001 to deliver a potentially consistent, sustained and therapeutically meaningful level of factor IX activity through one administration," said Dr. George. "Participants in the trial did not require prophylactic factor IX infusions to prevent bleeding, including one participant who we have followed for more than eight months as of the data cutoff. We look forward to reporting additional data as we continue to document the longer-term experience with SPK-9001."

As of Aug. 4, 2016, total consumption of clotting factor in all seven trial participants during a cumulative 724 days following vector administration was reduced by more than 540,000 international units, based on their pre-trial usage levels. Additionally, to date six of seven participants reported increased physical activity and improved quality of life, based on the Haemophilia Quality of Life Questionnaire for Adults, a validated instrument that measures health-related quality of life in adults with hemophilia.

Presentation Details:

SPK-9001: Adeno-Associated Virus Mediated Gene Transfer for Hemophilia B Achieves Sustained Mean Factor IX Activity Levels of >30% without Immunosuppression (Abstract # 91358)

Presenter: Lindsey George, M.D., Children's Hospital of Philadelphia
Date: Sunday, Dec. 4, 2016
Session time: 2-4 p.m. PST
Location: San Diego Convention Center, Hall AB

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 common 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 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 and SPK-9001

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. SPK-9001 has received breakthrough therapy and orphan product designations from the U.S. Food and Drug Administration.

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 investigational, potentially 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-mediated diseases such as hemophilia, and neurodegenerative diseases. Spark Therapeutics' validated platform successfully has delivered proof-of-concept data with investigational gene therapies in the retina and liver. Spark Therapeutics has reported

top-line results from a pivotal Phase 3 clinical trial for its most advanced product candidate, voretigene neparvovec (formerly referred to as SPK-RPE65), a potential treatment of a rare genetic blinding condition. Voretigene neparvovec has received both breakthrough therapy and orphan product designations. Spark Therapeutics' hemophilia franchise has two lead assets: SPK-9001 in a Phase 1/2 trial for hemophilia B being developed under a collaboration with Pfizer and SPK-8011, a preclinical candidate for hemophilia A to which Spark Therapeutics retains global commercialization rights. 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, 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 @PfizerNews, LinkedIn, YouTube and like us on Facebook at Facebook.com/Pfizer.

Pfizer Disclosure Notice:

The information contained in this release is as of Nov. 3, 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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