Pfizer Initiates Pivotal Phase 3 Program for Investigational Hemophilia B Gene Therapy

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Phase 3 lead-in study initiated following completion of the transfer of Spark Therapeutics' hemophilia B gene therapy program to Pfizer

NEW YORK & PHILADELPHIA--(BUSINESS WIRE)-- Pfizer Inc. (NYSE:PFE) and Spark Therapeutics (NASDAQ:ONCE) announced today that Pfizer initiated a Phase 3 open-label, multi-center, lead-in study (NCT03587116) to evaluate the efficacy and safety of current factor IX prophylaxis replacement therapy in the usual care setting. The factor IX prophylaxis efficacy data obtained in the lead-in study will serve as the within-subject control group for those patients that enroll into the next part of the Phase 3 study, which will evaluate the investigational gene therapy fidanacogene elaparvovec for the treatment of hemophilia B. The interventional portion of this pivotal Phase 3 study will enroll patients who have completed at least six months in the lead-in study. Fidanacogene elaparvovec is the official United States Adopted Name (USAN) and will become the Recommended International Nonproprietary Name (INN) for the therapy formerly known as SPK-9001 and PF-06838435.

The Phase 3 program was initiated following the transfer of the responsibility for Spark Therapeutics' hemophilia B gene therapy program to Pfizer. Fidanacogene elaparvovec is a novel, investigational vector that contains a bio-engineered adeno-associated virus (AAV) capsid (protein shell) and a high-activity human coagulation factor IX gene. It is hoped that, once treated, patients will be able to produce factor IX themselves, rather than having to regularly inject factor IX.

"With the lead-in study now open and actively recruiting patients, we are excited to begin our Phase 3 program evaluating fidanacogene elaparvovec for the treatment of hemophilia B," said Brenda Cooperstone, MD, Senior Vice President and Chief

Development Officer, Rare Disease, Pfizer Global Product Development. “The current data suggest immense promise for the use of this potential one-time treatment option. We look forward to the opportunity to continue the progress achieved by Spark Therapeutics for patients living with hemophilia B.”

“We are pleased to have transitioned fidanacogene elaparvovec to Pfizer following the positive results of the ongoing Phase 1/2 clinical trial,” said Katherine A. High, MD, President and Head of Research & Development, Spark Therapeutics. “The initiation of the Phase 3 program marks an important milestone toward our goal of one day potentially freeing patients with hemophilia B of the need for regular infusions, while potentially eliminating spontaneous bleeding.”

In May 2018, Pfizer and Spark Therapeutics announced data for 15 participants in the ongoing Phase 1/2 clinical trial of fidanacogene elaparvovec for the treatment of severe or moderately severe (FIX:C < 2 percent) hemophilia B. The findings showed all 15 patients had discontinued routine infusions of factor IX concentrates with no reported serious adverse events or thrombotic events as of the May 7, 2018 data cutoff.

About the Pfizer and Spark Therapeutics Agreement

Pfizer and Spark Therapeutics entered into a License Agreement in December 2014 for the hemophilia B gene therapy program. Under the terms of the agreement, Pfizer will now assume sole responsibility for all subsequent pivotal studies, all regulatory activities, manufacturing and global commercialization of any products resulting from the hemophilia B gene therapy program.

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, or rarely into other critical closed spaces such as the intracranial space, where bleeding can be fatal. 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.

Pfizer Rare Disease

Rare disease includes some of the most serious of all illnesses and impacts millions of patients worldwide, representing an opportunity to apply our knowledge and expertise to help make a significant impact on addressing unmet medical needs.1 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, neuromuscular, and inherited metabolic disorders.1

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.

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.

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 in the first FDA-approved gene therapy in the U.S. for a genetic disease, and currently have three programs in clinical trials, including 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 Therapeutics Cautionary note on forward-looking statements

This press 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) SPK-9001 may not free patients with hemophilia B of the need for regular infusions, while eliminating spontaneous bleeding 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 U.S. Securities and Exchange Commission. All information in this press release is as of the date of the press release, and Spark undertakes no duty to update this information unless required by law.

DISCLOSURE NOTICE: The information contained in this release is as of July 16, 2018. 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 fidnacogene elparvovec, the fidnacogene elparvovec program and the License Agreement between Pfizer and Spark,

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 fidnacogene elparvovec lead-in study and the subsequent Phase 3 study and/or additional clinical trials may be different from (including less favorable than) the initial data results and may not support further clinical development; the risk that clinical trial data are subject to differing interpretations, and, even when we view data as sufficient to support the safety and/or effectiveness of a product candidate, regulatory authorities may not share our views and may require additional data or may deny approval altogether; whether regulatory authorities will be satisfied with the design of and results from our clinical studies; whether and when any applications may be filed with regulatory authorities for fidnacogene elparvovec; 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 and, if approved, whether fidnacogene elparvovec will be commercially successful; decisions by regulatory authorities regarding labeling and other matters that could affect the availability or commercial potential of fidnacogene elparvovec; 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, 2017 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.

_____ 1 Pfizer Inc. Rare disease. http://www.pfizer.com/health-and-wellness/health-topics/rare-diseases/areas-of-focus. Accessed July 2018.

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