Pfizer Logo

Spark Therapeutics Announces $15 Million Milestone Payment from Pfizer for Progress in Hemophilia B Gene Therapy Program

Wednesday, January 04, 2017 - 08:00am

Second milestone achieved under 2014 global license agreement with Pfizer

PHILADELPHIA, Jan. 4, 2017 (GLOBE NEWSWIRE) -- Spark Therapeutics (NASDAQ:ONCE), a fully integrated gene therapy company dedicated to challenging the inevitability of genetic disease, today announced that it has earned a $15 million payment from Pfizer Inc. (NYSE:PFE) for achieving a prespecified safety and efficacy profile development milestone in the ongoing hemophilia B Phase 1/2 trial of investigational SPK-9001. SPK-9001 has received breakthrough therapy and orphan product designations from the U.S. Food and Drug Administration.

“We continue to make strong, tangible progress with our hemophilia pipeline, and achievement of this second milestone marks further advancement in the development of our investigational gene therapy for hemophilia B,” said Jeffrey D. Marrazzo, chief executive officer of Spark Therapeutics. “We look forward to reporting additional data as we continue to document the clinical experience with SPK9001.”

Under the terms of the license agreement with Pfizer, Spark Therapeutics received a $20 million upfront payment upon entering into the agreement in 2014, and a $15 million milestone payment in December 2015 for progress with the development program. The company is eligible to receive up to an additional $230 million in aggregate for achieving future development and commercial milestones, as well as royalties calculated as a low-teen percentage of net sales on any potential SPK-FIX products. Spark Therapeutics maintains responsibility for the clinical development of SPK-FIX product candidates through the completion of Phase 1/2 trials. Thereafter, Pfizer has responsibility for further clinical development, achieving regulatory approvals and potential global commercialization.

The SPK-FIX program leverages a more than two-decade long history of hemophilia gene therapy research and clinical development conducted by Spark Therapeutics and its founding scientific team. 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.

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 1 in the retina and liver. Our most advanced investigational candidate, voretigene neparvovec, in development for the treatment of

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 SPK7001, 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, SPK9001, 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.

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