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

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PHILADELPHIA, Dec. 8, 2015 – Spark Therapeutics, Inc. (“Spark”) (NASDAQ: ONCE) announced today that it has earned a $15 million milestone payment from Pfizer Inc. (“Pfizer”) (NYSE: PFE) under the companies’ global collaboration for the potential development and commercialization of SPK-FIX product candidates for the treatment of hemophilia B. This is the first milestone achieved under the agreement that was entered into in December 2014.

“We are delighted to have achieved this milestone in our collaboration with Pfizer, as it demonstrates the progress we have been making together in developing gene therapy for hemophilia B,” said Dr. Katherine A. High, co-founder, president and chief scientific officer of Spark. “We look forward to continuing to progress the current Phase 1/2 clinical trial.”

Under the terms of the collaboration agreement, Spark received a $20 million upfront payment and is eligible to receive up to $245 million in aggregate additional development and commercial milestones, as well as royalties calculated as a low-teen percentage of net product sales. Spark 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, any regulatory approvals and potential commercialization.

About Spark Therapeutics

Spark is a gene therapy leader seeking to transform the lives of patients with debilitating genetic diseases by developing one-time, life-altering treatments. Spark’s initial focus is on treating rare diseases where no, or only palliative, therapies exist. Spark’s most advanced product candidate, SPK-RPE65, which has received both breakthrough therapy and orphan product designation, recently reported positive top-line results from a pivotal Phase 3 clinical trial for the treatment of rare blinding conditions. Spark’s validated gene therapy platform is being applied to a range of clinical and preclinical programs addressing serious genetic diseases, including inherited retinal dystrophies, hematologic disorders and neurodegenerative diseases. Spark builds on two decades of research, development and manufacturing at The Children’s Hospital of Philadelphia, including human trials conducted across diverse therapeutic areas and routes of administration. To learn more, please visit www.sparktx.com.

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 forwardlooking 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 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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