Spark Therapeutics Enters into Licensing Agreement with Genethon

Includes exclusive worldwide rights to adeno-associated viral (AAV) gene therapy targeting the liver

PHILADELPHIA and EVRY, FRANCE, Sept. X, 2017 (GLOBE NEWS) -- Spark Therapeutics (NASDAQ:ONCE), a fully integrated gene therapy company dedicated to challenging the inevitability of genetic disease, today announced it has entered into a licensing agreement with Genethon, a non-profit research and development organization dedicated to the development of gene therapies for orphan genetic diseases from research to clinical validation, for the development and commercialization of an adeno-associated viral (AAV) gene therapy targeting the liver to address a rare genetic disease.

Spark Therapeutics has the option to develop and commercialize any gene therapy that results from the license agreement. Under the terms of the agreement, Genethon will collaborate with Spark Therapeutics on further preclinical research activities. The specific genetic target and financial terms of the agreement have not been disclosed.

“Capitalizing on our expertise and success to date in liver-directed AAV gene therapy, we look forward to collaborating with Genethon to develop a potential new gene therapy for a debilitating genetic disease,” said Katherine A. High, M.D., president and chief scientific officer of Spark Therapeutics. “We believe our agreement with Genethon expands our proven and proprietary gene therapy platform and helps deliver on our mission to challenge the inevitability of genetic disease.”

“Genethon is pleased to collaborate with Spark Therapeutics, a strong partner for the development of new gene therapies,” said Frédéric Revah, chief executive officer of Genethon.
"It is a recognition of the quality of translational research performed by Genethon's team with the objective of delivering effective therapies to patients affected with rare diseases."

**About Spark Therapeutics** 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, such as hemophilia. Spark Therapeutics has ongoing clinical trials investigating gene therapies in hemophilia A and B. **SPK-8011** is in an ongoing, dose-escalation Phase 1/2 clinical trial as a potential one-time therapy for hemophilia A. The company retains full global commercialization rights to **SPK-8011**. **SPK-9001** is in a Phase 1/2 clinical trial for hemophilia B and is being developed in collaboration with Pfizer. It has received both breakthrough therapy and orphan product designations from the Food and Drug Administration (FDA), and access to the PRIority MEdicines (PRIME) Program from the European Medicines Agency (EMA). Our most advanced investigational candidate, with proposed trade name LUXTURNA™ (voretigene neparvovec), is currently under Priority Review with FDA for the treatment of patients with vision loss due to confirmed biallelic RPE65-mediated IRD and has been designated as a drug for a rare pediatric disease. The Marketing Authorization Application for LUXTURNA has been validated by EMA for the treatment of patients with vision loss due to Leber congenital amaurosis (LCA) or retinitis pigmentosa (RP) IRDs caused by confirmed biallelic **RPE65** mutations. LUXTURNA has received breakthrough therapy and orphan product designations from FDA and orphan product designations from EMA. The pipeline also includes **SPK-7001** in an ongoing Phase 1/2 clinical trial for choroideremia. For more information, visit [www.sparktx.com](http://www.sparktx.com), and follow us on [Twitter](http://twitter.com) and [LinkedIn](http://linkedin.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. For example, our agreement with Genethon may not produce product candidates that warrant for further development and may not advance our gene therapy platform. 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.

**About Genethon**

Created by the AFM-Telethon, the French Muscular Dystrophy Association (AFM), Genethon, located in Evry, France, is a non-profit R&D organization dedicated to the development of biotherapies for orphan genetic diseases, from the research to clinical validation. Genethon is specialized in the discovery and development of gene therapy drugs and has multiple ongoing programs at clinical, preclinical and research stages for neuromuscular, blood, immune system, liver and eye diseases.

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