Sarepta Therapeutics and Genethon Announce a Gene Therapy Research Collaboration for the Treatment of Duchenne Muscular Dystrophy

CAMBRIDGE, Mass. and EVRY, France, June 21, 2017 (GLOBE NEWSWIRE) -- Sarepta Therapeutics, Inc. (NASDAQ:SRPT), a U.S. commercial-stage biopharmaceutical company focused on the discovery and development of unique RNA-targeted therapeutics for the treatment of rare neuromuscular diseases, and Genethon, a non-profit R&D organization dedicated to the development of biotherapies for orphan genetic diseases from research to clinical validation, have signed a gene therapy research collaboration to jointly develop treatments for Duchenne muscular dystrophy (DMD). Genethon's micro-dystrophin gene therapy approach can target the majority of patients with DMD. Genethon has demonstrated proof-of-concept of their micro-dystrophin program via robust gene expression in a large animal model of DMD.

Under the terms of the collaboration, Genethon will be responsible for the early development work. Sarepta has the option to co-develop Genethon's micro-dystrophin program, which includes exclusive U.S. commercial rights. Financial terms of the collaboration have not been disclosed.

Genethon has made significant investment in the development of gene therapies for neuromuscular diseases and employs one of the largest research and clinical groups in the world working to advance rare disease therapies. The Company's European-based research laboratory has a long-term commitment to neuromuscular diseases with a central focus on DMD. In addition, Genethon is affiliated with Europe's largest cGMP vector manufacturing facility, YposKesi, located in Evry (Essonne). YposKesi employs approximately 150 experts in bio-production at its current 54,000 square feet manufacturing facility, and plans significant future expansion to meet the growing demand of gene therapy products.

"Our agreement with Genethon strengthens our ongoing commitment to patients and is aligned with our strategy of building the industry's most comprehensive franchise in DMD," stated Edward Kaye, Sarepta's chief executive officer. "This partnership brings together our collective experience in Duchenne drug development and Genethon's particular expertise in gene therapy for rare diseases. We look forward to working with Genethon given their knowledge, large infrastructure and state-of the-art manufacturing capabilities to advance next generation therapies for DMD."

“Microdystrophin-based gene therapy is a very promising approach with potential application to a large majority of Duchenne patients. In order to accelerate the development of a treatment, we are very pleased to partner with Sarepta Therapeutics, which has demonstrated commitment and success for innovative therapies for Duchenne muscular dystrophy. This partnership brings together the highly complementary and synergistic expertises of Sarepta and Genethon, to the benefit of the patients,” said Frederic Revah, chief executive officer of Genethon.

About Sarepta Therapeutics

Sarepta Therapeutics is a U.S. commercial-stage biopharmaceutical company focused on the discovery and development of unique RNA-targeted therapeutics for the treatment of rare neuromuscular diseases. The Company is primarily focused on rapidly advancing the development of its potentially disease-modifying Duchenne muscular dystrophy (DMD) drug candidates. For more information, please visit www.sarepta.com.

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 stage for neuromuscular, blood, immune system, liver and eye diseases.

Forward-Looking Statement

This press release contains statements that are forward-looking. Any statements contained in this press release that are not statements of historical fact may be deemed to be forward-looking statements. Words such as "believes," "anticipates," "plans," "expects," "will," "intends," "potential," "possible" and similar expressions are intended to identify forward-looking statements. These forward-looking statements include statements about the gene therapy research collaboration agreement Sarepta has entered into with Genethon, Sarepta’s option to co-develop Genethon’s micro-dystrophin program, the potential for gene therapy to advance next generation therapies for DMD, the alignment of the collaboration agreement with Sarepta’s strategy to build the industry’s most comprehensive franchise in DMD, and the potential application of microdystrophin-based gene therapy to a large majority of Duchenne patients.

These forward-looking statements involve risks and uncertainties, many of which are beyond Sarepta's control. Known risk factors include, among others: the expected benefits and opportunities related to the

collaboration agreement between the parties may not be realized or may take longer to realize than expected due to challenges and uncertainties inherent in product research and development; the collaboration between Sarepta and Genethon may not result in any viable treatments suitable for clinical research or commercialization due to a variety of reasons including that the results of additional research may not be consistent with past results or may not be positive or may otherwise fail to meet regulatory approval requirements for the safety and efficacy of product candidates or may never become commercialized products due to other various reasons including any potential future inability of the parties to fulfill their commitments and obligations under the agreement; and even if the agreement results in commercialized products, the parties may not achieve any significant revenues from the sale of such products.

Any of the foregoing risks could adversely affect Sarepta's business, results of operations and the trading price of Sarepta's common stock. For a detailed description of risks and uncertainties Sarepta faces, you are encouraged to review Sarepta's 2016 Annual Report on Form 10-K and most recent Quarterly Report on Form 10-Q for the quarter ended March 31, 2017 filed with the Securities and Exchange Commission (SEC) as well as other SEC filings made by Sarepta. We caution investors not to place considerable reliance on the forward-looking statements contained in this press release. Sarepta does not undertake any obligation to publicly update its forward-looking statements based on events or circumstances after the date hereof.

Internet Posting of Information

We routinely post information that may be important to investors in the 'For Investors' section of our website at www.sarepta.com. We encourage investors and potential investors to consult our website regularly for important information about us.

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