Sarepta Therapeutics Announces its Partner, Genethon, Published New Micro-Dystrophin Gene Therapy Data in *Nature Communications*

-- Data show for the first time a systemic therapeutic effect in DMD dogs using a rAAV2/8 micro-dystrophin gene therapy approach without immunosuppressive treatment --

CAMBRIDGE, Mass., July 27, 2017 (GLOBE NEWSWIRE) -- Sarepta Therapeutics, Inc. (NASDAQ:SRPT), a commercial-stage biopharmaceutical company focused on the discovery and development of precision genetic medicines to treat rare neuromuscular diseases, today announced the publication of data validating Genethon's micro-dystrophin gene therapy approach in an animal model for Duchenne muscular dystrophy (DMD). The results were featured in the July 25, 2017 online issue of *Nature Communications*. Sarepta announced in June 2017 that it entered into an exclusive gene therapy research collaboration with Genethon to jointly develop treatments for DMD.

"While early, these data highlight the potential for Genethon's micro-dystrophin gene therapy program and once again underscore the significance of dystrophin production in the treatment of DMD," said Douglas Ingram, Sarepta's president and chief executive officer. "As the leader in the research and development of new treatments for DMD, we are taking a multi-front approach to advancing therapies for those afflicted with this debilitating disease."

The study was conducted in 12 dogs naturally affected by DMD and treated with intravenous micro-dystrophin, a shortened version of the dystrophin protein combined with an AAV-type viral vector. At two-year follow-up, muscle function was significantly restored and clinical symptoms had stabilized. Additionally, researchers noted that dystrophin expression had returned to a high level in the high-dose group. No immunosuppressive treatment was administered beforehand, and no side-effects were observed.

Under the terms of the previously announced 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. For more information, and to see a video illustrating the results of the aforementioned study, please click here.

Le Guiner, C. et al. Long-term micro-dystrophin gene therapy is effective in a canine model of Duchenne muscular dystrophy. Nature Communications. 2017: Accepted Article, ahead of print. DOI: 10.1038/ncomms161.

About Sarepta Therapeutics

Sarepta Therapeutics is a commercial-stage biopharmaceutical company focused on the discovery and development of precision genetic medicines to treat 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 Statements

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," "in-tends," "potential," "possible" and similar expressions are intended to identify forward-looking statements. These forward-looking statements include statements regarding the potential for Genethon's micro-dystrophin gene therapy program and the significance of dystrophin production in the treatment of DMD; Sarepta being the leader in the research and development of new treatments for DMD, and taking a multi-front approach to advancing therapies for those afflicted with this debilitating disease; and the rights and obligations of Sarepta and Genethon under the research collaboration agreement.

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 Sarepta and Genethon 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 uncertain-ties 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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