



## **Sarepta Therapeutics and Genethon Announce Gene Therapy Research Collaboration for Duchenne Muscular Disease (DMD) Therapies**

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 via robust gene expression with their micro-dystrophin program in a large animal model of DMD.

Under the terms of the collaboration, Genethon will be responsible for the early development work.

Sarepta will then have 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, CEO 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](http://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.

### **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](http://www.sarepta.com). We encourage investors and potential investors to consult our website regularly for important information about us.*

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