Sarepta Therapeutics Announces Positive Safety and Efficacy Data from the SRP-9001 Micro-Dystrophin Gene Therapy Trial Published in JAMA Neurology

6/15/20

-- Results at one year demonstrate continued safety and tolerability of SRP-9001 micro-dystrophin gene therapy in four patients with Duchenne muscular dystrophy --

-- Confirmed vector transduction and functional improvements maintained through one year --

CAMBRIDGE, Mass., June 15, 2020 (GLOBE NEWSWIRE) -- Sarepta Therapeutics, Inc. (NASDAQ:SRPT), the leader in precision genetic medicine for rare diseases, today announced safety and tolerability data at one year from four Duchenne muscular dystrophy (DMD) clinical trial participants who received SRP-9001 micro-dystrophin (AAVrh74.MHCK7.micro-dystrophin) have been published in *JAMA Neurology*. SRP-9001 is an investigational gene transfer therapy intended to deliver its micro-dystrophin-encoding gene to muscle tissue for the targeted production of micro-dystrophin protein.

"We are encouraged by the successful and safe systemic delivery of our micro-dystrophin transgene from our AAVrh74 viral capsid and targeted muscle expression results, demonstrating the safety and efficacy of SRP-9001 gene transfer maintained over one year in this cohort of participants living with Duchenne muscular dystrophy," said Louise Rodino-Klapac, Ph.D., senior vice president of gene therapy, Sarepta Therapeutics. "Following the 9-month update we shared last year, the peer-reviewed publication of these results in *JAMA Neurology* further supports the potential for SRP-9001 to provide clinically meaningful functional improvements in terms of speed and magnitude of improvement for patients with DMD. Study 102, our randomized, double-blind, placebo-controlled study of SRP-9001, is ongoing and we look forward to sharing the results in early 2021 as we work toward our ultimate goal of profoundly improving the lives of as many patients living with DMD as possible."

In the open-label trial, known as Study 101, four ambulatory participants between the ages of 4 and 7 were treated with an infusion of SRP-9001 at a dose of $2x10^{14}$ vg/kg. The therapy was safe and tolerable in all participants over the one-year time period. All adverse events were considered mild or moderate, and there were no serious adverse events or evidence of complement activation. At 12 weeks, muscle dystrophin levels demonstrated a mean of 81.2% muscle fibers expressing micro-dystrophin with a mean intensity at the sarcolemma by immunohistochemistry of 96% compared to normal biopsies. Adjusted for fat and fibrotic tissue, western blot showed a mean expression of 95.8%. All participants had confirmed vector transduction and showed functional improvement on the North Star Ambulatory Assessment scale (NSAA) and reduced creatine kinase (CK) levels that were maintained through one year.

"Duchenne muscular dystrophy is difficult to treat, and more options are needed to have the potential to alter the course of the disease. We are very pleased to report successful delivery of the transgene to the nuclei corresponding to robust expression and proper localization of micro-dystrophin. This coincides with improvements in functional measurements in study participants who received SRP-9001," said Jerry Mendell, M.D., the study's co-author and principal investigator with the Center for Gene Therapy in the Abigail Wexner Research Institute at Nationwide Children's Hospital. "These results, together with biological and clinical markers of efficacy, provide proof-of-concept support for continuation of clinical trials for assessment of SRP-9001 using single-dose gene therapy in participants with Duchenne."

About SRP-9001
SRP-9001 is an investigational gene transfer therapy intended to deliver the micro-dystrophin-encoding gene to muscle tissue for the targeted production of the micro-dystrophin protein. Sarepta is responsible for global development and manufacturing for SRP-9001 and plans to commercialize SRP-9001 in the United States. In December 2019, the Company announced a licensing agreement granting Roche the exclusive right to launch and commercialize SRP-9001 outside the United States. Sarepta has exclusive rights to the micro-dystrophin gene therapy program initially developed at the Abigail Wexner Research Institute at Nationwide Children's Hospital.

About Duchenne Muscular Dystrophy
Duchenne muscular dystrophy (DMD) is a rare, fatal neuromuscular genetic disease that occurs in approximately one in every 3,500-5,000 males worldwide. DMD is caused by a change or mutation in the gene that encodes instructions for dystrophin. Symptoms of DMD usually appear in infants and toddlers. Affected children may experience developmental delays such as difficulty in walking, climbing stairs or standing from a sitting position. As the disease progresses, muscle weakness in the lower limbs spreads to the arms, neck and other areas. Most patients require full-time use of a wheelchair in their early teens, and then progressively lose the ability to independently perform activities of daily living such as using the restroom, bathing and feeding. Eventually, increasing difficulty in breathing due to respiratory muscle dysfunction requires ventilation support, and cardiac dysfunction can lead to heart failure. The condition is universally fatal, and patients usually succumb to the disease in their twenties.

About Sarepta Therapeutics
At Sarepta, we are leading a revolution in precision genetic medicine and every day is an opportunity to change the lives of people living with rare disease. The Company has built an impressive position in Duchenne muscular dystrophy (DMD) and in gene therapies for limb-girdle muscular dystrophies (LGMDs), mucopolysaccharidosis type IIIA, Charcot-Marie-Tooth (CMT), and other CNS-related disorders, with more than 40 programs in various stages of development. The Company's programs and research focus span several therapeutic modalities, including RNA, gene therapy and gene editing. For more information, please visit www.sarepta.com or follow us on Twitter, LinkedIn, Instagram and Facebook.

Sarepta Forward-Looking Statement
This press release contains "forward-looking statements." 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 regarding SRP-9001's goal to deliver its micro-dystrophin-encoding gene to muscle tissue for the targeted production of micro-dystrophin protein; SRP-9001's potential to

provide clinically meaningful functional improvements in terms of speed and magnitude of improvement for patients with DMD; our plan to share the results of Study 102 in early 2021; our goal of profoundly improving the lives of as many patients living with DMD as possible; and the potential market opportunities with respect to DMD.

These forward-looking statements involve risks and uncertainties, many of which are beyond our control. Known risk factors include, among others: success in preclinical trials and early clinical trials, especially if based on a small patient sample, does not ensure that later clinical trials will be successful; the data presented in this release may not be consistent with the final data set and analysis thereof or result in a safe or effective treatment benefit; different methodologies, assumptions and applications we utilize to assess particular safety or efficacy parameters may yield different statistical results, and even if we believe the data collected from clinical trials of our product candidates are positive, these data may not be sufficient to support approval by the FDA or foreign regulatory authorities; we may not be able to execute on our business plans and goals, including meeting our expected or planned regulatory milestones and timelines, clinical development plans, and bringing our product candidates to market, due to a variety of reasons, some of which may be outside of our control, including possible limitations of company financial and other resources, manufacturing limitations that may not be anticipated or resolved for in a timely manner, regulatory, court or agency decisions, such as decisions by the United States Patent and Trademark Office with respect to patents that cover our product candidates and the COVID-19 pandemic; and even if Sarepta's programs result in new commercialized products, Sarepta may not achieve the expected revenues from the sale of such products; and those risks identified under the heading "Risk Factors" in Sarepta's most recent Annual Report on Form 10-K for the year ended December 31, 2019, and most recent Quarterly Report on Form 10-Q filed with the Securities and Exchange Commission (SEC) as well as other SEC filings made by the Company which you are encouraged to review.

Any of the foregoing risks could materially and adversely affect the Company'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 the 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.

Source: Sarepta Therapeutics, Inc.

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