Sarepta Therapeutics Announces Positive and Robust Expression and Biomarker Data from the First Three-Patient Cohort Dosed in the MYO-101 Gene Therapy Trial to Treat Limb-Girdle Muscular Dystrophy Type 2E, or Beta-Sarcoglycanopathy

-- In two-month post-treatment muscle biopsies, clinical trial participants showed a mean of 51% beta-sarcoglycan (beta-SG) positive fibers, as measured by immunohistochemistry (IHC), substantially exceeding the pre-defined 20% measure for success --

-- Robust expression was also quantified by Western Blot and via intensity on IHC --

-- 90% mean creatine kinase (CK) reduction from baseline --

-- Participants received a dose of $5x10^{13}$ vg/kg --

CAMBRIDGE, Mass., February 27, 2019 (GLOBE NEWSWIRE) – Sarepta Therapeutics, Inc. (NASDAQ:SRPT), the leader in precision genetic medicine for rare diseases, today announced positive results from three Limb-girdle muscular dystrophy (LGMD) Type 2E clinical trial participants who received MYO-101. MYO-101 is a novel gene therapy intended to transduce skeletal and cardiac muscle with a gene that codes for the full-length, native beta-SG protein, the lack of which causes LGMD2E. An autosomal recessive muscular dystrophy, persons with LGMD2E begin showing neuromuscular symptoms such as difficulty running, jumping and climbing stairs before age 10. The disease progresses to loss of ambulation in the teen years, and often leads to death before age 30. There is currently no treatment or cure for LGMD2E.

In Cohort 1 of the MYO-101 study, three participants ages 4-13, were treated with an infusion of MYO-101 at a dose of $5x10^{13}$vg/kg, with post-treatment biopsies taken at approximately two months. Preliminary results are as follows:

- All three participants in the study showed robust expression of transduced beta-SG, properly localized to the muscle sarcolemma, as measured by IHC. The pre-defined measure of success for expression in the study was 20% positive fibers. Actual mean protein expression, properly localized to the sarcolemma of the muscle, was 51%.
- Mean fiber intensity, as measured by IHC, was 47% compared to normal control.

- All participants showed robust quantification of beta-SG, as measured by Western blot, with mean beta-SG of 36.1% of normal control.
- All participants showed a striking decrease in serum creatine kinase (CK) levels from pre-treatment baseline measure to last measure, with a mean CK reduction of more than 90% from baseline. CK is an enzyme biomarker strongly associated with muscle damage.
- Two participants had elevated liver enzymes, one of which was designated a serious adverse event (SAE), as the patient had associated transient increase in bilirubin. Both events occurred when the participants were tapered off oral steroids and, in both instances, elevated liver enzymes returned to baseline and symptoms resolved quickly following supplemental steroid treatment. There were no other clinically significant laboratory findings and no decreases in platelet counts were observed.

"LGMD2E is a devastating neuromuscular disease, currently lacking any treatment options," said Jerry Mendell, M.D., Curran-Peters Chair of Pediatric Research at Nationwide Children's Hospital and lead investigator for the study. "Results in our first three clinical trial participants are consistent with what we have observed in preclinical models. We look forward to continuing this pivotal trial focused on development of MYO-101 for LGMD2E."

"The positive results in our first MYO-101 cohort strengthen our resolve to build out our gene therapy engine with speed and purpose," said Doug Ingram, Sarepta's president and chief executive officer. "Our gene therapy constructs have now produced high levels of expression of the missing protein of interest, and strong results in related biomarkers, in Duchenne and LGMD2E, both cruel, fatal genetic diseases. And these results have potential read through to our other 4 LGMD programs and further validate our gene therapy approach. Our success will come from the talent of our colleagues and our collaboration with the industry's best and brightest. In that vein, I would like to thank Dr. Jerry Mendell of Nationwide Children's Hospital for his hard work, ingenuity and extraordinary commitment to those living with rare neuromuscular disease."

About MYO-101 and the Phase I/IIa Gene Transfer Clinical Trial

MYO-101 uses the AAVrh74 vector, which is designed to be systemically and robustly delivered to skeletal, diaphragm and cardiac muscle without promiscuously crossing the blood brain barrier, making it an ideal candidate to treat peripheral neuromuscular diseases. As a rhesus monkey-derived AAV vector, AAVrh74

has lower immunogenicity rates than reported with other common human AAV vectors. The MHCK7 promoter has been chosen for its ability to robustly express in the heart, which is critically important for patients with LGMD2E, many of whom die from pulmonary or cardiac complications.

This first-in-human study is evaluating a single intravenous infusion of MYO-101 among children with LGMD2E between the ages of four and 15 years with significant symptoms of disease.

About Limb-Girdle Muscular Dystrophy

Limb girdle muscular dystrophies are genetic diseases that cause progressive, debilitating weakness and wasting that begins in muscles around the hips and shoulders before progressing to muscles in the arms and legs. Sarepta has five LGMD gene therapy programs in development, including LGMD2E, LGMD2D, LGMD2C, LGMD2B and LGMD2L.

About Sarepta Therapeutics

Sarepta is at the forefront of precision genetic medicine, having built an impressive and competitive position in Duchenne muscular dystrophy (DMD) and more recently in gene therapies for 5 Limb-girdle muscular dystrophy diseases (LGMD), Charcot-Marie-Tooth (CMT), MPS IIIA, Pompe and other CNS-related disorders, totaling over 20 therapies in various stages of development. The Company's programs and research focus span several therapeutic modalities, including RNA, gene therapy and gene editing. Sarepta is fueled by an audacious but important mission: to profoundly improve and extend the lives of patients with rare genetic-based diseases. For more information, please visit www.sarepta.com.

Forward-Looking Statements

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 the potential of MYO-101 to transduce skeletal and cardiac muscle with a gene that codes for the precise beta-SG protein; the results' potential read through to our other 4 LGMD programs; and Sarepta's mission to profoundly improve and extend the lives of patients with rare genetic-based diseases.

These forward-looking statements involve risks and uncertainties, many of which are beyond Sarepta's control. Known risk factors include, among others: success in preclinical testing and early clinical trials, especially if based on a small patient sample, does not ensure that later clinical trials will be successful, and initial results from a clinical trial do not necessarily predict final results; 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; Sarepta's ongoing research and development efforts may not result in any viable treatments suitable for clinical research or commercialization due to a variety of reasons, some of which may be outside of Sarepta's control, including possible limitations of company financial and other resources, manufacturing limitations that may not be anticipated or resolved for in a timely manner, and 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 even if Sarepta's programs result in new commercialized products, Sarepta may not achieve any significant 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, 2017, 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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