**Sarepta Therapeutics Announces Positive Functional Results from the SRP-9003 (MYO-101) Gene Therapy Trial to Treat Limb-Girdle Muscular Dystrophy Type 2E, or Beta-Sarcoglycanopathy**

-- Improvements on functional measures seen in all three participants --

-- Significant reduction in creatine kinase maintained over nine months --

-- Results follow positive and robust expression and biomarker data presented earlier in 2019 --

CAMBRIDGE, Mass., Oct. 4, 2019 (GLOBE NEWSWIRE) – Sarepta Therapeutics, Inc. (NASDAQ:SRPT), the leader in precision genetic medicine for rare diseases, today announced the nine-month functional results from three Limb-girdle muscular dystrophy Type 2E (LGMD2E) clinical trial participants who received SRP-9003. SRP-9003 is an investigational gene therapy intended to transduce skeletal and cardiac muscle with a gene that codes for the full-length, native beta-sarcoglycan protein, the lack of which is the sole cause of LGMD2E.

In Cohort 1 of the SRP-9003 study, three participants ages 4-13 were treated with an infusion of SRP-9003 at a dose of $5x10^{13}$vg/kg. Improvements in functional outcomes were observed at day 270 (nine months) for all three participants.

“We have now observed consistent functional improvements, in addition to high levels of expression of the missing protein of interest and strong results in related biomarkers, in both of our first cohorts for Duchenne muscular dystrophy (SRP-9001) and LGMD2E (SRP-9003). We intend to test one higher dose of SRP-9003 in LGMD2E participants, select our clinical dose and then advance our SRP-9003 program, along with our other five LGMD programs, as rapidly as possible,” said Doug Ingram, Sarepta’s president and chief executive officer. “With the results of our first LGMD2E cohort, Sarepta continues to build its gene therapy engine, an enduring model created to design, develop and bring to the medical and patient community transformative therapies for those living with, and too often dying from, rare genetic disease.”

At Day 270, mean creatine kinase (CK) was significantly reduced compared to baseline. CK is an enzyme biomarker strongly associated with muscle damage.

At Day 270, all three participants showed improvements from baseline across all functional measures, including the North Star Assessment for Dysferlinopathy (NSAD), time to rise, four-stair climb, 100-m walk test and 10-meter walk test. These results are distinctly different from what an age-matched, natural history group would predict.

No new safety signals were observed and the safety profile seen to date supports the ability to dose escalate in the next cohort of the study. As previously disclosed, two participants in the study had elevated liver enzymes, one of which was designated a serious adverse event (SAE), as the participant 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 following supplemental steroid treatment.

"LGMD2E is a devastating neuromuscular disease with no current treatment options so we are very pleased to observe a functional improvement in study participants who received SRP-9003," said Jerry Mendell, M.D., principal investigator at the Center for Gene Therapy in the Abigail Wexner Research Institute at Nationwide Children's Hospital and lead investigator for the study.

Sarepta had previously shared expression results from the study, which found that in two-month post-treatment muscle biopsies, clinical trial participants showed a mean of 51% beta-sarcoglycan positive fibers, as measured by immunohistochemistry (IHC), substantially exceeding the pre-defined 20% measure for success. Mean fiber intensity, as measured by IHC, was 47% compared to normal control.

**About SRP-9003 and the Phase I/IIa Gene Transfer Clinical Trial**

SRP-9003 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 SRP-9003 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 begin in muscles around the hips and shoulders before progressing to muscles in the arms and legs.

Patients with LGMD2E begin showing neuromuscular symptoms such as difficulty running, jumping and climbing stairs before age 10. The disease, which is an autosomal recessive subtype of LGMD, 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.

Sarepta has five LGMD gene therapy programs in development, including subtypes for LGMD2E, LGMD2D, LGMD2C, LGMD2B and LGMD2L, and holds an option for a sixth program for LGMD2A.

**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 Limb-girdle muscular dystrophy diseases (LGMD), 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 our intention to test one higher dose of SRP-9003 in LGMD2E participants, select our clinical dose and then advance our SRP-9003 program, along with our other five LGMD programs, as rapidly as possible; Sarepta continuing to build an enduring gene therapy model created to design, develop and bring to the medical and patient community transformative therapies for those living with rare genetic disease; the safety profile of SRP-9003 seen to date supporting the ability to dose escalate in the next cohort of the study; SRP-9003 being an ideal candidate to treat peripheral neuromuscular diseases; the potential benefits of the AAVrh74 vector and the MHCK7 promoter; and our 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; different methodologies, assumptions and applications Sarepta utilizes to assess particular safety or efficacy parameters may yield different statistical results, and even if Sarepta believes the data collected from clinical trials of its product candidates are positive, these data may not be sufficient to support approval by the FDA or foreign regulatory authorities; 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, 2018, 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.*

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