Sarepta Therapeutics Announces that at the 23rd International Congress of the World Muscle Society, Jerry Mendell, M.D., Presented Positive Updated Results from the Four Children Dosed in the Gene Therapy Micro-dystrophin Trial to Treat Patients with Duchenne Muscular Dystrophy

-- Biopsy of fourth patient showed robust micro-dystrophin expression as measured by Western blot and immunohistochemistry --

-- Positive functional improvements shown across all measures --

-- No serious adverse events (SAEs) observed --

CAMBRIDGE, Mass., October 3, 2018 (GLOBE NEWSWIRE) -- Sarepta Therapeutics, Inc. (NASDAQ:SRPT), a commercial-stage biopharmaceutical company focused on the discovery and development of precision genetic medicine to treat rare neuromuscular diseases, announced that at the 23rd International Congress of the World Muscle Society in Mendoza, Argentina, Jerry Mendell, M.D., of Nationwide Children's Hospital presented positive updated results from its gene therapy clinical trial assessing AAVrh74.MHCK7.micro-Dystrophin in individuals with Duchenne muscular dystrophy (DMD). Dr. Mendell presented the following updated data on the four patients enrolled in the study:

- All patients showed robust expression of transduced micro-dystrophin, which is properly localized to the muscle sarcolemma, as measured by immunohistochemistry. Mean gene expression for the study, as measured by percentage of micro-dystrophin positive fibers was 81.2% and the mean intensity of the fibers was 96.0% compared to normal control. All post-treatment biopsies showed robust levels of micro-dystrophin as measured by Western blot, with a mean of 74.3% compared to normal utilizing Sarepta's method, or 95.8% compared to normal pursuant to Nationwide Children's quantification of Sarepta's method that adjusts for fat and fibrotic tissue.
- Gene expression for the fourth patient was robust, as follows:
    - As measured by immunohistochemistry, micro-dystrophin positive fibers was 96.2% and the mean intensity of the fibers was 160.0% compared to normal control.
    - As measured by Western blot, patient 4 showed robust levels of micro-dystrophin, with a mean of 182.7% compared to normal utilizing Sarepta's method, or 222% compared to

normal pursuant to Nationwide Children's quantification of Sarepta's method that adjusts for fat and fibrotic tissue.

- In all patients, expression of micro-dystrophin was associated with significant expression and up-regulation of the dystrophin-associated protein complex, an additional indication of functionality of dystrophin.
- All patients showed significant decreases of serum creatine kinase (CK) levels at last measure, with a mean reduction of CK of over 78% from baseline.
- Dr. Mendell also provided an update on functional endpoints for all four patients, including North Star Ambulatory Assessment (NSAA), Time to Rise, 4 Stairs Up, and 100M. Patients showed improvements across all measured functions, with boys showing an average NSAA raw score improvement of 6.5 points from baseline to last measure, or on a linearized NSAA basis, 12 points of improvement in the first 90 days. While results suggest functional improvements across all measures significantly greater than natural history predictions, it should be cautioned that this is a small, uncontrolled data set and these positive results must be reconfirmed in the larger, controlled registration trial.
- No serious adverse events (SAEs) were observed in the study. Three patients had elevated gamma-glutamyl transferase (GGT) that resolved with increased steroids within a week. There were no other significant laboratory findings. Patients had transient nausea generally during the first week of therapy coincident with increased steroid dosing.

Dr. Mendell, the study's principal investigator, in collaboration with Louise Rodino-Klapac, Ph.D., empirically optimized the AAVrh74.MHCK7 specifically for DMD:

- The AAVrh74 vector 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 DMD, who typically die from pulmonary or cardiac complications. In pre-clinical models, micro-dystrophin expression in the heart was observed to be up to 120% of the micro-dystrophin levels observed in skeletal muscles.

- The transgene was designed to maintain spectrin-like repeats 2 and 3, which has been reported to be critical to maintaining the protective functional characteristics of dystrophin.

Dr. Mendell stated, “The goal of this study was to validate what we observed in pre-clinical models. We observed efficient transduction of our vector, AAVrh74, to all muscle types; robust expression in skeletal muscles via the MHCK7 promoter; a reduction in creatine kinase levels; and a favorable safety profile. Similar to pre-clinical models, we also observed in this early study that robust expression has the potential to positively impact the natural course of disease progression.”

Doug Ingram, Sarepta’s president and chief executive officer, added, “The encouraging results that we previously saw and reinforced in the fourth patient strengthen our resolve to rapidly move to a confirming trial and, assuming successful, to bring this therapy to the Duchenne community around the world with a sense of urgency.”

Mr. Ingram continued, “These results create for us an obligation to patients around the globe living with and being damaged by this cruel disease. We are investing our energy, resources and creativity to moving the development forward, planning meetings with the FDA and other agencies around the world to take their input, building a compelling access and reimbursement package, and establishing sufficient manufacturing capacity to fully serve the community if our program is successful.”

About Sarepta Therapeutics

Sarepta Therapeutics is a commercial-stage biopharmaceutical company focused on the discovery and development of precision genetic medicine 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.

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 results suggesting functional improvements across all measures significantly greater than natural history predictions; the potential of the AAVrh74 vector to systemically and robustly

be delivered to skeletal, diaphragm and cardiac muscle without promiscuously crossing the blood brain barrier, making it an ideal candidate to treat peripheral neuromuscular diseases; the ability of the MHCK7 promoter to robustly express in the heart; the potential of the transgene to maintain spectrin-like repeats 2 and 3; the potential of robust micro-dystrophin expression to positively impact the natural course of DMS progression; Sarepta's intention to rapidly move the micro-dystrophin gene therapy program to a confirming trial, and, if successful, to bring this therapy to the Duchenne community around the world with a sense of urgency; and Sarepta's plans to move the development forward, meet with the FDA and other agencies around the world to take their input, build a compelling access and reimbursement package, and establish sufficient manufacturing capacity to fully serve the community if the program is successful.

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; 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 promising, 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, 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 Sarepta's 2017 Annual Report on Form 10-K and most recent Quarterly Report on Form 10-Q filed with the 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 web-site at www.sarepta.com. We encourage investors and potential investors to consult our website regularly for important information about us.

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