Sarepta Therapeutics Announces that at its First R&D Day, Jerry Mendell, M.D. Presented Positive Preliminary Results from the First Three Children Dosed in the Phase 1/2a Gene Therapy Micro-dystrophin Trial to Treat Patients with Duchenne Muscular Dystrophy

-- Biopsies performed at Day 90 showed robust micro-dystrophin expression in muscle measured by all methods and observed in all three patients --

-- Significant decrease in levels of serum creatine kinase (CK), an enzyme biomarker strongly associated with muscle damage caused by Duchenne muscular dystrophy --

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

CAMBRIDGE, Mass., June 19, 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 Company's R&D Day, Jerry Mendell, M.D. of Nationwide Children's Hospital presented positive preliminary results from its Phase 1/2a gene therapy clinical trial assessing AAVrh74.MHCK7.micro-Dystrophin in individuals with Duchenne muscular dystrophy (DMD). Dr. Mendell presented the following preliminary data on the first three 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, as measured by percentage of micro-dystrophin positive fibers was 76.2% and the mean intensity of the fibers was 74.5% compared to normal control.
- All post-treatment biopsies showed robust levels of micro-dystrophin as measured by Western blot, with a mean of 38.2% compared to normal utilizing Sarepta's method, or 53.7% compared to normal pursuant to Nationwide Children's quantification of Sarepta's method that adjusts for fat and fibrotic tissue.
- A mean of 1.6 vector copies per cell nucleus was measured in patients, consistent with the high micro-dystrophin expression levels observed.
- All patients showed significant decreases of serum creatine kinase (CK) levels, with a mean reduction of CK of over 87% at Day 60. CK is an enzyme associated with muscle damage and patients

with DMD uniformly exhibit high levels of CK. Indeed, significantly elevated CK is often used as a preliminary diagnosis tool for DMD, which is then followed by confirmatory genetic testing.

- No serious adverse events (SAEs) were observed in the study. Two patients had elevated gamma-glutamyl transferase (GGT) that resolved with increased steroids within a week and returned to baseline levels. 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 can 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 neuromuscular diseases.
- As a rhesus monkey-derived AAV vector, AAVrh74 appears to show lower immunogenicity rates in existing early-stage clinical studies than expected with other 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 preclinical 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 important for maintaining the protective functional characteristics of dystrophin.

"As a genetic medicine company, our goal is to work with the world's leading clinicians and scientists to advance scientific discoveries to the clinic and, ultimately, to therapies that profoundly improve and extend the lives of those living with Duchenne muscular dystrophy and other rare, fatal diseases," stated Doug Ingram, Sarepta's president and chief executive officer. "Since the discovery of the dystrophin gene in 1986, scientists, clinicians, patient advocates and the biotech ecosystem have tirelessly searched for ways to restore or replace dystrophin and rescue boys with DMD from the damage and early death. Dr. Mendell's results, if confirmed in additional patients, studies, measures and time points, represent a monumental leap forward in the direction of our goal."

Dr. Mendell added, "I have been waiting my entire 49-year career to find a therapy that dramatically reduces CK levels and creates significant levels of dystrophin. Although the data are early and preliminary,

these results, if they persist and are confirmed in additional patients, will represent an unprecedented advancement in the treatment of DMD. I look forward to treating more patients in the clinical study to generate the data necessary to bring this therapy to patients with DMD, with the goal of dramatically changing the course of the disease."

"For years, PPMD has been interested in the potential of gene therapy as a treatment for Duchenne. At a critical moment in development in early 2017 – with the help and support of our amazing community – we were thrilled to be able to fund this important project of Drs. Mendell and Rodino-Klapac. To have reached this moment today is incredible and we are grateful to Sarepta for their investment and partnership in moving this therapeutic approach forward. While these are early days and work remains to fully understand the full potential of gene therapies, these first signals are encouraging. We remain hopeful that this will lead to a viable treatment for Duchenne," stated Pat Furlong, Parent Project Muscular Dystrophy's (PPMD) founding president and chief executive officer.

PPMD committed $2.2 million to the trial, with support from additional Duchenne foundations and families.

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 design and potential benefits of the AAVrh74 vector, including its ability to systemically and robustly being delivered to skeletal, diaphragm and cardiac muscle without promiscuously crossing the blood brain barrier and its potential to show lower immunogenicity rates; the ability of the MHCK7 promoter to robustly express in the heart; the potential of the transgene to maintain the protective functional characteristics of dystrophin; Sarepta's goal to work with the world's leading clinicians and scientists to advance scientific discoveries to the clinic and, ultimately, to therapies that profoundly improve

and extend the lives of those living with DMD and other rare, fatal diseases; and the potential of Dr. Mendell's results to represent a monumental leap forward in the direction of Sarepta's goal, an unprecedented advancement in the treatment of DMD and dramatically change the course of the disease.

These forward-looking statements involve risks and uncertainties, many of which are beyond Sarepta's control. Actual results could materially differ from those stated or implied by these forward-looking statements as a result of such risks and uncertainties. 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; Sarepta's ongoing research and development efforts may not result in any viable treatments suitable for commercialization due to a variety of reasons, some of which may be outside of Sarepta's control, including the results of future research may not be consistent with past positive results or may fail to meet regulatory approval requirements for the safety and efficacy of product candidates, 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 Sarepta's 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. 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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