Sarepta Therapeutics and Nationwide Children's Hospital Announce FDA Clearance of IND for Micro-Dystrophin Gene Therapy Program for the Treatment of Duchenne Muscular Dystrophy

-- Nationwide Children's Hospital to initiate a Phase 1/2a clinical trial at potentially therapeutic doses in children with DMD by mid-November 2017 --

-- First micro-dystrophin gene therapy program in clinical development for DMD --

CAMBRIDGE, Mass., November 6, 2017 (GLOBE NEWSWIRE) -- Sarepta Therapeutics, Inc. (NASDAQ: SRPT), a commercial-stage biopharmaceutical company focused on the discovery and development of precision genetic medicines to treat rare neuromuscular diseases, today announced that Nationwide Children's Hospital has received clearance from the U.S. Food and Drug Administration (FDA) for its Investigational New Drug (IND) application for the micro-dystrophin gene therapy program. The Phase 1/2a clinical trial, now open to enrollment and scheduled for initiation by mid-November, is designed to assess the safety and tolerability of AAVrh74.MHCK7.micro-Dystrophin in individuals with Duchenne muscular dystrophy (DMD). The program is being conducted pursuant to a sponsored research agreement with Sarepta, who has an option to worldwide exclusive rights.

The clinical trial will be conducted at Nationwide Children's with Jerry Mendell, M.D. and Louise Rodino-Klapac, Ph.D. as the principal investigators. The first patient in the clinical trial is anticipated to be dosed at potentially therapeutic levels in mid-November 2017. Patients with mutations between exons 18 and 58, or approximately 60 to 70 percent of the DMD population, are potential candidates for this treatment. The program uses the MHCK7 construct, which was optimized for this trial by Drs. Mendell and Rodino-Klapac to enhance the activity in DMD patients. The construct can be systemically delivered to skeletal, diaphragm and cardiac muscle, and has shown high levels of gene expression in preclinical studies. Patients will receive a biopsy at three months to measure micro-dystrophin expression.

Parent Project Muscular Dystrophy (PPMD) committed 2.2 million dollars to the trial, with support from additional Duchenne foundations and families. The grant was a key component to help expand and accelerate this opportunity.

“As the first systemic micro-dystrophin gene therapy program enters the clinic, we mark an important milestone in our journey to relentlessly pursue new therapies to treat DMD,” said Douglas Ingram, Sarepta’s president and chief executive officer. “This gene therapy, which is agnostic to genetic mutation and will be studied at potentially therapeutic doses in patients, exemplifies Sarepta’s unwavering commitment to partner with science’s best and brightest researchers to rapidly discover and develop new precision genetic medicines that may profoundly improve the lives of children suffering from this cruel disease, DMD. As we continue to develop the most comprehensive approaches to treating DMD, Dr. Mendell, Dr. Rodino-Klapac and Nationwide Children’s deep expertise in gene therapy will be instrumental to our mission.”

“This will be the first clinical trial for DMD to treat patients as young as three months old and is an important step forward in our quest to diagnose patients through newborn screenings,” said Dr. Mendell, leader of the neuromuscular gene therapy program at The Research Institute at Nationwide Children’s Hospital. “We are very encouraged by the promising preclinical results and are eager to complete enrollment. Sarepta’s unwavering commitment to develop disease-modifying therapies makes them an ideal partner and together we will continue to make significant strides to achieve what is possible to treat the underlying cause of DMD.”

“With this clinical trial, we are laser-focused on giving each patient the best possible chance of a successful outcome,” said Dr. Rodino-Klapac, principal investigator at the Center for Gene Therapy in The Research Institute at Nationwide Children’s Hospital. “We are taking a novel approach to trial design. The use of potentially therapeutic doses is a critical part of our mission to arm our patients for their one shot at gene therapy.”

“Parent Project Muscular Dystrophy is thrilled by the continued momentum of Nationwide Children’s micro-dystrophin gene therapy program and we are grateful for Sarepta’s support of this revolutionary approach to treat Duchenne. PPMD has long supported innovative approaches to slowing the progression of Duchenne and we could not be more pleased with the work of Drs. Mendell and Rodino-Klapac. This kind of collaboration amongst researchers, industry and patient advocacy groups has the potential to help us achieve our mission to end Duchenne,” said Pat Furlong, founding president and chief executive officer of Parent Project Muscular Dystrophy.

DMD is a fatal genetic neuromuscular disorder affecting an estimated one in approximately every 3,500 - 5,000 males born worldwide.

About the Phase 1/2a Clinical Trial

The Phase 1/2a clinical trial is a pilot study to evaluate safety, biological activity, and efficacy of the rAA-Vrh74.MHCK7.micro-Dystrophin vector administered through the circulation through an intravenous catheter in the arm. This study will evaluate the micro-dystrophin vector for its capacity to replace the missing muscle dystrophin in Duchenne muscular dystrophy. Twelve patients in two cohorts will undergo gene transfer to establish maximum tolerated dose (MTD) avoiding toxicity. Cohort 1 will include infants 3 months to 3 years of age with DMD. Cohort 2 will include DMD boys 4 years old to 7 years old. Patients will be enrolled sequentially starting with Cohort 2. All boys in this clinical trial will receive a total dose of 2E14 vg/kg.

About The Research Institute at Nationwide Children's Hospital

Named to the Top 10 Honor Roll on U.S. News & World Report's 2016-17 list of "America's Best Children's Hospitals," Nationwide Children's Hospital is one of America's largest not-for-profit freestanding pediatric healthcare systems providing wellness, preventive, diagnostic, treatment and rehabilitative care for infants, children and adolescents, as well as adult patients with congenital disease. As home to the Department of Pediatrics of The Ohio State University College of Medicine, Nationwide Children's faculty train the next generation of pediatricians, scientists and pediatric specialists. The Research Institute at Nationwide Children's Hospital is one of the Top 10 National Institutes of Health-funded free-standing pediatric research facilities in the U.S., supporting basic, clinical, translational and health services research at Nationwide Children's. The Research Institute encompasses three research facilities totaling 525,000 square feet dedicated to research. More information is available at www.nationwidechildrens.org/research.

About Sarepta Therapeutics

Sarepta Therapeutics is a commercial-stage biopharmaceutical company focused on the discovery and development of precision genetic medicines 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 Nationwide Children's Hospital's plan to initiate a Phase 1/2a clinical trial at potentially therapeutic doses in children with DMD by mid-November 2017 and dose the first patient at potentially therapeutic levels in mid-November 2017; the study design; the potential candidates for this treatment; Sarepta's goal to relentlessly pursue new therapies to treat DMD; this gene therapy exemplifying Sarepta's unwavering commitment to partner with science's best and brightest researchers to rapidly discover and develop new precision genetic medicines that may profoundly improve the lives of children suffering from DMD; Sarepta's plan to continue developing the most comprehensive approaches to treating DMD; Dr. Mendell, Dr. Rodino-Klapac and Nationwide Children's deep expertise in gene therapy being instrumental to Sarepta's mission; the clinical trial treating patients as young as three months old and being an important step forward in Sarepta's quest to diagnose patients through newborn screenings; Sarepta's unwavering commitment to develop disease-modifying therapies; Sarepta and Nationwide Children's plan to continue to make significant strides to achieve what is possible to treat the underlying cause of DMD; Nationwide Children's focus on giving each patient the best possible chance of a successful outcome and its mission to arm its patients for their one shot at gene therapy; and the collaboration amongst researchers, industry and patient advocacy groups having the potential to help achieve PPMD's mission to end DMD.

These forward-looking statements involve risks and uncertainties, many of which are beyond Sarepta's control. Known risk factors include, among others: the expected benefits and opportunities related to the collaboration with Nationwide Children's may not be realized or may take longer to realize than expected due to challenges and uncertainties inherent in product research and development; the collaboration with Nationwide Children's may not result in any viable treatments suitable for commercialization due to a variety of reasons 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 or may never become commercialized products due to other various reasons including any potential future inability of the parties to fulfill their commitments and obligations under the agreement, including any inability by Sarepta to fulfill its financial commitments to Nationwide Children's; and even if the collaboration results in commercialized products, the parties 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, 2016 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 2016 Annual Report on Form 10-K and most recent Quarterly Report on Form 10-Q for the quarter ended September 30, 2017 filed with the Securities and Exchange Commission (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 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.

Media and Investors:
Sarepta Therapeutics, Inc.
Ian Estepan, 617-274-4052
iestepan@sarepta.com
or
W2O Group
Brian Reid, 212-257-6725
breid@w2ogroup.com