Sarepta Therapeutics and Nationwide Children's Hospital Announce U.S. Food and Drug Administration (FDA) Clearance of the IND Application for the GALGT2 Gene Therapy Program

-- Nationwide Children's Hospital is on track to initiate a Phase 1/2a clinical trial in individuals with Duchenne muscular dystrophy (DMD) by year-end 2017 --

CAMBRIDGE, Mass., November 3, 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, and Nationwide Children's Hospital today announced that the Investigational New Drug (IND) application for the GALGT2 gene therapy program was cleared by the FDA. Nationwide Children's is on track to initiate the Phase 1/2a clinical trial in individuals with DMD by year-end 2017.

Kevin Flanigan, M.D., director of Nationwide Children's Center for Gene Therapy, is the principal investigator for the GALGT2 program, which was developed by Paul Martin, Ph.D., a principal investigator at the Center. The program is exploring a potential surrogate gene therapy approach to treat DMD, targeting the dystroglycan complex to preserve muscle function. In animal models, overexpression of GALGT2 results in muscle that function normally even in the absence of dystrophin.

"The field of gene therapy represents a potentially transformative approach to the treatment of Duchenne muscular dystrophy," said Douglas Ingram, Sarepta's president and chief executive officer. "We are very proud to be partnered with gene therapy luminaries Drs. Flanigan and Martin as they rapidly advance our partnered GALGT2 program and begin to treat DMD patients this year."

Mr. Ingram continued, "The GALGT2 program offers the potential to treat the majority of dystrophin gene mutations responsible for this devastating disease, DMD, as well as having potential utility in other muscular dystrophies, and perfectly exemplifies our strategy of working with urgency to find and progress

potentially life changing new treatments for DMD through agnostically investing in precision genetic medicine."

DMD, an incurable muscle-wasting disease, afflicts an estimated one in approximately every 3,500 - 5,000 males born worldwide.

"This approach represents a potential new pathway to treat the root cause of DMD. We look forward to continuing to collaborate with Sarepta, whose dedication to DMD, to the individuals impacted by the disease, and to rigorous scientific exploration, matches our own here at Nationwide Children's Hospital," said Dr. Flanigan. "This program would not have been possible without the generous support of the NIH and patient foundations, including Team Joseph, the Little Hercules Foundation, Walking Strong, Charley's Fund, JB's Keys, and Hope for Gus. We would also like to take this opportunity to extend our appreciation to those patient groups who will continue to help move this important program forward."

In January 2017, Sarepta announced a license agreement with Nationwide Children's for the GALGT2 gene therapy program.

About the GALGT2 Phase 1/2a Clinical Study

The study will enroll at least 6 subjects with DMD who will receive rAAVrh74.MCK.GALGT2 by direct injection into the femoral arteries of the legs, beginning with a dose that has shown robust gene expression in preclinical studies. In order to measure gene expression, which is the primary outcome measure, open muscle biopsies will be performed at baseline and at three months, and needle muscle biopsies will be performed at six and twelve months. Motor performance will be assessed using several tools, including the 6 minute walk test, the North Star Ambulatory Assessment, and the time to arise from the floor.

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 Nationwide Children's Hospital being on track to initiate the Phase 1/2a clinical trial in individuals with DMD by year-end 2017; the study design; the potential surrogate gene therapy approach to treat DMD; the field of gene therapy representing a potentially transformative approach to the treatment of DMD; Drs. Flanigan and Martin rapidly advancing the GALGT2 program and beginning to treat DMD patients this year; the GALGT2 program offering the potential to treat the majority of dystrophin gene mutations responsible for DMD, as well as having potential utility in other muscular dystrophies, and perfectly exemplifying Sarepta's strategy of working with urgency to find and progress potentially life changing new treatments for DMD through agnostically investing in precision genetic medicine; gene therapy representing a potential new pathway to treat the root cause of DMD; and Sarepta and Nationwide Children's dedication to the individuals impacted by DMD, and rigorous scientific exploration.

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.

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