Sarepta Announces Agreement with Nationwide Children's Hospital for Rights to its Gene Therapy Program to Treat Limb-Girdle Muscular Dystrophy Type 2A, the Most Common Form of Limb-Girdle Muscular Dystrophy

--Expands pipeline with 6th investigational gene therapy for Limb-girdle muscular dystrophy--

--Furthers the Company's commitment to finding treatments for neuromuscular conditions--

CAMBRIDGE, Mass., May 8, 2019 (GLOBE NEWSWIRE) -- Sarepta Therapeutics, Inc. (NASDAQ:SRPT), the leader in precision genetic medicine for rare diseases, today announced it has recently signed an agreement with the Research Institute at Nationwide Children's Hospital (Nationwide Children's) giving Sarepta the exclusive option to a Nationwide Children's gene therapy candidate, calpain 3 (CAPN-3), to treat Limb-girdle muscular dystrophy type 2A (LGMD2A).

LGMDs represent a group of distinct genetic neuromuscular diseases with a generally common set of symptoms, including progressive, debilitating weakness and wasting that begins in muscles around the hips and shoulders before progressing to muscles in the arms and legs. Many LGMD sub-types are seriously life-limiting and often life-ending diseases. Also known as calpainopathy, LGMD2A is caused by mutations in the CAPN-3 gene and is the most common type of LGMD, accounting for almost a third of cases.

Like Sarepta's micro-dystrophin and five other LGMD programs, the LGMD2A program employs the AAVrh74 vector, designed to systematically and robustly deliver treatment to skeletal muscle, including the diaphragm, without promiscuously crossing the blood brain barrier, making it an ideal candidate to treat muscle disease.

The CAPN-3 program is currently in pre-clinical trials. The program is led by Zarife Sahenk, M.D., Ph.D., an attending neurologist at Nationwide Children's, Director of Clinical and Experimental Neuromuscular Pathology at The Research Institute at Nationwide Children's and Professor of Pediatrics, Pathology and Neurology at The Ohio State University College of Medicine.

"We are pleased to expand and deepen our working relationship with Nationwide Children's and Dr. Sahenk, with whom we are already working on a gene therapy candidate to treat Charcot-Marie-Tooth. With six LGMD gene therapy programs now in our portfolio, our commitment and investment in

research for this group of neuromuscular diseases is unparalleled," said Doug Ingram, Sarepta's president and chief executive officer. "Recent positive early results from our LGMD2E program support expanding our development strategy to LGMD2A, as both programs utilize AAVrh74 vector, address sub-populations of LGMD, and address a well-characterized disease by directly replacing the missing protein which is the cause of the disease by transducing the native protein. We continue to fuel our gene therapy development engine aimed at building an enduring model that delivers potentially transformative therapies to treat genetically based diseases."

"LGMD2A is the most common form of limb-girdle muscular dystrophy and its relentless progression causes patients to lose the ability to walk in early adulthood," said Dr. Zahenk. "Our preclinical work suggests that a gene therapy approach has the potential to help those living with LGMD2A and we look forward to collaborating with Sarepta to advance this program in the clinic."

About Limb-Girdle Muscular Dystrophy

Limb-girdle muscular dystrophies are genetic diseases that cause progressive, debilitating weakness and wasting that begins in muscles around the hips and shoulders before progressing to muscles in the arms and legs. Sarepta's six LGMD gene therapy programs in development now include LGMD2E, LGMD2D, LGMD2C, LGMD2B, LGMD2L and 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), Charcot-Marie-Tooth (CMT), 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.

About Nationwide Children's Hospital

Named to the Top 10 Honor Roll on *U.S. News & World Report*'s 2018-19 list of "Best Children's Hospitals," Nationwide Children's Hospital is one of America's largest not-for-profit freestanding pediatric health care systems providing wellness, preventive, diagnostic, treatment and rehabilitative care for infants, children and adolescents, as well as adult patients with congenital disease. Nationwide Children's has a staff of more than 13,000 providing state-of-the-art pediatric care during more than 1.4 million patient visits annually. As home to the Department of Pediatrics of The Ohio State University College of Medicine, Nationwide Children's physicians train the next generation of pediatricians and

pediatric specialists. The Research Institute at Nationwide Children's Hospital is one of the Top 10 National Institutes of Health-funded freestanding pediatric research facilities. More information is available at NationwideChildrens.org.

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 potential benefits of the agreement with Nationwide Children's giving Sarepta the exclusive option to a Nationwide Children's gene therapy candidate, calpain 3 (CAPN-3), to treat LGMD2A; the design of the AAVrh74 vector to systematically and robustly deliver treatment to cardiac and skeletal muscle, including the diaphragm, without promiscuously crossing the blood brain barrier, making it an ideal candidate to treat muscle disease; the potential of our gene therapy development engine to deliver transformative therapies to treat genetically based diseases; the potential of a gene therapy approach to help those living with LGMD2A; and Sarepta's 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: the expected benefits and opportunities related to the agreement 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; in particular, the agreement may not result in any viable treatments suitable for commercialization due to a variety of reasons, including any inability of the parties to perform their commitments and obligations under the agreement, the results of research may not be consistent with past results or may not be positive or may otherwise 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 commercialization of any products under the agreement is achieved, this may not result in any significant revenues to the parties; if the actual number of patients suffering from LGMD2A is smaller than estimated, Sarepta's revenue and ability to achieve profitability may be adversely affected; 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 Sarepta's 2018 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 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.

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