Sarepta Announces Agreement with Nationwide Children's Hospital for Rights to its Gene Therapy Program to Treat Charcot-Marie-Tooth (CMT) Neuropathy, Globally the Most Common Inherited Neuromuscular Disorder

-- Furthers the Company's strategy to expand neuromuscular gene therapy franchise --

-- CMT is a group of hereditary, degenerative nerve diseases that in addition to causing extraordinary pain, can greatly impact peripheral motor skills and profoundly limit the ability to walk or use one's hands --

-- Clinical trial in patients scheduled to start in 2019 in the most prevalent subtype of CMT type 1A --

CAMBRIDGE, Mass., October 5, 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 today it has signed an agreement with Nationwide Children's Hospital (Nationwide Children's) giving Sarepta the exclusive option to a Nationwide Children's gene therapy candidate, neurotrophin 3 (NT-3), to treat Charcot-Marie-Tooth (CMT) neuropathies, including CMT type 1A. CMT is a group of hereditary, degenerative nerve diseases that can affect motor skills, resulting in muscle weakness, and limiting patients' ability to walk or use their hands. CMT is the most common inherited neuromuscular disorder, affecting over 2.8 million people worldwide. CMT type 1A is most often caused by an extra copy of the *PMP22* gene, and affects approximately 50,000 patients in the U.S. alone. Currently, there are no available treatment options.

The clinical trial to test NT-3 gene therapy is planned to commence dosing in 2019 for CMT type 1A. The delivery of the NT-3 gene may have applicability to other sub-types of CMT in addition to other muscle-wasting diseases. Pre-clinical research has shown the ability of the NT-3 gene construct to regenerate nerves. Further research is under way to explore its potential.

Dr. Zarife Sahenk, M.D., Ph.D. is the founder of the NT-3 program at Nationwide Children's. Over the past 15 years, Dr. Sahenk has accumulated a large body of evidence to support the efficacy of neurotrophin NT-3 to improve nerve regeneration and myelination, associated with increased Schwann Cell (SC) survival and differentiation along with the normalization of axonal neurofilament (NF) cytoskeleton.

Dr. Sahenk is 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, and a lead researcher in the anatomical and molecular basis of nerve degeneration and impaired regeneration in hereditary peripheral neuropathies.

“Our agreement today further builds on our strategy to establish a gene therapy engine delivering potentially transformative therapies to treat life-altering rare disease,” stated Doug Ingram, Sarepta’s president and chief executive officer. “CMT, globally the largest inherited neuromuscular disease, is potentially devastating and we are particularly honored to work with Dr. Sahenk, a world-leader in the field of CMT neuropathies, as she prepares to treat patients with CMT type 1A in 2019. Dr. Sahenk has dedicated her professional career, which spans over 40 years, to researching and treating life-limiting neuromuscular diseases such as CMT, Limb-girdle muscular dystrophy, Duchenne and Becker muscular dystrophy. Pursuing this work with Dr. Sahenk exemplifies our approach of success through partnering with the best and brightest gene therapy scientists.”

“Gene therapy represents a potential new pathway for the treatment of CMT Neuropathy. We look forward to collaborating with Sarepta, whose dedication to those impacted by neuromuscular disorders and to rigorous scientific exploration, echoes our own at Nationwide Children’s,” said Zarife Sahenk, M.D., Ph.D., attending neurologist and Director of Clinical and Experimental Neuromuscular Pathology at The Research Institute at Nationwide Children's Hospital.

About Charcot-Marie-Tooth Disease

Charcot-Marie-Tooth or CMT neuropathies are an inherited, heterogeneous group of peripheral nerve disorders affecting 1 in 2,500 persons. CMT is caused by mutations in genes that produce proteins involved in the structure and function of either the peripheral nerve axon or the myelin sheath. The degeneration of motor nerves results in muscle weakness and atrophy in the extremities (arms, legs, hands, or feet), and in some cases the degeneration of sensory nerves results in a reduced ability to feel heat, cold, and pain. Different mutations can cause mild to severe symptoms, with some patients losing their ability to walk or use their hands. Most patients are diagnosed at infancy, while other patients develop symptoms at adolescence.

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.

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 plan to commence the clinical trial to test NT-3 gene therapy in 2019 for CMT type 1A; the potential applicability of the delivery of the NT-3 gene to other sub-types of CMT in addition to other muscle-wasting diseases; the potential of the NT-3 gene construct to regenerate nerves; Sarepta's strategy to establish a gene therapy engine delivering potentially transformative therapies to treat life-altering rare disease; and gene therapy representing a potential new pathway for the treatment of CMT Neuropathy.

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 product under the agreement is achieved, this may not result in any significant revenues to the parties; if the actual number of patients suffering from CMT and/or CMT type 1A 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, 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 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
Rachel Hutman, 301-801-5540
rhutman@w2ogroup.com