



Sarepta Therapeutics Announces Partnership with Myonex Therapeutics for the Advancement of Multiple Gene Therapy Programs Aimed at Treating Distinct Forms of Limb-Girdle Muscular Dystrophies

-- Sarepta strengthens position as a leader in gene therapy; expands rare disease franchise --

-- The program is designed, if successful, to offer first-ever corrective treatments for five distinct forms of Limb-girdle muscular dystrophy (LGMD) --

-- Sarepta is granted exclusive option to acquire Myonex --

CAMBRIDGE, Mass., May 3, 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 that it has entered into an exclusive partnership with Myonex Therapeutics, Inc., a clinical-stage biotechnology company developing transformative gene therapies for various forms of Limb-girdle muscular dystrophies (LGMDs). Myonex' five LGMD gene therapy candidates target the most severe and common forms of the disease and include three clinical and two pre-clinical stage programs.

The most advanced of Myonex' programs, MYO-101, aims to treat LGMD2E, also known as beta-sarcoglycanopathy, a severe and debilitating form of LGMD characterized by progressive muscle fiber loss, inflammation and muscle fiber replacement with fat and fibrotic tissue. MYO-101 is designed to transfect a gene that codes for and restores beta-sarcoglycan protein with the goal of restoring the dystroglycan complex. MYO-101 has generated strong pre-clinical safety and efficacy data utilizing the AAVrh.74 vector system, the same vector used in the micro-dystrophin gene therapy program Sarepta is developing with Nationwide Children's Hospital. A Phase 1/2a study of MYO-101 is scheduled to begin in mid-2018. The companies plan to report on 60-day biopsy data in late-2018 or early 2019. Additionally, Myonex is advancing MYO-102 for LGMD2D, MYO-103 for LGMD2C, MYO-201 for LGMD2B, and MYO-301 for LGMD2L. Like MYO-101, all programs rely upon transfecting a restorative gene utilizing the AAVrh.74 vector.

Louise Rodino-Klapac, Ph.D., the principal investigator for the Center for Gene Therapy at Nationwide Children's Hospital and associate professor in the Department of Pediatrics at Ohio State University, is the

inventor of the Myonexus portfolio of LGMD candidates and the chief scientific officer and co-founder of Myonexus, a spinout of Nationwide Children's Hospital, Center for Gene Therapy. Sarepta and Nationwide Children's are also developing and currently dosing patients in a micro-dystrophin gene therapy for the treatment of Duchenne muscular dystrophy (DMD). Dr. Rodino-Klapac is the co-inventor and serves as one of the principal investigators of the micro-dystrophin program.

Under the terms of the agreement, Sarepta will make an upfront payment of \$60 million and additional development-related milestone payments to purchase an exclusive option to acquire Myonexus at a pre-negotiated, fixed price with sales-related contingent payments. If all development-related milestone payments are met, Sarepta will make payments of up to \$45 million over an approximately two-year evaluation period. Sarepta has the option to purchase Myonexus at any time, including upon review of proof-of-concept data.

"We are very excited to enter a partnership with an option to acquire Myonexus. Myonexus and its focus on gene therapy using the AAVrh.74 vector to treat forms of LGMD aligns brilliantly with our vision to emerge as one of the most meaningful global precision genetic medicine companies by focusing on the use of genetic medicine to improve the lives of those with rare fatal diseases," stated Doug Ingram, Sarepta's president and chief executive officer. "We are also delighted to extend our collaboration with Dr. Rodino-Klapac, a renowned thought leader in the field of gene therapy. We share her dedication to rapidly bring life-changing medicines to children suffering and dying from genetic disease."

Mr. Ingram continued, "With this partnership, we continue to expand our genetic medicine platform of development compounds from 16 to 21, across RNA and gene therapy development and gene editing research. Further, consistent with the goal we expressed at the January 2018 J.P. Morgan Healthcare Conference, this partnership allows us to leverage our genetic medicine expertise beyond DMD and into new rare diseases in need of therapeutic options."

Mike Triplett, Myonexus' president and chief executive officer added, "Our partnership with Sarepta reflects the teams' mutual commitment to the commercialization of our LGMD product candidates and treating this debilitating and fatal set of diseases."

Dr. Rodino-Klapac stated, "The culmination of this partnership with Sarepta is important to Myonexus' mission of rapidly advancing our LGMD pipeline. As the inventor of Myonexus' LGMD approach and co-inventor of the DMD gene therapy approach, both of which utilize the AAVrh.74 vector system, we are

excited to leverage this knowledge from our work in DMD and now apply it to our LGMD portfolio. We are particularly excited to be collaborating again with Sarepta on the development of potentially transformative therapies to treat muscular dystrophies, which represent vast areas of unmet medical need.”

Dr. Rodino-Klapac added, “To date, we are encouraged by the data we have generated in our lead MYO-101 program and I look forward to presenting data from the entire program at Sarepta’s upcoming R&D day. We intend to initiate our clinical trial by mid-year and soon thereafter generate biopsy data to inform what could be the first-ever corrective treatment for Limb-girdle muscular dystrophy.”

William Blair acted as the exclusive financial advisor and Thompson Hine acted as legal counsel to Myonexus for the transaction.

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.

About Myonexus Therapeutics

Myonexus Therapeutics is a clinical stage, rare disease gene therapy company developing first ever treatments for Limb-girdle muscular dystrophies (LGMDs) based on research at Nationwide Children’s Hospital, a leader in neuromuscular gene therapy discovery and translational research. Myonexus Therapeutics’ pipeline includes three clinical stage gene therapy programs (LGMD2E, LGMD2D, and LGMD2B) and two preclinical gene therapy programs (LGMD2C and LGMD2L). Founded in 2017, Myonexus is headquartered in New Albany, Ohio. More information is available at www.myonexustx.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 potential of the program in collaboration with Myonexus to offer first-ever corrective treatments for five distinct forms of LGMD; MYO-101’s design and goal of restoring the dystrogly-

can complex; expected milestones, including initiating a Phase 1/2a study of MYO-101 in mid-2018, presenting data from Myonexus' program at Sarepta's upcoming R&D day and reporting on 60-day biopsy data in late-2018 or early 2019; payments that Sarepta is expected to make under the agreement; Myonexus and its focus on gene therapy using the AAVrh.74 vector to treat forms of LGMD aligning brilliantly with Sarepta's vision to emerge as one of the most meaningful global precision genetic medicines companies by focusing on the use of genetic medicine to improve the lives of those with rare fatal diseases; the expansion of Sarepta's genetic medicine platform of development compounds from 16 to 21; the partnership with Myonexus allowing Sarepta to leverage its genetic medicine expertise beyond DMD and into new rare diseases in need of therapeutic options; and Myonexus' LGMD's portfolio involving potentially transformative therapies to treat muscular dystrophies, which represent vast areas of unmet medical need.

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 Myonexus may not be realized or may take longer to realize than expected due to challenges and uncertainties inherent in product research and development; the partnership with Myonexus may not result in any viable treatments suitable for clinical research or 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 Myonexus; and even if the agreement results 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. 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 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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