Sarepta Exercises Option to Acquire Myonexus Therapeutics

-- Exercise Fee is $165 Million --

-- Sarepta to Acquire Myonexus' Portfolio of Five Gene Therapy Candidates to Treat Distinct Forms of Limb-Girdle Muscular Dystrophy (LGMD) --

CAMBRIDGE, Mass., February 27, 2019 (GLOBE NEWSWIRE) -- Sarepta Therapeutics, Inc. (NASDAQ:SRPT), the leader in precision genetic medicine for rare diseases, announced today that it has exercised its option to acquire Myonexus Therapeutics, a clinical-stage biotechnology company developing transformative gene therapies for five LGMDs: LGMD2E, LGMD2D, LGMD2B, LGMD2C and LGMD2L. Subject to satisfaction of closing conditions, Sarepta will pay the Myonexus shareholders $165 million.

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, including the five programs progressing with Myonexus, are seriously life-limiting and often life-ending diseases.

In May 2018, Sarepta and Myonexus entered into an exclusive partnership to develop Myonexus' five LGMD gene therapy candidates, which target the most severe and common forms of the disease. Three of the programs are in clinical development and two are in the pre-clinical stage and ready to progress into the clinic. As part of the agreement, Sarepta had an exclusive option to acquire Myonexus.

As previously announced, Sarepta will host a webcast and conference call at 8:00 am ET today, February 27, 2019, during which the Company will present results from the first 3-patient cohort of the MYO-101 study in patients with LGMD2E. Details to participate in the call are below.

"The five LGMD gene therapies being developed fit brilliantly with Sarepta's mission to develop therapies with the potential to rescue the lives of patients with serious life-limiting rare genetic diseases," said Doug Ingram, president and chief executive officer, Sarepta. "Our confidence in these programs has come from the fact that our micro-dystrophin gene therapy and the Myonexus programs have much in common, including inventors from Nationwide Children's Hospital, a shared vector in AAVrh74 and, to date, similar pre-clinical safety data. We are excited to acquire Myonexus, which will allow us to move rapidly to find solutions for LGMD patients and continue to build out and validate our gene therapy engine."

Mr. Ingram continued, “We would also like to take this opportunity to thank Myonexus and Nationwide Children’s Hospital for their contributions, as it was their dedication and tireless efforts that advanced these programs to where they are today.”

Like Sarepta’s micro-dystrophin program, all five Myonexus LGMD sub-type programs employ the AAVrh74 vector, designed to systemically 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 MHCK7 promoter used in MYO-101, which is also used in the micro-dystrophin program, was chosen for 3 of the 5 LGMD programs because it is generally more productive in muscle than other MCK promoters and it robustly expresses in the heart, which is critically important for patients with LGMD2E, LGMD2B, and LGMD2C, many of whom die from pulmonary or cardiac complications.

"We partnered with Sarepta less than a year ago, as we shared the mutual goal of developing LGMD therapies on behalf of patients with debilitating and fatal disease,” said Michael Triplett, Ph.D., president and chief executive officer, Myonexus. “This acquisition solidifies a commitment to rapidly advance therapies on behalf of patients who currently don’t have treatment options.”

Conference Call Details

The 8:00 a.m. ET conference call presenting the MYO-101 LGMD results may be accessed by dialing (844) 534-7313 for domestic callers and (574) 990-1451 for international callers. The passcode for the call is 1693875. Please specify to the operator that you would like to join the “Sarepta-hosted LGMD Results Call.”

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 5 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.

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 satisfaction of closing conditions related to the acquisition; Sarepta's mission to develop therapies with the potential to rescue the lives of patients with serious life-limiting rare genetic diseases; Sarepta's confidence in the Myonexus' programs; the expectation that the acquisition of Myonexus will allow Sarepta to move rapidly to find solutions for LGMD patients and continue to build out and validate its gene therapy engine; the potential benefits of the AAVrh74 vector and the MHCK7 promoter; 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: there can be no assurance that Sarepta will be able to complete the acquisition of Myonexus on the anticipated terms, or at all; Sarepta may not realize the anticipated benefits of the acquisition, which involves various risks, including disruption of Sarepta's ongoing business and distraction of its management and employees from other opportunities and challenges, potential failure of the due diligence processes to identify significant problems, liabilities or other shortcomings or challenges of Myonexus or the product candidates, liability for activities of Myonexus before the acquisition, including intellectual property infringement claims, violations of laws, commercial disputes, tax liabilities, and other known and unknown liabilities; success in preclinical testing and early clinical trials, especially if based on a small patient sample, does not ensure that later clinical trials will be successful, and initial results from a clinical trial do not necessarily predict final results; Sarepta's ongoing research and development efforts may not result in any viable treatments suitable for clinical research or commercialization due to a variety of reasons, some of which may be outside of Sarepta's control, including 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 our product candidates; and even if Sarepta's programs result 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 the 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 web-site 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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