Sarepta Therapeutics to Commence Dosing of the Next Study with Commercial Process Material for the SRP-9001 Gene Therapy Program for the Treatment of Duchenne Muscular Dystrophy

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-- Dosing with SRP-9001 commercial-process material to proceed following discussions with U.S. FDA --

-- Clinical dosing is expected to begin before the end of 2020 --

CAMBRIDGE, Mass., Nov. 05, 2020 (GLOBE NEWSWIRE) -- Sarepta Therapeutics, Inc. (NASDAQ:SRPT), the leader in precision genetic medicine for rare diseases, today announced important updates related to the development plan for SRP-9001 (AAVrh74.MHCK7.micro-dystrophin), the Company's investigational gene transfer therapy for the treatment of Duchenne muscular dystrophy.

In light of the risks that the ongoing COVID-19 pandemic posed to the execution of a large, multi-center trial, Sarepta proposed a new clinical study in the September 2020 Type C meeting to support the Company's clinical and regulatory efforts -- Study SRP-9001-103 (Study 103). Study 103 is an open-label study in up to 10 patients evaluating the safety and expression of commercial process material for SRP-9001 and is intended to accelerate Sarepta's validation of commercial process material.

In the Type C meeting written response, the Office of Tissues and Advanced Therapies (OTAT), part of the Center for Biologics Evaluation and Research (CBER) at the U.S. Food and Drug Administration (FDA), requested, among other things, an additional potency assay to be used for the release of SRP-9001 commercial process material. Subsequent to the written feedback, Sarepta and OTAT have engaged in informal and productive dialog and as a result of those discussions, Sarepta anticipates commencing dosing in Study 103 this year.

"Our ability to rapidly address outstanding issues regarding our potency assay approach and commence Study 103 is an important milestone for SRP-9001 and for Duchenne muscular dystrophy patients who daily degenerate from this cruel disease," said Doug Ingram, president and CEO, Sarepta. "Time and delay are not on the side of patients with Duchenne. This rapid resolution would not have occurred without the willingness of OTAT to engage with us informally and to discuss collaboratively how we could allow the program to advance. I want to thank the professionals at OTAT for their willingness, in midst of many competing priorities and in the face of a pandemic, to work with us to find an appropriate solution that will allow us to dose patients this year with our commercial process material of SRP-9001. We look forward to commencing Study 103 this year and having a data read-out from our ongoing blinded, placebo-controlled trial, Study 102, in early 2021."

About SRP-9001 (AAVrh74.MHCK7.micro-dystrophin)

SRP-9001 is an investigational gene transfer therapy intended to deliver the micro-dystrophin-encoding gene to muscle tissue for the targeted production of the micro-dystrophin protein. Sarepta is responsible for global development and manufacturing of SRP-9001 and plans to commercialize SRP-9001 in the United States. In December 2019, the Company announced a licensing agreement granting Roche the exclusive right to launch and commercialize SRP-9001 outside the United States upon approval. Sarepta has exclusive rights to the micro-dystrophin gene therapy program initially developed at the Abigail Wexner Research Institute at Nationwide Children's Hospital.

About Sarepta Therapeutics

At Sarepta, we are leading a revolution in precision genetic medicine and every day is an opportunity to change the lives of people living with rare disease. The Company has built an impressive and competitive position in Duchenne muscular dystrophy (DMD) and in gene therapies for limb-girdle muscular dystrophies (LGMDs), mucopolysaccharidosis type IIIA, Charcot-Marie-Tooth (CMT), and other CNS-related disorders, with more than 40 programs in various stages of development. The Company's programs and research focus span several therapeutic modalities, including RNA, gene therapy and gene editing. For more information, please visit www.sarepta.com or follow us on Twitter, LinkedIn, Instagram and Facebook.

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 the expectation to commence clinical dosing with commercial-process material for SRP-9001 (study 103) before the end of 2020; the potential of Study 103 to accelerate Sarepta's validation of commercial-process material; and the expectation to have a data read-out from our ongoing blinded, placebo-controlled trial (Study 102), in early 2021.

These forward-looking statements involve risks and uncertainties, many of which are beyond our control. Known risk factors include, among others: success in pre-clinical trials and clinical trials, especially if based on a small patient sample, does not ensure that later clinical trials will be successful; the commencement and completion of our clinical trials and announcement of results may be delayed or prevented for a number of reasons, including, among others, denial by the regulatory agencies of permission to proceed with our clinical trials, or placement of a clinical trial on hold, challenges in identifying, recruiting, enrolling and retaining patients to participate in clinical trials and inadequate quantity or quality of supplies of a product candidate or other materials necessary to conduct clinical trials; our data for SRP-9001 may not be sufficient for obtaining regulatory approval; we may not be able to execute on our business plans and goals, including meeting our expected or planned regulatory milestones and timelines, clinical development plans, and bringing our product candidates to market, due to a variety of reasons, some of which may be outside of our control, including possible limitations of company financial and other resources, manufacturing limitations that may not be anticipated or resolved for in a timely manner, 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 the COVID-19 pandemic; and even if Sarepta's programs result in new commercialized products, Sarepta may not achieve the expected 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, 2019, 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 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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