Sarepta Therapeutics Receives Fast Track Designation for SRP-9001 Micro-Dystrophin Gene Therapy for the Treatment of Duchenne Muscular Dystrophy

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CAMBRIDGE, Mass., July 24, 2020 (GLOBE NEWSWIRE) -- Sarepta Therapeutics, Inc. (NASDAQ:SRPT), the leader in precision genetic medicine for rare diseases, today announced that the U.S. Food and Drug Administration (FDA) has granted Fast Track designation to SRP-9001 (AAVrh74.MHCK7.micro-dystrophin). SRP-9001 is an investigational gene transfer therapy intended to deliver its micro-dystrophin-encoding gene to muscle tissue for the targeted production of the micro-dystrophin protein. Safety and tolerability data at one year from four clinical trial participants who received SRP-9001 in Study 101 were recently published in *JAMA Neurology,* and Study 102, a randomized, double-blind, placebo-controlled study of SRP-9001, is ongoing with results expected in early 2021.

The Fast Track designation is a process designed to facilitate the development and expedited review of drugs that treat serious conditions and fill unmet medical needs. In addition to Fast Track, SRP-9001 has also been granted Rare Pediatric Disease (RPD) designation. SRP-9001 was previously granted Orphan Drug status in the United States, the European Union and Japan.

About SRP-9001

SRP-9001 is an investigational gene transfer therapy designed 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 for 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. 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 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 statements regarding SRP-9001's intention to deliver the micro-dystrophin-encoding gene to muscle tissue for the targeted production of the micro-dystrophin protein; Sarepta's expectation to have results from Study 102 in early 2021; and Sarepta's plan to commercialize SRP-9001 in the United States.

These forward-looking statements involve risks and uncertainties, many of which are beyond Sarepta's control. Known risk factors include, among others: fast track designation by the FDA may not lead to faster development or regulatory review or approval process, and does not increase the likelihood that SRP-9001 will receive marketing approval; Sarepta may not be able to complete clinical trials required by the FDA or other regulatory authorities for approval of SRP-9001; SRP-9001 may not result in a viable treatment suitable for 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; Sarepta may not be able to execute on its business plans and goals, including meeting its expected or planned regulatory milestones and timelines, clinical development plans, and bringing its product candidates to market, due to a variety of reasons, many 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, 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 the COVID-19 pandemic; 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 Sarepta which you are encouraged to review.

Any of the foregoing risks could materially and adversely affect Sarepta'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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