Sarepta Therapeutics Announces That U.S. FDA has Accepted for Filing and Granted Priority Review for the Biologics License Application for SRP-9001, Sarepta's Gene Therapy for the Treatment of Ambulant Individuals with Duchenne Muscular Dystrophy

11/28/22

– Regulatory action date of May 29, 2023

– SRP-9001 would be the first gene therapy for Duchenne, a one-time treatment designed to treat the underlying cause of DMD by delivering a functional shortened dystrophin to muscle

CAMBRIDGE, Mass., Nov. 28, 2022 (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 accepted the Company's Biologics License Application (BLA) seeking accelerated approval of SRP-9001 (delandistrogene moxeparvovec) for the treatment of ambulant individuals with Duchenne muscular dystrophy. SRP-9001 has been granted Priority Review by the FDA, with a regulatory action date of May 29, 2023.

"We are delighted to announce that the FDA has accepted Sarepta's BLA for SRP-9001 for filing and priority review," said Doug Ingram, president and chief executive officer, Sarepta Therapeutics. "Duchenne is a relentlessly degenerative and invariably fatal disease, robbing children of muscle and function hourly and daily. Our BLA submission for an accelerated approval, along with the FDA's acceptance of that BLA for filing and review, is a tremendously important milestone in our effort to bring a potentially life-changing gene therapy to Duchenne patients as rapidly as possible and we look forward to working with the FDA through the review process."

SRP-9001 is an investigational gene therapy for Duchenne being developed in partnership with Roche. Duchenne is characterized by a mutation in the dystrophin gene that results in the lack of dystrophin, which acts as a shock absorber for muscle at the membrane. SRP-9001 is designed to treat the proximate cause of Duchenne by delivering to muscle a gene that codes for a shortened, functional form of dystrophin. In addition to a wealth of pre-clinical evidence, the BLA for SRP-9001 included efficacy and safety data from Study SRP-9001-103 (also known as ENDEAVOR), as well as from Studies SRP-9001-101 and SRP-9001-102, and an integrated analysis across these three clinical studies comparing functional results to a propensity-score-weighted external control (EC). In clinical results from more than 80 treated patients, SRP-9001 has demonstrated positive results at multiple time points, including one-, two- and up to four-years after treatment, in addition to demonstrating a consistent safety profile.

In addition to Studies SRP-9001-101, SRP-9001-102 and SRP-9001-103, SRP-9001 is also being studied in EMBARK (Study SRP-9001-301), a global, randomized, double-blind, placebo-controlled clinical trial of SRP-9001 which has recruited 125 participants with Duchenne between the ages of 4 to 7. EMBARK is fully enrolled with results expected by the end of 2023. Sarepta has proposed EMBARK as the post-marketing confirmatory trial for SRP-9001.

About SRP-9001 (delandistrogene moxeparvovec)
SRP-9001 (delandistrogene moxeparvovec) is an investigational gene transfer therapy intended to deliver SRP-9001 to muscle tissue for the targeted production of functional components of dystrophin. Sarepta is responsible for global development and manufacturing for SRP-9001 and plans to commercialize SRP-9001 in the United States upon receiving FDA approval. In December 2019, Roche partnered with Sarepta to combine Roche's global reach, commercial presence and regulatory expertise with Sarepta's gene therapy candidate for Duchenne to accelerate access to SRP-9001 for patients outside the United States.

About Duchenne Muscular Dystrophy
Duchenne muscular dystrophy (DMD) is a rare, fatal neuromuscular genetic disease that occurs in approximately one in every 3,500-5,000 newborn males worldwide. DMD is caused by a change or mutation in the gene that encodes instructions for dystrophin. Symptoms of DMD usually appear in infants and toddlers. Affected children may experience developmental delays such as difficulty in walking, climbing stairs or standing from a sitting position. As the disease progresses, muscle weakness in the lower limbs spreads to the arms and other areas. Most patients require full-time use of a wheelchair in their early teens, and then progressively lose the ability to independently perform activities of daily living such as using the restroom, bathing and feeding. Eventually, increasing difficulty in breathing due to respiratory muscle dysfunction requires ventilation support, and cardiac dysfunction can lead to heart failure. The condition is universally fatal, and patients usually succumb to the disease in their twenties.

About Sarepta Therapeutics
Sarepta is on an urgent mission: engineer precision genetic medicine for rare diseases that devastate lives and cut futures short. We hold leadership positions in Duchenne muscular dystrophy (DMD) and limb-girdle muscular dystrophies (LGMDs), and we currently have more than 40 programs in various stages of development. Our vast pipeline is driven by our multi-platform Precision Genetic Medicine Engine in gene therapy, RNA and gene editing. For more information, please visit www.sarepta.com or follow us on Twitter, LinkedIn, Instagram and Facebook.

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.

Forward-Looking Statements
This press release contains "forward-looking statements." Any statements that are not statements of historical fact may be deemed to be forward-looking statements. Words such as "believe," "anticipate," "plan," "expect," "will," "may," "intend," "prepare," "look," "potential," "possible" and similar expressions are intended to identify forward-looking statements. These forward-looking statements include statements relating to the potential benefits of SRP-9001; our effort to bring a potentially life-changing gene therapy to Duchenne patients as rapidly as possible; the regulatory action date of May 29, 2023; our plan to work with the FDA through the review process; and our expectation that study EMBARK (Study SRP-9001-301) will serve as the

post-marketing confirmatory trial for SRP-9001 and that we will have results by the end of 2023.

These forward-looking statements involve risks and uncertainties, many of which are beyond our control. Known risk factors include, among others: the possible impact of regulations and regulatory decisions by the FDA and other regulatory agencies on our business, as well as the development of our product candidates and our financial and contractual obligations; that 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; success in pre-clinical trials and early clinical trials, especially if based on a small patient sample, does not ensure that later clinical trials will be successful; different methodologies, assumptions and applications we use to assess particular safety or efficacy parameters may yield different statistical results, and even if we believe the data collected from clinical trials of our product candidates are positive, these data may not be sufficient to support approval by the FDA or other global regulatory authorities; 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, 2021, 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, except as required by law.

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