# Sarepta Therapeutics Submits Biologics License Application for SRP-9001 for the Treatment of Ambulant Patients with Duchenne Muscular Dystrophy

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CAMBRIDGE, Mass., Sept. 29, 2022 (GLOBE NEWSWIRE) -- Sarepta Therapeutics, Inc. (NASDAQ:SRPT), the leader in precision genetic medicine for rare diseases, today announced that it has submitted a Biologics License Application (BLA) to the U.S. Food and Drug Administration (FDA) for the accelerated approval of SRP-9001 (delandistrogene moxeparvovec) to treat ambulant patients with Duchenne muscular dystrophy. SRP-9001 is an investigational gene therapy for Duchenne being developed in partnership with Roche.

The BLA is submitted for accelerated approval based on the expression of SRP-9001 dystrophin protein, an internally shortened and functional version of dystrophin, as a surrogate endpoint reasonably likely to predict clinical benefit. Among other things, the BLA is based on positive pre-clinical, biomarker and clinical functional results. In clinical trials, SRP-9001 demonstrated positive results at multiple time points, including one-, two- and four-years after treatment, in addition to a consistent safety profile. The submitted BLA for SRP-9001 includes efficacy and safety data from Studies SRP-9001-101, SRP-9001-102, SRP-9001-103 (also known as ENDEAVOR), as well as an integrated analysis across these three clinical studies comparing functional results to a propensity-score-matched external control (EC). Quantification of the SRP-9001-protein expression is measured by western blot and supported by immunofluorescence and efficacy is further supported by biomarker and clinical functional benefit as measured by the North Star Ambulatory Assessment (NSAA) and secondary timed tests.

Sarepta has proposed its fully-enrolled study EMBARK (Study SRP-9001-301) as the post-marketing confirmatory study to support the accelerated approval. EMBARK is a global, randomized, double-blind, placebo-controlled clinical trial. The primary endpoint for EMBARK is the assessment of the change in NSAA total score from baseline to week 52 compared to placebo.

“Every hour of every day, this ruthless disease, Duchenne, robs thousands of children in the United States of muscle as it steals their future from them. Sarepta’s BLA submission for an accelerated approval of SRP-9001 is a significant milestone in our quest to intervene with urgency on behalf of the children we serve,” said Doug Ingram, president and chief executive officer, Sarepta Therapeutics. “If approved, SRP-9001 will be the first gene therapy available for Duchenne patients. We are enormously grateful to the courageous families who have participated in the SRP-9001 trials and to the participating clinical investigators and experts who have guided us and played a crucial part in reaching this milestone.”

SRP-9001 was granted Fast Track designation in July 2020, an FDA process designed to facilitate the development and expedited review of therapies 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 in the United States, and Orphan Drug status in the United States, the European Union, Switzerland and Japan.

***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 benefits of SRP-9001; the potential for SRP-9001 to be the first approved gene therapy for Duchenne available to the majority of those with Duchenne; our expectation to use our fully-enrolled study EMBARK (Study SRP-9001-301) as the post-marketing confirmatory study to support the accelerated approval and our quest to intervene with urgency on behalf of the children we serve.*

*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.*

Source: Sarepta Therapeutics, Inc.

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