Sarepta Therapeutics Announces Intent to Submit an Accelerated Approval Biologics License Application for its Gene Therapy SRP-9001 to Treat Duchenne Muscular Dystrophy

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CAMBRIDGE, Mass., July 29, 2022 (GLOBE NEWSWIRE) -- Sarepta Therapeutics, Inc. (NASDAQ:SRPT), the leader in precision genetic medicine for rare diseases, today announced its intent to submit a Biologics License Application (BLA) seeking accelerated approval for SRP-9001 (delandistrogene moxeparvovec) to treat ambulant individuals with Duchenne muscular dystrophy. SRP-9001 is an investigational gene therapy for Duchenne being developed in partnership with Roche.

"We are delighted to confirm that based on FDA feedback received following a thorough and in-depth review, we intend to submit a BLA for our SRP-9001 gene therapy to treat Duchenne muscular dystrophy this fall. We look forward to a collaborative review commencing this year and running through the first half of 2023," said Doug Ingram, president and chief executive officer, Sarepta Therapeutics. "Duchenne robs children daily and hourly of their muscle, stealing them bit by bit from their families and loved ones. Guided by rigorous science and productive regulatory discussions, our goal is to move with the urgency desperately needed by the patient community, and our upcoming BLA filing for SRP-9001 serves that goal."

SRP-9001 was granted Fast Track designation in July 2020, an FDA 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 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 Company's intent to submit a BLA this fall and the Company anticipating a collaborative review commencing this year and running through the first half 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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