Sarepta Therapeutics Announces Initiation of EMBARK, a Global Pivotal Study of SRP-9001, a Gene Therapy for the Treatment of Duchenne Muscular Dystrophy

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- Sarepta to host "SRP-9001 Micro-dystrophin R&D Day" at 8:30 a.m. Eastern Time on Monday, Oct. 11, 2021

CAMBRIDGE, Mass., Oct. 04, 2021 (GLOBE NEWSWIRE) -- Sarepta Therapeutics, Inc. (NASDAQ:SRPT), the leader in precision genetic medicine for rare diseases, today announced the initiation, in partnership with Roche, in the U.S. and countries around the world, of study SRP-9001-301, also known as EMBARK, a pivotal study of SRP-9001 (rAAVrh74.MHCK7.micro-dystrophin) for the treatment of Duchenne muscular dystrophy. 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.

"We are delighted to announce the initiation of EMBARK, representing the first pivotal double-blind gene therapy trial in Duchenne which will be initiated in US, Europe and Asia," said Doug Ingram, president and chief executive officer, Sarepta. "The initiation of EMBARK represents the culmination of enormous effort and success from a research, development and manufacturing perspective and is an extraordinarily important moment for the patient community and a leap forward in our effort to change the course of Duchenne. In addition to our team, our investigators and the families who have participated in our trials, I want to thank the professionals within FDA's Office of Tissues and Advanced Therapies for their collaborative guidance and insight as we finalized our EMBARK study and advance the SRP-9001 program. We look forward to sharing the particulars of EMBARK and additional functional data from our prior studies in our upcoming 'Micro-dystrophin Day'."

The Company will host an SRP-9001 Micro-dystrophin R&D Day on Monday, Oct. 11, 2021, at 8:30 am Eastern Time.

The presentation will be webcast live under the investor relations section of Sarepta's website at https://investorrelations.sarepta.com/events-presentations and slides will be archived there following the call for one year. Please connect to Sarepta's website several minutes prior to the start of the broadcast to ensure adequate time for any software download that may be necessary. The conference call may be accessed by dialing (844) 534-7313 for domestic callers and (574) 990-1451 for international callers. The passcode for the call is 3878397. Please specify to the operator that you would like to join the "SRP-9001 Micro-dystrophin R&D Day" call.

About SRP-9001 (rAAVrh74.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 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. 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 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 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.

Forward-Looking Statements
This presentation 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 potential market opportunities; the potential benefits of SRP-9001; the potential of our collaborations and partnerships; the potential of our efforts to change the course of Duchenne; and expected plans and milestones, including initiating SRP-9001-301 in the U.S., Europe and Asia, and our plans to share the study design of SRP-9001-301 and additional functional data at our upcoming SRP-9001 R&D Day.

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; different methodologies, assumptions and applications we utilize 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 regulatory authorities; 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; 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, 2020, 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.

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.

Source: Sarepta Therapeutics, Inc.

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