Sarepta Therapeutics' Investigational Gene Therapy for the Treatment of Duchenne Muscular Dystrophy, SRP-9001, Demonstrates Robust Expression and Consistent Safety Profile Using Sarepta's Commercial Process Material

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- ***Results from the first 11 participants enrolled in Study 9001-103 ENDEAVOR showed robust transduction, delivering mean vector genome copies of 3.87 per nucleus***
- ***Treated patients achieved mean micro-dystrophin expression levels of 55.4% of normal as measured by western blot***
- ***Micro-dystrophin was properly localized to the muscle sarcolemma, with patients achieving mean percentage of dystrophin positive fibers of 70.5% and intensity of micro-dystrophin expression of 116.9% of normal control, as measured by immunofluorescence (IF)***
- ***Safety profile consistent with prior studies and no new safety signals identified***

CAMBRIDGE, Mass., May 18, 2021 (GLOBE NEWSWIRE) -- Sarepta Therapeutics, Inc. (NASDAQ:SRPT), the leader in precision genetic medicine for rare diseases, today announced positive 12-week expression and safety results from the first 11 participants enrolled in Study SRP-9001-103, an open-label study known as ENDEAVOR being conducted in partnership with Roche. In results from the first clinical study using commercially representative material, SRP-9001 (rAAVrh74.MHCK7.micro-dystrophin) demonstrated robust expression of micro-dystrophin and no new safety signals from prior studies, supporting its potentially differentiated profile 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 by these seminal results from the ENDEAVOR Study, our first trial results with SRP-9001 made by our commercial-scale manufacturing process. These data show strong transduction of the micro-dystrophin gene, resulting in robust expression of the properly localized micro-dystrophin protein, and did so with no new or unexpected safety signals," said Doug Ingram, president and chief executive officer, Sarepta. "In addition to characterizing and differentiating SRP-9001, these results confirm the extraordinary work done over the last two and a half years to build an at-scale gene therapy manufacturing process and corresponding analytics sufficient to meet the needs of the Duchenne population with what we believe will be a potentially life-changing therapy. Armed with these data, we will seek a meeting with the FDA with the goal of rapidly starting our registrational study."

In the open-label study, 20 participants between the ages of four and seven were treated with a single infusion of SRP-9001 at a dose of $1.33x10^{14}$ vg/kg. In muscle biopsies from the first 11 patients taken 12 weeks after treatment, the following results were observed:

- All patients demonstrated robust transduction, with mean micro-dystrophin expression of 55.4% of normal, as measured by western blot.
- Muscle dystrophin levels demonstrated a mean of 70.5% (baseline 12.8%) muscle fibers expressing micro-dystrophin at 12 weeks with a mean intensity at the sarcolemma of 116.9% (baseline 41.0%) compared to normal biopsies, as measured by immunofluorescence. Comparisons between baseline and post-treatment measures were statistically significant (p=0.001 for positive fibers, and p=0.002 for intensity).
- Mean vector genome copies per nucleus reached 3.87.

The safety profile of SRP-9001 observed in the first 11 participants in ENDEAVOR is consistent with the safety seen in earlier studies using clinical manufacturing process material. In line with previously reported clinical data, no clinically relevant complement activation was observed in these 11 patients. Two patients experienced serious adverse events (transaminase elevation in one patient and nausea and vomiting in a second patient) that fully resolved.

About SRP-9001-103 (ENDEAVOR)
Study SRP-9001-103 (Study 103) is an open-label clinical trial of SRP-9001 that has enrolled 20 participants with Duchenne muscular dystrophy between the ages of 4-7. Study 103 uses commercially representative SRP-9001 and the primary endpoint is the change from baseline in the quantity of micro-dystrophin protein expression measured by western blot at 12 weeks. Secondary outcome measures include change from baseline in micro-dystrophin expression fiber intensity as measured by immunofluorescence (IF) and micro-dystrophin expression measured by IF percent dystrophin positive fibers at 12 weeks. Exploratory endpoints include the change in vector genome copies per nucleus, North Star Ambulatory Assessment (NSAA) and certain timed functional tests. Including the initial 12-week period, patients will be followed for a total of five years.

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.

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 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 the potentially differentiated profile of SRP-9001 for the treatment of Duchenne muscular dystrophy; the potential for SRP-9001 to deliver micro-dystrophin-encoding gene to muscle tissue for the targeted production of the micro-dystrophin protein; the potential of our gene therapy manufacturing process and corresponding analytics to meet the needs of the Duchenne population with what we believe will be a potentially life-changing therapy and our plan to meet with the FDA with the goal of rapidly starting our registrational study.

These forward-looking statements involve risks and uncertainties that may cause actual results to differ materially from those expressed or implied in the forward-looking statements. Many of these risks and uncertainties are beyond our control. Known risk factors include, among others: success in preclinical and clinical trials, especially if based on a small patient sample, does not ensure that later clinical trials will be successful; the data presented in this release may not be consistent with the final data set and analysis or result in an assessment that SRP-9001 provides a safe or effective treatment benefit; different methodologies or assumptions than 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 are positive, the data may not be sufficient to support approval by the FDA or foreign 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, many of which are outside of our control, including possible limitations on 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; the impact of the COVID-19 pandemic; and those risks identified under the heading "Risk Factors" in our 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 we make, 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 we face, we encourage you to review our SEC filings. We caution investors not to place considerable reliance on the forward-looking statements contained in this press release. We undertake no obligation to update forward-looking statements based on events or circumstances after the date of this press release.

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