Sarepta Therapeutics Announces Top-line Results for Part 1 of Study 102 Evaluating SRP-9001, its Investigational Gene Therapy for the Treatment of Duchenne Muscular Dystrophy

-- Study met the primary biological endpoint of micro-dystrophin protein expression at 12 weeks post-treatment, as measured by western blot, in SRP-9001-treated participants versus placebo --

-- SRP-9001-treated participants showed an increase in NSAA total score compared to placebo at 48 weeks; however, the study did not achieve statistical significance on the primary functional endpoint of improvement in NSAA total score compared to placebo at 48 weeks post-treatment --

-- In the pre-specified analysis by age-group, by which the randomization was stratified, participants aged 4-5 years at time of treatment with SRP-9001 demonstrated a statistically significant improvement in NSAA total score versus the age-matched placebo cohort, achieving a 4.3-point improvement on NSAA at 48 weeks post-treatment from baseline --

-- No new safety signals identified for SRP-9001, reinforcing the favorable safety profile observed to date --

-- Sarepta to host conference call at 4:30 p.m. Eastern time --

CAMBRIDGE, Mass., Jan. 7, 2021 (GLOBE NEWSWIRE) -- Sarepta Therapeutics, Inc. (NASDAQ:SRPT), the leader in precision genetic medicine for rare diseases, today announced top-line results from Part 1 of Study SRP-9001-102 (Study 102), an ongoing, randomized, double-blind, placebo-controlled clinical trial to evaluate the safety, efficacy and tolerability of a single dose of SRP-9001 (rAAVrh74.MHCK7.micro-dystrophin) in 41 patients with 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.

At 12 weeks post-treatment compared to baseline, the study met its primary biological endpoint of micro-dystrophin protein expression ($P<0.0001$). Participants who received SRP-9001 (n=20) had mean micro-dystrophin expression of 28.1%, as measured by western blot. Accompanying secondary biological endpoints including vector genome copies per nucleus, percent positive fibers, intensity, and reduction in creatine kinase (exploratory) were also met.

In the primary functional endpoint, SRP-9001-treated participants showed an increase in NSAA total score compared to placebo at 48 weeks; however, the difference was not statistically significant (P=0.37). At every time point measured, the cohort of SRP-9001 treated participants outperformed the placebo group, and, at 48 weeks, participants in the treatment group demonstrated a statistically significant increase of 1.7 points in NSAA total score compared to baseline (P=0.009), while participants in the placebo group saw an increase of 0.9 points on the NSAA total score compared to baseline, which was not statistically significant (n=21, P=0.1411).

Study randomization was stratified by age group and, in the pre-specified analysis of participants aged 4-5 (n=16) at the time of treatment, the treatment group demonstrated a statistically significant 4.3-point improvement on NSAA total score at 48 weeks post treatment compared to a 1.9-point improvement in the age-matched placebo group (P=0.0172). The functional status at baseline for participants in the 4-5 age group was balanced across the placebo and treatment cohorts. A statistically significant imbalance (P=0.0046) in baseline NSAA total score was present in the cohort of 6-7-year-old participants (n=25), resulting in milder participants in the placebo arm (n=13) than in the treated arm (n=12). The significantly different baseline characteristics between treatment and control groups in the 6-7 age group may have contributed to the inability to observe a treatment effect in the 6-7 age group at the week 48 timepoint in Part 1.

The results from Study 102 reinforce the favorable safety and tolerability profile of SRP-9001 with no new safety signals identified. In line with previously reported clinical data, no clinical complement activation was observed. 85% of participants in the treatment group experienced at least one treatment-related adverse event compared to 43% in the placebo group. Among participants with treatment-related adverse events, 82% were mild or moderate in severity, and 4 participants experienced serious treatment-related adverse events including 3 participants in the treatment group (2 cases of rhabdomyolysis, 2 transaminase elevations) and 1 participant in the placebo (rhabdomyolysis).

Study 102 is ongoing and remains blinded to participants, investigators, site staff and sponsor staff with direct site interaction. All 41 participants have completed their Part 1, 48-week assessment and have entered the Part 2 crossover phase. Participants continue to be monitored for safety and will undergo another biopsy at week 12 in Part 2 to assess expression and biological markers, in addition to longer-term assessments of functional outcomes.

“Study 102 reinforces our confidence in the potentially transformative benefits of SRP-9001, including among other things, the fact that in the Study’s pre-specified analysis, the participants in the 4-5 age group robustly achieved a statistically significant and clinically meaningful improvement in NSAA over placebo, as predicted by our prior Study 101. For the entire population, while we saw separation at every time point between the active and placebo cohorts, Study 102 did not achieve statistical significance on the primary functional endpoint. In this regard, we are very disappointed that the randomization process resulted in a significant imbalance in baseline NSAA scores between the active and placebo cohorts of the participants ages 6-7, making the 6-7 age groups non-comparable and likely substantially contributing to the inability to achieve statistical significance,” said Doug Ingram, president and chief executive officer, Sarepta. “Study 102 remains blinded and we will analyze the functional results for all patients, including cross-over participants, once they have achieved the 48-week timepoint in Part 2. We have already enrolled and dosed 11 participants in Study 103, using our commercial process material, and we will have biomarker and safety results from that cohort in the second quarter. And very importantly, Study 102 has provided us with a wealth of information and insight which we will use to refine and complete the protocol for our upcoming trial using commercial process material. We intend to continue to move forward with diligence and urgency to generate the evidence necessary to bring SRP-9001 to waiting Duchenne patients around the world.”

Sarepta will host an investor webcast and conference call on Thursday, Jan. 7, 2021 at 4:30 pm Eastern Time, to discuss these results. 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 2538387. Please specify to the operator that you would like to join the "Micro-dystrophin SRP-9001 Study 102 Top-line Results Call."

*The NSAA is a 17-item rating scale that is used to measure functional motor abilities in ambulant children with Duchenne. It is used to monitor the progression of the disease and treatment effects which makes it suitable as an endpoint in clinical trials for Duchenne.

About SRP-9001-102

Study SRP-9001-102 (Study 102) is a double-blind, 1:1 randomized, placebo-controlled clinical trial of SRP-9001 in 41 participants with Duchenne muscular dystrophy between the ages of 4-7. Study 102 uses clinical process SRP-9001 and has two primary endpoints: micro-dystrophin expression at 12 weeks and change in NSAA total score at 48 weeks compared to placebo. Secondary endpoints include certain timed functional tests; micro-dystrophin expression measured by immunofluorescence (IF) fiber intensity; and micro-dystrophin expression measured by IF percent dystrophin positive fibers. In Part 1, results from the treatment and placebo groups are compared through 48 weeks following treatment. In Part 2, the study remains blinded while all participants in the placebo group cross over to active treatment and all participants are followed for another 48 weeks while safety and efficacy continue to be evaluated.

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, the Company announced a licensing agreement granting Roche the exclusive right to launch and commercialize SRP-9001 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, neck 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

At Sarepta, we are leading a revolution in precision genetic medicine and every day is an opportunity to change the lives of people living with rare disease. The Company has built an impressive position in Duchenne muscular dystrophy (DMD) and in gene therapies for limb-girdle muscular dystrophies (LGMDs), mucopolysaccharidosis type IIIA, Charcot-Marie-Tooth (CMT), and other CNS-related disorders, with more than 40 programs in various stages of development. The Company's programs and research focus span several therapeutic modalities, including RNA, gene therapy and gene editing. For more information, please visit www.sarepta.com or follow us on Twitter, LinkedIn, Instagram and Facebook.

Sarepta Forward-Looking Statement

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 transformative benefits of SRP-9001; our plan to analyze the functional results for all the patients in Study 102 once they have achieved the 48-week timepoint; the expectation to have biomarker and safety results from Study 103 in the second quarter of 2021; our plan to use the information and insight from Study 102 to refine and complete the protocol for our upcoming trial using commercial process material; and our intention to continue to move forward with diligence and urgency to generate the evidence necessary to bring SRP-9001 to waiting Duchenne patients around the world.

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, 2019, 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.

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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