Sarepta Therapeutics Reports Sustained Functional Improvement Two Years After Treatment with SRP-9001, its Investigational Micro-dystrophin Gene Therapy for Duchenne Muscular Dystrophy

9/28/20

--Results demonstrate continued safety and tolerability of SRP-9001 in four participants with Duchenne --

--All four participants demonstrated improvements in NSAA scores compared to baseline and showed a durable response two years after administration of SRP-9001 --

CAMBRIDGE, Mass., Sept. 28, 2020 (GLOBE NEWSWIRE) -- Sarepta Therapeutics, Inc. (NASDAQ:SRPT), the leader in precision genetic medicine for rare diseases, today announced two-year follow up results from four Duchenne muscular dystrophy (DMD) clinical trial participants who received SRP-9001 (AAVrh74.MHCK7.micro-dystrophin). SRP-9001 is an investigational gene transfer therapy intended to deliver its micro-dystrophin-encoding gene to muscle tissue for the targeted production of micro-dystrophin protein. Results presented at the 25th International Annual Congress of the World Muscle Society demonstrated that two years after a one-time infusion of SRP-9001, trial participants exhibited a mean 7.0 point improvement on the North Star Ambulatory Assessment (NSAA) compared to baseline.

"We continue to be encouraged by the safety profile and enduring treatment response that we have seen to date with SRP-9001 gene transfer therapy," said Doug Ingram, President and CEO, Sarepta. "The consistent results and functional improvements sustained over two years give us added confidence as we prepare for the results from Study 102, our randomized, double-blind, placebo-controlled study of SRP-9001. We continue to work with urgency to bring this potentially transformative treatment to patients as quickly as possible."

In the open-label trial, known as Study 101, four ambulatory participants between the ages of 4 and 7 were treated with an infusion of SRP-9001 at a dose of $2x10^{14}$ vg/kg. The therapy was well-tolerated in all participants over the two-year time period. All adverse events were considered mild or moderate, and occurred within 90 days of treatment. There were no serious adverse events or evidence of complement activation.

At day 90, all participants had confirmed vector transduction and showed functional improvement on the NSAA scale and reduced creatine kinase (CK) levels. Participants demonstrated a mean increase of 5.5 points from baseline one year after treatment and 7.0 points from baseline two years after treatment. The NSAA is a validated scale developed to measure functional motor abilities in ambulant children with Duchenne, with scores ranging from 0-34.

As previously disclosed, micro-dystrophin protein levels for participants in Study 101 were as follows: 12-weeks post-infusion, a mean of 81.2% muscle fibers expressing micro-dystrophin with a mean intensity at the sarcolemma by immunohistochemistry of 96% compared to normal biopsies. Adjusted for fat and fibrotic tissue, western blot showed a mean expression of 95.8%.

About SRP-9001
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. 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 SRP-9001's intention to deliver the micro-dystrophin-encoding gene to muscle tissue for the targeted production of the micro-dystrophin protein, the potential benefits of SRP-9001 and its potential to provide a transformative treatment for DMD, the future results from Study 102 and potential market opportunities.

These forward-looking statements involve risks and uncertainties, many of which are beyond our control. Known risk factors include, among others:

success in preclinical trials 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 thereof or result in a safe or effective treatment benefit; 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 the FDA or foreign regulatory authorities; if the actual number of patients suffering from DMD is smaller than estimated, our revenue and ability to achieve profitability may be adversely affected; 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; and 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, 2019, 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.

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.

Investors:
Ian Estepan, 617-274-4052
iestepan@sarepta.com

Media:
Tracy Sorrentino, 617-301-8566
tsorrentino@sarepta.com