Sarepta Therapeutics Provides Update on SRP-5051 for the Treatment of Duchenne Muscular Dystrophy

6/23/22

- ***Sarepta to host conference call at 4:15 p.m. Eastern time***

CAMBRIDGE, Mass., June 23, 2022 (GLOBE NEWSWIRE) -- Sarepta Therapeutics, Inc. (NASDAQ:SRPT), the leader in precision genetic medicine for rare diseases, today announced that the U.S. Food and Drug Administration (FDA) has placed a clinical hold on SRP-5051 (vesleteplirsen), the Company's next-generation peptide-conjugated phosphorodiamidate morpholino oligomer (PPMO) to treat patients with Duchenne muscular dystrophy who are amenable to exon 51 skipping. The hold in Part B of Study 5051-201, also known as MOMENTUM, follows a serious adverse event of hypomagnesemia. FDA is requesting information on all cases of hypomagnesemia, including a small number of non-serious grade 2 cases, and to assess the adequacy of the risk mitigation and safety monitoring plan. In the next few days we will respond to the Agency with this information and proposed changes to the monitoring plan.

"Patient safety is always our top priority. The hypomagnesemia was identified through lab tests conducted as part of the monitoring outlined in the protocol of the MOMENTUM study and is similar to previously observed cases of hypomagnesemia in clinical trials of SRP-5051. The hypomagnesemia was transient and patients' magnesium levels returned to normal following additional supplementation," said Louise Rodino-Klapac, Ph.D., executive vice president and chief scientific officer, Sarepta Therapeutics. "Globally, we have enrolled approximately half of the planned patients in Part B of MOMENTUM. The study is ongoing, and we remain on track to complete enrollment by the end of the year. We will work to share information with FDA with the goal of resuming screening and dosing in the U.S. as quickly as possible."

Sarepta will host an investor conference call on Thurs., June 23, 2022 at 4:15 pm Eastern time, to discuss this update. The presentation will be webcast live under the investor relations section of Sarepta's website at **https://investorrelations.sarepta.com/events-presentations** and 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 6055778. Please specify to the operator that you would like to join the "SRP-5051 Program Update."

About SRP-5051 (vesleteplirsen)
SRP-5051 is an investigational agent using Sarepta's PPMO chemistry and exon-skipping technology to skip exon 51 of the dystrophin gene. SRP-5051 is designed to bind to exon 51 of dystrophin pre-mRNA, resulting in exclusion of this exon during mRNA processing in patients with genetic mutations that are amenable to exon 51 skipping. Exon skipping is intended to allow for production of an internally shortened functional dystrophin protein. PPMO is Sarepta's next-generation chemistry platform designed around a proprietary cell-penetrating peptide conjugated to the PMO backbone, with the goal of increasing tissue penetration, increasing exon skipping, and significantly increasing dystrophin production. Around 13% of DMD patients have mutations that make them amenable to skipping exon 51. If successful, the PPMO offers the potential for improved efficacy and less frequent dosing for patients.

About MOMENTUM (Study SRP-5051-201)
MOMENTUM is Phase 2, multi-arm, ascending dose study of SRP-5051, infused monthly and will assess dystrophin protein levels in skeletal muscle tissue following SRP-5051 treatment. The study will enroll up to 60 participants, both ambulant and non-ambulant, between the ages of 7 to 21 at sites in the U.S., Canada, and the European Union. The study will also assess safety and tolerability.

In 2021, the Company announced results from Part A of MOMENTUM showing that after 12 weeks, 30 mg/kg of SRP-5051 dosed monthly resulted in 18 times the exon skipping and eight times the dystrophin production as eteplirsen, dosed weekly for 24 weeks. Reversible hypomagnesemia was identified in patients taking SRP-5051. The protocol for Part B of MOMENTUM includes magnesium supplementation and monitoring of magnesium levels.

More information can be found on www.clinicaltrials.gov.

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 potential benefits of PPMO and SRP-5051; our belief that the hypomagnesemia identified in MOMENTUM is similar to previously observed cases in clinical trials of SRP-5051; our approach to monitoring and managing hypomagnesemia; and our expected timelines, plans, and milestones, including completing enrollment of Part B of MOMENTUM by the end of the year, our plan to respond to the Agency with the requested information and

proposed changes to the monitoring plan in the next few days, and resuming screening and dosing in the U.S. as quickly as possible.

These forward-looking statements involve risks and uncertainties, many of which 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, and 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; 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 ongoing COVID-19 pandemic; 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.

Source: Sarepta Therapeutics, Inc.

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

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