Sarepta Therapeutics Announces That FDA has Lifted its Clinical Hold on SRP-5051 for the Treatment of Duchenne Muscular Dystrophy

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CAMBRIDGE, Mass., Sept. 06, 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 removed the clinical hold on SRP-5051 (vesleteplirsen), the Company's investigational, next-generation peptide-conjugated phosphorodiamidate morpholino oligomer (PPMO) to treat patients with Duchenne muscular dystrophy who are amenable to exon 51 skipping. After discussions with FDA and as part of the lift, Sarepta will adjust the global trial protocol to include expanded monitoring of urine biomarkers.

The hold in Part B of Study 5051-201, also known as MOMENTUM, followed a serious adverse event of hypomagnesemia. Information was provided by the Company to FDA to assess the adequacy of the risk mitigation and safety monitoring plan.

"We would like to thank FDA for working closely with us to expeditiously resolve this clinical hold. We will implement the changes in the protocol to resume dosing in the U.S. as quickly as possible," said Louise Rodino-Klapac, Ph.D., executive vice president and chief scientific officer, Sarepta Therapeutics. "Our monitoring plan is designed to mitigate the risks of hypomagnesemia. MOMENTUM has continued enrolling participants outside the U.S., and we remain on track to complete enrollment by the end of 2022. Sarepta is committed to the SRP-5051 program and excited about the PPMO platform as a next-generation exon-skipping approach for the treatment of Duchenne."

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 a Phase 2, multi-arm, ascending dose trial of SRP-5051, infused monthly and will assess dystrophin protein levels in skeletal muscle tissue following SRP-5051 treatment. The trial 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 trial 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 adjustments to our global trial protocol for SRP-5051; the potential benefits of PPMO and 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 2022, 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 studies 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.

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