Sarepta Therapeutics to Initiate Part B of MOMENTUM Study of SRP-5051 in Patients with Duchenne Muscular Dystrophy Amenable to Exon 51 Skipping Following Positive Interactions with FDA

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- Company Anticipates Part B of MOMENTUM to Serve as Pivotal Study for SRP-5051 and to Seek Accelerated Approval if Successful

- Ambulatory and Non-Ambulatory Patients Between the Ages of 7 to 21 Will Be Eligible to Enroll in Part B of MOMENTUM

CAMBRIDGE, Mass., Sept. 27, 2021 (GLOBE NEWSWIRE) -- Sarepta Therapeutics, Inc. (NASDAQ:SRPT), the leader in precision genetic medicine for rare diseases, today announced that following positive interactions with the U.S. Food and Drug Administration (FDA), the Company plans to initiate Part B of the MOMENTUM study (Study 5051-201), in the fourth quarter. MOMENTUM is a global trial investigating the use of SRP-5051, 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 study will enroll between 20-40 patients between ages 7 to 21 amenable to exon 51 skipping who are naïve to SRP-5051. Additionally, those previously dosed in Study 5051-201, Part A or Study 5051-102 who meet the entrance criteria will be eligible to participate. Both ambulatory and non-ambulatory patients are eligible for participation. The Company will submit the protocol in the next week.

"If proven safe and as efficacious as our initial data suggest it may be, SRP-5051 could be the first in a host of transformative therapies from our next-generation PPMO platform to treat and improve the lives of children living with Duchenne muscular dystrophy. In addition to the fantastic work of our team, and the dedication of Duchenne families and the program's investigators, I would like to thank FDA's Division of Neurology I for their diligence and thoughtful advice and input, without which we would not be able to commence Part B of the MOMEMTUM study ahead of schedule," said Doug Ingram, Sarepta's president and chief executive officer.

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

In May of this year, the Company announced results from Part A of the MOMENTUM study 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 will include magnesium supplementation and monitoring of magnesium levels.

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

About SRP-5051
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 truncated 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 which make them amenable to skipping exon 51. If successful, the PPMO offers the potential for improved efficacy and less frequent dosing for patients.

About Duchenne Muscular Dystrophy (DMD)
DMD is an X-linked rare degenerative neuromuscular disorder causing severe progressive muscle loss and premature death. One of the most common fatal genetic disorders, DMD affects approximately one in every 3,500 - 5,000 male births worldwide. A devastating and incurable muscle wasting disease, DMD is associated with specific errors in the gene that codes for dystrophin, a protein that plays a key structural role in muscle fiber function. Progressive muscle weakness in the lower limbs spreads to the arms, neck and other areas of the body. The condition is universally fatal, and death usually occurs before the age of 30 due to respiratory or cardiac failure.

About EXONDYS 51
EXONDYS 51 (eteplirsen) uses Sarepta's proprietary phosphorodiamidate morpholino oligomer (PMO) chemistry and exon-skipping technology to bind to exon 51 of dystrophin pre-mRNA, resulting in "skipping" 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 truncated dystrophin protein.

EXONDYS 51 is indicated for the treatment of Duchenne muscular dystrophy in patients who have a confirmed mutation of the DMD gene that is amenable to exon 51 skipping.

This indication is approved under accelerated approval based on an increase in dystrophin production in skeletal muscle observed in some patients treated with EXONDYS 51. Continued approval may be contingent upon verification of a clinical benefit in confirmatory trials.

EXONDYS 51 has met the full statutory standards for safety and effectiveness and as such is not considered investigational or experimental.

Important Safety Information About EXONDYS 51

Hypersensitivity reactions, including rash and urticaria, pyrexia, flushing, cough, dyspnea, bronchospasm, and hypotension, have occurred in patients who were treated with EXONDYS 51. If a hypersensitivity reaction occurs, institute appropriate medical treatment and consider slowing the infusion or interrupting the EXONDYS 51 therapy.

Adverse reactions in DMD patients (N=8) treated with EXONDYS 51 30 mg or 50 mg/kg/week by intravenous (IV) infusion with an incidence of at least 25% more than placebo (N=4) (Study 1, 24 weeks) were (EXONDYS 51, placebo): balance disorder (38%, 0%), vomiting (38%, 0%) and contact dermatitis (25%, 0%). The most common adverse reactions were balance disorder and vomiting. Because of the small numbers of patients, these represent crude frequencies that may not reflect the frequencies observed in practice. The 50 mg/kg once weekly dosing regimen of EXONDYS 51 is not recommended.

In the 88 patients who received ≥30 mg/kg/week of EXONDYS 51 for up to 208 weeks in clinical studies, the following events were reported in ≥10% of patients and occurred more frequently than on the same dose in Study 1: vomiting, contusion, excoriation, arthralgia, rash, catheter site pain, and upper respiratory tract infection.

For further information, please see the full **Prescribing Information**.

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.

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 our expected timing for submission of the Part B MOMENTUM Study Protocol to the FDA; the potential benefits of SRP-5051; our plans to seek accelerated approval for SRP-5051; and the potential benefits of PPMO, including offering greater efficacy with less frequent dosing.

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; 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, 2020, Sarepta's quarterly reports on Form 10-Q, as well as other Securities and Exchange Commission (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, except as required by law.

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.

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

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