



Sarepta Therapeutics Completes Submission of New Drug Application Seeking Approval of Casimersen (SRP-4045) for Patients with Duchenne Muscular Dystrophy Amenable to Skipping Exon 45

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-- Casimersen is designed for the treatment of exon 45 amenable patients, approximately eight percent of patients with Duchenne --

-- Casimersen is the third exon-skipping medicine using the Company's proprietary PMO RNA-based platform --

CAMBRIDGE, Mass., June 26, 2020 (GLOBE NEWSWIRE) -- Sarepta Therapeutics, Inc. (NASDAQ:SRPT), the leader in precision genetic medicine for rare diseases, today announced the Company has completed the submission of a rolling New Drug Application (NDA) to the U.S. Food and Drug Administration (FDA) seeking accelerated approval for casimersen (SRP-4045). Casimersen, a phosphorodiamidate morpholino oligomer (PMO), is engineered to treat patients with Duchenne muscular dystrophy (DMD) who have genetic mutations that are amenable to skipping exon 45 of the Duchenne gene.

The completion of the rolling submission includes data from the casimersen arm of the ESSENCE study (also known as study 4045-301), a global, randomized, double-blind, placebo-controlled Phase 3 study evaluating efficacy and safety in patients amenable to skipping exons 45 and 53. An [interim analysis from ESSENCE](#) demonstrated a statistically significant increase in dystrophin production as measured by western blot in patients who received casimersen compared to baseline and placebo. The study is ongoing, and remains blinded to collect additional efficacy and safety data. If the casimersen NDA is accepted and granted accelerated approval, the completed ESSENCE study will serve as a post-marketing confirmatory study.

"The completion of our casimersen submission is an important milestone in our journey to advance treatments for the greatest possible number of people living with Duchenne muscular dystrophy," said Doug Ingram, president and chief executive officer, Sarepta Therapeutics. "If approved, casimersen will be our third approved therapy for sub-populations of Duchenne. Together with our other approved therapies, we have the potential to treat nearly 30% of Duchenne patients in the United States. Our proprietary PMO platform is an important focus of our pipeline, and we owe our clinical progress to the patients and families participating in our studies."

About Casimersen

Casimersen uses Sarepta's proprietary phosphorodiamidate morpholino oligomer (PMO) chemistry and exon-skipping technology to skip exon 45 of the DMD gene. Casimersen is designed to bind to exon 45 of dystrophin pre-mRNA, resulting in exclusion, or "skipping," of this exon during mRNA processing in patients with genetic mutations that are amenable to exon 45 skipping. Exon skipping is intended to allow for production of an internally truncated dystrophin protein.

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](#).

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 potential benefits of casimersen and the PMO technology; the potential of casimersen, if approved, to treat approximately 8% of patients with DMD; Sarepta's potential to treat nearly 30% of DMD patients in the United States; the expectation that the completed ESSENCE study will serve as a post-marketing confirmatory study if the casimersen NDA is accepted and granted accelerated approval; and Sarepta's goal to advance treatments for the greatest possible number of people living with DMD.

These forward-looking statements involve risks and uncertainties, many of which are beyond Sarepta's control. Known risk factors include, among others: Sarepta may not be able to complete clinical trials required by the FDA or other regulatory authorities for approval of casimersen; casimersen may not result in a viable treatment suitable for commercialization due to a variety of reasons including the results of future research may not be consistent with past positive results or may fail to meet regulatory approval requirements for the safety and efficacy of product candidates; and even if casimersen results in a commercialized product, Sarepta may not achieve any significant revenues from the sale of such product; if the actual number of patients suffering from DMD is smaller than estimated, our revenue and ability to achieve profitability may be adversely affected; Sarepta may not

be able to execute on its business plans and goals, including meeting its expected or planned regulatory milestones and timelines, clinical development plans, and bringing its product candidates to market, due to a variety of reasons, many of which may be outside of Sarepta's 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 Sarepta's product candidates and the 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, 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 Sarepta which you are encouraged to review.

Any of the foregoing risks could materially and adversely affect Sarepta'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.

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