



Sarepta Therapeutics Announces FDA Accelerated Approval of EXONDYS 51™ (eteplirsen) injection, an Exon Skipping Therapy to Treat Duchenne Muscular Dystrophy (DMD) Patients Amenable to Skipping Exon 51

-- EXONDYS 51™ the first DMD treatment approved in the US, targets dystrophin deficiency, the underlying cause of Duchenne --

--U.S. Commercial Launch planned to commence immediately--

--Conference call Scheduled for September 19, 2016, 4:00 p.m. EST--

CAMBRIDGE, Mass.--(BUSINESS WIRE)—September 19, 2016--Sarepta Therapeutics, Inc. (NASDAQ:SRPT), a developer of innovative RNA-targeted therapeutics, today announced that the U.S. Food and Drug Administration (FDA) has granted accelerated approval for EXONDYS 51™ (eteplirsen) as a once weekly intravenous infusion of 30 milligrams per kilogram for the treatment of Duchenne muscular dystrophy (DMD) in patients who have a confirmed mutation in the DMD gene that is amenable to exon 51 skipping. This indication is based on an increase in dystrophin in skeletal muscles observed in some patients treated with EXONDYS 51. A clinical benefit of EXONDYS 51 has not been established. Continued approval for this indication may be contingent upon verification of a clinical benefit in confirmatory trials. The most common adverse reactions compared to a placebo group were vomiting (38%) and balance disorder (38%) with contusion, excoriation, arthralgia, rash, catheter site pain, and upper respiratory tract infection also reported more frequently than placebo ($\geq 10\%$).

“Today’s accelerated approval of EXONDYS 51 represents a major milestone in the treatment of Duchenne Muscular Dystrophy for patients amenable to skipping exon 51 by targeting the underlying genetic cause of the disease – the lack of the dystrophin protein,” said Edward Kaye, M.D., Sarepta’s interim chief executive officer and chief medical officer. “We are grateful to the many patients and investigators who participated in EXONDYS 51’s clinical studies. EXONDYS 51 represents the culmination of many years of work across our entire organization and the Duchenne community to address a critical unmet need by bringing this novel medicine to patients. We will continue to leverage what we have learned from EXONDYS 51 to facilitate future development of potential new treatments targeting additional exons with the goal of one day treating all DMD patients amenable to exon skipping.”

The underlying cause of Duchenne muscular dystrophy is a mutation or error in the gene for dystrophin, an essential protein involved in muscle fiber function. Certain genetic mutations in DMD involve the deletion of exons, which interrupt proper translation of the genetic code into protein.

Duchenne muscular dystrophy is a fatal genetic neuromuscular disorder affecting an estimated one in approximately every 3,500 – 5,000 males born worldwide. It is estimated that up to thirteen percent of people with DMD have mutations addressable by EXONDYS 51.

Patients and physicians can access information by visiting www.SareptAssist.com or calling 1-888-727-3782.

Conference Call

The Company will be hosting a conference call at 4:00 p.m. EST. The conference call may be accessed by dialing (844) 534-7313 for domestic callers and (574) 990-1451 international callers. The passcode for the call is 85217990. Please specify to the operator that you would like to join the “Sarepta Corporate Update.” The conference call will be webcast live under the investor relations section of Sarepta’s website at www.sarepta.com and will be archived there following the call for 90 days. 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.

About Duchenne Muscular Dystrophy (DMD)

DMD is an X-linked rare degenerative neuromuscular disorder causing severe progressive muscle loss and premature death. DMD is estimated to affect approximately one in every 3,500-5000 males born 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. 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 death usually occurs before the age of 30.

About EXONDYS 51™

EXONDYS 51 uses Sarepta’s proprietary phosphorodiamidate morpholino oligomer (PMO) chemistry and exon-skipping technology to skip exon 51 of the dystrophin gene. EXONDYS 51 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. Data from clinical studies of EXONDYS 51 in a small number of DMD patients have demonstrated a consistent safety and tolerability profile. The pivotal trials were not designed to evaluate long-term safety and a clinical benefit of EXONDYS 51 has not been established.

Important Safety Information

- Adverse reactions observed in patients (N=8) treated with 30 or 50 mg/kg/wk of EXONDYS 51 with incidence \geq 25% and higher than in the placebo group (N=4) (Study 1) were: balance disorder (38%), vomiting (38%) and contact dermatitis (25%). The most common adverse reactions were balance disorder and vomiting.
- The following events were reported in \geq 10% of patients treated with EXONDYS 51 for up to 208 weeks (N=88) and occurred more frequently than placebo in a controlled trial for 24 weeks (Study 1): vomiting, contusion, excoriation, arthralgia, rash, catheter site pain, and upper respiratory tract infection.
- There have been reports of transient erythema, facial flushing, and elevated temperature occurring on the day of EXONDYS 51 infusion.

For the full prescribing information please refer to U.S. Full Prescribing Information at www.EXONDYS51.com

About Sarepta Therapeutics

Sarepta Therapeutics is a biopharmaceutical company focused on the discovery and development of unique RNA-targeted therapeutics for the treatment of rare neuromuscular diseases. The Company is primarily focused on rapidly advancing the development of its potentially disease-modifying DMD drug candidates, including EXONDYS 51, designed to skip exon 51 and approved under the accelerated approval pathway. For more information, please visit us at www.sarepta.com.

Forward Looking Statements

This press release contains "forward-looking statements" within the meaning of the safe harbor provisions of the U.S. Private Securities Litigation Reform Act of 1995. 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 EXONDYS 51's continued approval for its indication potentially being contingent upon verification of a clinical benefit in confirmatory trials; the potential benefits and risks of EXONDYS 51; the accelerated approval of EXONDYS 51 representing a major milestone in the treatment of DMD for patients amenable to skipping exon 51 by targeting the underlying genetic cause of the disease; EXONDYS 51's potential to address an unmet need and being a novel medicine; Sarepta's plans to continue to leverage what it has learned from EXONDYS 51 to facilitate future development of potential new treatments targeting additional exons with the goal of someday treating all DMD patients amenable to exon skipping; the estimated number of patients worldwide of patients with DMD and the estimated percentage of people with DMD that have mutations addressable by EXONDYS 51 and Sarepta's plans for a US Commercial launch to commence immediately.

These forward-looking statements involve risks and uncertainties, many of which are beyond Sarepta's control or unknown. Known risk factors include, among others: the planned commercial launch in the US for EXONDYS 51 may not be successful in part or at all for various reasons including the actual market size and drug supply needed may not be consistent with the company's expectations and its executed commercial readiness plans, the degree to which EXONDYS 51 is accepted by patients and prescribed by physicians, the efficiency of our manufacturing, sales, distribution and specialty pharmacy network in getting Exondys51 to the market and future economic, competitive, reimbursement and regulatory conditions that could negatively impact the commercial launch of EXONDYS 51; the confirmatory and other studies for Exondys51 may not yield data consistent with prior results or demonstrate a benefit that supports continued or full regulatory approval; we may not be able to complete clinical trials required by the FDA or other regulatory authorities for approval of our pipeline of exon-skipping products; the results of our ongoing research and development efforts

and clinical trials for our product candidates and our technologies may not be positive or consistent with prior results or demonstrate a safe treatment benefit or support an NDA filing, positive advisory committee recommendation or marketing approval by the FDA or other regulatory authority; we may not be able to execute on our business plans including meeting our expected or planned regulatory milestones and timelines, clinical development plans and bringing our product candidates to market, including the commercialization of EXONDYS 51, for various reasons, including factors outside of the Company's control, such as possible limitations of Company financial and other resources, manufacturing limitations that may not be anticipated or resolved for in a timely manner or at all, and 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 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, 2015 or Quarterly Report on Form 10-Q for the quarter ended June 30, 2016 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 Company's filings with the SEC. 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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