

Sarepta Therapeutics Announces Plans to Submit New Drug Application to FDA for Eteplirsen for the Treatment of Duchenne Muscular Dystrophy in First Half of 2014

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CAMBRIDGE, MA -- (Marketwired) -- 07/24/13 -- Sarepta Therapeutics, Inc. (NASDAQ: SRPT), a developer of innovative RNA-based therapeutics, today announced it plans to submit a New Drug Application (NDA) to the U.S. Food and Drug Administration (FDA) in the first half of 2014 for the approval of eteplirsen for the treatment of Duchenne muscular dystrophy (DMD). Eteplirsen is Sarepta's lead exon-skipping compound in development for the treatment of patients with DMD who have a genotype amenable to skipping of exon 51.

The decision to submit an NDA for eteplirsen in 2014 is based on productive interactions with the FDA in a meeting that occurred this week. That meeting was a follow-up to the FDA's review of two recently submitted summary documents that included data on dystrophin and clinical outcomes from the existing eteplirsen studies. The FDA stated in pre-meeting comments that the Agency is "open to considering an NDA based on these data for filing." The Agency, however, requested additional information related to the methodology and verification of dystrophin quantification. Sarepta believes the requests from the Agency can be addressed and incorporated into an NDA submission in the first half of 2014.

"We are encouraged by the feedback from the FDA and believe that data from our ongoing clinical study merits review by the Agency and will be sufficient for an NDA filing," said Chris Garabedian, president and chief executive officer of Sarepta Therapeutics. "We plan to work closely with the FDA to prepare an NDA submission in the first half of 2014 as we continue to prepare for our confirmatory study and our manufacturing scale up."

The Agency would not commit to declaring dystrophin an acceptable surrogate endpoint under the CFR 314 Subpart H Accelerated Approval pathway prior to an NDA filing and commented that a decision by the Agency to file "the NDA would not indicate that we have accepted dystrophin expression as a biomarker reasonably likely to predict clinical benefit. A filing would only indicate that the question merits review, and that we deem the data to be reviewable."

Sarepta anticipates submitting an NDA for eteplirsen in the first half of 2014; however, the exact timing of the submission will be dependent on further discussions and agreement with the FDA on the information needed for an acceptable filing. Sarepta also intends to have an End-of-Phase II meeting with the agency later this quarter to discuss the requirements for the Chemistry, Manufacturing, and Controls (CMC) section of the NDA.

Conference Call Information

Sarepta will hold a conference call to discuss this update today at 8:00 a.m. EDT (5:00 a.m. PDT). The conference call may be accessed by dialing 800.446.2782 for domestic callers and 847.413.3235 for international callers. The passcode for the call is 35357086. Please specify to the operator that you would like to join the "Sarepta Therapeutics Regulatory Update Call." The conference call will be webcast live under the events 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. An audio replay will be available through August 14, 2013 by calling 888.843.7419 or 630.652.3042 and entering access code 35357086.

About Duchenne Muscular Dystrophy

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 boys 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 patients usually succumb to the disease in their twenties.

About Eteplirsen

Eteplirsen is Sarepta's lead drug candidate and is designed to address the underlying cause of DMD by enabling the production of a functional dystrophin protein. Data from clinical studies of eteplirsen in DMD patients have demonstrated a broadly favorable safety and tolerability profile and restoration of dystrophin protein expression.

Eteplirsen uses Sarepta's novel phosphorodiamidate morpholino oligomer (PMO)-based chemistry and proprietary exon-skipping technology to skip exon 51 of the dystrophin gene enabling the repair of specific genetic mutations that affect approximately 13 percent of the total DMD population. By skipping exon 51, eteplirsen may restore the gene's ability to make a shorter, but still functional, form of dystrophin from messenger RNA, or mRNA. Promoting the synthesis of a truncated dystrophin protein is intended to stabilize or significantly slow the disease process and prolong and improve the quality of life for patients with DMD.

Sarepta is also developing other PMO-based exon-skipping drug candidates intended to treat additional patients with DMD.

About Sarepta Therapeutics

Sarepta Therapeutics is focused on developing first-in-class RNA-based therapeutics to improve and save the lives of people affected by serious and life-threatening rare and infectious diseases. The Company's diverse pipeline includes its lead program eteplirsen, for Duchenne muscular dystrophy, as well as potential treatments for some of the world's most lethal infectious diseases. Sarepta aims to build a leading, independent biotech company dedicated to translating its RNA-based science into transformational therapeutics for patients who face significant unmet medical needs. For more information, please visit us at www.sarepta.com.

Forward Looking Statement

This press release contains forward-looking statements. These forward-looking statements generally can be identified by use of words such as "believes or belief," "anticipates," "plans," "expects," "will," "intends," "potential," "possible," "advance" and similar expressions. These forward-looking statements include statements about the timing of an NDA submission for eteplirsen in the treatment of DMD; the potential filing and acceptance of an NDA for eteplirsen; the information necessary for the Agency to make regulatory determinations and our ability to provide such information; the potential regulatory approval of eteplirsen (including under Subpart H Accelerated Approval); and the potential and timing for manufacturing scale up.

Each forward-looking statement contained in this press release is subject to risks and uncertainties that could cause actual results to differ materially from those expressed or implied by such statement. Applicable risks and uncertainties include, among others: the FDA may determine that our NDA submission for eteplirsen does not qualify for filing or that substantial additional data is required for accelerated or other approvals; other decisions by regulatory authorities; and those identified under the heading "Risk Factors" in Sarepta's Quarterly Report on Form 10-Q for the quarter ended March 31, 2012, and filed with the Securities and Exchange Commission, and Sarepta's other filings with the SEC.

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 Securities and Exchange Commission. 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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