Sarepta Therapeutics Announces FDA Will Consider Accelerated Approval for Eteplirsen After Further Review of Data on Dystrophin and Clinical Outcomes

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Eteplirsen Manufacturing and Clinical Activities Continue as Planned

CAMBRIDGE, MA -- (Marketwired) -- 04/15/13 -- Sarepta Therapeutics, Inc. (NASDAQ: SRPT) today provided an update on its discussions with the U.S. Food and Drug Administration (FDA) regarding a potential application for accelerated approval of eteplirsen for the treatment of Duchenne muscular dystrophy (DMD). The FDA has requested that Sarepta provide additional information from the existing eteplirsen dataset to inform a decision on the acceptability of this dataset for a New Drug Application (NDA) filing under the Subpart H Accelerated Approval regulatory pathway. This feedback was provided in meeting minutes from Sarepta's End-of-Phase II meeting with the FDA's Division of Neurology Products that occurred last month.

Specifically, Sarepta received feedback on both the acceptability of dystrophin as a surrogate endpoint that would reasonably predict clinical benefit in DMD patients and the acceptability of the eteplirsen safety database for a Subpart H Accelerated Approval filing.

"We are encouraged by our interactions with the FDA and their Division of Neurology Products and we view their request for more data as a reflection of the thorough and comprehensive approach that the Agency takes in evaluating a new surrogate marker," said Chris Garabedian, president and chief executive officer of Sarepta Therapeutics. "We are confident that the method in which we've collected dystrophin, the degree and consistency of the dystrophin levels, and the supporting clinical data will provide the Agency the information it needs to determine if dystrophin is a feasible surrogate marker that is reasonably likely to predict clinical benefit."

At the End-of-Phase II Clinical Meeting in March, there was a productive discussion on the suitability of dystrophin as a surrogate marker, including a presentation by Sarepta detailing the methodologies used to analyze dystrophin in the studies and supportive data suggesting that the dystrophin produced is functional. As follow up to the discussion, and as reflected in the meeting minutes from the Division of Neurology Products, the Agency requested two written summaries: *"a coherent and comprehensive summary to support dystrophin as a surrogate"* and *"a detailed discussion of all clinical outcomes in the eteplirsen study."*

Furthermore, the meeting minutes contained the following statement:

"The Agency stated that they had not made a final decision regarding acceptability of the proposed Subpart H (Accelerated Approval) NDA filing, and that the Agency would consider the additional data submitted by the sponsor before making a final decision."

Sarepta also discussed the eteplirsen and phosphorodiamidate morpholino oligomer (PMO) safety database at the End-of-Phase II meeting and asked if the 38 patient eteplirsen safety database was sufficient for a Subpart H Accelerated Approval filing. While Sarepta will continue to collect long-term safety from the ongoing eteplirsen extension study for a potential submission, the meeting minutes stated that: *"In the event (the Agency) agrees to file the Subpart H NDA submission, additional safety data to support approval could come from the first few months of the... pivotal confirmatory study."* Sarepta still intends to begin dosing patients in a pivotal confirmatory study in the first quarter of 2014.

Sarepta is preparing to submit the dystrophin and clinical outcomes summaries and will be requesting a follow-up meeting with the FDA to discuss these later this quarter. As a result, the End-of-Phase II CMC meeting with the FDA is now expected to occur in the third quarter, however all eteplirsen manufacturing and clinical development activities continue as planned and are not anticipated to delay the potential timeline of an Accelerated Approval NDA submission.

Conference Call Information

Sarepta will hold a conference call to discuss this update today at 5:00 p.m. EDT. 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 34696256. Please specify to the operator that you would like to join the "Sarepta Therapeutics Corporate Conference Call." The conference call will

be webcast live under the events section of Sarepta's website at www.sareptatherapeutics.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 May 9, 2013 by calling 888.843.7419 or 630.652.3042 and entering access code 34696256.

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 eventually 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 Eteplirsen

Eteplirsen is Sarepta's lead drug candidate that is designed to address the underlying cause of DMD by enabling the production of a functional dystrophin protein. Eteplirsen uses Sarepta's phosphorodiamidate morpholino oligomer (PMO)-based chemistry and 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 improve, stabilize or significantly slow the disease process and prolong and improve the quality of life for 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.sareptatherapeutics.com.

About the Accelerated Approval Regulatory Pathway (21 CFR 314 Subpart H)

To speed the development and availability of new medicines for serious and life-threatening diseases, the FDA has the authority to grant accelerated approval based on evidence that an investigational new drug provides a meaningful therapeutic benefit over existing treatments. Under this authority, marketing approval may be granted on the basis of adequate and well-controlled clinical trials that show the drug has an effect on a surrogate endpoint that is reasonably likely to predict clinical benefit or on the basis of a clinical endpoint other than survival or irreversible morbidity. If granted, the FDA requires post-marketing studies to verify the clinical benefit of the approved medicine. For more information, visit the FDA web site at: http://www.accessdata.fda.gov/scripts/cdrh/cfdocs/cfcfr/CFRSearch.cfm?CFRPart=314&showFR=1&subpartNode=21:5.0.1.1.4.8.

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 development of eteplirsen and its efficacy, safety, potency and utility in the treatment of DMD; the potential for the creation of novel dystrophin to lead to significant clinical benefit or its suitability as a valid surrogate marker; the amount and type of dystrophin, safety and other information necessary for the Agency to make regulatory determinations; the impact of manufacturing and development activities on NDA submission timelines; and the potential use of the accelerated approval pathway for eteplirsen.

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: subsequent clinical trials may fail to demonstrate the safety and efficacy of eteplirsen or replicate results; treatment of patients with DMD using eteplirsen over a longer duration may not lead to significant clinical benefit; any of Sarepta's drug candidates, including eteplirsen, may fail in development, may not qualify for filing under Subpart H accelerated approval or receive required regulatory approvals at all, or may not become commercially viable due to delays, decisions by regulatory authorities, or other reasons; and those identified under the heading "Risk Factors" in Sarepta's Annual Report on Form 10-K for the full year ended December 31, 2012, and filed with the Securities and Exchange Commission.

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.

"Safe Harbor" Statement under the Private Securities Litigation Reform Act of 1995: The statements that are not historical facts contained in this release are forward-looking statements that involve risks and uncertainties, including, but not limited to, the results of research and development efforts, the results of preclinical and clinical testing, the effect of regulation by the FDA and other agencies, the impact of competitive products, product development, commercialization and technological difficulties, and other risks detailed in the company's Securities and Exchange Commission filings.

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