Sarepta Therapeutics Announces Regulatory Update on Eteplirsen

Updated and additional guidance received from FDA on specific data requirements for NDA;
FDA states further discussion needed to determine what constitutes a "complete" NDA submission;
NDA submission planned for mid-year 2015;
Company to hold teleconference today at 8:00a.m. EDT

CAMBRIDGE, MA -- (Marketwire) -- 10/27/14 -- Sarepta Therapeutics, Inc. (NASDAQ: SRPT), a developer of innovative RNA-based therapeutics, today provided an update on its discussions with the U.S. Food and Drug Administration (FDA) regarding its planned New Drug Application (NDA) submission for the approval of eteplirsen for the treatment of Duchenne muscular dystrophy (DMD).

In meeting minutes received last week from a Type B Pre-NDA meeting that took place in September 2014, the FDA provided updated guidance regarding the specific data to be included as part of, or at the time of, Sarepta's NDA submission. The guidance states that additional data are now required as part of the NDA submission, including the results from an independent assessment of dystrophin images and the 168-week clinical data from study 202. Additionally, the guidance requests more specific data including a minimum duration of safety in new patients exposed to eteplirsen, patient-level natural history data to be obtained by Sarepta from independent academic institutions, and MRI data from a recent study conducted by an independent academic group. The FDA indicated that further discussion with Sarepta "will be necessary to determine what would constitute a complete NDA." Based on these requests, Sarepta plans to submit an NDA by mid-year 2015, pending any additional requests from further discussions with the FDA.

"We are committed to satisfying the FDA's updated requests for these specific data to be included as part of an NDA submission and will continue to work with the Agency toward the goal of a complete and acceptable NDA filing." said Chris Garabedian, president and chief executive officer of Sarepta Therapeutics. "We believe all of the data requests and additional FDA discussions that have currently been outlined can be completed in time for an NDA submission by mid-year 2015. Obtaining an FDA approval of eteplirsen for the DMD patients who may benefit from the drug continues to be our highest priority."

Excerpts from the Pre-NDA Meeting Minutes related to information that the FDA is requesting as part of an NDA submission included:

"The sponsor should include 3-month data from at least 12 to 24 newly exposed patients at the time the NDA is submitted."

"Available data from the other patients enrolled in the new eteplirsen studies (studies 301, 203, 204) should also be included at the time the NDA is submitted, even if exposure is less than 3 months in duration."

"Additional data from later time points and from newly enrolled patients should be submitted in the 120-Day Safety Update."

"FDA strongly advises the sponsor to obtain and submit patient-level natural history data. FDA is prepared to appeal to the academic groups holding the data to allow the sponsor a means to acquire the data."

"The study 201/202 clinical site inspection conducted in May, 2014, after the issuance of the April 15, 2014, guidance letter, uncovered marked disparities in the immunohistochemistry methodology and concerns about the reproducibility of the data. The lack of confirmation of robust dystrophin measurement during the site visit necessitates including the independent assessment of dystrophin-positive fibers and 168-week efficacy data from study 201/202 in the NDA."

"FDA strongly urged the sponsor to submit the MRI data with appropriate natural history controls."

The FDA also stated that *"[a]dditional discussion between the sponsor and the FDA will be necessary to determine what would constitute a complete 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 800.708.4539 for domestic callers and 847.619.6396 for international callers. The passcode for the call is 38376370. Please specify to the operator that you would like to join the "Sarepta Regulatory Update Call." 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. An audio replay will be available through November 3, 2014 by calling 888.843.7419 or 630.652.3042 and entering access code 38376370.

About Duchenne Muscular Dystrophy

DMD is an X-linked rare degenerative neuromuscular disorder causing severe progressive muscle loss and premature death. DMD affects approximately one in every 3,500 boys 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 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 mutations affecting exon 51 of the dystrophin gene. Approximately 13 percent of the total DMD population is amenable to exon 51 skipping. 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 DMD, 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 Statements and Information
This press release contains forward-looking statements. These forward-looking statements generally can be identified by the 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 Sarepta's planned timing for an NDA submission for eteplirsen in the treatment of DMD; Sarepta's plans to work with the FDA towards the goal of a complete and acceptable NDA filing; Sarepta's ability to satisfy the additional FDA requests; the timing and submission of additional data, analysis and other information to the FDA necessary for the FDA to make regulatory determinations; the timing of and ability to initiate additional studies for eteplirsen and other follow-on exons; and the potential regulatory approval of 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: we may not be able to comply with all FDA requests; the FDA may determine that substantial additional data is required for accelerated or other approval of eteplirsen or that our NDA submission for eteplirsen does not qualify for filing, even with additional information; the results of our ongoing and new clinical trials may not be positive; there may be delays in timelines relating to an NDA submission, initiating clinical trials, or making a product commercially available for regulatory or internal reasons; we may not be able to manufacture sufficient supply for clinical trials or commercialization; agency or court decisions with respect to our patents or those of third parties may

negatively impact our business and those identified under the heading "Risk Factors" in Sarepta's Quarterly Report on Form 10-Qfor the quarter ended June 30, 2014 filed with the Securities and Exchange Commission (SEC), 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 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.