



Sarepta Therapeutics Receives Complete Response Letter from the US Food and Drug Administration for Golodirsen New Drug Application

CAMBRIDGE, Mass., August 19, 2019 (GLOBE NEWSWIRE) – Sarepta Therapeutics, Inc. (NASDAQ:SRPT), the leader in precision genetic medicine for rare diseases, today announced it had received a Complete Response Letter (CRL) from the U.S. Food and Drug Administration (FDA) regarding the New Drug Application (NDA) seeking accelerated approval of golodirsen injection for the treatment of Duchenne muscular dystrophy (DMD) in patients with a confirmed mutation amenable to exon 53 skipping.

The CRL generally cites two concerns: the risk of infections related to intravenous infusion ports and renal toxicity seen in pre-clinical models of golodirsen and observed following administration of other antisense oligonucleotides. Renal toxicity with golodirsen was observed in pre-clinical models at doses that were ten-fold higher than the dose used in clinical studies. Renal toxicity was not observed in Study 4053-101, on which the application for golodirsen was based.

“We are very surprised to have received the complete response letter this afternoon. Over the entire course of its review, the Agency did not raise any issues suggesting the non-approvability of golodirsen, including the issues that formed the basis of the complete response letter,” said Doug Ingram, president and chief executive officer, Sarepta. “We will work with the Division to address the issues raised in the letter and, to the fullest extent possible, find an expeditious pathway forward for the approval of golodirsen. We know that the patient community is waiting.”

Sarepta will immediately request a meeting with the FDA to determine next steps.

The ESSENCE study (4045-301), a global, randomized double-blind, placebo-controlled study assessing the efficacy and safety of golodirsen and casimersen, our exon-45 skipping agent, is ongoing.

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 our ability to work with the Division to address the issues raised in the

letter and find an expeditious pathway forward for the approval of golodirsen, and our immediate request for a meeting with the FDA.

These forward-looking statements involve risks and uncertainties, many of which are beyond Sarepta's control. Known risk factors include, among others: we may not be able to obtain FDA approval of golodirsen; we may not be able to complete clinical trials required by the FDA or other regulatory authorities for approval of our product candidates; the results of our ongoing research and development efforts and clinical trials for our products and product candidates may not be positive or consistent with prior results or demonstrate a safe treatment benefit or support an NDA or a BLA 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 VYONDYS 53, for various reasons, including factors outside of our 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, 2018 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 the Company which you are encouraged to review.

Any of the foregoing risks could materially and adversely affect the Company'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 Sarepta's 2018 Annual Report on Form 10-K and most recent Quarterly Report on Form 10-Q filed with the SEC as well as other 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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