Sarepta Announces Clinical Hold Lifted for its Duchenne Muscular Dystrophy Micro-dystrophin Gene Therapy Program

CAMBRIDGE, Mass., September 24, 2018 (GLOBE NEWSWIRE) – Sarepta Therapeutics, Inc. (NASDAQ:SRPT), a commercial-stage biopharmaceutical company focused on the discovery and development of precision genetic medicine to treat rare neuromuscular diseases, announced today that the Food and Drug Administration (FDA) has lifted the clinical hold for the Company's Duchenne muscular dystrophy (DMD) micro-dystrophin gene therapy program. Sarepta previously announced on July 25, 2018, that the FDA placed the program on clinical hold due to the presence of trace amounts of DNA fragment in research-grade third-party supplied plasmid in a manufacturing lot. In response, and in collaboration with Nationwide Children's Hospital, an action plan was developed and submitted to the FDA, including an audit of the plasmid supplier and a commitment to use GMP-s plasmid for all future production lots.

"Thanks to the diligent and rapid work of my Sarepta colleagues and Nationwide Children's Hospital in compiling and submitting a complete response and the expeditious evaluation by the FDA in reviewing the response and removing this clinical hold, we have been able to address the clinical hold in record time and without delay to this profoundly important clinical program," stated Doug Ingram, Sarepta's president and chief executive officer. "Our focus now is on meeting with the Division to take guidance and gain alignment around what we hope to be our registration trial for our micro-dystrophin program and achieving our goal of commencing that trial by year-end 2018."

About Sarepta Therapeutics

Sarepta Therapeutics is a commercial-stage biopharmaceutical company focused on the discovery and development of precision genetic medicine to treat rare neuromuscular diseases. The Company is primarily focused on rapidly advancing the development of its potentially disease-modifying Duchenne muscular dystrophy (DMD) drug candidates. For more information, please visit www.sarepta.com.

Forward-Looking Statements

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 Sarepta's focus on meeting with the Division to take guidance and gain alignment around what Sarepta hopes to be its registration trial for its micro-dystrophin program; Sarepta's expectation that the DMD micro-dystrophin gene therapy program will not be delayed due to the clinical hold; and Sarepta's goal to commence the DMD micro-dystrophin gene therapy by year-end 2018.

These forward-looking statements involve risks and uncertainties, many of which are beyond Sarepta's control. Known risk factors include, among others: Sarepta may not be able to execute on its business plans, including meeting its expected or planned regulatory milestones and timelines, research and clinical development plans and timelines, and bringing its product candidates to market, for various reasons including that study data may not consistently or sufficiently demonstrate the safety or efficacy of any of Sarepta's product candidates, possible limitations of Company financial and other resources, manufacturing limitations that may not be anticipated or resolved for in a timely manner, and regulatory, court or agency decisions, such as decisions by the CHMP on eteplirsen or the United States Patent and Trademark Office with respect to patents that cover Sarepta's product candidates; and even if Sarepta's programs result in new commercialized products, Sarepta may not achieve any significant revenues from the sale of such products; 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, 2017 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 2017 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.

Source: Sarepta Therapeutics, Inc.

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