



Sarepta Therapeutics Announces that Phase 1/2a Duchenne Muscular Dystrophy (DMD) Micro-Dystrophin Gene Therapy Trial Placed on Clinical Hold Due to an Out-of-Specification Production Lot; No Observed Safety Events

- Out-of-specification lot resulted from the presence of trace levels of DNA fragment in research-grade raw material plasmid sourced from third-party manufacturer --
- Fragment fully characterized; preliminary testing and analysis indicates no safety signals --
- Subject to FDA review of a corrective action plan, which will include the use of GMP-s plasmid for all future production lots --
- Clinical timeline to commence dosing of patients in pivotal trial by year-end 2018 remains on track --
- Sarepta to host conference call on Wednesday, July 25, 2018 at 5:00 p.m. ET --

CAMBRIDGE, Mass., July 25, 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, has been notified by the Research Institute at Nationwide Children's Hospital (the Research Institute) that they have received a letter from the Food and Drug Administration (FDA) on July 24, 2018, stating that their Phase 1/2a Duchenne Muscular Dystrophy (DMD) Micro-Dystrophin Gene Therapy Trial has been placed on clinical hold due to the presence of a trace amount of DNA fragment in research-grade third-party supplied plasmid. Preliminary *in-vivo* testing performed by the Research Institute indicates that the trace fragment does not result in protein expression and is quickly cleared.

The Research Institute, working with Sarepta, has developed their action plan with immediate plans to submit for review by the FDA, which will include the use of GMP-s plasmid for the program. Subject to the FDA's acceptance of the action plan, Sarepta does not anticipate any material delay in dosing patients as originally planned by year-end 2018.

"Patient safety is our top priority at Sarepta as we know it is for Nationwide Children's Research Institute," stated Doug Ingram, Sarepta's president and chief executive officer. "We intend to rapidly respond to the

FDA's clinical hold letter, including a commitment to the Agency to only use GMP-s plasmid. Independently, we will also request a meeting with the Agency to discuss the micro-dystrophin program with the goal of commencing a pivotal trial by year-end 2018."

Sarepta will host a conference call today, Wednesday, July 25, 2018 at 5:00 p.m. ET. The conference call may be accessed by dialing 844-534-7313 for domestic callers and +1-574-990-1451 for international callers. The passcode for the call is 8689739. Please specify to the operator that you would like to join the "Sarepta Conference 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.

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 Research Institute's action plan, including the plan to use GMP-s plasmid for all future production lots; the preliminary in-vivo tests performed by the Research Institute and the indications regarding safety; the clinical timeline to commence dosing of patients in pivotal trial by year-end 2018 remaining on track; Sarepta not anticipating any material delay in dosing patients as originally planned by year-end 2018, subject to FDA's acceptance of the corrective action plan; the intention to rapidly respond to the FDA's clinical hold letter, including a commitment to the FDA to only use GMP-s plasmid; and Sarepta's plan to request a meeting with the FDA to discuss the micro-dystrophin program, with the goal of commencing a pivotal trial by year-end 2018.

These forward-looking statements involve risks and uncertainties, many of which are beyond Sarepta's control. Actual results could materially differ from those stated or implied by these forward-looking statements as a result of such risks and uncertainties. Known risk factors include, among others: preliminary testing results do not necessarily predict final results; the response to the FDA's clinical hold letter may take longer than expected; the expected clinical timeline for the micro-dystrophin gene therapy trial may be delayed; we may not be able to execute on our business plans, including meeting our expected or planned regulatory milestones and time-lines, research and clinical development plans, and bringing our product candidates to market, for various reasons including that study data may not consistently or sufficiently demonstrate the safety or efficacy of any of our 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 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, 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. 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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