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FDA lifts clinical hold on Sarepta's DMD gene therapy program

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The FDA instituted the clinical hold two months ago due to the presence of trace amounts of DNA fragment in a manufacturing lot of research-grade plasmid supplied by a third party.

Singapore - Sarepta Therapeutics, a commercial-stage biopharmaceutical company focused on the discovery and development of precision genetic medicine to treat rare neuromuscular diseases, announced 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."