



## **Sarepta Therapeutics Comments on Erroneous Submission to US FDA Adverse Event Reporting System (FAERS)**

CAMBRIDGE, Mass., August 8, 2019 (GLOBE NEWSWIRE) -- Sarepta Therapeutics, Inc. (NASDAQ:SRPT), the leader in precision genetic medicine for rare diseases, was informed earlier today that an adverse event report was erroneously submitted to the FDA's adverse event reporting system (FAERs), a post-marketing surveillance database for approved therapies. Our investigation to date indicates that this report was not submitted to the FAERs database by a Sarepta employee or the study's principal investigator.

The submission reported a case of rhabdomyolysis in a participant in Sarepta's Study SRP-9001-102, a blinded, placebo-controlled trial investigating the use of Sarepta's micro-dystrophin gene therapy candidate in patients with Duchenne muscular dystrophy. Two weeks post-infusion, the patient presented with dark colored urine and elevated creatine phosphokinase (CK) levels but was otherwise asymptomatic. He was hospitalized for observation, discharged the following day and test results returned to baseline.

Study 102 is a one-to-one blinded study and thus a subject presenting an adverse event could be either on active therapy or in the placebo arm of the trial.

While Sarepta and its principal investigator remain blinded to the study, the study drug safety monitoring board is unblinded to the event and has reviewed the issue and recommended the study continue uninterrupted. No stopping rule in Study 102 was triggered.

Rhabdomyolysis is a commonly understood risk associated with Duchenne muscular dystrophy.

### **About Sarepta Therapeutics**

Sarepta is at the forefront of precision genetic medicine, having built an impressive and competitive position in Duchenne muscular dystrophy (DMD) and more recently in gene therapies for 6 Limb-girdle muscular dystrophy diseases (LGMD), Charcot-Marie-Tooth (CMT), MPS IIIA, Pompe and other CNS-related disorders, totaling over 20 therapies in various stages of development. The Company's programs and research focus span several therapeutic modalities, including RNA, gene therapy and gene editing. Sarepta is fueled by an audacious but important mission: to profoundly improve and extend the lives of patients with rare genetic-based diseases. For more information, please visit [www.sarepta.com](http://www.sarepta.com).

### **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](http://www.sarepta.com). We encourage investors and potential investors to consult our website regularly for important information about us.*

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