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Safety scare shakes Sarepta

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After previous toxicity concerns with gene therapies, news of adverse events is sure to give investors the jitters. Sarepta found this out the hard way yesterday, sinking as much as 19% after a report to the US FAERS database of rhabdomyolysis in the phase II trial of its Duchenne muscular dystrophy gene therapy contender SRP-9001. Still, the company managed to claw back some of its losses, ending the day down just 7% after eventually releasing a statement saying the report had been submitted "erroneously" since the FAERS is a post-marketing database. True, rhabdomyolysis – the breakdown of skeletal muscle – is associated with DMD itself, and was previously seen in a study of Sarepta's exon skipper golodirsen; that project, now branded Vyondys 53, is due a US approval decision this month. And the trial of SRP-9001 remains blinded, so it is possible that the affected patient received placebo. The patient apparently recovered quickly and the study has continued. Crucially, the latest adverse event does not appear similar to those seen with other DMD gene therapy contenders from Solid Biosciences and Pfizer, which both use an AAV9 vector; SRP-9001 uses a vector called AAVrh74.

DMD gene therapy forecasts			
Project	**Company**	**2024e sales ($m)**	**Note**
SRP-9001	Sarepta Therapeutics	2,352	1 case of rhabdomyolysis reported in phase II trial (NCT03769116)
SGT-001	Solid Biosciences	131	1 case of complement activation that led to clinical hold on Ignite-DMD trial; later case of decreased platelet count and increased liver enzymes with higher dose in same trial (NCT03368742)
PF-06939926	Pfizer	-	1 case of complement activation in phase I trial (NCT03362502)
Source: EvaluatePharma.			