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November 12, 2019

# Another dystrophy catastrophe for Solid Biosciences

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A third clinical hold on Solid Biosciences' Duchenne muscular dystrophy gene therapy SGT-001 leaves the company facing an existential question: what to do with a project that is ineffective at its low dose and appears to be dangerous at its high one? The company revealed today that the FDA had suspended the Ignite DMD trial after the sixth patient to be enrolled, and the third to receive the highest $2x10^{14}$vg/kg dose, suffered serious side-effects including complement activation, thrombocytopenia, decreased red blood cell count, acute kidney injury and cardiopulmonary insufficiency. Further data on all subjects, including biopsy information that will give an idea of how well the microdystrophin gene is being transfected, will be released by the year end, the company said. However, executives' refusal to answer questions on a conference call this afternoon – on, for example, how the patient was rescued – will give investors little reason to hang around. Solid shares slumped 69% this morning to a record low. Sarepta, which is viewed as having the leading DMD gene therapy, SRP-9001, was up 3% in early trade.