Patient’s ‘serious’ kidney injury halts gene therapy trial for Duchenne muscular dystrophy

The Food and Drug Administration has halted a clinical trial involving a Duchenne muscular dystrophy gene therapy from Solid Biosciences (SLDB) after a patient suffered serious kidney and blood-related injuries, the company said [November 12].

This is the third time that the Cambridge, Mass.-based Solid has run into a serious safety problem with its gene therapy, called SGT-001. The FDA placed similar clinical holds on the same clinical trial after each prior incident, but later allowed the company to proceed with patient dosing.

SGT-001 uses an inactivated virus to deliver a miniaturized but functional version of the dystrophin gene to muscle cells. The gene therapy is designed to be a one-time and potentially curative treatment for all Duchenne patients, regardless of the mutation that causes their disease.

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All of the toxicities were deemed related to SGT-001 by the patient’s treating doctor. The patient is being treated and is recovering, Solid said.

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In a statement, the company said it “will work with the FDA in an effort to resolve the hold and determine next steps” for the clinical trial.

Read full, original post: Solid Biosciences’ Duchenne gene therapy trial halted after patient suffers serious toxicity