

Gene therapy setback? Animal deaths in treating muscular dystrophy spark new concerns over high doses

An influential scientist involved in gene therapy's biggest setback, the death of a study volunteer [19 years ago](#), has issued a surprise warning over the dangers of the gene-replacement technique.

James Wilson of the University of Pennsylvania [reported](#) ... that monkeys and pigs given super-high doses of gene therapy died.

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The warning comes amidst a scramble by three companies—Sarepta Therapeutics, Pfizer, and Solid Biosciences—to be [the first to use the technique to cure muscular dystrophy](#). That disease strikes young boys, destroys their muscles, and kills them by their 20s.

To attack the disease, researchers replace patients' damaged copies of a gene called dystrophin by introducing viral particles that carry a correct copy. Reaching the countless muscle cells in a boy's body requires extremely high doses of these particles—400 trillion or more per pound of body weight.

Image provided by James Wilson
[1-31-2018 drjameswilson_1024xx2400-1354-0-296](#)

That's where the danger could come in, says Wilson. At those doses, he says, his team found that two different viruses caused extreme and sudden immunological effects.

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Studies of the extra-high doses have only recently begun. Among them, Wilson said in his paper, are the one initiated by Solid Biosciences as well as a study at Nationwide Children's Hospital, in Columbus, Ohio, where at least 15 infants got megadoses in a successful effort to treat a different disorder, [spinal muscular atrophy](#).

Read full, original post: [The doctor responsible for gene therapy's greatest setback is sounding a new alarm](#)