

How early failures dogged gene therapy — and why the future looks so much brighter now

Jesse Gelsinger was 18 years old in 1999, when he joined one of the first clinical trials of [gene therapy](#). Gelsinger suffered from an inherited genetic disorder called ornithine transcarbamylase (OTC) deficiency, which causes toxic levels of ammonia to build up in the blood.

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The gene therapy trial he enrolled in used a type of cold virus known as an adenovirus that had been engineered to deliver a working version of the OTC gene to his liver cells. Gelsinger was one of two participants receiving the highest dose. Within days of the treatment, however, his condition declined rapidly. His body launched a severe inflammatory response that led to organ failure and, ultimately, brain death.

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Gene therapy has come a long way since Gelsinger died.... Yet researchers remain vigilant about the specter of side effects. “We’re in a very different place now,” says Mark Batshaw, the physician who helped to lead the trial involving Gelsinger more than 20 years ago and is now a developmental pediatrician at Children’s National Hospital.* “We know a lot more about vectors. We know a lot more about the immunity that is associated with that. And I think there’s a lot more care.”

[This is an excerpt. Read the original post here.](#)