

Nature editorial: Gene therapy must proceed slowly in light of past perils

The GLP aggregated and excerpted this blog/article to reflect the diversity of news, opinion and analysis.

Despite having a condition called ornithine transcarbamylase deficiency (OTC), Jesse Gelsinger was relatively healthy thanks to a combination of diet and medication. In 1999, he died at age 18 during a gene-therapy experiment.

That memory was firmly on the agenda at a meeting of the US National Institutes of Health's Recombinant DNA Advisory Committee when Sam Wadsworth and Cary Harding were proposing the first new trial of gene therapy for OTC.

Harding and the researchers at Dimension argue that the technology and our understanding of physiology have advanced enough since 1999 to try it again in people. However, such assurances were not enough for the RAC, and particularly not for its bioethicists and historians.

After some discussion, the RAC voted unanimously to approve the trial under a long list of conditions, including that the treatment first be tested in a second animal species. However, the researchers disagree with most of the conditions, believing that more expensive animal trials will add nothing.

"These patients have been waiting a long time," Wadsworth said.

Read full, original post: [Gene-therapy trials must proceed with caution](#)