‘We are out on the far edge of experimentation’: Lone volunteer in Duchenne muscular dystrophy CRISPR gene editing study dies

The lone volunteer in a unique study involving a gene-editing technique has died, and those behind the trial are now trying to figure out what killed him.

Terry Horgan, a 27-year-old who had Duchenne muscular dystrophy, died last month, according to Cure Rare Disease, a Connecticut-based nonprofit founded by his brother, Rich, to try and save him from the fatal condition.

Although little is known about how he died, his death occurred during one of the first studies to test a gene editing treatment built for one person. It’s raising questions about the overall prospect of such therapies, which have buoyed hopes among many families facing rare and devastating diseases.

“This whole notion that we can do designer genetic therapies is, I would say, uncertain,” said Arthur Caplan, a medical ethicist at New York University who is not involved in the study. “We are out on the far edge of experimentation.”

A statement from Cure Rare Disease said multiple teams across the country are looking into the details of the trial and its outcome, and the company intends to share findings with the scientific community.

“It will probably be 3-4 months to come up with a full conclusion,” said spokesman Scott Bauman. “At this stage of the game, saying anything is pure speculation.”

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