Duchenne muscular dystrophy (DMD): Background on the first personalized CRISPR therapy approved for trial

The FDA has given the greenlight to test the first personalized CRISPR therapy, which was developed specifically to treat one man's unique genetic disorder.

The gene therapy, created by a team of scientists assembled by his brother, may not only extend his life but potentially herald a new age of personalized genetic treatments.

The disease: Duchenne muscular dystrophy (DMD) is a rare disorder caused by mutations in the DMD gene.

Terry Horgan is now 27 — and of the hundreds of different mutations that can cause DMD, his is one of the rarest.

Because of that, he wouldn't have benefitted from many recently trialed gene therapies for DMD. Those trials also typically involve children, so even the therapies that might have helped Terry when he was younger were now out of reach.

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In an attempt to help his brother overcome DMD, Richard founded the nonprofit Cure Rare Disease in 2017, when he was still a student at Harvard Business School.

Within a few years, the nonprofit had researchers from UMass Chan, Yale University, and other institutions developing and testing a unique DMD treatment for Terry's mutation. And in July 2022, the FDA approved the treatment (CRD-TMH-001) for a clinical trial.

"We're finally here," Richard told the Boston Globe. "We're cautiously optimistic."

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