

Intellia's disease-eradicating CRISPR tool is injected directly into the bloodstream. Here's why that's such a big deal

CRISPR gives us the ability to correct genetic mutations, and given that such mutations are responsible for more than [6,000 human diseases](#), the tech has the potential to dramatically improve human health.

...

Injecting a CRISPR therapy right into the bloodstream has been a problem, though, because the therapy has to find the right cells to edit... Now, researchers from Intellia Therapeutics and Regeneron Pharmaceuticals have [demonstrated](#) for the first time that a CRISPR therapy delivered into the bloodstream can travel to desired tissues to make edits.

...

During a [phase 1 clinical trial](#), Intellia researchers injected a CRISPR therapy dubbed NTLA-2001 into the bloodstreams of six people with a rare, potentially fatal genetic disorder called transthyretin amyloidosis... After just one injection of NTLA-2001, the three patients given a higher dose saw their levels of the protein drop by 80% to 96%.

Follow the latest news and policy debates on sustainable agriculture, biomedicine, and other 'disruptive' innovations. Subscribe to our newsletter.

[SIGN UP](#)

"This is a wonderful day for the future of gene-editing as a medicine," Fyodor Urnov, a UC Berkeley professor of genetics, who wasn't involved in the trial, told NPR. "We as a species are watching this remarkable new show called: our gene-edited future."

[Read the original post](#)