CRISPR poised to move from the research laboratory to direct patient care

Preliminary results from an ongoing trial by Intellia Therapeutics show that a CRISPR-Cas9-based drug can be delivered into the body to target the liver and reduce expression of the gene that causes transthyretin amyloidosis (ATTR). This is the first clinical trial demonstrating successful in vivo gene editing; the results suggest that it may be possible to safely edit the genomes of cells in the body.

“For in vivo delivery, the goal is that you can administer CRISPR as a medicine to the patient,” said Laura Sepp-Lorenzino, chief scientific officer of Intellia Therapeutics.

ATTR is characterized by a misfolded version of the transthyretin (TTR) protein that builds up in the heart, nervous system, and kidneys. Patients generally experience pain, weakness, and the inability to control basic body functions.

The potential to treat ATTR by merely reducing TTR—and the limited effectiveness of prior attempts to do so—made the disease an ideal scenario for CRISPR-Cas9, [lead researcher Julian] Gilmore said. The molecular scissors could reduce TTR levels by disrupting the gene that encodes it. This snip is a more powerful way to dampen expression, and a single infusion of CRISPR edits the cells to decrease TTR in perpetuity, which could also repair prior damage.

This is an excerpt. Read the original post.