A CRISPR gene-editing tool has been added to three people with HIV, the virus that causes AIDS. Is it working?

In a remarkable experiment, a biotechnology company called Excision BioTherapeutics says it added the gene-editing tool to the bodies of three people living with HIV and commanded it to cut, and destroy, the virus wherever it is hiding.

The early-stage study is a probing step toward the company’s eventual goal of curing HIV infection with a single intravenous dose of a gene-editing drug. Excision, which is based in San Francisco, says the first patient received treatment about a year ago.

Today, doctors involved in the study reported at a meeting in Brussels that the treatment appeared safe and did not have major side-effects. However, they withheld early data about the treatment’s effects, leaving outside experts guessing whether it had worked.

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“Although the concept of using CRISPR against a virus looks novel, it stems from what was going on in nature already,” [Kamel Khalili, a professor at Temple University] says.

Initial lab tests showed that CRISPR could find and destroy the HIV genes in cells and, later, that it was able to functionally cure about 20% of HIV-infected mice treated with a gene-editing drug dripped into their veins, says Khalili.

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