



HIV Gene Therapy Looks Safe—but Does It Work?

An early human trial seeks answers.

January 8, 2024 By [Liz Highleyman](#)

In October, researchers reported that a CRISPR-based gene-editing therapy known as EBT-101 appears safe so far in a pilot study, but they didn't say whether it works. Nevertheless, the U.K. Daily Mail ran a headline stating that a cure for HIV "could be months away."

Antiretroviral therapy keeps HIV suppressed indefinitely, but the virus inserts its genetic blueprints into human cells and establishes a long-lasting reservoir that makes a cure nearly impossible. Kamel Khalili, PhD, of Temple University in Philadelphia, and colleagues developed a CRISPR-Cas9 tool that acts as "molecular scissors," showing that it can snip viral DNA out of cells in mice and monkeys.

An early human trial of EBT-101, from Excision BioTherapeutics, is enrolling HIV-positive people on antiretroviral therapy with an undetectable viral load. The first three treated participants experienced no serious adverse effects. They were supposed to stop antiretrovirals 12 weeks after EBT-101 administration to see whether their virus would rebound, but the first patient, who received the gene therapy in July 2022, is well past that point. Excision has not provided further information about his status but indicated that additional data will be presented in 2024.

"It's encouraging that this research is progressing, but there is as yet zero evidence that this approach can cure HIV in people, let alone within months," says Richard Jefferys of the Treatment Action Group, adding that misleading media reports can "create false hopes."

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