



Cure: CRISPR Cuts

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Excision BioTherapeutics recently announced that the first participant in a Phase I/II clinical trial has received a CRISPR-based gene-editing therapy designed to snip HIV out of human cells, potentially leading to a functional cure. Antiretroviral therapy can keep HIV replication suppressed, but the virus inserts its genetic blueprints—known as a provirus—into the DNA of human cells and establishes a long-lasting reservoir that is unreachable by antiretrovirals and invisible to the immune system. The therapy, dubbed EBT-101, uses an adeno-associated virus to deliver CRISPR-Cas9 nucleases—enzymes that act as “molecular scissors”—and dual guide RNAs targeting three sites in the HIV genome that tell the enzymes where to cut. This study is evaluating a single IV infusion of EBT-101 in nine men with an undetectable viral load on daily antiretroviral therapy. The first participant was treated last July, and the therapy has been well tolerated so far. The man is expected to start a carefully monitored antiretroviral treatment interruption to see whether his HIV rebounds.

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