

Cut It Out!

Can gene editing rid us of HIV?

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Despite many great advances in HIV prevention and treatment, the cure for this chronic disease continues to elude the world's top scientists.

Among the reasons HIV poses such a challenge are the virus's knack for incorporating itself into the genetic material of cells, its constant replication and the tendency of some amount of virus to remain latent, or dormant, in the body in so-called reservoirs—despite antiretroviral treatment. (Such latent virus evades meds and may be activated by a number of factors; additionally, these hidden agents likely contribute to the chronic inflammation that afflicts people living with HIV.) But research that was published in the journal *Molecular Therapy* suggests that the gene-editing technology CRISPR/Cas9 may be able to thwart the virus's wily ways, bringing us one step closer to a cure.

CRISPR (pronounced "crisper")/Cas9 is sometimes referred to as a genetic scalpel. It consists of two molecules: gRNA, which matches a sequence of DNA, and the enzyme Cas9, which "snips" DNA and facilitates changes in genetic code.

Using CRISPR/Cas9, researchers from Temple University and the University of Pittsburgh successfully excised HIV from three different groups of mice: mice altered to have humanlike genetic qualities and infected with human HIV, rodents acutely infected with EcoHIV (the mouse equivalent of HIV) and mice permeated with human immune cells and infected with latent HIV.

Scientists employed bioluminescent imaging, a light-based technology, to see and track the HIV-infected cells at a molecular level and gauge replication in order to assess the success of the gene editing. Among the mice in the first two groups, the gene-editing tool blocked viral replication and potentially prevented systemic infection. What's more, among the mice infected with EcoHIV, CRISPR/Cas9 had a 96 percent excision efficacy. In the final group of rodents, after a single treatment, CRISPR/Cas9 removed latent virus that was rooted in the tissues and organs of the animals.

By shutting down replication and eradicating both active and latent virus, this exciting new research signals the possibility of a cure. The next challenge is to replicate the study in primates with the ultimate goal of starting clinical trials among humans and—one day—ending the epidemic.

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