CRISPR Snips Simian HIV From of Monkeys' Cells, Even From Viral Reservoir

Trials for HIV-positive people of a treatment using the CRISPR-Cas9 gene editing tool may be on the horizon.

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Researchers have succeeded in using a CRISPR-based gene-editing mechanism to edit SIV, HIV's simian cousin, out of the cells of monkeys that received an infusion of the treatment.

The treatment even targeted cells in the viral reservoir—the collection of SIV-infected cells that, because they are not replicating, remain under the radar of standard antiretroviral treatment, which works only on cells that are actively churning out new copies of virus.

The investigators did not indicate that they had cured the animals of SIV. But their findings amount to a substantial advance in HIV cure field.

Publishing their findings in Nature Communications, a research team led by Kamel Khalili, PhD, of Temple University in Phladelphia, designed an SIV-specific CRISPR-Cas9 gene-editing tool meant to snip the virus's genetic code from the genome of infected cells.

In a previous study, the investigators successfully used this tool to excise SIV DNA from cells in cell cultures in the lab. They found that the editing was precise enough to yield a limited risk of potentially harmful off-target effects.

They proceeded to find that the treatment was effective in cell and tissue cultures and in small-animal models, including in mice genetically engineered to have human-like immune systems.

Next, the investigators packaged the editing tool with a viral carrier meant to transport it toward SIV-infected cells within the bodies of macaque monkeys. They randomly selected three such SIV-positive primates, leaving a fourth infected monkey as their control. Then they provided a single infusion of the treatment, called AAV9-CRISPR-Cas9, to the three experimental animals.

Three weeks later, the scientists harvested blood and tissues from the four animals. They found that in the three animals that received AAV9-CRISPR-Cas9, the treatment had been distributed to a

broad range of tissues, including the bone marrow, lymph nodes and spleen. They also discovered that the treatment had targeted the viral reservoir of latently infected CD4 immune cells.

The SIV genome, the scientists discovered, had been effectively cleaved from infected cells.

The study authors are hopeful that they will be able to move this treatment into clinical trials in people living with HIV.

To read the study abstract.

To read a press release about the study, click here.
