Scientists Take First Step Toward Using CRISPR to Cure HIV in Humans

Trying to mimic the "Berlin Patient" cure, researchers edited the CCR5 gene in the immune stem cells of a man with leukemia and HIV.

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Marking an important debut effort in what may be a long road toward using the CRISPR gene-editing method to cure HIV in humans, researchers have found that their use of the technology to edit immune stem cells proved safe, even if their effort to eradicate the virus failed, NPR reports.

Publishing their findings in The New England Journal of Medicine, a Chinese research team led by Hongkui Deng, PhD, a professor of cell biology at Peking University in Beijing, used CRISPR to edit the hematopoietic stem and progenitor cells (HSPCs) of a 27-year-old man who had HIV and acute lymphoblastic leukemia. HSPCs give rise to the blood cells that make up the immune system.

The goal was to mimic the same success achieved in Timothy Ray Brown, aka "the Berlin Patient." As a part of his own leukemia treatment over a decade ago, Brown received a pair of stem cell transplants from a donor who had a genetic mutation that interfered with the CCR5 coreceptor on the surface of CD4 cells, to which most HIV attaches to begin the process of infecting the cell.

Brown is the only person in history confirmed to have been cured of HIV, but he has potential company: "the London Patient." According to a case report presented at the 2019 Conference on Retroviruses and Opportunistic Infections (CROI) in Seattle in March, this British man received a similar treatment and, if he continues to experience no viral rebound while not on antiretroviral treatment, should ultimately be pronounced cured as well.

In 2017, the Chinese researchers published a paper in which they succeeded in using CRISPR to excise HIV from the immune cells of mice.

In the new study, the team drew HSPCs from the HIV-positive man who had acute lymphoblasticleukemia and used CRISPR to edit those cells so that they would generate immune cells lacking the CCR5 coreceptor. The edited cells were then returned to his body as a stem cell transplant.

While the transplant has apparently sent the man's leukemia into remission, it did not cure his

HIV. When his antiretroviral therapy was briefly stopped in a monitored treatment interruption, his viral load rebounded. The bright side is that the gene-edited cells have persisted in his body for more than 19 months. However, only about 5% of his CD4 cells have the CCR5 edit. Going forward, the researchers would like to develop means of increasing that rate closer to 100% in other individuals in hopes that this would eradicate the virus.

In an accompanying editorial, Carl June, MD, a professor of immunology at the University of Pennsylvania praised the Chinese scientists' work with CRISPR, saying, "It's a successful use of the technology, and it's the first one. It's a good first step."

To read the NPR report, click here.

To read the study abstract, click here.

To read the editorial abstract, click here.

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