

Headline	Gene therapy seems safe, may help control HIV		
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Gene therapy seems safe, may help control HIV

Scientists have modified genes in the blood cells of HIV patients to help them resist the AIDS virus, and say the treatment seems safe and promising. The results give hope that this approach might one day free at least some people from needing medicines to keep HIV under control, a form of cure.

The idea came from an AIDS patient who appears cured after getting a cell transplant seven years ago in Berlin from a donor with natural immunity to HIV. Only about 1 percent of people have two copies of the gene that gives this protection.

Researchers are seeking a more practical way to get similar results by using gene therapy to modify patients' own blood cells.

A study of this in 12 patients was led by Dr. Carl June at the University of Pennsylvania. Results are in Thursday's the New England Journal of Medicine. These are the first published results from this method, which also has been tried in several smaller studies of patients in California.

HIV usually infects blood cells through a protein on their surface, a "docking station" called CCR5. A California company, Sangamo BioSciences Inc., makes a treatment that can knock out a gene that makes CCR5.

The 12 HIV patients had their blood filtered to remove some of their cells. The gene-snipping compound was added in the lab, and the cells were infused back into the patients.

Four weeks later, half of the patients were temporarily taken off AIDS medicines to see the gene therapy's effect. The virus returned in all but one of them, but the modified cells seemed to be protected from HIV infection and were more likely to survive than the cells that had not been treated.

"We knew that the virus was going to come back in most of the patients," but the hope is that the modified cells eventually will outnumber the rest and give the patient a way to control viral levels without medicines, said Dr. Pablo Tebas, one of the Penn researchers. That would be what doctors call a "functional cure," because the virus would still be present but held in check without treatment.

The lone patient whose HIV did not return turned out to have one copy of the protective gene, so "nature had done half of the job already," Tebas said.

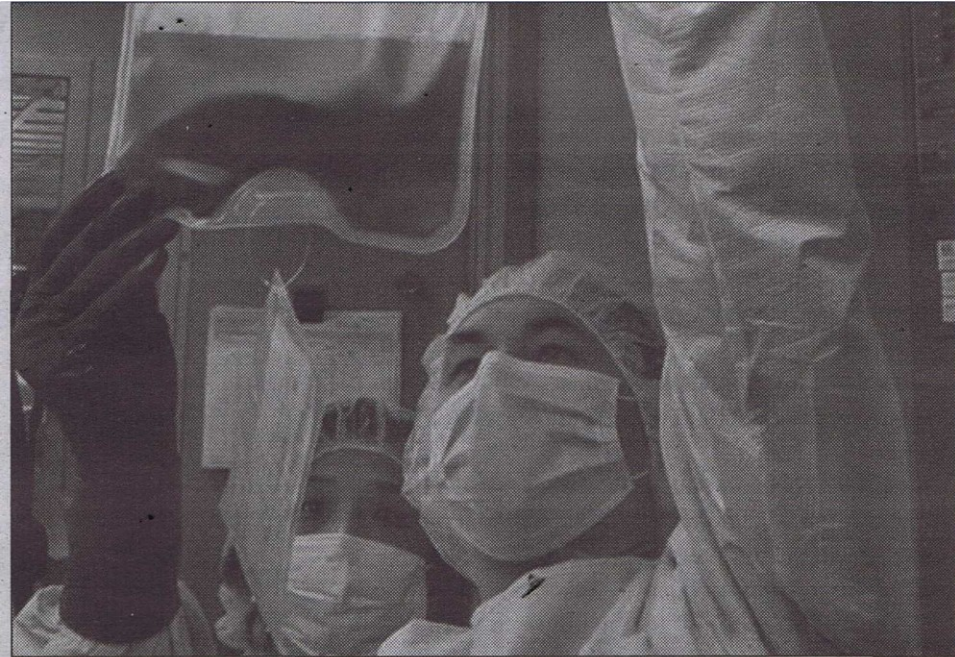
The National Institute of Allergy and Infectious Diseases sponsored the work with Sangamo and Penn.

"The ultimate goal is to create an immune system in the body that's been edited genetically so the cells are not capable of being infected with HIV," said institute director Dr. Anthony Fauci, "but we are a long way from there at this point."

Jay Johnson, 53, who works for Action AIDS, an advocacy and service organization in Philadelphia, had the treatment more than three years ago. Although the virus rebounded when he temporarily went off HIV medicines, tests show his modified blood cells are still multiplying.

"Hopefully one day I'll be able to say I'm HIV negative again," he said. ©ap

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In this January 2013 photo provided by Penn Medicine, technicians in Penn Medicine's Clinical Cell and Vaccine Production Facility in Philadelphia hold up a bag of modified T cells genetically modified to resist HIV infection. ©ap