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'Major advance' seen in gene therapy

PARIS: The world's largest experiment using gene therapy to combat the AIDS virus has yielded "a major advance," demonstrating that the technique is both beneficial and safe, scientists said yesterday.

Data from an advanced phase of the test process confirms that the quest to use transplanted genes to roll back the human immunodeficiency virus (HIV) is valid, they said.

Doctors led by Ronald Mitsuyasu of the University of California in Los Angeles recruited 74 HIV-infected volunteers for the experiment, whose results are reported online by the journal *Nature Medicine*.

Half the group were given blood stemcells that had been infiltrated by a crippled virus containing a key gene, while the other were given a harmless lookalike substance. The gene encodes something called an RNA enzyme, or ribozyme for short – a small molecule that, like a spanner thrown into a machine, is intended to block HIV from replicating once it infects a cell.

Stemcells are progenitor cells, which means that when they replicate, future generations of those cells will carry the same genetic code. The goal was to see whether these novel stemcells, by being shielded from HIV thanks to the ribozyme, would survive the body's immune defences and whether HIV, denied a haven for reproduction, would retreat.

Forty-eight weeks after the so-called OZ1 experiment began, there was no statistical difference between those who had received the gene and those who were given the placebo. But at the 100-week mark, there was encouraging news: in the gene group, the viral count was significantly lower. And the count of CD4 cells – immune cells that are depleted by HIV – was higher.

The stock of new blood cells, though, became rather depleted. Four weeks after they were introduced, a DNA test found the modified cells were present in 94% of participants in the OZ1 group, which fell to 12% by week 48 and to just 7% at week 100.

None of the gene group experienced any adverse reaction to the therapy.

The treatment "is safe and has efficacy, albeit modest," the study says.

"It shows the potential of the gene therapy approach for the treatment of HIV and represents a major advance in the field... it can be developed as a conventional therapeutic product."

Gene therapy arose in the latter years of the Nineties as a golden dawn in medical research. It conjured a vision whereby a gene, slotted into cells, would either correct a flawed gene that caused disease or, as in the case of the OZ1 trial, block progression of a pathogen. But the prospects suddenly darkened when an 18-year-old American, Jesse Gelsinger, tragically died in an experiment in 1999 to reverse a rare metabolic disorder. In several other incidents, gene-based treatments caused leukaemia. – AFP