Gene Therapy Shows Promise in Controlling HIV

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For the first time, scientists have proved that it is possible to engineer immune cells to control HIV for a sustained period of time without the need for ongoing antiretroviral (ARV) therapy. Representatives from Sangamo BioSciences presented data from the biotech company's ongoing Phase II clinical trial of its ZFP Therapeutic, SB-728-T, at the 53rd Interscience Conference on Antimicrobial Agents and Chemotherapy (ICAAC).

This genetic therapy focuses on CCR5 receptors on the surface of CD4 cells. HIV latches onto these receptors in order to begin its assault. Two different genes express the CCR5 receptors, and for this therapy Sangamo researchers removed blood cells from people with HIV who were born with one non-functional version of the two genes. After knocking out the other CCR5-expressing gene, they then reinfused the genetically engineered cells into the body.

The study had a total of 11 participants. Three of them were not included in the evaluation for the ICAAC presentation because it was found they had low levels of virus that latches onto both the CCR5 and the CXCR4 receptors, and this treatment only works for virus that is "CCR5 tropic," meaning that it only uses CCR5 receptors. One other participant is not far along enough in the study to receive an evaluation.

Three out of the remaining seven study participants achieved an undetectable viral load during an ARV treatment interruption. One maintained an undetectable level from week 11 of the treatment interruption through to week 19, when the last measurement was taken. The two other participants, however, experienced only a transient undetectable status.

The treatment has also led to a long-term increase of CD4 cells in those treated, which was correlated to a corresponding drop in the viral reservoir, as measured by the total HIV DNA in peripheral blood mononuclear cells.

"The data…demonstrate that a single infusion of SB-728-T can lead to profound suppression of viral load in the blood and sustained functional control of the virus," Dale Ando, MD, Sangamo's vice president of therapeutic development and chief medical officer, said in a release. "This is the first evidence that sustained functional control of HIV in the absence of ART is possible."

To read the Sangamo release, click here.