Stanford’s Gene Therapy Helps Block HIV’s Ability to Bind to CD4s

January 25, 2013

In a step forward for genetic therapy research, scientists at Stanford University School of Medicine have created an advanced method of engineering CD4 cells to resist HIV infection, the San Francisco Chronicle reports. Ongoing research conducted by Sangamo BioSciences and the University of Pennsylvania has used gene therapy to effectively block the CCR5 receptor on the surface of CD4 cells to which HIV latches in order to ultimately gain entry into the cell. Building on that model, the Stanford study has gone further by creating another splice in the CCR5 receptor’s DNA and adding two additional genes—a method known as stacking. Consequently, the total of three synthetic genes helped prevent HIV’s entry into the cells through either the CCR5 or CXCR4 receptors. Their results were published in the journal Molecular Therapy.

The researchers tested their gene therapy in a laboratory setting (not in humans), inserting one, two or all three of the genes into CD4 cells before exposing them to the virus. Those cells that were exposed to either one or two gene modifications were somewhat protected against infection. But those that received the triple modification had more than a 1,200-fold protection against HIV molecules that use the CCR5 receptor for entry—called being “CCR5-tropic”—and a 1,700-fold protection against the CXCR4-tropic viruses.

In a statement, Matthew Porteus, MD, an associate professor of pediatrics at Stanford, said such tailored gene therapy could one day replace daily antiretrovirals as a treatment for HIV. However, actual human trials aren’t expected for another five years.

To read the Stanford statement, click here.

To read the San Francisco Chronicle story, click here.