HIV’s Power Harnessed for Good in New Gene Therapies

July 18, 2013

HIV has a new role in cutting-edge genetic therapies in which the virus is rendered harmless but retains the power to infect cells and introduce genetic code, the Los Angeles Times reports. In December, physicians at the University of Pennsylvania announced they had cured a girl dying of leukemia using a variation of this technique. And now Italian scientists have successfully used deactivated HIV to cure children of two rare, fatal genetic diseases, including three young people with metachromatic leukodystrophy and three others with Wiskott-Aldrich syndrome. Both hereditary conditions are the consequence of genetic mutations that inhibit the body’s capacity to produce certain important enzymes. The investigators published their findings in the journal Science.

The investigators drew bone marrow stem cells from the study participants and then used an HIV-derived lentivirus to replace disease-causing genes in those cells with normal ones. Next they conducted a bone marrow transplant with the modified cells along with chemotherapy to kill off the old immune cells with the faulty genetic code.

Throughout a nearly two-year follow-up period, 45 to 80 percent of the transplanted bone marrow cells produced the necessary proteins in the children with metachromatic leukodystrophy; the number was 25 to 40 percent for those with Wiskott-Aldrich syndrome.

“Three years after the start of the clinical trial, the results obtained from the first six patients are very encouraging: The therapy is not only safe, but also effective and able to change the clinical history of these severe diseases,” Luigi Naldini, MD, PhD, director of San Raffaele Telethon Institute for Gene Therapy (TIGET) in Milan, Italy, and the study’s head researcher, said in a release. “After 15 years of effort and our successes in the laboratory, but frustration as well, it’s really exciting to be able to give a concrete solution to the first patients.”

To read the LA Times story, click here.

To read a release on the study, click here.