Cure: CRISPR Gene Editing

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A novel CRISPR gene therapy that removes an HIV-like virus from infected cells in monkeys is now being tested in humans. HIV inserts its genetic blueprints into the DNA of host cells, establishing a long-lasting reservoir that antiretrovirals can’t reach, making a cure nearly impossible. Researchers at Temple University administered a single injection of EBT-100—a CRISPR-Cas9 therapy that targets three sites on the integrated viral genome—to monkeys on daily antiretrovirals, starting 28 days after simian immunodeficiency virus (SIV) infection. They observed gene editing of SIV DNA in all significant reservoir sites with no off-target effects or other evidence of toxicity. Monkeys that received the two higher EBT-100 doses showed an improvement in lymphocyte counts, appeared healthier and gained weight. Last year, Excision BioTherapeutics announced that the first participant received EBT-100 in a Phase I/II clinical trial. If the gene therapy appears safe and well tolerated, participants will undergo a careful antiretroviral treatment interruption to see whether their HIV rebounds.
