Cure: CRISPR Gene Therapy

Findings suggest CRISPR-based therapies might play a role in a combination functional cure strategy

November 11, 2024 By Liz Highleyman

A CRISPR-based gene-editing therapy was safe and well tolerated in a Phase I study, but it did not prevent viral rebound after stopping antiretroviral treatment. Antiretrovirals can keep HIV suppressed, but the virus inserts its genetic blueprints into human cells and establishes a long-lasting reservoir that makes a cure nearly impossible. EBT-101, from Excision BioTherapeutics, acts as “molecular scissors” to cut viral DNA out of cells. The study enrolled people on antiretrovirals with an undetectable viral load. They received a single IV infusion of EBT-101. Three of the first four participants who undertook an analytical treatment interruption experienced viral rebound at 2, 3.5 and 4 weeks. But the fourth man had a shrinking viral reservoir and went 16 weeks before he experienced viral rebound, considerably longer than it usually takes for the virus to return after stopping antiretrovirals. Rebound likely occurred because the gene therapy did not reach all cells harboring latent HIV. The findings suggest CRISPR-based therapies might play a role in a combination functional cure strategy.
