Cure: CRISPR Disappoints

A Phase 1 study enrolled people on antiretroviral therapy with an undetectable viral load.

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A CRISPR-based gene therapy was safe and well tolerated in a Phase I study, but it did not prevent viral rebound after stopping antiretroviral therapy (ART). 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 ART with an undetectable viral load. They received a single infusion of EBT-101. Three people who maintained viral suppression 12 weeks later started an analytic treatment interruption. Unfortunately, all three experienced viral rebound. This likely occurred because the gene therapy did not reach all cells harboring latent HIV. However, one recipient was able to maintain viral suppression for four months, longer than it typically takes for the virus to rebound after stopping antiretrovirals. This suggests that CRISPR-based therapies like EBT-101 might play a role in a combination functional cure strategy.
