One-and-done intravenous gene editing infusion might eventually be able to cure HIV

In 2014, Temple University researchers proved they could use state-of-the-art molecular scissors to cut out dormant HIV hiding in human cells in lab dishes.

Now, seven years later — the blink of an eye in basic research — the approach has received Food and Drug Administration approval for testing in humans.

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The approach is also one-and-done — a single intravenous infusion.

“Our strategy from the get-go was to make it easy to be given, not just in well-equipped labs,” [microbiologist Kamel] Khalili said. “If this works, it could be taken anywhere,” including sub-Saharan Africa, where HIV infection is rampant. In 2018, there were 800,000 new HIV infections there, just under half of the global total, according to UNAIDS.

However, the genetic editing does not completely eradicate HIV. In the macaques, for example, CRISPR’s efficiency was 37%, 65%, and 92%, depending on the region of the viral genome that was being snipped out. In the mouse model, two-thirds of the mice still had some latent infection. That raises the question: How good is good enough?

“That’s the question in the [research] field: Do we need to completely eliminate every viral copy to achieve the cure?” [molecular biologist Tricia] Burdo said. “We don’t know yet.”