‘One-and-done’ CRISPR gene therapy treatment could be lifetime cure for high cholesterol — but the FDA remains cautious

A biotech startup has successfully lowered the cholesterol levels of monkeys using a version of CRISPR that doesn’t permanently alter DNA — suggesting a safer way to take advantage of the groundbreaking technology.

Tune Therapeutics is developing a CRISPR-based platform, called TEMPO, that targets these epigenetic marks to control gene *expression*, rather than editing the genes themselves. The idea is that this will allow them to influence the proteins that play a pivotal role in our health, without the risk of permanent off-target effects.

Biotech startup Verve Therapeutics is using a CRISPR-based therapy, called “base editing,” to permanently turn off the PCSK9 gene by swapping out a single letter of DNA. In monkeys, it slashed LDL-C levels by 70% in two weeks, and the effect appeared to last indefinitely — suggesting it could be a lifetime cure for high cholesterol.

Verve is now running a human clinical trial in New Zealand and the UK, but it has had trouble getting FDA approval to start it in the US, in part because of concerns about potential off-target edits.

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