Adjustment to CRISPR method could make gene editing therapy a reality

A tweak to a technique that edits DNA with pinpoint precision has boosted its ability to correct defective genes in people. Called CRISPR, the method is already used in the lab to insert and remove genome defects in animal embryos. But the genetic instructions for the machinery on which CRISPR relies — a gene-editing enzyme called Cas9 and RNA molecules that guide it to its target — are simply too large to be efficiently ferried into most of the human body's cells.

Researchers report a possible way around that obstacle: a Cas9 enzyme that is encoded by a gene about three-quarters the size of the one currently used. The finding, published on 1 April in *Nature*, could open the door to new treatments for a host of genetic maladies.

"There are thousands of diseases in humans associated with specific genetic changes," says David Liu, a chemical biologist at Harvard University in Cambridge, Massachusetts, who was not involved in the latest study. "A fairly large fraction of those have the potential to be addressed using genome editing."

Genome editing has generated controversy, with unconfirmed reports of its use in human embryos. Some scientists have expressed concern that the technique might be used by fertility doctors to edit the genes of human embryos before its safety is established. That concern is exacerbated by the fact that changes made by the procedure in embryos would be passed to all subsequent generations without giving anyone affected the opportunity to consent. But in the non-reproductive cells of children and adults, where inter-generational issues are not a concern, researchers and companies are already racing to develop CRISPR as a clinical tool.

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