CRISPR gene editing is in clinical trials to treat sickle cell disease, cancer, HIV/AIDS and some rare inherited diseases. Here's what we can expect going forward

Examples of Crispr's promise arrive frequently, as new clinical trials report their results. Most are still at an early stage, but the sheer breadth of diseases targeted, and the mainly positive results so far, mean it is easy to be optimistic about the future of this technique.

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The furthest along the clinical pathway is a treatment for the blood disorders sickle cell disease and betathalassemia. Both of these are caused by a defective form of the gene that produces haemoglobin in red blood cells, and a Crispr-based drug called exa-cel, developed by Crispr Therapeutics and Vertex Pharmaceuticals, has shown great promise in curing both diseases with a single dose.

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Crispr is also being developed into treatments that target not faulty versions of our own DNA, but foreign invasions as well. Excision Biotherapeutics is testing a treatment for HIV that has the potential to cure the infection with a single dose. 'We're using Crispr for the same purpose it evolved in nature, to defend against a virus,' says <u>Daniel Dornbusch</u>, the company's chief executive.

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There are more than 5000 diseases caused by mutations in a single gene, and Crispr could help deal with a huge number of them.

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