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From Bacterial Defence to Medical Revolution

CRISPR originally appeared as a fundamental immune defense mechanism in bacteria, enabling them to recognize and eliminate invading genetic material such as viral DNA or RNA (Barrangou et al. 2007). Over time, what began as a bacterial survival strategy—centered around the Cas (CRISPR-associated) genes—evolved into one of the most groundbreaking tools in molecular biology. The Cas9 protein became a mechanism, so-called molecular scissors, capable of cutting DNA at precise locations (Jinek et al. 2012).

If you come from a biological background, chances are your first real encounter with this technology was through the remarkable story of two women—Emmanuelle Charpentier and Jennifer Doudna—who transformed CRISPR–Cas9 into a programmable gene-editing tool. Their journey was not simple, and the real beginning of this revolution was marked not by fame, but by years of foundational work and scientific perseverance.

The Discovery That Changed Everything

What truly captured my attention was the breakthrough in 2012, when CRISPR–Cas9 was successfully applied in mammalian cells (Cong et al. 2013; Mali et al. 2013). That moment redefined the potential of gene editing, moving from theoretical possibility to practical application.

As the world began to recognize its potential, the revolution culminated in 2020 when Charpentier and Doudna were awarded the Nobel Prize in Chemistry for their pioneering work. Their achievement not only validated decades of research but also opened the door to new frontiers in medicine, agriculture, and biotechnology.

CRISPR in the Clinic: Blood Disorders and Beyond

One of the first advancements regarding this tool was the beginning of diagnostics for two previously lesser-known diseases—sickle cell disease, which is caused by a mutation in a gene and is widely known, and β-thalassemia, which has a major impact on oxygenation in the body. The results from clinical trials were more than successful, with a significant increase in fetal hemoglobin throughout the duration of the trial, which is considered the starting point (Frangoul et al. 2020).

The Diagnostic Frontier: CRISPR for COVID, HPV, Influenza

As CRISPR continued to prove itself in treating blood disorders, the natural next step was to make the technology even more precise, personalized, and applicable to a broader range of diseases. In early 2020, one of the most significant discoveries in modern medicine appeared as prime editing was introduced. It’s designed for more precise manipulation, and one of the most advanced parts of this tool is that it does not rely on the creation of double-stranded DNA breaks but instead uses a so-called search-and-replace approach (Anzalone, Koblan and Liu 2019).

The next step was using CRISPR-Cas13 as a diagnostic tool for more widely recognized diseases, such as COVID-19, HPV, and influenza. CRISPR/Cas13 has appeared as a promising tool for diagnosing COVID-19 due to its ability to precisely target viral RNA. Early studies proved its effectiveness in finding viral sequences using guide RNAs (crRNAs) (Yan & Finnigan 2018; Yan et al. 2019).

Scientists even managed to program Cas13d to recognize and degrade SARS-CoV-2 RNA, specifically targeting regions such as ORF1ab, the spike (S) protein, and RdRp. The enzyme delivery can be enhanced via adeno-associated virus (AAV) vectors, even in symptomatic patients (Abbott et al. 2020; Nguyen et al. 2020).

Also, CRISPR-based SHERLOCK tests have shown high diagnostic accuracy. One study reported 100% sensitivity and specificity using fluorescence detection, while another showed 97% sensitivity and 100% specificity with lateral flow assays (Subsoontorn et al. 2020).

The Tipping Point: The First Approved Gene Therapy

By the end of 2023, the biggest star at the end of the tunnel became a bit brighter, as the first-ever genetic therapy based on CRISPR was approved for widespread global use. This treatment can save not thousands, but millions of people worldwide. The disease in question is transthyretin amyloidosis (TTR amyloidosis)—a rare genetic disorder where faulty genes cause harmful amyloid proteins to build up in organs and nerves, leading to severe damage. Although this disease is not yet well known to the public, awareness is growing thanks to this initial treatment.

The CRISPR method used here is called in vivo gene editing, where the gene-editing tools are delivered directly into the patient's body to disable the faulty gene responsible for the disease. This breakthrough marks a historic moment in medicine and opens the door for many more life-saving gene therapies in the future (Gilmore et al. 2021).

If you are familiar with this topic by now, I have another breath-taking piece of information for you—one that may sound like science fiction to some. But I assure you, it is very real. The sci-fi feel might belong in movies, but as I mentioned earlier, this is science grounded in clinical application. The information I am presenting here is the case of a newborn with CPS1 deficiency, who received a patient-specific base-editing therapy named k-abe, developed within just six months of diagnosis. This therapy precisely corrected a pathogenic mutation (Q335X) in the CPS1 gene without cutting DNA, marking one of the first real examples of bespoke genome editing in humans—safe, efficient, and potentially lifesaving (Musunuru et al. 2025).

As a more common example of the otherwise unimaginable use of this discovery, the base-editing therapy VERVE-102 has been shown to permanently lower LDL cholesterol by up to 69% with a single infusion—offering a glimpse into a future where one-shot gene editing may replace lifelong medications for millions suffering from heart disease (Trembley et al. 2025).

Lately, there has been major research into the use of CRISPR in the treatment of diseases such as Down syndrome. This research is still in the early clinical trial stages, having started in the first half of 2025. So far, there have been considerable results that seem more than promising for the future of this potential treatment. The most promising finding is that the elimination of the third copy happened in vitro in fibroblast cells as well as in induced pluripotent stem cells, which showed a significant improvement in restored karyotype (Hashizume et al. 2025).

If you're thinking something like "Are you joking?" after reading this—you are not alone. I had similar thoughts. But deep down, I have always had the feeling that this tool might help not only people, but generations far beyond ours.

Looking Ahead: What's Next for CRISPR?

As for the future of this discovery, no one really knows what will happen in the next 5 to 10 years. But we do know that with this tool, almost nothing is impossible when driven by the determination of scientists. There are already ideas in the early stages of clinical trials.

Across all clinical and preclinical trials, one field always catches my eye when something intriguing is happening—and that's gene therapy and its potential for the future of many diseases. Beyond its scope in human medicine, this tool might help our planet in numerous ways as well. For instance, it is being used in various applications that may help species or plants become more adaptable in today's changing climate.

If we focus more on studies currently undergoing clinical or preclinical testing, there are several therapies that have already shown significant results, such as those aimed at curing diseases that were previously considered incurable, like herpes simplex or even HIV (Hassan et al. 2025). Other clinical trials in Phase 3 have even more significant implications for the future. One example involves CRISPR being evaluated as a potential cure for COVID-19 in mice (Blanchard et al. 2021). Another, even more complex possibility is that CRISPR could be used to treat neurodegenerative diseases like Alzheimer's—though this topic is still highly complex for now.

Looking toward the future, the application of CRISPR technologies in gene therapy continues to expand rapidly. One particularly promising direction lies in in vivo editing strategies, enabled by novel delivery systems such as lipid nanoparticles and AAV vectors (Gan et al. 2025). These may soon allow direct correction of disease-causing mutations within human tissues.

For instance, early experimental work has shown that CRISPR-Cas13 can target and degrade SARS-CoV-2 RNA in mouse lungs, significantly reducing viral load (Blanchard et al. 2021). While not yet in human trials, this paves the way for potential antiviral therapies using RNA-targeting CRISPR tools.

Another long-term aspiration is the treatment of neurodegenerative diseases like Alzheimer's, where CRISPR could potentially modulate key genetic risk factors such as APOE4. Although still in its infancy, this area highlights the versatility of genome editing beyond traditional single-gene disorders (Khan et al. 2025).

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