

# CRISPR–Cas9 Gene Editing: Precision Technology with Transformative Potential and Ethical Implications

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**Citation:** M. Anupama (2025). CRISPR-Cas9 Gene Editing: Precision Technology with Transformative Potential and Ethical Implications. *Journal of Food and Biotechnology*. **22** to **26**. DOI: <https://doi.org/10.51470/FAB.2025.6.1.22>

27 January 2025: Received | 28 February 2025: Revised | 22 March 2025: Accepted | 25 April 2025: Available Online

## Abstract

The CRISPR-Cas9 technology has completely changed the fields of molecular biology and biotechnology. It provides a precise and easy way to alter genes. This system was first found in bacteria and archaea as a part of their immune defence against viruses. In 2012, scientists Jennifer Doudna and Emmanuelle Charpentier took this system and modified it so it could work in eukaryotic cells, turning it into a powerful tool for editing genomes. The process uses the Cas9 enzyme, which is directed by a specially designed single guide RNA to find a matching DNA sequence. When it gets to that spot, it creates a break in both strands of the DNA. The cell then tries to fix this break through its natural repair methods, which can either lead to gene being disrupted or new piece being added in it. The appeal of CRISPR-Cas9 lies in its straightforwardness, precision, and ability to be programmed, making it popular in fields like medicine, farming, and basic research. However, this technology isn't without its problems. It raises ethical issues and technical concerns, such as the risk of unintended changes and the implications of editing genes in reproductive cells, highlighting the need for ongoing study. The CRISPR-Cas9 system has changed the way we think about gene editing. It allows us to make precise and efficient changes to genetic material in many different organisms, and it does so at a low cost.

**Keywords:** CRISPR-Cas9, Gene Editing, genetic scissors, crops, DNA and animals.

## 1. Introduction

In 2020, the Nobel Prize in Chemistry recognized a ground-breaking method for editing genomes using CRISPR-Cas9 technology. This award came less than ten years after the key elements of the system were identified [1]. For the first time ever, two women, Emmanuelle Charpentier and Jennifer Doudna, were honored for their significant contributions to DNA manipulation with what are known as “genetic scissors.” The impact of this technique is hard to exaggerate [2]. It allows scientists to alter the genomes of model organisms in experiments, modify traits in important crops and animals, and it might even bring about major changes in medicine, especially for treating genetic disorders. CRISPR, which stands for clustered regularly interspaced short palindromic repeats, was first found in the DNA of *Escherichia coli* bacteria [3]. This discovery was made in 1987 by Ishino and colleagues from Osaka University in Japan. Back then, figuring out these tricky DNA pieces took months. The researchers didn't really know where these sequences came from or what they did in the bacteria. Even though the role of the CRISPR system wasn't clear at first, scientists started to think about how the information in CRISPR could be useful in medical research [4]. They looked into using it for identifying different strains of bacteria, starting with *Mycobacterium tuberculosis* and later on, *Streptococcus pyogenes*. It turned out that CRISPR sequences varied a lot among different strains of the same bacteria, which helped in identifying bacterial strains in clinical settings. In 1995, Francisco Mojica from the University of Alicante made an important discovery that changed how we view CRISPR loci. He found similar structures in the archaeal genome of *Haloferax mediterranei*.

This discovery highlighted the possible importance of these elements, especially since they appeared in two very different domains of life. It motivated further investigation into their roles. Mojica observed that the structures he identified in archaea were similar to DNA repeats already known in bacteria. He was among the first to suggest that these unique loci might contain pieces of foreign DNA, acting as a part of the immune defense for both bacteria and archaea [5]. Remarkably, around the same time, two other labs reached similar conclusions, marking the start of a new chapter in studying this fascinating natural phenomenon. The first experimental information about the mechanism of action of the CRISPR system was obtained in 2007 in the studies of two French food scientists, Rodolphe Barrangou and Philippe Horvath, who worked with yoghurt cultures of bacteria *Streptococcus thermophilus* for the Danish company Danisco [6]. Due to the company's rich collection of bacterial strains collected since the 1980s, scientists have been able to trace the historical course of the bacterial acquisition of spacers at the CRISPR locus in response to viral attacks by bacteriophages. The addition of new spacers in this work caused acquired immunity to the corresponding new types of bacteriophages in *S. thermophilus*: observation which subsequently led to the authors obtaining one of the first patents in this area and the start of bacterial cultures' “vaccination” with the use of CRISPR-based technology by Danisco in 2005.

## Mechanism

First, we need to figure out which specific genes are causing the problem. Once we have that information, we can create an RNA molecule that acts like a search tool to find this gene sequence in the DNA, much like using the search function on a computer [7].

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Then, we use Cas9 to cut the DNA at precise locations and get rid of the faulty part. When DNA is cut, it naturally tries to heal itself, but if we let that happen without intervention, the bad gene can come back. So, scientists step in during this healing process and provide the right genetic sequence to attach to the broken DNA strand. It's similar to cutting out a damaged section of a zipper and replacing it with a new, working piece.

### Applications of the Revolutionary CRISPR-Cas9 System for Precision Gene Editing

CRISPR-Cas9 (Clustered Regularly Interspaced Short Palindromic Repeats and CRISPR-associated protein 9) has emerged as a transformative tool in the field of genetic engineering [8]. By enabling precise, targeted changes to DNA, this technology has opened new frontiers in medical, agricultural, and basic biological research. Among its most impactful applications are those in **biomedical research** and **human gene therapy**, where it is revolutionizing our ability to understand and treat genetic diseases.

#### 1. Biomedical Research

##### Understanding Gene Functions

CRISPR-Cas9 allows scientists to knock out or modify specific genes in a wide variety of organisms, including human cells, mice, zebrafish, and organoids (miniature 3D organ models grown in vitro). By observing the effects of gene disruption, researchers can infer the gene's role in development, physiology, or disease processes [9]. This has greatly accelerated the pace of functional genomics.

##### Modeling Human Diseases

One of the most valuable applications of CRISPR in research is the generation of accurate disease models. By introducing precise mutations that mimic human genetic conditions, scientists can study how these mutations affect biology in controlled environments. For example:

- Mouse models of cancer can be engineered to carry mutations in tumor suppressor genes or oncogenes.
- Brain organoids with mutations related to Alzheimer's disease help in understanding neurodegenerative processes.
- Models of rare diseases enable drug screening and therapeutic research.

These models are critical for unraveling disease mechanisms and for the preclinical testing of new treatments.

#### 2. Human Gene Therapy

##### Correcting Genetic Disorders

CRISPR-Cas9 is currently being tested as a therapeutic tool for a variety of inherited genetic disorders. By repairing, replacing, or silencing disease-causing genes directly in patients' cells, CRISPR holds the promise of long-lasting cures. Some of the most notable applications include:

- **Sickle Cell Anemia:** CRISPR is used to edit bone marrow stem cells to produce fetal hemoglobin, which can substitute for the defective adult hemoglobin in these patients.

- **Beta-Thalassemia:** Similar strategies are being used to restore normal hemoglobin production by editing key regulatory genes.

- **Duchenne Muscular Dystrophy (DMD):** CRISPR is employed to correct mutations in the dystrophin gene, which is essential for muscle function.

- **Cystic Fibrosis (CF):** Research is underway to repair the CFTR gene mutation in lung epithelial cells, potentially offering a cure for this life-threatening condition.

#### Clinical Trials and Therapeutic Advances

Several clinical trials are currently investigating CRISPR-based therapies. Notably:

- CTX001, developed by CRISPR Therapeutics and Vertex Pharmaceuticals, is a CRISPR-edited therapy in trials for sickle cell disease and beta-thalassemia.

- In oncology, CRISPR is being used to modify T cells to enhance their ability to target and kill cancer cells. Trials in non-small cell lung cancer and multiple myeloma are exploring the effectiveness of such engineered T cell therapies.

- Trials for Leber congenital amaurosis, a rare inherited eye disease, have also commenced, representing one of the first in vivo CRISPR therapies delivered directly to human tissues.

The CRISPR-Cas9 system represents a landmark innovation in gene editing, with vast potential to redefine our approach to genetic research and therapy. From elucidating gene function to correcting inherited disorders, CRISPR is at the forefront of personalized medicine and precision biology [10]. As ongoing clinical trials continue to demonstrate its safety and efficacy, CRISPR is expected to become an essential tool in the treatment of numerous diseases, ushering in a new era of molecular medicine.

#### 3. Infectious Disease Control

##### Viral Disease Resistance

CRISPR-Cas9 is being harnessed to create resistance against viral infections in both plants and animals, including humans. In agriculture, researchers have developed virus-resistant crops by editing genes that viruses use to replicate, thus enhancing food security. In human medicine, one of the most significant breakthroughs is the potential to edit genes like CCR5, a receptor on white blood cells that HIV uses to enter the body. By knocking out the CCR5 gene using CRISPR, scientists can render human cells resistant to HIV infection, offering hope for long-term control or even a cure.

##### CRISPR-Based Diagnostics

Advanced CRISPR platforms such as SHERLOCK (Specific High-sensitivity Enzymatic Reporter unLOCKing) and DETECTR (DNA Endonuclease-Targeted CRISPR Trans Reporter) have been developed for rapid, accurate, and portable detection of viral pathogens. These diagnostic tools have been crucial in detecting viruses like SARS-CoV-2, Zika, and Ebola, especially in resource-limited settings.

CRISPR-based diagnostics offer significant advantages in terms of speed, sensitivity, and specificity, making them invaluable in pandemic preparedness and outbreak response.

#### 4. Synthetic Biology and Bioengineering

##### Genome Reprogramming

CRISPR is a foundational technology in synthetic biology, allowing the precise engineering of microbial genomes for various industrial applications. By reprogramming the genetic code of bacteria or yeast, scientists can enhance the production of biofuels, bioplastics, vaccines, and pharmaceutical compounds. For example, CRISPR has been used to create strains of *E. coli* and yeast that produce high yields of artemisinin (an anti-malarial drug), insulin, and other valuable biochemicals.

##### Gene Drives

A revolutionary application of CRISPR is the creation of gene drives, which are genetic systems that increase the likelihood of a particular gene being inherited, thereby spreading specific traits rapidly through populations. This technology has shown promise in controlling the populations of disease vectors like mosquitoes, aiming to eradicate malaria, dengue, and Zika by making the vectors sterile or resistant to the parasites [11]. While powerful, gene drives raise ecological and ethical concerns due to their potential to disrupt ecosystems.

#### 5. Ethical and Regulatory Considerations

The powerful capabilities of CRISPR technology have sparked global debates about its ethical, legal, and social implications. Particularly controversial is germline editing—modifying genes in human embryos—which can result in heritable changes. While this could theoretically eliminate genetic diseases, it also opens the door to "designer babies" and unknown long-term consequences. Governments and bioethics bodies worldwide are actively engaged in drafting regulations and guidelines to ensure the responsible use of gene-editing technologies. Key considerations include:

- **Informed consent**
- **Risk assessment and biosafety**
- **Transparency in research**
- **Equitable access to technologies**

Leading international organizations like the World Health Organization (WHO) and National Academies of Sciences are working on frameworks to guide ethical CRISPR use, particularly in clinical applications.

#### 6. Bioethical Concerns and Future Potential of CRISPR-Cas9

The transformative power of CRISPR-Cas9 in genetic editing brings with it not only medical and scientific promise but also deep ethical questions. As this technology moves closer to clinical application and public acceptance, its societal implications must be carefully evaluated.

##### 6.1 Germline Editing and Heritability

###### What It Is

Germline editing refers to the genetic modification of reproductive cells—sperm, eggs, or embryos.

Any changes made are heritable, meaning they can be passed on to future generations.

###### Why It Matters

Unlike somatic (non-reproductive) gene editing, germline editing affects not just an individual but also their descendants, who have no say in the genetic changes made on their behalf.

###### Ethical Concerns

- **Irreversibility and Unknown Risks:** Permanent alterations to the human gene pool could have unintended consequences that may not manifest for generations.
- **New Genetic Disorders:** Missteps in editing might introduce or propagate harmful mutations.

##### 6.2 Designer Babies and Genetic Enhancement

###### The Possibility

CRISPR's precision opens the door to enhancements beyond disease correction—altering traits like intelligence, physical appearance, or athletic ability.

###### Ethical Concerns

- **Social Inequality:** Enhancement could exacerbate existing inequalities, creating a division between "genetically privileged" and "natural" individuals.
- **Commodification of Human Life:** Turning human traits into customizable features risks reducing individuals to products.
- **Philosophical Debate:** Some argue that such manipulation equates to "playing God," redefining the natural human experience.

##### 6.3 Informed Consent and Equitable Access

###### The Challenge

Ensuring informed consent is particularly complicated in cases involving embryos or minors who cannot speak for themselves.

###### Access Issues

- **Affordability:** Advanced gene-editing therapies may only be accessible to wealthy individuals or countries, leading to global health disparities.
- **Justice and Equity:** This raises critical questions about fairness and the right to benefit from medical innovations.

##### 6.4 Safety and Off-Target Effects

###### The Risk

The advances in precision, CRISPR can cause off-target mutations, editing unintended parts of the genome.

###### Ethical Dilemma

- **Risk vs. Reward:** Should clinical trials proceed when there is still uncertainty about the safety of the technology?
- **Patient Protection:** How do we ensure the safety of individuals undergoing experimental treatments?

## 6.5 Regulatory Oversight and Global Governance

### Current Status

There is a significant lack of international consensus on how to regulate CRISPR technology.

### Case in Point

The controversial 2018 birth of gene-edited twins in China highlighted the danger of unregulated scientific experimentation, sparking global outrage and calls for stricter oversight.

### Future Needs

- **Robust Ethical Frameworks:** Clear global standards for permissible use.
- **Transparent Guidelines:** Involving scientists, policymakers, ethicists, and the public in shaping governance.

## 7. Psychological and Social Impacts of Gene Editing

As CRISPR-Cas9 technology becomes more prominent in discussions around healthcare and genetics, it is essential to address not just its scientific and ethical dimensions but also the psychological and social ramifications for individuals and communities [11-15].

### 7.1 Stigmatization and Social Perception

#### Potential Issue

Widespread use of gene editing to "correct" or eliminate certain genetic conditions may unintentionally stigmatize individuals who live with these conditions.

#### Implications

- People with genetic disorders might be perceived as "defective" or "less than" in a society where these conditions are deemed curable or preventable.
- There could be increased discrimination and marginalization, especially in communities where access to gene editing is limited.

### 7.2 Parental and Societal Pressures

#### Concern

Parents might feel pressured to genetically enhance their children—not for health reasons, but to meet cultural ideals of intelligence, appearance, or performance.

#### Risks

- Children may be raised with unrealistic expectations or lose autonomy over their identity.

- Societal trends could emerge where people are judged based on genetic desirability, potentially leading to new forms of inequality.

## 8. Moral Status of Embryos and Ethical Debate

The use of embryos in gene editing research—especially for germline modifications—raises complex philosophical and religious questions.

### 8.1 Defining Moral Status

#### The Dilemma

What rights and protections should be afforded to embryos used in CRISPR research? Is it ethical to experiment on human embryos, especially if they will not be implanted?

#### Diverse Perspectives

- Some cultures and religions believe life begins at conception, and therefore any manipulation of embryos constitutes the manipulation of a human life.
- Others believe moral status increases with development and that research on early-stage embryos is ethically permissible, especially if it can prevent suffering in future generations.

### 8.2 Global Variability

#### Challenge

Because ethical views on embryonic research vary significantly across countries and cultures, international consensus is difficult to achieve.

#### Examples

- Some countries, like the UK, allow embryo research under strict regulation (up to 14 days post-fertilization).
- Others, such as Germany and many religious nations, have strict prohibitions on any embryo manipulation.

Gene editing technologies like CRISPR not only challenge our understanding of health and biology but also force us to confront profound psychological, social, and philosophical questions. As we advance toward integrating such tools into medical practice, it is critical to address how they may reshape our values, identities, and interpersonal relationships. Thoughtful dialogue across disciplines, cultures, and communities will be necessary to navigate these uncharted territories with empathy and responsibility [12].

**Table 1: Applications of CRISPR-Cas9 in Biomedical Research**

Application Area	Example	Purpose	Impact
Disease Modeling	Mouse models of Alzheimer's	Study disease mechanisms	Improved understanding of neurodegenerative diseases
Functional Genomics	Gene knockout in stem cells	Identify gene functions	Accelerated gene-function discovery
Cancer Research	CRISPR-modified T-cells	Enhance immune response to tumors	Promising immunotherapy trials
Rare Genetic Disorders	Sickle cell disease	Correct mutations in hematopoietic stem cells	Initial clinical success in gene therapy
Drug Development	High-throughput gene screens	Identify drug targets and resistance genes	Faster and targeted drug development



Table 2: Key Ethical and Safety Concerns in CRISPR-Cas9 Applications

Concern Area	Description	Example/Case	Potential Risk
Germline Editing	Genetic changes passed to future generations	He Jiankui's twin gene-editing case (2018)	Unknown long-term effects
Off-target Effects	CRISPR edits unintended genomic regions	Observed in early animal trials	Unintended mutations and health complications
Accessibility and Equity	Limited access to CRISPR therapies in low-resource settings	High cost of gene therapies	Global healthcare inequality
Designer Babies	Editing traits beyond health, such as intelligence or appearance	Potential future misuse	Ethical and societal imbalance
Informed Consent	Difficult in cases involving embryos or minors	Clinical trials involving children	Ethical dilemma in consent and autonomy

## Conclusion

The advent of CRISPR-Cas9 technology marks a revolutionary leap in genetic engineering, offering unprecedented precision, efficiency, and accessibility in genome editing. Its applications range from curing genetic diseases and improving crop traits to combating infectious diseases and advancing fundamental biological research, its transformative potential, the technology also raises serious ethical, legal, and social concerns, particularly in the context of germline editing, off-target effects, and equitable access, it is imperative that scientific innovation is accompanied by robust ethical frameworks, transparent regulatory guidelines, and inclusive public dialogue. Balancing the promise of CRISPR-Cas9 with responsible governance will be essential to harness its full potential while safeguarding human rights, biodiversity, and societal values.

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