

CRISPR and Genome Editing in Modern Medicine: Opportunities and Ethical Challenges

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Introduction

The emergence of CRISPR-Cas9 and related genome-editing technologies has revolutionized modern medicine by enabling precise, efficient, and relatively cost-effective modification of genetic material. Originally adapted from bacterial immune defense mechanisms, CRISPR offers a programmable method to target specific DNA sequences for editing, correction, or regulation. This technology has rapidly moved from laboratory experimentation to clinical applications, opening new possibilities for treating genetic disorders, cancers, infectious diseases, and beyond. In the past decade, CRISPR has shifted the paradigm of medical research, accelerating the development of curative strategies for conditions once deemed untreatable. Its ability to correct disease-causing mutations has already translated into clinical trials, and several therapies have reached regulatory approval. However, with such transformative power comes complex ethical, safety, and societal questions. Balancing opportunities with responsible oversight will be key to shaping the future of genome editing in medicine [1].

Description

CRISPR's therapeutic potential spans a broad spectrum of diseases. In hematology, exagamglogene autotemcel (Casgevy) became the first FDA-approved CRISPR-based therapy in 2023 for sickle cell disease and beta thalassemia, marking a historic milestone. In oncology, CRISPR is being applied to engineer CAR-T cells with enhanced tumor-targeting capacity, while early trials are investigating in vivo CRISPR delivery against cancers with defined genetic drivers. Infectious diseases represent another frontier, with CRISPR-based platforms under investigation to target viral genomes such as HIV and hepatitis B, aiming for functional cures. In ophthalmology, experimental CRISPR therapies are testing gene editing for Leber congenital amaurosis and Stargardt disease, offering hope to patients with inherited blindness. These diverse applications highlight CRISPR's versatility across medical specialties [2].

Since the advent of CRISPR-Cas9, genome-editing technologies have diversified into more refined approaches. Base editors enable single-nucleotide changes without

introducing double-strand breaks, reducing off-target risks. Prime editing extends this precision by allowing targeted insertions, deletions, and substitutions, functioning as a "genetic word processor." Additionally, epigenome editors harness CRISPR for gene regulation without altering the DNA sequence, offering therapeutic potential for complex diseases influenced by epigenetic states. More recently, in vivo CRISPR delivery, including lipid nanoparticles and viral vectors, has enabled direct editing inside the human body, as demonstrated in a 2025 case where a newborn received a personalized CRISPR treatment to correct a rare metabolic disorder. These innovations extend CRISPR's reach and broaden its clinical promise [3].

Despite its promise, CRISPR raises profound ethical challenges. Germline editing, which introduces heritable genetic modifications, remains highly controversial due to concerns about unintended mutations, long-term effects, and potential misuse for human enhancement. The 2018 case of gene-edited babies in China sparked international debate, leading to stronger global regulatory scrutiny. Somatic editing—targeting only the treated individual—poses fewer ethical issues but still raises safety concerns, particularly regarding off-target edits, immune reactions, and long-term durability of effects. Furthermore, unequal access to CRISPR therapies risks exacerbating global health disparities, as high development and treatment costs may restrict availability to wealthy nations or individuals. Responsible governance, informed consent, and global collaboration are essential to address these ethical complexities [4].

Looking ahead, CRISPR and genome editing are poised to play central roles in the era of precision medicine. Integration with artificial intelligence and multi-omics data will enable more accurate identification of genetic targets and prediction of treatment responses. Advances in delivery systems will further enhance the safety and efficacy of in vivo editing. On the societal level, fostering equitable access to these transformative therapies will be vital to ensure that breakthroughs in genome editing benefit patients worldwide, not just those in resource-rich settings. Ethical frameworks must evolve alongside technology, balancing innovation with the protection of human rights and dignity. International dialogue and consensus will be necessary to establish boundaries between therapeutic applications and controversial

enhancements, ensuring CRISPR's use aligns with shared values of justice and responsibility. CRISPR-Cas9 has rapidly transformed modern medicine by enabling precise genetic editing, with ongoing clinical trials showing promise across multiple fields: ophthalmology trials are testing direct eye injections to restore vision in Leber Congenital Amaurosis, cancer studies are advancing CRISPR-edited CAR-T and TIL therapies for solid and gastrointestinal tumors, infectious disease research is exploring therapies like EBT-101 for HIV, CRISPR-modified immune cells for COVID-19, and phage-based approaches for resistant bacterial infections such as *E. coli* UTIs. While these innovations offer groundbreaking opportunities, challenges remain off-target risks and long-term safety, high treatment costs that may worsen healthcare inequalities, and ethical debates around human germline editing highlight the need for strict regulation and global oversight to ensure responsible and equitable use of this transformative technology [5].

Conclusion

CRISPR and genome editing represent one of the most powerful scientific revolutions in modern medicine, offering unprecedented opportunities to treat and even cure genetic diseases. From laboratory innovations to FDA-approved therapies, the transition from bench to bedside has been remarkably rapid. Yet, alongside these opportunities, ethical challenges particularly those involving germline editing, safety risks, and equitable access demand thoughtful consideration and global oversight. As CRISPR technology continues to evolve, its future will depend not only on scientific progress but also on society's ability to establish responsible frameworks that maximize benefits while minimizing risks. In this balance lies the promise of CRISPR as both a medical breakthrough and a test of our ethical stewardship in the genomic era.

Acknowledgment

None.

Conflict of Interest

None.

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