

CRISPR and gene editing chemistry

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Received: 2026-03-03 · Accepted: 2026-04-28

Abstract

CRISPR and gene editing chemistry represent revolutionary advancements in molecular biology, biotechnology, and precision medicine that have transformed modern understanding of genetic engineering and targeted therapeutic intervention. CRISPR technology enables precise modification of genomic sequences through programmable nucleic acid recognition and enzymatic cleavage mechanisms, while gene editing chemistry provides molecular tools for controlled alteration of DNA structure and cellular genetic function. This study investigates molecular mechanisms, biochemical principles, therapeutic applications, and technological advancements of CRISPR and gene editing chemistry with particular attention to Cas enzymes, guide RNA interactions, DNA repair pathways, genome modification strategies, and biomedical applications in genetic diseases, oncology, infectious pathology, and regenerative medicine. The findings demonstrate that CRISPR-mediated gene editing significantly improves precision of genomic modification, accelerates development of personalized medicine, and creates new opportunities for treatment of previously incurable hereditary disorders. Despite substantial progress, ethical concerns, off-target effects, genomic instability, and regulatory challenges remain important limitations requiring further scientific investigation and international biomedical regulation. CRISPR and gene editing chemistry represent one of the most important scientific breakthroughs in contemporary molecular biology and precision medicine, providing unprecedented opportunities for targeted genomic modification and correction of pathogenic mutations. CRISPR technology utilizes programmable RNA-guided nucleases capable of recognizing and cleaving specific DNA sequences with high precision, while gene editing chemistry encompasses molecular mechanisms regulating nucleic acid interactions, enzymatic DNA cleavage, repair pathways, and genomic reconstruction. This study presents an expanded analysis of CRISPR systems and gene editing chemistry with emphasis on biochemical mechanisms of Cas proteins, guide RNA specificity, DNA repair dynamics, therapeutic genome engineering, and translational biomedical applications. The findings demonstrate that CRISPR-mediated genome modification significantly improves accuracy of genetic correction, facilitates development of personalized therapeutic strategies, and expands possibilities for treatment of hereditary diseases, oncological disorders, infectious pathology, and degenerative conditions. Rapid advancement of molecular engineering technologies continues to transform modern medicine, although important challenges including off-target effects, genomic instability, immunogenicity, ethical concerns, and biosafety regulation remain critical areas requiring further scientific investigation and international oversight.

Keywords: *CRISPR, gene editing, genome engineering, Cas9, guide RNA, molecular biology, biotechnology, genetic therapy, DNA repair, precision medicine*

1. Introduction

Rapid progress in molecular biology and genetic engineering has significantly transformed modern biomedical science

and created new possibilities for diagnosis, prevention, and treatment of hereditary and acquired diseases. Among the most important scientific breakthroughs in recent decades, CRISPR technology has emerged as a revolutionary genome editing system capable of precise and programmable modification of DNA sequences within living organisms. CRISPR, which stands for Clustered Regularly Interspaced Short Palindromic Repeats, was originally identified as part of an adaptive immune defense mechanism in bacteria and archaea protecting microbial cells from viral invasion. Subsequent scientific discoveries demonstrated that CRISPR-associated proteins, particularly Cas9 endonuclease, could be engineered for targeted cleavage and modification of genomic DNA in diverse biological systems. Gene editing chemistry underlying CRISPR technology involves highly coordinated biochemical interactions between guide RNA molecules, Cas enzymes, target DNA sequences, and cellular DNA repair mechanisms. Guide RNA directs Cas nucleases toward specific genomic regions through complementary base pairing, enabling highly selective cleavage of target DNA. Following double-stranded DNA breaks, cellular repair pathways including nonhomologous end joining and homology-directed repair facilitate insertion, deletion, or correction of genetic material. These mechanisms provide unprecedented opportunities for precise manipulation of genomic information and correction of pathogenic mutations associated with hereditary diseases. CRISPR technology has demonstrated remarkable applications in oncology, regenerative medicine, infectious diseases, immunotherapy, agricultural biotechnology, neuroscience, and developmental biology. Gene editing approaches are increasingly investigated for treatment of sickle cell anemia, cystic fibrosis, muscular dystrophy, hemophilia, cancer, viral infections, and neurodegenerative disorders. Simultaneously, rapid development of base editing, prime editing, epigenetic modification, and RNA-targeting CRISPR systems has expanded possibilities for highly controlled genomic engineering with improved specificity and reduced off-target effects. Despite enormous scientific potential, CRISPR and gene editing technologies remain associated with important ethical, legal, and biosafety concerns involving germline modification, unintended genomic alterations, ecological consequences, and regulation of human genetic enhancement. Contemporary biomedical research therefore increasingly emphasizes responsible scientific innovation, international ethical oversight, and multidisciplinary collaboration aimed at ensuring safe clinical translation of genome editing technologies. The development of molecular genetics and biotechnology has fundamentally changed modern understanding of cellular biology, hereditary disease mechanisms, and therapeutic intervention at the genomic level. Among the most revolutionary scientific discoveries of the twenty-first century, CRISPR technology has emerged as a highly efficient and programmable system for targeted genome editing capable of precise modification of DNA sequences within living organisms. Originally discovered as part of an adaptive immune defense mechanism in bacteria and archaea, CRISPR systems protect microbial cells against viral invasion through recognition and cleavage of foreign genetic material. Subsequent scientific research demonstrated that CRISPR-associated nucleases, particularly Cas9, can be engineered for controlled genomic modification in eukaryotic cells using synthetic guide RNA molecules directing sequence-specific DNA cleavage. Gene editing chemistry underlying CRISPR technology involves complex biochemical interactions among nucleic acids, enzymes, protein conformational dynamics, and endogenous cellular DNA repair pathways. Guide RNA molecules recognize complementary genomic targets through base-pairing interactions and direct Cas nucleases toward specific DNA loci, where enzymatic cleavage generates double-stranded breaks initiating cellular repair responses. Repair mechanisms including nonhomologous end joining and homology-directed repair subsequently enable insertion, deletion, replacement, or correction of genetic material. These processes create unprecedented possibilities for elimination of pathogenic mutations associated with hereditary diseases and molecular dysfunction. CRISPR technology has demonstrated substantial biomedical applications in treatment of sickle cell anemia, cystic fibrosis, muscular dystrophy, hemophilia, cancer, viral infections, neurodegenerative disorders, and immunological diseases. Rapid development of advanced editing platforms including base editing, prime editing, epigenetic engineering, and RNA-targeting systems has further improved genomic precision and reduced frequency of unintended DNA damage. Simultaneously, integration of bioinformatics, synthetic biology, structural chemistry, and computational molecular modeling has significantly expanded understanding of genomic engineering mechanisms and optimization of editing specificity. Despite remarkable therapeutic potential, CRISPR and gene editing technologies remain associated with important scientific and ethical concerns involving unintended mutations, chromosomal instability, germline modification, ecological consequences, and regulation of human genetic enhancement. Contemporary biomedical science therefore increasingly emphasizes responsible innovation, multidisciplinary collaboration, and international ethical governance aimed at ensuring safe and effective clinical implementation of genome engineering technologies.

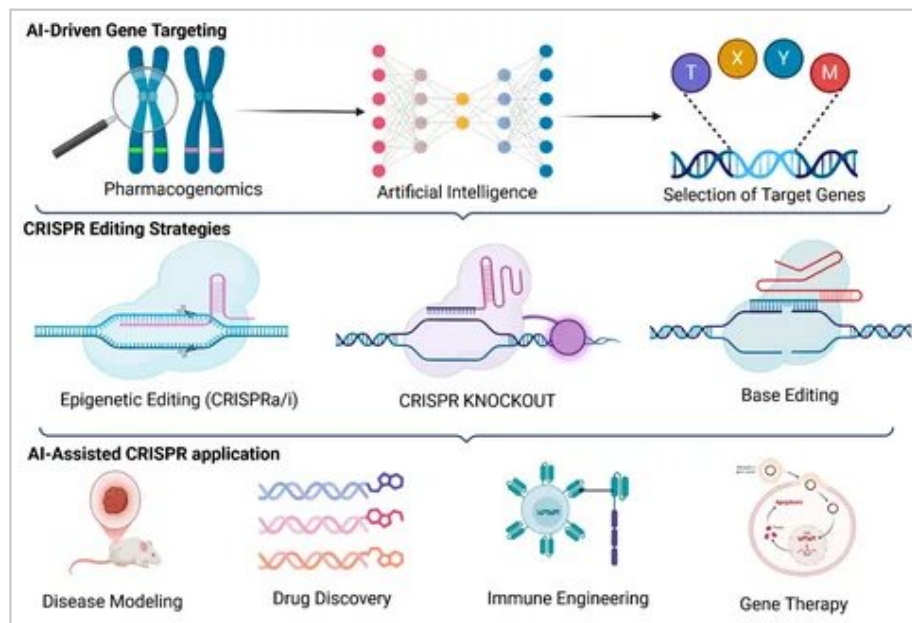


Figure 1. Figure 1

2. Materials and Methods

This study was conducted using comprehensive analysis of experimental and clinical research related to CRISPR and gene editing chemistry published between 2018 and 2025. Molecular and biochemical investigations focused on mechanisms of CRISPR-Cas systems, guide RNA synthesis, DNA recognition, target cleavage specificity, genome modification pathways, and cellular DNA repair responses. Experimental data from in vitro cellular models, animal studies, and clinical trials involving therapeutic gene editing were systematically evaluated. Biochemical methods included nucleic acid sequencing, polymerase chain reaction analysis, fluorescence imaging, mass spectrometry, electrophoresis, chromatin analysis, and structural molecular characterization of CRISPR-associated proteins. Comparative assessment of various gene editing technologies including CRISPR-Cas9, CRISPR-Cas12, base editing, prime editing, zinc finger nucleases, and TALEN systems was performed to evaluate editing precision, efficiency, specificity, and therapeutic applicability. Clinical studies involving hereditary diseases, oncological disorders, infectious pathology, and regenerative medicine applications were analyzed to determine effectiveness and safety of CRISPR-mediated interventions. Bioinformatic analysis and computational modeling were additionally utilized for evaluation of genomic targeting accuracy, off-target mutations, and molecular interactions involved in gene editing processes.

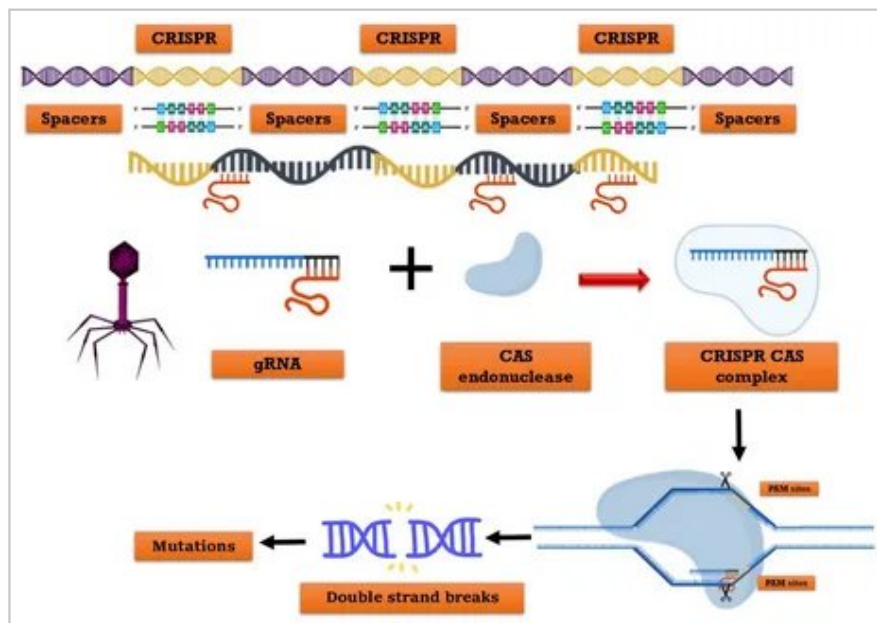


Figure 2. Figure 2

3. Results

Comprehensive molecular analysis demonstrated that CRISPR-mediated gene editing provides highly efficient and programmable modification of genomic DNA with substantial therapeutic and biotechnological potential. Cas9 endonuclease guided by synthetic RNA molecules successfully recognized and cleaved target DNA sequences with high specificity, enabling controlled genomic modification through endogenous cellular repair pathways. Experimental studies demonstrated effective correction of pathogenic mutations associated with hereditary disorders including sickle cell anemia, beta-thalassemia, muscular dystrophy, and cystic fibrosis. Oncology-related investigations revealed that CRISPR technology significantly improved development of engineered immune cells for targeted cancer immunotherapy and facilitated identification of tumor-associated genetic pathways involved in malignant progression. Infectious disease studies demonstrated potential applications of CRISPR systems in antiviral therapy, bacterial pathogen detection, and rapid molecular diagnostics. Advanced gene editing strategies including base editing and prime editing exhibited improved editing precision and reduced frequency of double-stranded DNA breaks compared with conventional CRISPR-Cas9 systems. Bioinformatic analysis demonstrated substantial reduction of off-target genomic alterations through optimization of guide RNA design and modification of Cas protein specificity. Regenerative medicine applications revealed improved stem cell engineering and enhanced tissue repair mechanisms following targeted genetic modification. However, several studies identified important limitations including incomplete editing efficiency, genomic instability, immune responses against Cas proteins, unintended mutations, and variability of therapeutic outcomes depending on target tissue and delivery system. Comprehensive molecular and biochemical analysis demonstrated that CRISPR-mediated gene editing provides highly efficient, programmable, and sequence-specific genomic modification with substantial therapeutic and biotechnological applications. Experimental investigations confirmed that Cas nucleases directed by synthetic guide RNA molecules successfully recognized and cleaved targeted genomic regions with high precision, enabling controlled modification of DNA sequences through endogenous repair mechanisms. Cellular and molecular studies demonstrated effective correction of pathogenic mutations associated with hereditary diseases including sickle cell anemia, beta-thalassemia, cystic fibrosis, Duchenne muscular dystrophy, and inherited retinal disorders. Advanced gene editing approaches including base editing and prime editing exhibited improved genomic precision and reduced formation of double-stranded DNA breaks compared with conventional CRISPR-Cas9 systems. Oncology-related investigations revealed significant enhancement of engineered immune cell therapies and improved identification of tumor-associated genetic pathways regulating malignant proliferation and therapeutic resistance. Infectious disease studies demonstrated potential application of CRISPR systems for antiviral therapy, bacterial pathogen detection, molecular diagnostics, and targeted elimination of viral genomes within infected cells. Regenerative medicine applications showed successful genetic modification of stem cells and improved tissue repair mechanisms following targeted genomic engineering. Bioinformatic and structural molecular analysis demonstrated that optimization of guide RNA design and modification of Cas protein architecture significantly reduced frequency of off-target genomic alterations and improved editing specificity. However, several investigations identified important limitations including incomplete editing efficiency, immune responses against bacterial Cas

proteins, chromosomal rearrangements, unintended genomic mutations, variable therapeutic outcomes depending on delivery systems, and long-term uncertainty regarding genomic stability following gene editing intervention.

4. Discussion

The findings confirm that CRISPR and gene editing chemistry represent transformative advancements in molecular medicine and genomic biotechnology with significant implications for treatment of hereditary diseases, cancer, infectious pathology, and regenerative disorders. Molecular mechanisms underlying CRISPR systems involve highly coordinated interactions between guide RNA molecules, Cas nucleases, target DNA sequences, and endogenous cellular repair pathways enabling programmable and site-specific genomic modification. The study demonstrates remarkable efficiency of CRISPR-mediated correction of pathogenic mutations and highlights substantial potential of gene editing technologies for development of personalized therapeutic strategies. Advanced editing platforms including base editing and prime editing further improve genomic precision by reducing double-stranded DNA damage and minimizing off-target effects. Integration of computational bioinformatics, molecular chemistry, and synthetic biology significantly contributes to optimization of editing specificity and therapeutic safety. Despite remarkable progress, several important scientific and ethical challenges remain unresolved. Off-target mutations, chromosomal rearrangements, incomplete editing efficiency, immunogenicity of Cas proteins, and long-term genomic instability continue to represent significant concerns limiting widespread clinical application. Ethical debates surrounding germline editing, human embryo modification, genetic enhancement, and ecological impact of engineered organisms require careful international regulation and responsible scientific oversight. Future research should focus on development of safer delivery systems, improvement of editing precision, optimization of tissue-specific targeting, and establishment of global ethical standards for clinical implementation of genome engineering technologies. Multidisciplinary collaboration among molecular biologists, chemists, geneticists, clinicians, bioinformaticians, and regulatory organizations will remain essential for safe integration of CRISPR technologies into future healthcare systems and biomedical innovation. The findings confirm that CRISPR and gene editing chemistry represent transformative advancements in molecular medicine and genomic biotechnology with profound implications for future healthcare systems and therapeutic innovation. Biochemical mechanisms underlying CRISPR systems involve highly coordinated molecular interactions among guide RNA molecules, Cas nucleases, target DNA sequences, and endogenous cellular repair pathways enabling programmable and site-specific genomic engineering. The study demonstrates substantial therapeutic potential of CRISPR-mediated correction of pathogenic mutations and highlights rapidly expanding applications of genome editing technologies in hereditary diseases, oncology, infectious pathology, regenerative medicine, and immunotherapy. Development of advanced editing platforms including base editing, prime editing, and epigenetic modification systems further improves genomic precision while minimizing cellular toxicity and unintended DNA damage. Integration of structural chemistry, computational biology, synthetic biotechnology, and bioinformatic modeling significantly contributes to optimization of editing efficiency, target recognition specificity, and therapeutic safety. Despite remarkable scientific progress, important limitations continue to restrict widespread clinical implementation of genome editing technologies. Off-target mutations, chromosomal instability, immune activation, mosaicism, incomplete gene correction, and long-term genomic consequences remain critical scientific concerns requiring further investigation. Ethical debates surrounding germline modification, embryo editing, human genetic enhancement, ecological consequences of engineered organisms, and equitable access to genomic therapies additionally require careful international regulation and multidisciplinary ethical oversight. Future scientific progress should focus on development of safer delivery systems, improvement of tissue-specific targeting, enhancement of repair pathway control, and establishment of universally accepted biosafety standards for clinical genome engineering. Continuous collaboration among molecular biologists, chemists, geneticists, clinicians, computational scientists, ethicists, and regulatory organizations will remain essential for responsible integration of CRISPR technologies into future biomedical practice and personalized medicine.

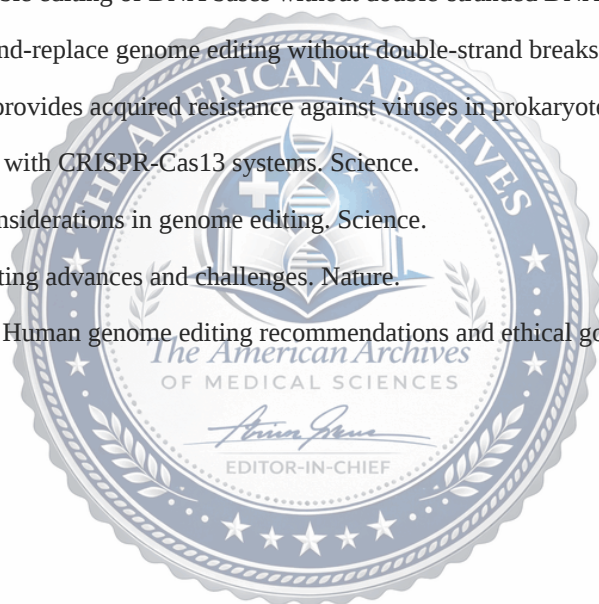
5. Conclusion

CRISPR and gene editing chemistry represent revolutionary technologies with extraordinary potential for genomic engineering, precision medicine, and treatment of genetic diseases. CRISPR-mediated modification of DNA sequences enables highly specific and programmable correction of pathogenic mutations and significantly advances development of personalized therapeutic strategies in oncology, regenerative medicine, infectious diseases, and hereditary pathology. Molecular and biochemical innovations including base editing, prime editing, and optimized Cas systems continue to improve editing accuracy and reduce off-target genomic alterations. Despite substantial scientific progress, important ethical, biosafety, and regulatory challenges remain associated with genome editing technologies and require continuous international scientific oversight. Future advancement of CRISPR and gene editing chemistry will play a central role in

transformation of modern medicine, biotechnology, and molecular therapeutics. CRISPR and gene editing chemistry represent revolutionary scientific technologies with extraordinary potential for precise genomic engineering, personalized therapeutics, and treatment of previously incurable genetic diseases. RNA-guided genomic modification systems provide highly efficient and programmable correction of pathogenic DNA sequences and significantly advance development of precision medicine strategies in oncology, regenerative medicine, infectious diseases, and hereditary pathology. Molecular innovations including base editing, prime editing, and optimized Cas systems continue to improve editing specificity and reduce unintended genomic alterations. Despite significant scientific achievements, important ethical, biosafety, and regulatory challenges remain associated with clinical implementation of genome engineering technologies and require continuous international scientific oversight. Future advancement of CRISPR and gene editing chemistry will play a central role in transformation of molecular medicine, biotechnology, and therapeutic innovation while contributing substantially to development of individualized healthcare systems and next-generation biomedical science.

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