SKU शोध संचार

(INTERNATIONAL PEER REVIEWED E-RESEARCH JOURNAL)

Impact Factor SJIF 2025 – 5.306

Vol-4, Issue-3 July - September 2026 E-ISSN: 2584-2900

CRISPR Gene Editing: A Comprehensive Review of Its Applications in the Pharmaceutical Industry

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ABSTRACT

Clustered Regularly Interspaced Short Palindromic Repeats (CRISPR)-based gene editing has emerged as one of the most significant advancements in pharmaceutical research and biotechnology. This technology enables precise modification of DNA sequences and has created new opportunities for drug discovery, gene therapy, cancer treatment, and personalized medicine. Originally discovered as a bacterial defense mechanism against viral infections, CRISPR has been adapted into a programmable genome-editing tool using guide RNA and Cas proteins, particularly Cas9. The pharmaceutical industry has adopted CRISPR for identifying disease-associated genes, developing new therapeutic targets, creating disease models, and designing innovative treatments for genetic disorders. Recent clinical advances demonstrate the potential of CRISPR-based therapies for conditions such as sickle cell disease and beta-thalassemia. Despite its advantages, challenges including off-target effects, delivery limitations, ethical concerns, and regulatory requirements remain important barriers. This review summarizes the mechanism of CRISPR gene editing, its applications in pharmaceutical research, current challenges, and future perspectives. CRISPR technology is expected to play a central role in the development of next-generation medicines and precision healthcare.

KEYWORDS

CRISPR, Cas9, gene editing, pharmaceutical industry, gene therapy, drug discovery, precision medicine

1. INTRODUCTION

The pharmaceutical industry continuously seeks innovative approaches to improve disease diagnosis, drug discovery, and treatment strategies. Traditional drug development methods often require extensive research periods, high costs, and multiple stages of testing before a medicine reaches patients. Advances in molecular biology and genetic engineering have introduced new approaches that allow scientists to investigate diseases at the genetic level. Among these innovations, CRISPR gene-editing technology has become one of the most powerful tools in modern biomedical research. CRISPR (Clustered Regularly Interspaced Short Palindromic Repeats) is a genome-editing technology that allows scientists to modify specific DNA

sequences with high accuracy. Unlike earlier gene-editing techniques, CRISPR is simpler, faster, and more cost-effective. Its ability to remove, replace, or modify genes has created new possibilities for treating inherited diseases, developing targeted therapies, and improving pharmaceutical research.

2. OVERVIEW OF CRISPR GENE EDITING

CRISPR is a naturally occurring genetic defense system found in bacteria and archaea. These microorganisms use CRISPR sequences to recognize and destroy genetic material from invading viruses. Scientists discovered that this bacterial immune mechanism could be modified and used as a programmable gene-editing tool .The most widely used CRISPR system is **CRISPR-Cas9**, which consists of two major components:

2.1 Guide RNA (gRNA): Guide RNA is a specially designed RNA molecule that identifies the specific DNA sequence to be edited. It works by matching with the complementary DNA sequence inside the genome, directing the Cas9 enzyme to the correct location.

2.2 Cas9 Enzyme: Cas9 is a nuclease enzyme that functions as molecular scissors. Once guided to the target DNA sequence by gRNA, Cas9 creates a double-stranded break in the DNA. This break allows researchers to modify the genetic information through the cell's natural repair mechanisms .Together, gRNA and Cas9 provide a highly programmable system capable of targeting almost any region of the genome.

3. MECHANISM OF CRISPR GENE EDITING

CRISPR-mediated gene editing occurs through several important steps.

3.1 Target Gene Identification: The first step involves identifying the gene responsible for a disease or biological function. Researchers use genomic sequencing and molecular analysis to determine the DNA region requiring modification.

3.2 Guide RNA Design: A guide RNA is designed to specifically recognize the selected DNA sequence. The accuracy of gRNA design is essential to ensure efficient editing and reduce unwanted genetic changes.

3.3 Delivery of CRISPR Components: The CRISPR system is introduced into target cells using different delivery methods, including viral vectors, lipid nanoparticles, and electroporation. Effective delivery is necessary for successful gene editing.

3.4 DNA Recognition and Cutting: After entering the cell, the guide RNA directs Cas9 to the target DNA sequence. Cas9 then cuts both strands of DNA, producing a controlled double-strand break.

3.5 DNA Repair and Genetic Modification: Cells naturally repair damaged DNA through two major pathways:

Non-Homologous End Joining (NHEJ):

This pathway rapidly reconnects broken DNA ends and often introduces small mutations. It is commonly used to disable unwanted genes.

Homology-Directed Repair (HDR):

HDR uses a DNA template to accurately correct or replace genetic sequences. This method is useful for repairing disease-causing mutations .Through these repair mechanisms, CRISPR can delete, correct, or insert genetic information, resulting in the desired biological change.

CRISPR Editing Process

Target Gene Selection
↓
ssGuide RNA Design
↓
gRNA + Cas9 Delivery
↓
DNA Recognition
↓
Cas9 DNA Cutting
↓
Cellular DNA Repair
↓
Gene Modification

4. APPLICATIONS OF CRISPR GENE EDITING IN THE PHARMACEUTICAL INDUSTRY

CRISPR technology has significantly influenced pharmaceutical research by providing advanced methods for understanding diseases, discovering new drug targets, and developing innovative therapies. Its ability to modify genetic information has made it a valuable tool in drug development and precision medicine.

4.1 Drug Discovery and Development: Drug discovery is a complex and costly process that requires identifying disease-related targets and evaluating potential therapies. CRISPR has improved this process by enabling precise study of gene functions involved in disease pathways. CRISPR-based screening allows researchers to activate or deactivate thousands of genes to identify new drug targets and understand mechanisms of drug resistance effective therapeutics.

4.2 Gene Therapy: One of the most promising applications of CRISPR is gene therapy. Many inherited disorders occur due to mutations in specific genes. Traditional treatments often focus on managing symptoms, whereas CRISPR-based therapies aim to correct the underlying genetic defect. CRISPR has been investigated for treating diseases including sickle cell disease, beta-

thalassemia, cystic fibrosis, muscular dystrophy, and inherited retinal disorders. By directly modifying disease-causing genes..

4.3 Cancer Therapy: Cancer is primarily driven by genetic alterations that affect cell growth, division, and immune responses. CRISPR provides researchers with powerful tools to study cancer-related genes and develop new treatment strategies. A major application of CRISPR in cancer therapy is the modification of immune cells. Scientists can edit T cells to improve their ability to recognize and destroy cancer cells. CRISPR is also used to identify genes involved in cancer progression, metastasis, and resistance to chemotherapy .These approaches support the development of more effective and personalized cancer treatments.

4.4 Personalized Medicine: Personalized medicine aims to provide treatments based on an individual's genetic characteristics. Since genetic differences influence disease risk and drug response, CRISPR technology can help develop patient-specific therapies..

4.5 Disease Modeling and Pharmaceutical Research: Understanding disease mechanisms is essential for developing effective medicines. CRISPR allows researchers to create accurate disease models by introducing specific genetic mutations into cells, organoids and laboratory animals .These models help scientists:

- Study disease progression.
- Test potential drug candidates.
- Understand molecular pathways.
- Identify new therapeutic targets.

4.6 Vaccine and Infectious Disease Research: CRISPR technology has contributed to infectious disease research by helping scientists understand pathogen-host interactions. Researchers use CRISPR to study viral genes, identify infection mechanisms, and discover potential therapeutic targets. CRISPR-based diagnostic technologies have also been developed for rapid detection of infectious diseases. These systems demonstrate the broader potential of CRISPR beyond traditional gene editing.

5. ADVANTAGES OF CRISPR IN PHARMACEUTICAL RESEARCH

CRISPR offers several advantages that have accelerated innovation in the pharmaceutical industry:

High Precision: CRISPR can target specific DNA sequences, allowing accurate genetic modifications compared with earlier gene-editing methods.

Faster Research and Development: CRISPR reduces the time required for gene studies, disease modeling, and identification of therapeutic targets.

Cost Effectiveness: The design and implementation of CRISPR experiments are relatively simple and less expensive compared with older genome-editing technologies.

Development of Curative Therapies: Unlike treatments that only manage symptoms, CRISPR has the potential to correct the genetic causes of diseases.

Support for Precision Medicine: CRISPR enables personalized treatment strategies based on individual genetic profiles.

6. CHALLENGES AND LIMITATIONS OF CRISPR TECHNOLOGY

Although CRISPR has enormous potential, several challenges must be addressed before its widespread clinical application.

6.1 Off-Target Effects: One major concern is unintended genetic modifications at locations other than the target site. These off-target changes may affect normal gene function and create safety risks. Researchers are developing improved CRISPR systems with greater accuracy to reduce these unwanted effects.

6.2 Delivery Challenges: Delivering CRISPR components into specific tissues and cells remains a significant challenge. Different organs require different delivery strategies, and achieving efficient delivery without causing immune reactions is an important research focus.

6.3 Ethical Concerns: Genome editing raises ethical questions, particularly regarding editing human embryos and germline cells. Changes made to germline DNA could be inherited by future generations, requiring careful scientific and ethical consideration.

7. FUTURE PERSPECTIVES

The future of CRISPR in pharmaceutical research is highly promising. Advanced gene-editing technologies, such as base editing and prime editing, offer greater precision with fewer off-target effects, improving the safety and effectiveness of gene therapies. CRISPR is expected to accelerate drug discovery, support the development of personalized medicine, and enable more effective treatments for genetic disorders, cancer, and infectious diseases. The integration of artificial intelligence (AI**)** and improved delivery systems will further enhance its applications. Although challenges related to ethics, safety, and regulation remain, continued research and technological advancements are likely to establish CRISPR as a key tool in the future of pharmaceutical research and precision medicine.

8. CONCLUSION

CRISPR gene-editing technology has transformed the pharmaceutical industry by providing a powerful method for precise genetic modification. Through the interaction of guide RNA and Cas enzymes, CRISPR enables researchers to identify, cut, and modify specific DNA sequences. This capability has improved drug discovery, accelerated gene therapy development, advanced cancer treatment, and supported personalized medicine. Despite challenges related to safety, delivery, ethics, and regulation, continuous research is improving the accuracy and effectiveness

of CRISPR systems. The development of advanced editing technologies and successful clinical applications demonstrates the significant potential of CRISPR in future medicine.

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