Proceedings of ICMMGH 2026 Symposium: Biomedical Imaging and AI Applications in Neurorehabilitation
DOI: 10.54254/2753-8818/2026.AU29302

Core Application and Practical Exploration of Genetic Engineering Technology

Yiru Huang

Nanchang No.2 High School Sino-Canadian Program, Nanchang, China
hyr19100133206@outlook.com

Abstract. In the face of rising global medical demand, genetic engineering technology has emerged as a significant driving force in the biomedical field, helping to address health issues like cancer, infectious diseases, and genetic diseases. Recombinant protein medications, like human insulin, have emerged as the primary clinical medications, according to significant research findings, and mRNA vaccines have been crucial during the COVID-19 pandemic, demonstrating their quick adaptation. CRISPR-Cas9 technology has enabled precise gene editing for genetic diseases, although off-target effects remain a concern. AAV vectors are a delivery system that can improve efficacy but have several issues with respect to the immune response. Heterogeneous immune responses, protein stability issues, and the high cost of therapies such as CAR-T are current difficulties and challenges that may limit equitable access. To ensure the safe and equal use of these technologies, this paper highlights the role that genetic engineering plays in advancing precision medicine and proposes solutions such as translational collaboration and optimized regulation.

Keywords: Genetic engineering, recombinant protein drugs, genetically engineered vaccines, gene therapy, cell therapy

1. Introduction

In recent decades, biomedicine has become an important means to address global health challenges, including population aging, emerging infectious diseases such as COVID- 19 and chronic diseases, which have driven the development of innovative treatment technologies. Genetic engineering is a transformative force of the new era. By enabling genes and proteins to operate at the molecular level, it provides new insights into the treatment of diseases in this field.

At present, the application of the gene editing tool CRISPR-Cas9 has been accelerated, along with the progress in this core field. This tool makes genomic targeting easier by using guide RNA, avoiding the expensive protein engineering steps in previous technologies [1, 2]. Diseases such as hemophilia and cystic fibrosis may benefit from long-term therapy gene expression in non-dividing cells, which is precisely what AAV vectors achieve. AAV vectors are both safe and efficient when delivered in the body [3]. The mRNA vaccine can instruct cells to produce viral antigens. It has demonstrated an astonishing speed in combating COVID-19 and does not use eggs for production; it

© 2025 The Authors. This is an open access article distributed under the terms of the Creative Commons Attribution License 4.0 (https://creativecommons.org/licenses/by/4.0/).

DOI: 10.54254/2753-8818/2026.AU29302

bypasses the traditional steps that rely on eggs in the manufacturing process [4]. These advances have solidified genetic engineering's role in tackling urgent health needs.

Global research on genetic engineering has yielded impactful progress across three key areas. In recombinant protein drugs recombinant insulin, the first commercialized product and monoclonal antibodies, such as obinutuzumab, have revolutionized diabetes and cancer treatment with clinical trials confirming safety and efficacy [5]. In genetically engineered vaccines mRNA platforms like Moderna or Pfizer COVID-19 vaccines and viral vector vaccines such as AstraZeneca's ChAdOx1 have shown high protection rates, though mRNA vaccines face cold chain stability challenges [4]. In gene or cell therapy, CRISPR-Cas9 has been tested for sickle cell anemia and CAR-T therapy has achieved remission in relapsed blood cancers, though long-term safety data remains limited [1, 5].

However, research gaps persist. Technically, CRISPR-Cas9's off-target effects and AAV vectors' immune rejection hinder wider use [1, 3]. Clinically, CAR-T's high manufacturing costs, ranging from $300000 to $500000 per patient, create equity issues [5]. Ethically, human germline editing raises concerns about heritable genetic changes with few global consensus guidelines. Additionally, most studies focus on single technologies such as mRNA vaccines alone rather than cross-domain comparisons, lacking systematic analysis of their strengths, limitations and complementary roles. Existing research on genetic engineering is fragmented. It emphasizes individual technologies over systemic integration, overlooks cross-domain synergies such as combining CRISPR with AAV delivery and inadequately addresses practical barriers, including cost regulation and ethics. This gap limits the field's ability to translate breakthroughs into accessible and equitable therapies. Thus, a comprehensive review is needed to synthesize progress, identify bottlenecks and propose targeted solutions. The goal is to provide a holistic reference for researchers, policymakers and clinicians to advance genetic engineering in biomedicine.

2. Core application and practical exploration of genetic engineering technology

Genetic engineering was born in the 1970s, and the innovation of experimental research and technology has now made it widely used in the field of biomedicine. It makes up for the missing active substances in nature, and allows human beings to directly intervene and adjust the genetic code of life for disease treatment. This chapter will systematically describe the important application and practical exploration of this technology in the three core fields of recombinant protein drug development, genetically engineered vaccines, gene and cell therapy, and put forward suggestions and comparative analysis for its future development.

2.1. Recombinant protein drugs: the cornerstone of the biomedical industry

Recombinant protein drugs were among the earliest commercially successful areas of genetic engineering. in 1982, Humulin, a recombinant protein-based therapeutic drug, was approved by the FDA. As of 2024, the global market size for protein-based drugs is approaching $400 billion, with hundreds of candidates that have been approved and are in clinical trials [4]. The process of genetic recombination, which entails inserting particular DNA sequences into host organisms like bacteria, yeast, or mammalian cells, is the foundation of recombinant protein therapy.

In the field of biomedicine, the birth of human insulin was a milestone. Human insulin, composed of 51 amino acids (21 aa in the A chain and 30 aa in the B chain), was the first therapeutically useful protein product derived from recombinant DNA technology. Before 1982, when the US Federal Drug Administration (FDA) approved Humulin for diabetes treatment, people had relied on porcine

DOI: 10.54254/2753-8818/2026.AU29302

and bovine insulin [5]. This breakthrough not only ensured the safety of genetically engineered drugs but also marked a more advanced era in the commercial pharmaceutical industry.

After the introduction of Humulin, recombinant protein technology led to a series of drugs. Firstly, the first monoclonal antibody (muromonab-CD3) was approved in 1986. Subsequently, protein fusion drugs (etanercept) entered the market in 1998, followed by glycoengineered drugs (pegfilgrastim) in 2002, engineered antibody glycoforms (obinutuzumab) in 2014, bispecific antibodies (blinatumomab) in 2014, and antibody conjugates (gemtuzumab ozogamicin) in 2000. As of 2021, the number of monoclonal antibodies approved by the US FDA alone has exceeded one hundred. This further demonstrates the strong innovation capabilities and significant clinical value of recombinant protein technology as the core of biopharmaceuticals [4].

Although recombinant protein technology has achieved many accomplishments, it still faces several challenges. These challenges include: the need for a deeper understanding of the relationship between protein structure and function; the need to develop reliable computational algorithms for protein design; and the need to improve high-throughput screening techniques in order to select and identify protein variants. For instance, sugar engineering is a powerful technique used to optimize the properties of proteins. However, it also has some challenges: currently, it is impossible to predict in advance how altering the sugar structure will affect the properties of proteins, and changing the type and location of glycosylation may lead to the loss of protein activity [4]. To address these issues, people have explored emerging technological solutions. In terms of protein structure-function research, cryo-electron microscopy (cryo-EM) combined with artificial intelligence (AI) has enabled more accurate prediction of protein tertiary structures and their interaction mechanisms with targets, providing a theoretical basis for rational protein design. For high-throughput screening, microfluidic chip technology has been applied to rapidly screen thousands of protein variants, significantly improving the efficiency of variant selection. In glycoengineering, machine learning models trained on large-scale glycoengineering data are being developed to predict the impact of glycosylation modifications on protein activity, laying the groundwork for precise glycoengineering design.

2.2. Genetically engineered vaccines: an effective tool to tackle infectious diseases

Vaccination can significantly reduce the incidence of most bacterial and viral infections worldwide. According to data from the World Health Organization (WHO), vaccines save between 200,000 and 300,000 lives annually worldwide [6]. Compared to conventional vaccines, genetic engineering technology has made vaccines safer and more stable, and it also played a crucial role in responding to the recent novel coronavirus outbreak [6].

Among all the genetically engineered vaccines, a prominent example is the recombinant subunit vaccine. This is a highly secure technology that recombines and expresses the specific antigen genes on the surface of pathogens that can trigger an immune response. The hepatitis B vaccine is a successful example. Some traditional vaccines work by delivering viral proteins to cells. Although the methods used to produce those protein fragments vary, businesses are using yeast and insect cells. The viral protein used in the hepatitis B vaccine was produced by genetically modified yeast [6]. The HPV vaccine is also a successful example, and it represents a major breakthrough in the history of cancer prevention for humanity.

mRNA vaccines have been a hot topic of discussion in recent years. Within weeks of the virus sequence being released on January 11, 2020, Moderna's mRNA-1273, one of several mRNA vaccines targeting the SARS-CoV-2 spike (S) protein, was first given to human volunteers on March 16, 2020. One of the biggest benefits of mRNA technology is its biological role as a template for protein translation. In contrast to conventional vaccine technology, which relies on mass-producing a

DOI: 10.54254/2753-8818/2026.AU29302

vaccine in a bioreactor using chicken eggs or mammalian cells, mRNA vaccines are only complete once they are inside the patient's cells [7]. mRNA boasts an astonishingly rapid development speed, high efficacy, and the ability for large-scale production. From the release of the vaccine sequence to the commencement of clinical trials, it only takes a few months. This technology has brought many new possibilities to the field of vaccines.

Viral vector vaccines are also very important. Both the AstraZeneca vaccine (using the ChAdOx1 vector) and the Johnson & Johnson vaccine employ this technology, and they have been used in both Ebola and COVID-19. This demonstrates the ability to rapidly develop vaccines and also advances the vaccine research. Without involving the entire dangerous pathogen, viral vectored vaccines are safe and elicit both innate and adaptive immune responses.

Overall, genetically engineered vaccines have multiple advantages. Through codon optimization and RNA structure modification, the expression efficiency of plasmids can be improved; meanwhile, engineering modifications can be easily carried out using synthetic biology and PCR technology. In manufacturing, these vaccines have quick development cycles, are easy to scale up, and have high repeatability. Since they do not require handling live pathogens, they are highly safe. Compared to live vaccines, genetically engineered vaccines are less likely to cause virulence rebound; in addition, their preparation process does not require toxic reagents, and their immune response is safer than that of some inactivated vaccines. Furthermore, genetically engineered vaccines usually have longer shelf lives, better thermal stability, and more convenient storage and shipping conditions. However, this technology still has some limitations. When viruses undergo major mutations, vaccines based on the original strain may become less effective. Some SARS-CoV-2 strains have exhibited this behavior, allowing them to partially circumvent the immune protection provided by earlier vaccinations. Therefore, timely updates of vaccine components and continuous surveillance of virus mutations are essential to maintain vaccine efficacy. Additionally, some mRNA vaccines require ultra-low-temperature storage and transportation, which increases the difficulty of popularization in regions with underdeveloped cold chain systems.

2.3. Genetics and cell therapy

Gene and cell therapy represent the cutting-edge of genetic engineering technology. Its primary goal is to eradicate diseases entirely, not just their symptoms. It primarily uses two approaches to accomplish this goal: "gene therapy," which directly fixes the defective genes in patients' bodies, and "cell therapy," which alters and fortifies patients' immune cells in vitro before reintroducing them into the body to combat the illness. This method of treatment, which goes straight after the disease's underlying cause, signals that medicine is entering a new era of precision where customized, one-time cures are possible.

The CRISPR-Cas9 system is a revolutionary genome-editing technology that has transformed biomedical research. CRISPR-Cas9-mediated genome engineering holds great promise for treating or even curing a variety of genetic disorders, including sickle cell anemia, cystic fibrosis, Duchenne muscular dystrophy, viral infections, immunological disorders, cardiovascular diseases, and various types of cancer and neurodegenerative diseases [8]. Since the potential of the CRISPR-Cas9 system in gene editing was first discovered, this field has developed rapidly.

The working principle of CRISPR-Cas9 is based on the adaptive immune mechanism of bacteria: the Cas9 protein acts as "molecular scissors", while the guide RNA (gRNA) with specific sequences guides Cas9 to bind to the target DNA site in the genome [9]. After binding, Cas9 will cut the double-stranded DNA at the target site, triggering the cell's own DNA repair mechanism. During the repair process, researchers can introduce specific genetic modifications (such as gene insertion,

DOI: 10.54254/2753-8818/2026.AU29302

deletion or substitution) to achieve gene editing. Compared with previous technologies (such as ZFNs and TALENs), CRISPR-Cas9 has significant advantages: it uses gRNA sequences to identify specific DNA sites instead of relying on complex protein design. This avoids the time-consuming protein engineering for each new target and speeds up its application in research, enabling large-scale genomic projects to be carried out.

In cancer immunotherapy, chimeric antigen receptor (CAR)-T cell therapy is a rapidly developing new field, and it has achieved remarkable clinical results in patients with B- cell leukemia or lymphoma. The first clinical application of CAR-T cells was the use of CAR-T cells targeting CAIX for the treatment of metastatic renal cell carcinoma. These CAIX CAR-T cells could effectively target tumor cells expressing CAIX, but they also recognized normal tissues expressing CAIX, leading to "on-target, off-tumor" toxicity and grade 2-4 enzyme disruptions [10].

Although gene and cell therapy hold great promise, this field still faces significant challenges. There are still issues with safety and ethics, particularly with germline editing and unexpected genetic effects. As scientists continue to develop CRISPR-Cas9 technology, resolving these problems is crucial to ensuring its safe and effective application in a range of fields. CAR-T cells target the antigen expressed on the surface of tumor cells. Tumor cells do not express this antigen exclusively; normal cells usually do. CAR-T cells target healthy tissues in order to eradicate tumor cells, which results in "on-target, off-tumor" side effects in healthy tissues.

2.4. Development suggestions

In order to advance genetic engineering technology, it is necessary to strengthen translational medical research. This is because it will enable the laboratory research results to be applied to patients more quickly. Translational medical research serves as a platform for collaboration among enterprises, academic institutions, and research centers. To promote innovation while ensuring stability and safety, new regulatory strategies and guidelines should also be formulated for cutting-edge technologies such as gene editing. Additionally, efforts should be made to promote technological progress in the production field, such as developing universal CAR-T cell therapies and improving production processes, in order to reduce costs and increase the chances for patients to access the drugs. Lastly, but equally important, it is crucial to establish relevant laws and regulations through promoting international public debates and define the boundaries of human gene editing, which is essential for maintaining moral norms and social consensus.

Overall, in the field of biomedicine, the three main and most significant applications of genetic engineering technology are gene or cell therapy, genetically modified vaccines, and recombinant protein drugs. They differ in their representative products, technical nature, benefits, and difficulties.

Drugs made from recombinant proteins are produced in vitro. Their benefits include advanced technology and high safety because the medications only work inside the body and don't change a person's genetic makeup. The problem is that these medications typically require repeated, prolonged use and are unable to fully cure illnesses. "Insulin" is a common example of a product that helps diabetics manage their blood sugar levels. Vaccines created through genetic engineering trigger the body's immune system. This instruction will cause our cells to temporarily produce pieces of the virus's proteins, which will help the immune system identify and remember it beforehand. It can react fast when the actual virus infiltrates. The main benefits of this approach are its quick development and production, as well as its relative safety due to the fact that it does not contain the entire virus. The problem is that certain vaccines must be transported and stored at very low temperatures, and their efficacy may decrease as the virus mutates. Typical examples include

DOI: 10.54254/2753-8818/2026.AU29302

the mRNA vaccines we used during the COVID-19 pandemic, including those made by Moderna and Pfizer.

Directly altering genetic material or cells is known as gene/cell therapy. It primarily uses two methods: "gene therapy," which treats the disease by introducing the correct gene into the patient's damaged cells using a safe "vector" (typically a modified virus); and "cell therapy," of which CAR-T therapy is the most well-known. In order to precisely target tumors, this entails removing the patient's immune cells, utilizing genetic engineering techniques to give them instruments that can recognize cancer cells, and then reintroducing them into the body. Its ability to cure some genetic diseases or advanced cancers all at once is its greatest benefit. But there are also significant drawbacks and restrictions, such as incredibly complicated technology, exorbitant prices, and potential unidentified long-term safety hazards. Some representative products are currently available, such as gene therapy medications used to treat a hereditary form of blindness and CAR-T drugs used to treat a particular type of leukemia.

3. Conclusion

This has thoroughly investigated the fundamental uses and real-world applications of genetic engineering technology in the three main fields of biomedicine: gene and cell therapy, genetically modified vaccines, and recombinant protein medications. From the early success of human insulin, showed how recombinant protein technology has developed into a wide variety of biologics, such as fusion proteins and monoclonal antibodies, radically altering the way chronic disease treatments are approached. In the field of vaccines, genetic engineering has facilitated the quick development of innovative platforms like viral vector and mRNA vaccines, which were crucial during the COVID-19 pandemic. Additionally, the cutting edge of precision medicine is represented by gene editing tools like CRISPR-Cas9 and cell-based therapies like CAR- T, which may be able to treat genetic diseases and cancers that were previously incurable. This study is important because it offers a comprehensive view of how genetic engineering is advancing biomedical innovation from fundamental research to clinical application by integrating an analysis of technological advancements and real-world difficulties. Nonetheless, several restrictions still exist, such as the high expense of sophisticated treatments, the technical difficulties of delivery methods, moral dilemmas with germline editing, and doubts about long-term safety profiles. More research is needed to address problems like CAR-T therapy's "on-target, off-tumor" effects and some gene edits' short lifespan. Future developments should concentrate on building strong translational research platforms that enable industry-academia collaboration, developing flexible regulatory frameworks that strike a balance between safety and innovation, and promoting international ethical discussions to establish responsible research boundaries. Enhancing accessibility and therapeutic efficacy will also require developing more accurate gene editing systems, improving cold chain logistics for vaccine distribution, and developing production technologies to cut costs. Genetic engineering has the potential to revolutionize healthcare paradigms and provide new hope for the treatment and possible cure of a wide range of human diseases, provided that interdisciplinary innovation and responsible governance continue.

References

[1] Wang, H., La Russa, M., & Qi, L. S. (2016). CRISPR/Cas9 in Genome Editing and Beyond. Annual Review of Biochemistry, 85, 227–264.

[2] JINEK M, CHYLINSKI K, FONFARA I, et al. A programmable dual-RNA-guided DNA endonuclease in adaptive bacterial immunity [J]. Science, 2012, 337(6096): 816-821.

DOI: 10.54254/2753-8818/2026.AU29302

[3] WANG D, TAI P W L, GAO G. Adeno-associated virus vector as a platform for gene therapy delivery [J]. Nature Reviews Drug Discovery, 2019, 18(5): 358-378.
[4] Pascoe, M. J., Kitchin, N., Mansfield, K. L., et al. (2023). Next-generation vaccines: the role of mRNA in pandemic preparedness and response. Expert Review of Vaccines, 22(1), 241–255.
[5] ZHANG C, LIU J, ZHONG J F, et al. Engineering CAR-T cells [J]. Biomarker Research, 2017, 5: Article 22.
[6] LI D, LI X, ZHOU W L, et al. Next-generation engineered cell therapies for cancer [J]. Nature Reviews Immunology, 2023, 23: 335-350.
[7] CHEN Y, LI Y, NARAYANAN C P, et al. Global landscape of engineered cell therapy: from basic research to clinical application [J]. Endocrine Reviews, 2021, 42(3): 374-408.
[8] LIU C, ZHANG W, LI Y, et al. MicroRNA-21 is a therapeutic target in myeloid leukemia [J]. EMBO Molecular Medicine, 2015, 7(2): 132-147.
[9] Lamers, C. H., Sleijfer, S., Vulto, A. G., Kruit, W. H., Kliffen, M., Debets, R., Gratama, J. W., Stoter, G., & Oosterwijk, E. (2006). Treatment of metastatic renal cell carcinoma with autologous T-lymphocytes genetically retargeted against carbonic anhydrase IX: first clinical experience. Journal of Clinical Oncology, 24(13), e20–e22.
[10] Doudna, J. A., & Charpentier, E. (2014). The new frontier of genome engineering with CRISPR-Cas9. Science, 346(6213), 1258096.