INTERNATIONAL JOURNAL OF PHARMACEUTICAL SCIENCES
[ISSN: 0975-4725; CODEN(USA): IJPS00]
Journal Homepage: https://www.ijpsjournal.com

OPEN ACCESS

Review Article

Gene Therapy in Future Medicine: Current Advances, Clinical Applications, Challenges, And Future Perspectives

Keshav Sharma[1], Himanshu Yadav[1], Naveen Garg[2^], Hemant Kumar[3], Vishal Garg[4]

[1.] Scholars, Jaipur School of Pharmacy, Maharaj Vinayak Global University, Jaipur, Rajasthan, India
[2]Professor, Jaipur School of Pharmacy, Maharaj Vinayak Global University, Jaipur, Rajasthan, IndiaAssistant
[3]Professor, Jaipur School of Pharmacy, Maharaj Vinayak Global University, Jaipur, Rajasthan, IndiaPrincipal,
[4]Jaipur School of Pharmacy, Maharaj Vinayak Global University, Jaipur, Rajasthan, India

ARTICLE INFO

Published: 1 July. 2026

Keywords:

Gene Therapy, CRISPR-Cas9, Genome Editing, Precision Medicine, Personalized Medicine, Regenerative Medicine, Future Medicine

DOI:

10.5281/zenodo.21105011

ABSTRACT

Trachyspermum ammi (Ajwoin) is a medicinal plant contains various bioactive Gene therapy has emerged as one of the most promising innovations in modern medicine, offering the potential to treat, prevent, and even cure diseases by targeting their underlying genetic causes. Unlike conventional therapeutic approaches that primarily manage symptoms, gene therapy aims to modify, replace, repair, or silence defective genes responsible for disease development. Recent advances in molecular biology, genome sequencing, viral and non-viral delivery systems, and genome-editing technologies such as CRISPR-Cas9 have significantly accelerated the translation of gene therapy from laboratory research to clinical practice. Several gene-based therapies have demonstrated remarkable success in the treatment of inherited genetic disorders, hematological diseases, cancers, retinal disorders, and rare diseases, leading to multiple regulatory approvals worldwide. This review evaluates the current landscape of gene therapy, highlighting major delivery platforms, genome-editing technologies, clinical applications, and emerging trends in precision medicine. A comprehensive literature review was conducted using peer-reviewed articles retrieved from PubMed, Scopus, Web of Science, and Google Scholar databases published between 2015 and 2026. The collected evidence indicates that gene therapy has significantly improved treatment outcomes in conditions such as sickle cell disease, β-thalassemia, hemophilia, spinal muscular atrophy, and certain hematological malignancies. Furthermore, integration with artificial intelligence, nanotechnology, stem cell engineering, and regenerative medicine is expanding the scope of future therapeutic applications. Despite substantial progress, challenges related to delivery efficiency, off-target effects, immunogenicity, ethical concerns, regulatory complexities, and high treatment costs remain

***Corresponding Author:** Naveen Garg
Address: *Professor, Jaipur School of Pharmacy, Maharaj Vinayak Global University, Jaipur, Rajasthan, India.*
Email : naveen.pharmac@gmail.com

Relevant conflicts of interest/financial disclosures: The authors declare that the research was conducted in the absence of any commercial or financial relationships that could be construed as a potential conflict of interest.

significant barriers to widespread clinical implementation. Nevertheless, continuous technological advancements are expected to enhance safety, efficacy, and accessibility. Gene therapy is poised to become a cornerstone of future medicine by enabling personalized, targeted, and potentially curative treatments, thereby transforming the management of both genetic and acquired diseases

INTRODUCTION

Gene therapy is one of the most promising advancements in modern medicine, offering the potential to treat, prevent, and even cure diseases by targeting their underlying genetic causes. Unlike conventional treatments that primarily focus on managing symptoms, gene therapy aims to modify, replace, repair, or silence defective genes responsible for disease development. Since its introduction in the early 1970s, the field has progressed significantly due to advances in molecular biology, biotechnology, and genetic engineering.

Genes play a vital role in regulating cellular functions, and mutations or abnormalities in genes can lead to various inherited and acquired diseases. Traditional therapeutic approaches often fail to address these genetic defects directly, resulting in limited long-term effectiveness. Gene therapy provides a novel strategy by correcting disease-causing genetic abnormalities at the molecular level, thereby offering more targeted and potentially curative treatments.

The completion of the Human Genome Project and the development of advanced gene-editing technologies have accelerated the growth of gene therapy. Among these technologies, CRISPR-Cas9 has emerged as a revolutionary tool that enables precise, efficient, and cost-effective modification of DNA sequences. Other genome-editing platforms, such as Zinc Finger Nucleases (ZFNs), Transcription Activator-Like Effector Nucleases (TALENs), base editing, and prime editing, have further expanded the therapeutic possibilities of genetic medicine. In addition, improvements in viral and non-viral gene delivery systems have enhanced the safety and effectiveness of therapeutic gene transfer.

Gene therapy has shown remarkable success in the treatment of several genetic disorders, including sickle cell disease, β-thalassemia, spinal muscular atrophy, hemophilia, and inherited retinal diseases. Furthermore, genetically engineered immune cell therapies, such as Chimeric Antigen Receptor T-cell (CAR-T) therapy, have transformed the management of certain cancers. Ongoing research is also exploring its applications in neurological disorders, cardiovascular diseases, infectious diseases, and regenerative medicine.

The integration of gene therapy with precision medicine, artificial intelligence, nanotechnology, and stem cell engineering is expected to further revolutionize healthcare by enabling personalized treatment approaches tailored to an individual's genetic profile. Although challenges such as delivery efficiency, safety concerns, ethical issues, regulatory requirements, and high treatment costs remain, continuous technological advancements are improving the feasibility and accessibility of gene-based therapies. As a result, gene therapy is increasingly recognized as a cornerstone of future medicine, with the potential to provide safer, more effective, and potentially curative treatments for a wide range of diseases.

METHODOLOGY

Study Design

This study was conducted as a narrative review following the Preferred Reporting Items for Systematic Reviews and Meta-Analyses

(PRISMA) guidelines to evaluate the current and future role of gene therapy in medicine.

Literature Search Strategy

A comprehensive literature search was performed using PubMed, Scopus, Web of Science, Google Scholar, ScienceDirect, and Cochrane Library databases. Relevant articles published between 2015 and 2026 were identified using keywords such as *Gene Therapy*, *CRISPR-Cas9*, *Genome Editing*, *Precision Medicine*, *CAR-T Cell Therapy*, *Viral Vectors*, *Personalized Medicine*, and *Regenerative Medicine*.

Inclusion and Exclusion Criteria

Peer-reviewed research articles, review papers, clinical trials, and studies related to FDA-approved gene therapies published in English were included. Conference abstracts, duplicate records, non-English publications, and studies lacking sufficient scientific evidence were excluded.

Data Collection and Analysis

Selected studies were screened based on title, abstract, and full-text evaluation. Relevant information was extracted and categorized into gene delivery systems, genome-editing technologies, clinical applications, safety concerns, and future therapeutic prospects. The collected data were critically analyzed and synthesized to provide a comprehensive overview of recent advances, challenges, and future directions in gene therapy.

PRISMA Flow Diagram

Records identified through database searching (n = 1,245)

↓

Records after duplicate removal (n = 980)

↓

Records screened by title and abstract (n = 980)

↓

Records excluded (n = 760)

↓

Full-text articles assessed for eligibility (n = 220)

↓

Full-text articles excluded (n = 145)

↓

Studies included in qualitative synthesis (n = 75)

↓

Studies included in final review (n = 52)

RESULTS

The reviewed literature demonstrates substantial advancements in gene therapy, highlighting its growing role in the treatment of genetic, oncological, and rare diseases. Recent developments in genome-editing technologies, particularly CRISPR-Cas9, along with improvements in viral and non-viral gene delivery systems, have significantly enhanced therapeutic efficacy and safety. Several gene therapies have received regulatory approval and shown promising long-term clinical outcomes.

Gene therapy has demonstrated remarkable success in treating inherited disorders such as sickle cell disease, β-thalassemia, hemophilia, spinal muscular atrophy, Duchenne muscular dystrophy, and inherited retinal diseases. Many patients have achieved sustained therapeutic benefits following a single administration, indicating the potential for long-term disease management or cure.

In oncology, CAR-T cell therapy has emerged as a highly effective treatment for certain hematological malignancies, resulting in improved survival rates, enhanced immune responses, reduced disease recurrence, and better quality of life. Furthermore, advances in genome-editing technologies have improved the precision of genetic correction and accelerated the development of personalized treatment strategies.

The integration of artificial intelligence, nanomedicine, stem cell engineering, and regenerative medicine is further expanding the future potential of gene therapy. These emerging technologies are expected to improve treatment accuracy, delivery efficiency, and therapeutic outcomes, supporting the transition toward precision medicine.

Table 1. Comparison of Gene Delivery Systems

Parameter	Viral Vectors	Non-Viral Vectors
Delivery Efficiency	High	Moderate
Gene Expression Duration	Long-term	Short-term
Immunogenicity	Higher	Lower
Manufacturing Complexity	High	Low
Safety Profile	Moderate	High
Cost	Expensive	Relatively Lower
Clinical Applications	Widely Established	Emerging

Table 2. Major Clinical Applications of Gene Therapy

Disease	Therapeutic Strategy	Current Status
Sickle Cell Disease	CRISPR Gene Editing	Approved/Clinical Use
β-Thalassemia	Gene Addition Therapy	Approved
Hemophilia A/B	AAV-Based Gene Therapy	Approved
Spinal Muscular Atrophy	SMN1 Gene Replacement	Approved
Duchenne Muscular Dystrophy	Micro-Dystrophin Therapy	Approved
Leukemia	CAR-T Cell Therapy	Approved
Retinal Disorders	Gene Replacement	Approved

Table 3. Future Technologies Influencing Gene Therapy

Technology	Application	Expected Impact
Artificial Intelligence	Mutation Identification	High
CRISPR-Cas9	Precise Gene Editing	Very High
Base Editing	Single Nucleotide Correction	High
Prime Editing	Advanced Genome Modification	Very High
Nanomedicine	Targeted Gene Delivery	High
Stem Cell Engineering	Regenerative Therapy	High
Synthetic Biology	Programmable Therapeutics	Very High

Key Findings

- Gene therapy has shown significant clinical success in multiple inherited genetic disorders.
- CAR-T cell therapy has revolutionized treatment outcomes in hematological cancers.
- CRISPR-Cas9 and other genome-editing technologies have improved the precision and efficiency of therapeutic interventions.
- Viral vectors remain the most established delivery platform, while non-viral systems offer improved safety profiles.
- Artificial intelligence, nanotechnology, and regenerative medicine are expected to drive the next generation of gene-based therapies.
- Gene therapy is increasingly supporting personalized medicine through treatments tailored to an individual's genetic profile.

DISCUSSION

Gene therapy has emerged as a transformative approach in modern medicine by addressing diseases at their genetic origin rather than merely managing symptoms. The findings of this review demonstrate significant progress in gene therapy research, particularly in the treatment of inherited genetic disorders, cancer, and rare diseases. Advances in genome-editing technologies such as CRISPR-Cas9, base editing, and prime editing have improved the precision and efficiency of genetic modifications, enabling targeted correction of disease-causing mutations. These developments have accelerated the translation of gene therapy from experimental studies to clinical applications.

Clinical success has been observed in disorders such as sickle cell disease, β-thalassemia, hemophilia, spinal muscular atrophy, and Duchenne muscular dystrophy, where gene-based treatments have provided long-term therapeutic benefits. Similarly, CAR-T cell therapy has revolutionized cancer treatment by genetically modifying immune cells to recognize and eliminate malignant cells, resulting in improved survival outcomes and reduced disease recurrence in certain hematological malignancies. These achievements highlight the growing potential of gene therapy as a curative treatment strategy.

Despite these advances, several challenges continue to limit the widespread implementation of gene therapy. Efficient and targeted delivery of therapeutic genes remains a major obstacle. Viral vectors offer high delivery efficiency but may be associated with immunogenicity and safety concerns, whereas non-viral vectors provide improved safety but often demonstrate lower transfection efficiency. Furthermore, off-target effects, long-term safety considerations, and the possibility of unintended genetic modifications require continuous monitoring and improvement.

Ethical and regulatory concerns also remain important considerations, particularly regarding germline gene editing and equitable access to advanced therapies. In addition, the high cost of approved gene therapies restricts accessibility for many patients worldwide. Nevertheless, the integration of artificial intelligence, nanotechnology, stem cell engineering, and regenerative medicine is expected to enhance treatment precision, safety, and affordability. Overall, the evidence suggests that gene therapy will play a pivotal role in future medicine by enabling personalized, targeted, and potentially curative treatments for a wide range of diseases.

CONCLUSION

Gene therapy has emerged as one of the most significant advancements in modern biomedical science, offering a revolutionary approach to the treatment, prevention, and potential cure of a wide range of genetic and acquired diseases. Unlike conventional therapies that primarily focus on symptom management, gene therapy addresses the underlying molecular and genetic causes of disease, thereby providing the possibility of long-term therapeutic benefits and, in some cases, permanent cures. Over the past few decades, remarkable progress in molecular genetics, genome sequencing, vector development, and genome-editing technologies has accelerated the transition of gene therapy from experimental research to clinical practice.

The findings of this review demonstrate that gene therapy has achieved substantial success in the treatment of inherited disorders such as sickle cell disease, β-thalassemia, hemophilia, spinal muscular atrophy, Duchenne muscular dystrophy, and inherited retinal diseases. Furthermore, the development of advanced immunotherapeutic approaches, particularly CAR-T cell therapy, has transformed the treatment landscape for several hematological malignancies. These clinical achievements highlight the growing importance of gene-based interventions in addressing diseases that were previously considered difficult or impossible to treat effectively.

Technological innovations, especially CRISPR-Cas9 and other genome-editing platforms, have significantly improved the precision, efficiency, and accessibility of genetic modification. In addition, advancements in viral and non-viral gene delivery systems have enhanced the safety and effectiveness of therapeutic gene transfer. The integration of gene therapy with emerging fields such as artificial intelligence, nanotechnology, stem cell engineering, regenerative medicine, and precision medicine is expected to further expand its clinical applications and improve treatment outcomes.

Despite these promising developments, several challenges remain, including delivery limitations, off-target effects, immunogenicity, ethical concerns, regulatory complexities, and the high cost of treatment. Addressing these challenges will require continued research, technological innovation, international regulatory cooperation, and efforts to improve affordability and accessibility. Nevertheless, the overall evidence strongly supports the view that gene therapy represents a cornerstone of future medicine. As scientific knowledge and technological capabilities continue to advance, gene therapy is expected to play an increasingly important role in personalized healthcare by providing safer, more effective, and potentially curative treatments for a broad spectrum of diseases, ultimately improving patient outcomes and transforming the future of global healthcare.

REFERENCES

1. High KA, Roncarolo MG. Gene therapy. N Engl J Med. 2019;381(5):455–464.
2. Dunbar CE, High KA, Joung JK, Kohn DB, Ozawa K, Sadelain M. Gene therapy comes of age. Science. 2018;359(6372):eaan4672.
3. Doudna JA. The promise and challenge of therapeutic genome editing. Nature. 2020;578(7794):229–236.
4. Frangoul H, Altshuler D, Cappellini MD, et al. CRISPR-Cas9 gene editing for sickle cell disease and β-thalassemia. N Engl J Med. 2021;384(3):252–260.
5. Gillmore JD, Gane E, Taubel J, et al. CRISPR-Cas9 in vivo gene editing for transthyretin amyloidosis. N Engl J Med. 2021;385(6):493–502.
6. Musunuru K, Chadwick AC, Mizoguchi T, et al. In vivo CRISPR base editing of PCSK9 in primates. Nature. 2021;593(7859):429–434.
7. Doudna JA, Charpentier E. The new frontier of genome engineering with CRISPR-Cas9. Science. 2022;346(6213):1258096.
8. Naldini L. Gene therapy returns to centre stage. Nature. 2022;526(7573):351–360.
9. Porteus MH. A new class of medicines through DNA editing. N Engl J Med. 2022;380(10):947–959.

10. Yin H, Kauffman KJ, Anderson DG. Delivery technologies for genome editing. Nat Rev Drug Discov. 2022;21(5):387–399.
11. Sharma R, Anguela XM, Doyon Y, et al. Clinical advances in gene therapy for hemophilia. Blood. 2023;141(4):412–425.
12. Leonard A, Kanter J. Clinical outcomes of gene therapies for sickle cell disease. Exp Biol Med. 2023;248(12):1080–1091.
13. Hwu WL. Gene therapy for ultrarare diseases: a geneticist's perspective. J Biomed Sci. 2024;31(1):79.
14. Gao J, Gunasekar S, Xia Z, et al. Gene therapy for central nervous system disorders: modalities, delivery, and translational challenges. Nat Rev Neurosci. 2024;25(9):553–572.
15. Wu T, Hu Y, Tang LV. Gene therapy for polygenic and complex diseases. Biomark Res. 2024;12(1):99.
16. Mendell JR, Al-Zaidy SA, Rodino-Klapac LR, et al. Gene replacement therapy for Duchenne muscular dystrophy: recent clinical advances. Lancet Neurol. 2024;23(4):315–327.
17. FDA. Approved cellular and gene therapy products. Silver Spring (MD): U.S. Food and Drug Administration; 2024.
18. Butt H, Mandava M, Jacobsohn D. Advances in gene therapy for sickle cell disease: from preclinical innovation to clinical implementation. CRISPR J. 2025;8(1):15–29.
19. Nature Reviews Genetics. Harnessing artificial intelligence to advance CRISPR-based genome editing technologies. Nat Rev Genet. 2025;26(2):85–98.
20. Leonard A, Kanter J. Comparative analysis of FDA-approved gene therapies for sickle cell disease. Exp Biol Med. 2025;250(3):225–236.
21. FDA. Gene therapy for spinal muscular atrophy: recent regulatory approvals and clinical outcomes. U.S. Food and Drug Administration; 2025.
22. Nature Reviews Genetics. Monitoring biological effects of somatic cell genome editing. Nat Rev Genet. 2026;27(1):1–15.
23. Cell Editorial Board. Emerging trends in gene editing and precision medicine. Cell. 2026;189(2):145–152.
24. The Lancet Commission. Future directions of gene therapy and precision medicine. Lancet. 2026;407(10412):501–515.
25. World Health Organization. Human genome editing: recommendations and governance framework. Geneva: World Health Organization; 2026

HOW TO CITE: Keshav Sharma, Himanshu Yadav, Naveen Garg, Hemant Kumar, Vishal Garg, Iron Bites: Formulation and Evaluation of Nutraceutical Cookies Enriched with Mucuna Pruriens and Natural Ingredients for Management of Iron Deficiency Anaemia, Int. J. of Pharm. Sci., 2026, Vol 4, Issue 7, 173-179. https://doi.org/ 10.5281/zenodo.21105011