

www.pharmaerudition.org

ISSN: 2249-3875



International Journal of Pharmaceutical Erudition

Research for Present and Next Generation

MAY 2026

Vol: 16 Issue:01
(36-40)





Review Article

A REVIEW ON GENE THERAPY IN CANCER TREATMENT

Mushtaq Basit*, Mistry Kamalesh , Zahid, Tamheed , Sharma Tanya

Faculty of Pharmaceutical Science, Mewar University, Chittorgarh, Rajasthan, India

Gene therapy has emerged as a promising and innovative approach in the treatment of cancer by targeting the genetic abnormalities responsible for tumor development and progression. Unlike conventional therapies such as chemotherapy and radiotherapy, which often affect both normal and cancerous cells, gene therapy offers a more specific and targeted strategy by modifying or manipulating genetic material within cells. This project provides an overview of the principles, mechanisms, and applications of gene therapy in cancer treatment. Various strategies such as replacement of mutated genes, inactivation of oncogenes, introduction of therapeutic (suicide) genes, and enhancement of the immune response have been discussed. The role of gene delivery systems, including viral vectors like adenoviruses and retroviruses, as well as non-viral methods such as liposomes and nanoparticles, has also been highlighted. Furthermore, the project explores different gene therapy approaches including immunotherapy, oncolytic virotherapy, and tumor suppressor gene therapy. Clinical applications in various cancers, along with the advantages such as target specificity and reduced side effects, are discussed. However, limitations such as delivery challenges, immune responses, high cost, and safety concerns remain significant barriers. Recent advancements, including the development of CRISPR-Cas9 gene editing and improved vector systems, have shown great potential in overcoming these challenges. In conclusion, gene therapy represents a rapidly evolving field with the potential to revolutionize cancer treatment by providing more effective, personalized, and targeted therapeutic options.

Keywords: Cancer, etiology, diagnostic advances, therapeutic strategies, oncolytic virotherapy.

INTRODUCTION

Cancer is a complex genetic disease characterized by uncontrolled cell growth caused by mutations in genes regulating cell division and survival. Conventional treatments such as chemotherapy, radiotherapy, and surgery often lack specificity and may damage normal cells. Gene therapy has emerged as a promising and targeted approach that aims to treat cancer at the molecular level by modifying genetic material within cells. Gene therapy involves the introduction, removal, or modification of genetic material to correct defective genes or to enhance the body's ability

to fight disease. It represents a shift from traditional therapies toward precision medicine, offering more targeted and potentially less toxic treatments.⁽¹⁾

Concept and Mechanism of Gene Therapy

Gene therapy is a modern therapeutic approach that focuses on modifying the genetic material (DNA or RNA) inside a patient's cells to treat or prevent disease. In cancer, it specifically targets the genetic mutations and abnormal gene expression responsible for uncontrolled cell growth. Unlike conventional treatments, gene therapy acts at the molecular



level, correcting or manipulating genes to restore normal cellular function or to destroy cancer cells.⁽²⁾

Replacement of Mutated Genes

Cancer often occurs due to mutations in important regulatory genes, especially tumor suppressor genes such as p53. These genes normally control cell division, repair DNA damage, and induce apoptosis (programmed cell death). In gene therapy, a normal (wild-type) copy of the defective gene is introduced into cancer cells. This restores the lost function, helping cells regain control over growth and division. For example, restoring p53 function can trigger apoptosis in cancer cells. Outcome: Stops tumor progression and promotes cancer cell death.⁽³⁾

Inactivation of Oncogenes

Oncogenes are mutated or over expressed versions of normal genes (proto-oncogenes) that drive uncontrolled cell proliferation. Gene therapy can silence or block these oncogenes using techniques such as: RNA interference (RNAi), Antisense oligonucleotide. These methods prevent the production of proteins that promote tumor growth. Outcome: Slows or halts cancer cell multiplication.⁽⁴⁾

Introduction of Therapeutic Genes

New genes can be introduced into cancer cells to actively destroy them. This includes: Suicide Gene Therapy. A gene is inserted into cancer

cells that converts a non-toxic drug (prodrug) into a toxic substance. Only cancer cells with this gene are killed when the drug is administered. Example: Herpes simplex virus thymidine kinase (HSV-TK) gene + ganciclovir drug. Pro-apoptotic Genes, Genes that directly induce apoptosis are introduced into tumor cells. Outcome: Selective killing of cancer cells with minimal damage to normal tissues.⁽⁵⁾

Enhancement of Immune Response

Gene therapy can strengthen the body's immune system to recognize and destroy cancer cells. Immune cells (like T-cells) are genetically modified to better target tumors. Example: CAR-T cell therapy. T-cells are engineered to express receptors that recognize cancer-specific antigens. Genes encoding cytokines (immune-stimulating proteins) can also be introduced to boost immune activity. Outcome: Improved immune-mediated destruction of tumors.⁽⁶⁾

Overall Therapeutic Effect

All these mechanisms work together to:

- Eliminate cancer cells directly
- Inhibit tumor growth and metastasis
- Increase sensitivity to chemotherapy and radiotherapy
- Reduce damage to normal healthy cells⁽⁷⁾

Methods of Gene Delivery



Efficient delivery of genetic material is critical for successful gene therapy. Delivery methods are broadly classified into: Viral Vectors. Viruses are commonly used because of their natural ability to deliver genetic material into cells. Common viral vectors include: Adenoviruses, Retroviruses, Lentiviruses, Adeno-associated viruses (AAV). These vectors can either integrate into the host genome for long-term expression or remain non-integrating for temporary effects.⁽⁸⁾

Non-Viral Methods

These include:

Liposomes

Nanoparticles

Electroporation

Gene gun

Types of Gene Therapy Strategies in Cancer

Several strategies are used in cancer gene therapy:

Tumor Suppressor Gene Therapy

This involves introducing normal copies of tumor suppressor genes (e.g., p53) to restore their function and inhibit tumor growth. Suicide Gene Therapy. Genes are introduced into cancer cells that convert non-toxic drugs into toxic compounds.⁽⁹⁾

selectively killing cancer cells. Immunotherapy (Gene-based). Genetically modified immune cells are used to recognize and destroy cancer cells. For example: CAR-T cell therapy.

Oncolytic Virotherapy. Genetically engineered

viruses infect and destroy cancer cells while sparing normal cells. Anti-angiogenic Gene Therapy. Targets blood vessel formation in tumors, cutting off nutrient supply. RNA-based Therapy includes antisense RNA and RNA interference (RNAi) to block harmful gene expression.⁽¹⁰⁾

Clinical Applications

Gene therapy has been tested in various cancers such as:

Lung cancer

Breast cancer

Prostate cancer

Melanoma

Brain tumors

Some approved gene therapy products include:

Gendicine (China) – p53 gene therapy

Oncorine– oncolytic virus therapy

Clinical trials show that gene therapy can:

Reduce tumor size

Improve survival rates

Enhance effectiveness of chemotherapy and radiotherapy.⁽¹¹⁾

Advantages of Gene Therapy in Cancer

Target specificity – acts directly on cancer cells

Reduced side effects compared to chemotherapy

Potential for long-term effects. Combination therapy – works with chemo/radiotherapy

Personalized treatment based on genetic profile.⁽¹²⁾

Limitations and Challenges



Despite its potential, gene therapy faces several challenges: Delivery issues (efficient targeting of tumor cells) Immune response against vectors Short duration of gene expression (in some cases) High cost Ethical concerns Limited success in clinical trials so far Future Perspectives Gene therapy is rapidly advancing with innovations such as:⁽¹³⁾

CRISPR-Cas9 gene editing Personalized gene therapy .Combination with immunotherapy Improved delivery systems (nanotechnology) Ongoing research aims to overcome current limitations and expand its application to more cancer types . As technology improves, gene therapy may become a standard cancer treatment in the future .⁽¹⁴⁻¹⁵⁾

CONCLUSION

Gene therapy represents a revolutionary and rapidly advancing approach in the field of cancer treatment, focusing on correcting or manipulating the genetic defects responsible for tumor development . Unlike conventional therapies, gene therapy provides a targeted and precise strategy by acting at the molecular level, thereby improving treatment efficacy while minimizing damage to normal cells . Various gene therapy strategies, including tumor suppressor gene replacement, oncogene inhibition, suicide gene therapy, immunotherapy, and oncolytic virotherapy, have demonstrated significant potential in preclinical and clinical studies . The

www.pharmaerudition.org May 2026, 16(1), 36-40

development of efficient gene delivery systems, particularly viral vectors such as adenoviruses and retroviruses, has played a crucial role in enhancing therapeutic outcomes . Despite these promising advancements, several challenges remain, including issues related to safe and efficient gene delivery, immune responses, high treatment costs, and long-term safety concerns .However, recent technological innovations such as CRISPR-Cas9 gene editing, improved vector design, and personalized medicine approaches are expected to overcome these limitations in the near future . In conclusion, gene therapy holds great promise as a next-generation cancer treatment modality . With continued research, clinical trials, and technological advancements, it has the potential to become a standard and highly effective treatment option, offering improved survival rates and better quality of life for cancer patients.

REFERENCE

1. Qi L, Li G, Li P, Wang H, Fang X, He T, et al. Twenty years of Gendicine rAd-p53 cancer gene therapy: The first-in-class human cancer gene therapy in the era of personalized oncology. *Genes & Diseases*. 2023;11(4):101155.
2. Li Y, Qin X, Liang C, Wang L. Progress in the research and development of oncolytic virus therapies. *Front Pharmacol*. 2026;17:1751206.



3. Geng G, et al. Viral and non-viral vectors in gene therapy: current state and clinical perspectives. *eBioMedicine*. 2025;112:105542.
4. Chesney J, et al. Talimogene Laherparepvec: A Review in Advanced Melanoma. *Target Oncol*. 2023;18(2):285-296.
5. Ginn SL, et al. Gene therapy clinical trials worldwide to 2023—an update. *J Gene Med*. 2024;26(1):e3641.
6. Khoshandam M, et al. CRISPR, CAR-T, and NK: Current applications and future perspectives in cancer immunotherapy. *J Control Release*. 2024;365:123-145.
7. Lei T, et al. Leveraging CRISPR gene editing technology to optimize CAR T-cells therapy. *Leukemia*. 2024;38:215-230.
8. Cervera ST, et al. Suicide gene therapy targeting Ewing sarcoma via an oncolytic adenovirus. *Sci Rep*. 2025;15:14945.
9. Oishi T, et al. Efficacy of HSV-TK/GCV system suicide gene therapy using various delivery methods. *Mol Ther Oncolytics*. 2022;26:312-325.
10. Dhuri A, et al. Current advances in non-viral nanoparticle-based gene delivery systems for cancer therapy. *J Drug Deliv Sci Technol*. 2024;91:105241.
11. Zhang Y, et al. CRISPR-Cas9 technology: a breakthrough in cancer gene therapy. *J Genet Eng Biotechnol*. 2024;22:15.
12. Wang Z, et al. Angiogenic signaling pathways and anti-angiogenic therapy for cancer. *Signal Transduct Target Ther*. 2023;8:198.
13. Li Y, et al. Expert consensus on the clinical application of Gendicine in head and neck squamous cell carcinoma. *Int J Oral Sci*. 2021;13:15.
14. Amiri M, et al. The synergistic potential of CAR-T cell therapy and CRISPR/Cas9 gene editing in cancer treatment. *Front Immunol*. 2024;15:1462697.
15. Ingle RG, et al. Advancements and applications of gene therapy in severe genetic disorders and cancer. *J Pers Med*. 2025;15(1):93.

Conflict of Interest

The authors declare that they have no conflict of interest