

## GENE THERAPY: EMERGING TRENDS AND ITS TRANSFORMATIVE ROLE IN MODERN MEDICINE

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### ABSTRACT

Gene therapy has rapidly evolved as an innovative therapeutic strategy aimed at modifying genetic material to treat or prevent diseases at their molecular origin. Unlike conventional pharmacotherapy, which primarily alleviates symptoms, gene therapy focuses on correcting or regulating defective genes responsible for disease progression. This review provides a critical overview of gene therapy, including delivery systems, genome-editing technologies such as CRISPR-Cas9, and various therapeutic approaches. Recent clinical successes, particularly in chimeric antigen receptor T-cell (CAR-T) therapy and monogenic disorders, highlight its translational significance. However, challenges such as immune responses, off-target effects, ethical concerns, and high treatment costs remain barriers to widespread adoption. Regulatory frameworks and ongoing clinical trials are continuously addressing these issues. The integration of gene therapy with

personalized medicine further enhances its potential in targeted treatment. Overall, gene therapy represents a paradigm shift toward precision medicine and holds promise as a future cornerstone of healthcare systems.

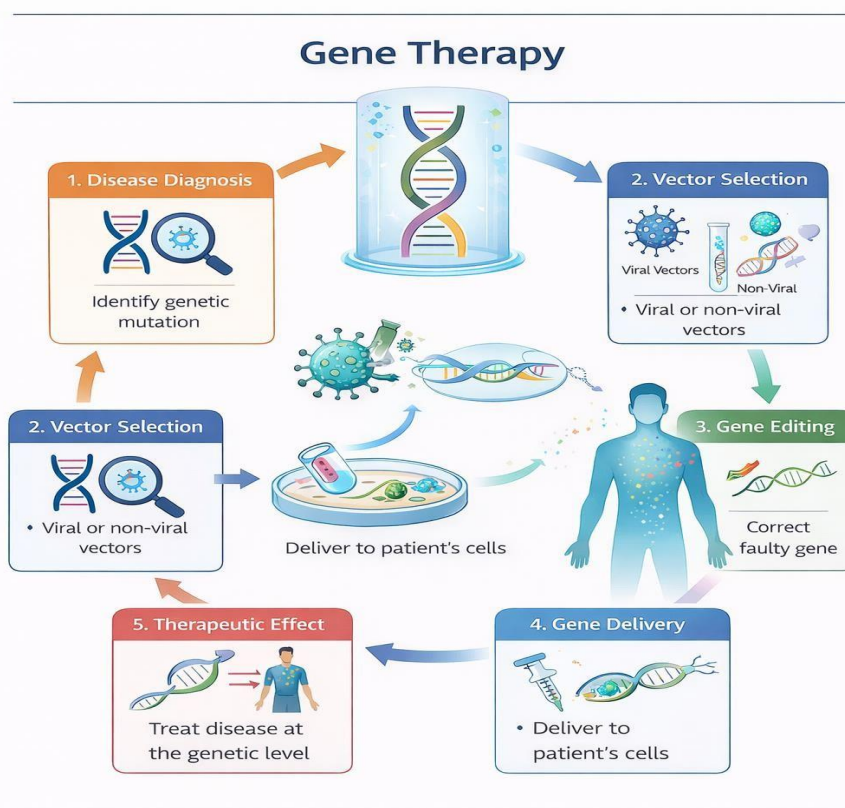
**KEYWORDS:** Gene therapy; CRISPR-Cas9; Viral vectors; Personalized medicine; Genome editing; CAR-T therapy.

## 1. INTRODUCTION

Gene therapy is a rapidly advancing field in biomedical science that involves the deliberate modification of genetic material to treat or prevent diseases. This strategy aims to address the fundamental genetic abnormalities responsible for disease development rather than merely managing clinical symptoms. By introducing, removing, or altering specific DNA sequences, gene therapy provides a targeted and potentially curative approach to a wide range of disorders.<sup>[1]</sup>

Technological advancements in molecular biology, particularly in recombinant DNA technology and genome editing tools such as CRISPR-Cas9, have significantly improved the accuracy and feasibility of gene-based interventions.<sup>[2]</sup> These innovations have expanded the therapeutic scope of gene therapy beyond rare inherited disorders to include complex diseases such as cancer and neurological conditions.

Despite these advancements, several challenges remain, including efficient gene delivery, immunogenicity, long-term safety, and ethical considerations. Continuous research and clinical evaluation are essential to overcome these limitations and ensure safe clinical translation.<sup>[3]</sup>

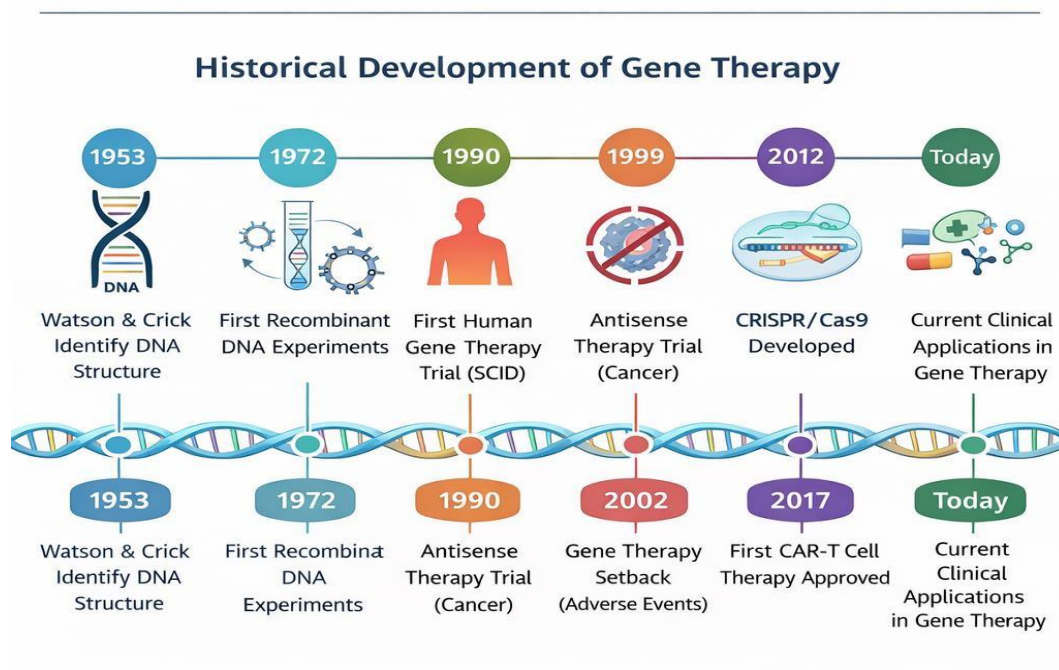


## 2. Historical Development of Gene Therapy

The conceptual foundation of gene therapy was established following the discovery of the DNA double-helix structure in 1953. Subsequent developments in genetic engineering during the 1970s enabled scientists to manipulate DNA sequences, laying the groundwork for therapeutic applications.<sup>[4]</sup>

The first successful human gene therapy trial was conducted in 1990 to treat severe combined immunodeficiency (SCID), marking a milestone in medical history.<sup>[5]</sup> However, early clinical setbacks, including adverse immune reactions, highlighted safety concerns and led to stricter regulatory oversight.

The emergence of advanced genome-editing technologies, particularly CRISPR-Cas9, has significantly enhanced the precision and efficiency of gene modification, revitalizing interest in gene therapy as a viable clinical approach.<sup>[6]</sup>



## 3. Classification of Gene Therapy

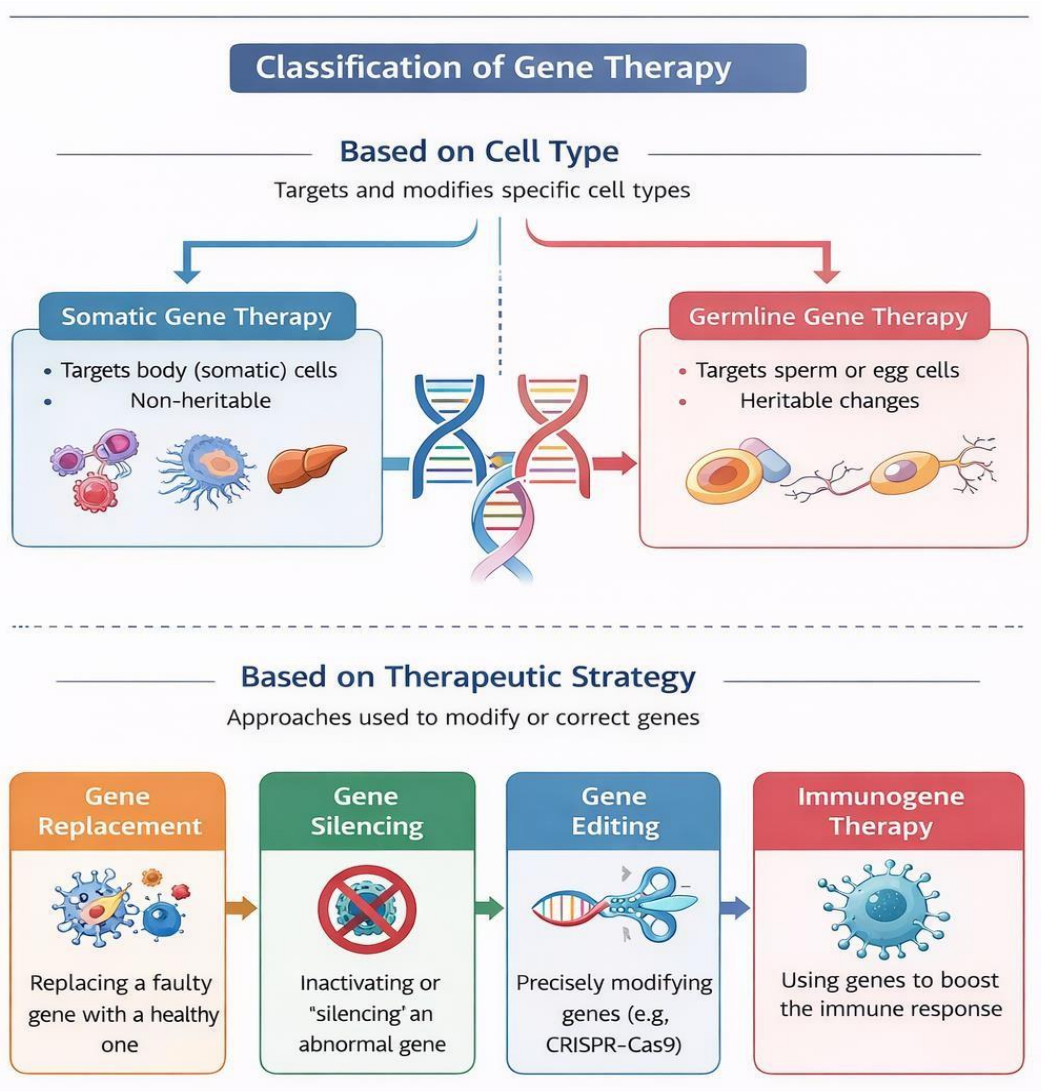
### 3.1 Based on Target Cells

- **Somatic Gene Therapy:** Targets non-reproductive cells; modifications are not inherited
- **Germline Gene Therapy:** Involves genetic changes in reproductive cells; ethically restricted due to heritable implications.

### 3.2 Based on Therapeutic Strategy

- Gene replacement
- Gene silencing
- Gene augmentation
- Gene editing
- Immunogenic therapy

These strategies collectively provide diverse approaches for correcting genetic abnormalities and improving therapeutic outcomes.<sup>[7]</sup>

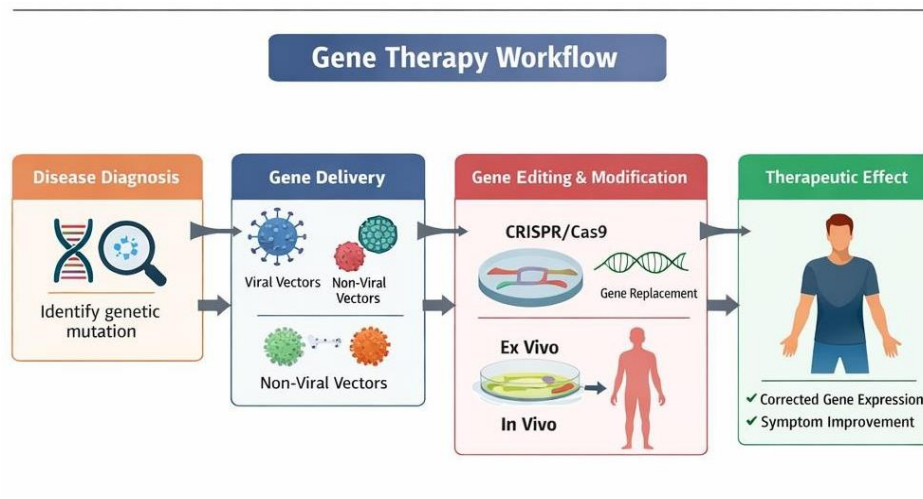


### 4. Gene Delivery Techniques

Efficient delivery of therapeutic genes is critical for successful gene therapy. Delivery systems are broadly categorized into viral and non-viral approaches.

**Viral vectors**, including adenoviruses, retroviruses, lentiviruses, and adeno-associated viruses (AAV), are widely used due to their high transduction efficiency. However, they may induce immune responses and pose risks such as insertional mutagenesis.<sup>[8]</sup>

**Non-viral methods**, such as liposomes, nanoparticles, and plasmid DNA, offer improved safety profiles but generally exhibit lower delivery efficiency. Ongoing research focuses on optimizing these systems for enhanced stability and targeted delivery.<sup>[9]</sup>



#### Comparison of Viral and Non-Viral Vectors

Feature	Viral Vectors	Non-Viral Vectors
<b>Efficiency</b>	High	Moderate
<b>Safety</b>	Risk of immune response	Low immunogenicity
<b>Capacity</b>	Limited (8-10 kb)	High (20 kb+)
<b>Stability</b>	Potential for insertional mutagenesis	Stable, low integration

### 5. Applications of Gene Therapy

Gene therapy has demonstrated significant potential across multiple therapeutic areas:

- **Genetic disorders:** Hemophilia, cystic fibrosis, SCID
- **Cancer:** CAR-T cell therapy targeting malignant cells
- **Neurological disorders:** Parkinson's disease, Alzheimer's disease
- **Infectious diseases:** Emerging applications in viral infections

These applications highlight the expanding clinical relevance of gene therapy in modern medicine.<sup>[10]</sup>

## 6. Advantages of Gene Therapy

Gene therapy offers several advantages over conventional treatment approaches:

- Targets underlying genetic causes
- Potential for long-term or permanent therapeutic effects
- Reduced systemic side effects
- Enables personalized treatment strategies

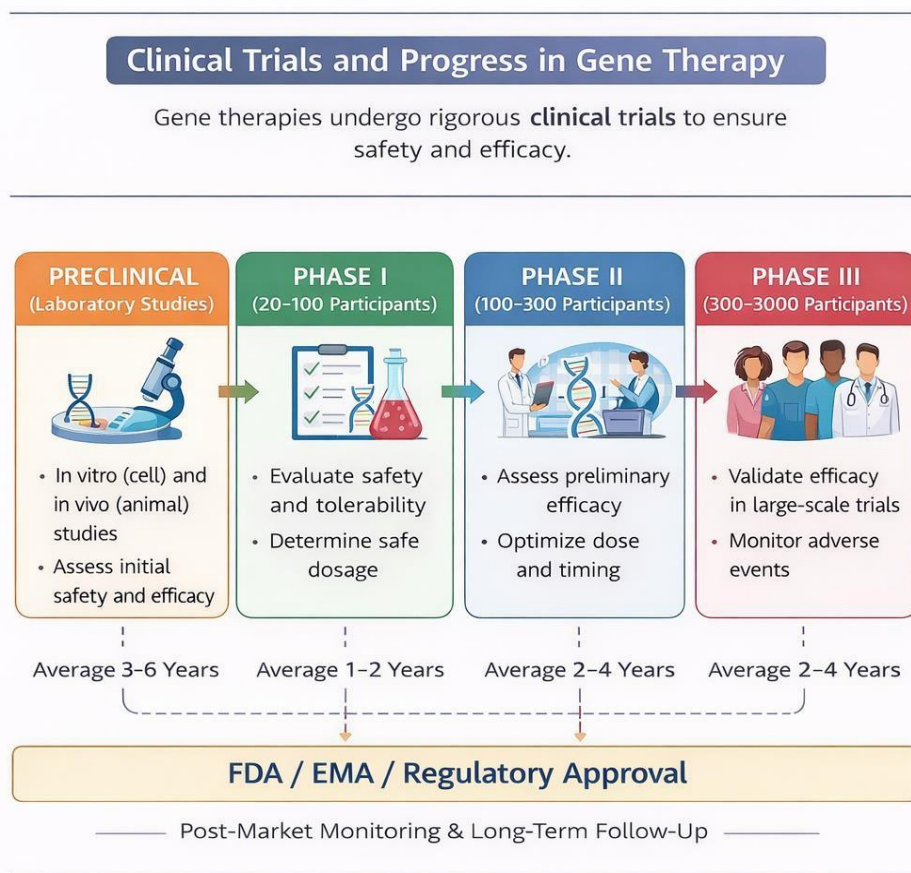
These benefits position gene therapy as a promising alternative to traditional pharmacological interventions.<sup>[11]</sup>

## 7. Clinical Trials and Progress

Gene therapy undergoes rigorous clinical evaluation through phased trials:

- **Phase I:** Safety and tolerability
- **Phase II:** Preliminary efficacy and dose optimization
- **Phase III:** Large-scale validation

Recent clinical approvals of gene therapies indicate substantial progress toward clinical implementation.<sup>[12]</sup>



## 8. Role in Personalized Medicine

Gene therapy plays a pivotal role in personalized medicine by enabling treatments tailored to an individual's genetic profile. This approach enhances therapeutic precision and reduces adverse effects, particularly in oncology and rare genetic disorders.<sup>[13]</sup>

## 9. Challenges and Safety Concerns

Despite its potential, gene therapy faces several limitations:

- Immune responses to vectors
- Off-target genetic modifications
- High treatment costs
- Ethical concerns related to germline editing

Long-term safety monitoring is essential to ensure the reliability of these therapies.<sup>[14]</sup>

## 10. Regulatory Framework

Gene therapy is regulated by international and national agencies, including:

- U.S. Food and Drug Administration (FDA)
- European Medicines Agency (EMA)
- Central Drugs Standard Control Organization (CDSCO, India)

These organizations ensure compliance with safety, ethical, and clinical standards.<sup>[15]</sup>

## 11. Future Perspectives

Advancements in genome editing, artificial intelligence, and nanotechnology are expected to further enhance gene therapy applications. The integration of these technologies may lead to more efficient, cost-effective, and widely accessible treatments in the future.<sup>[16]</sup>

## 12. CONCLUSION

Gene therapy represents a transformative approach in modern medicine by enabling targeted intervention at the genetic level. Although challenges such as safety, cost, and ethical considerations persist, continuous advancements in technology and regulatory frameworks are facilitating its clinical translation. With further research and innovation, gene therapy is expected to play a central role in the evolution of precision medicine and future healthcare systems.

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