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Review ETPM

A Review on Design of Gene Therapy and Its Potential for Craniofacial Regeneration

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Abstract

One of the most talked-about topics of the 21st century is gene therapy, which holds the promise of treating many diseases. Current gene therapy research explores a wide range of potential treatments, such as enhancing the body's immune response to tumors, promoting the formation of new blood vessels in the heart to mitigate heart attacks, and preventing HIV replication in AIDS patients. Gene therapy involves the introduction, alteration, or replacement of genetic material within a patient's cells to treat or prevent disease. This innovative approach has evolved significantly over the years, overcoming early challenges related to gene delivery, immune responses, and ethical considerations. Advances in viral and non-viral vector systems have improved the efficiency and safety of gene transfer, making it possible to target specific diseases more effectively. Materials and Methods: Relevant articles were thoroughly analyzed and reviewed to assess the historical, current, and future perspectives of gene therapy. Various clinical trials and experimental studies have provided valuable insights into gene-editing techniques, including CRISPR-Cas9, zinc finger nucleases, and RNA interference, all of which have expanded the therapeutic possibilities of gene-based treatments. Unlike traditional gene replacement therapy, craniofacial regeneration aims to utilize genetic vectors as supplementary elements for tissue growth and repair. The combination of viral gene therapy with craniofacial tissue engineering presents a powerful synergy that can greatly improve our capacity to repair and regenerate tissues directly within the body.

Keywords: Gene therapy, genetic disease, perspective, methods of gene therapy, viral and non-viral vectors

INTRODUCTION

In recent years, significant progress in recombinant DNA technology and cell biology has brought the once-distant dream of certain medical breakthroughs closer to reality.

1. Scientists are developing new methods for efficiently producing essential proteins, such as insulin for diabetic patients. Engineered bacteria, when introduced into the body, can be cultivated and used to produce these proteins in individuals who cannot naturally produce them.
2. Gene therapy is broadly defined as the transfer of genetic material aimed at curing a disease or, at the very least, improving a patient's clinical condition [1].
3. Genes consist of long strands of DNA molecules and are organized in a sequential manner within chromosomes.
4. The genetic message is encoded by DNA subunits known as nucleotides. Based on the type of viral genome, gene therapy vectors can be classified into RNA and/or DNA viral vectors. Among DNA virus vectors, adenoviruses and adeno-associated viruses (AAVs) are the most commonly used [2].
5. Simple retroviruses, like the murine leukemia virus, are the source of most RNA virus-based vectors. However, the inability of these vectors to transduce cells that are not dividing is a major drawback [3].

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GENE THERAPY

Gene therapy is the process of altering an individual's DNA to treat or cure illnesses. It can function through various mechanisms, such as replacing a disease-causing gene with a healthy version or deactivating a malfunctioning gene [4].

Gene therapy aims to replace a defective gene or introduce a new one to potentially cure a disease or enhance the body's capacity to combat it. This approach offers hope for treating a diverse array of conditions (Figure 1).

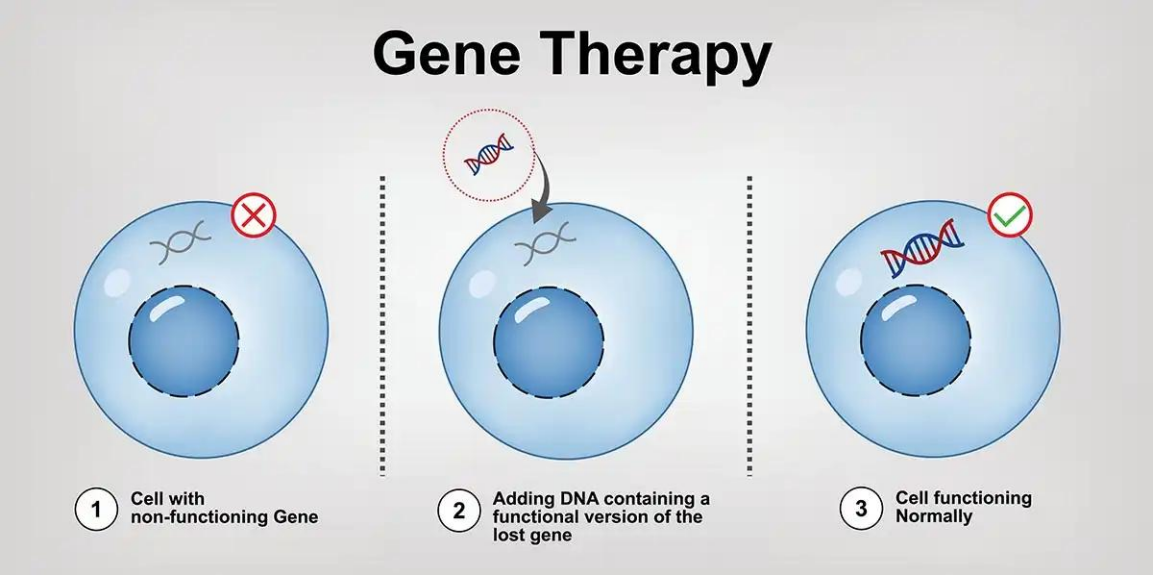

Figure 1. Gene therapy.

Methods of Gene Therapy

Gene therapy employs various methods to alter genes for treating or curing diseases. These include:

1. Activation of genes with related functions.
2. Targeted gene transfer through receptors.
3. Site-directed recombination.
4. Use of artificial chromosomes.
5. Retroviral vectors.
6. Other viral vectors.
7. Electroporation.

CALCIUM PHOSPHATE TRANSFECTION

The most popular technique in gene therapy is substituting a healthy gene with a damaged one. Other methods include swapping an abnormal gene with a normal one and repairing the defective gene.

Molecular testing is the preferred approach for genetic analysis. Direct DNA testing is frequently the most effective for small DNA mutations, particularly when the protein's function is unknown and a biochemical test cannot be developed. This type of testing requires very little material and can be performed on any tissue sample.

Gene therapies typically involve five distinct steps, each with a unique process, but they share some core steps:

- *Step 1:* Consultation (may include multiple visits over time).
- *Step 2:* Preparation.
- *Step 3:* Treatment.

- *Step 4:* Recovery.
- *Step 5:* Follow-up and Monitoring [5].

Gene Therapy Offers Several Advantages

Provides a treatment option where no other medication is effective. Generally, it requires only a single administration. Produces long-lasting effects. The advantages are transferable to subsequent generations [6]. Continually evolving with technological advancements.

Genetic testing is used in three main ways:

1. Before symptoms appear, find out if you have a genetic condition that runs in your family.
2. To determine whether a genetic condition will be present in a current or future pregnancy (Figure 2).
3. To identify a genetic condition when you or your child exhibit symptoms [7].

Figure 2. The diagram of genetic testing.

APPROACHES

Gene therapy can be categorized into two main types: gene addition and gene editing. Gene editing itself includes two approaches:

1. Gene Silencing.
2. Gene Correction.

Although each of these methods results in different gene-based modifications, they follow a similar initial process: stem cells are collected from the patient and sent to a laboratory for modification (Figures 3–4) [8].

Genetic Diseases

Numerous illnesses could be effectively treated with gene therapy, such as:

1. Cancer.
2. Cystic Fibrosis.
3. Heart Disease
4. Diabetes.
5. AIDS.
6. Hemophilia [9].

METHODS FOR GENE THERAPY IN CANCER

1. Viruses.
2. Electroporation.
3. Gene gun.
4. Protein-DNA complexes.
5. Calcium phosphate precipitation.
6. Liposomes [10].

A therapeutic strategy involves a comprehensive plan of targeted measures aimed at addressing a specific pathological condition. A drug forms a part of this strategy, alongside diagnosis, environmental considerations, and other medicinal or non-medicinal interventions (Figure 5) [11].

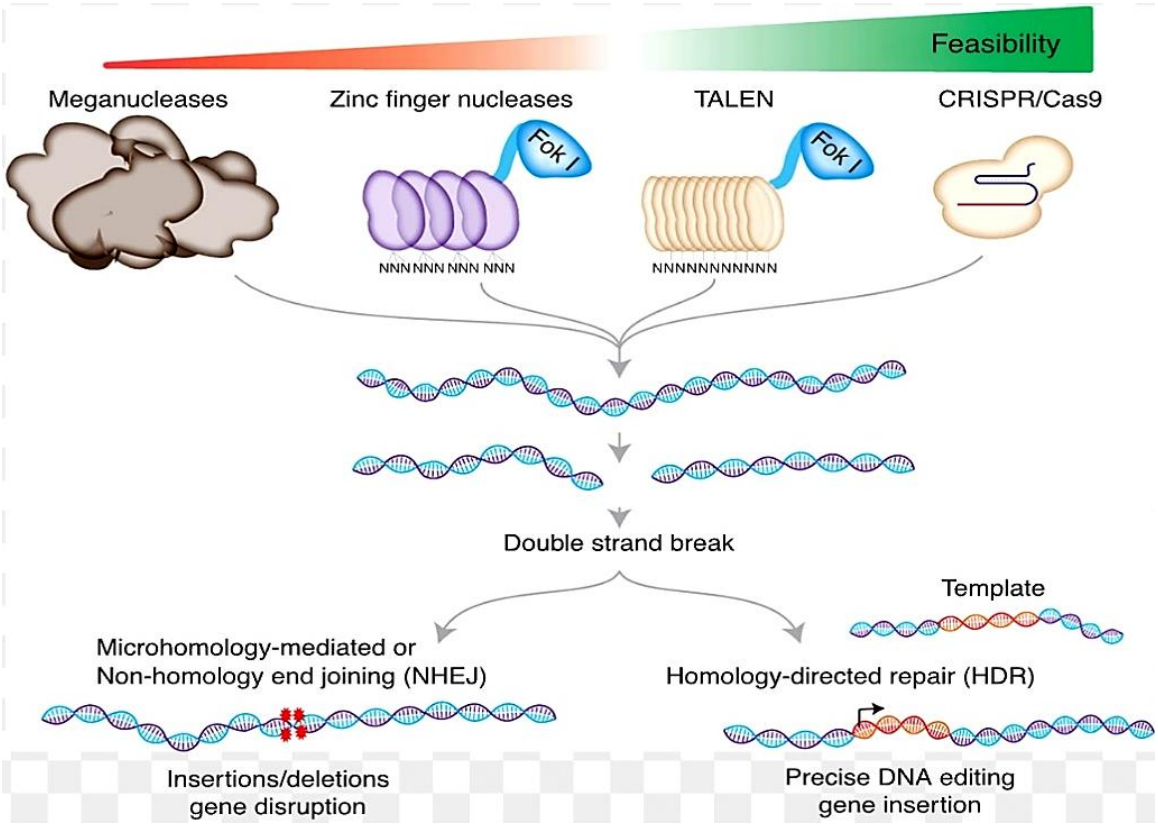

Figure 3. Gene correction.

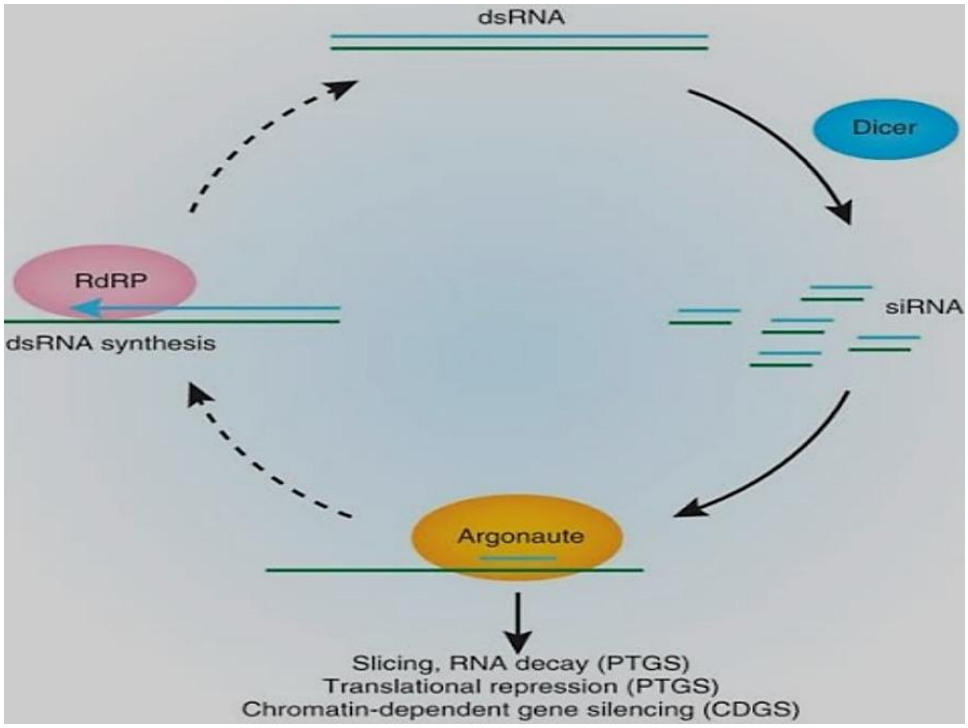

Figure 4. Gene silencing.

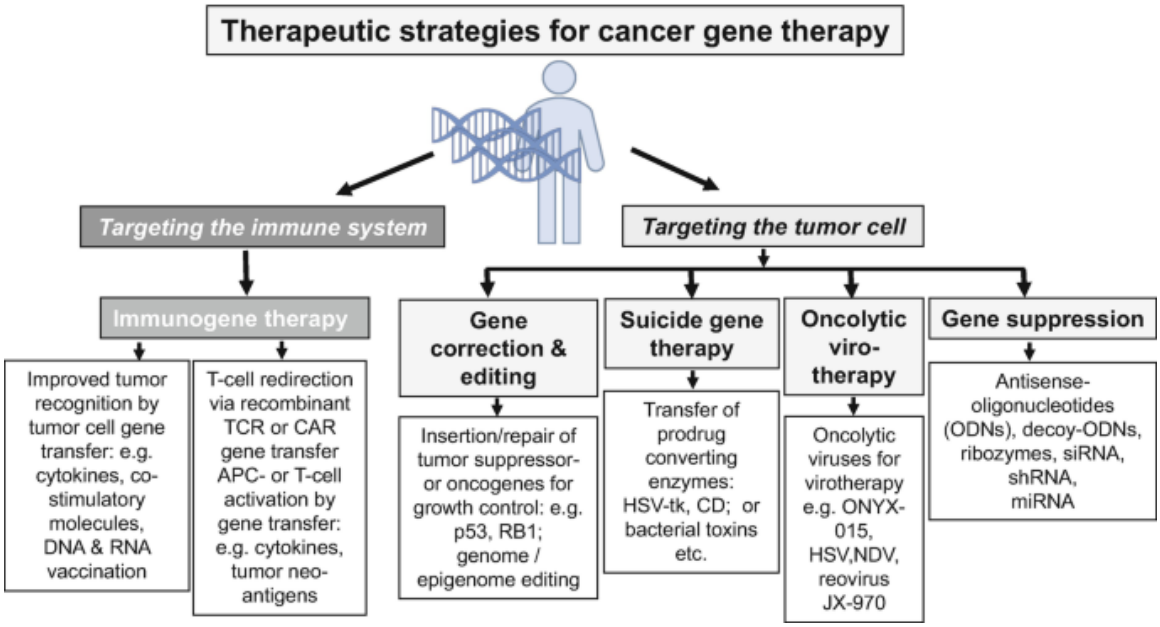

Figure 5. Therapeutic strategies for cancer gene therapy.

CRISPR Cas9 gene editing technology has become a pivotal tool in gene therapy, offering a groundbreaking solution to many previous challenges. It has helped overcome limitations related to disease types (such as recessive or dominant conditions), gene length, and the development of experimental models both ex vivo and in vivo [12].

- *Gene Therapy in the body:* This strategy aims to treat a mutated or missing gene in the body by directly inserting functional copies of a gene into target cells using a vector [13].
- *Ex vivo Gene Therapy:* This method involves genetically modifying a patient's stem cells outside the body, which are then reintroduced to replace target cells with missing or malfunctioning genes (Figure 6) [14].

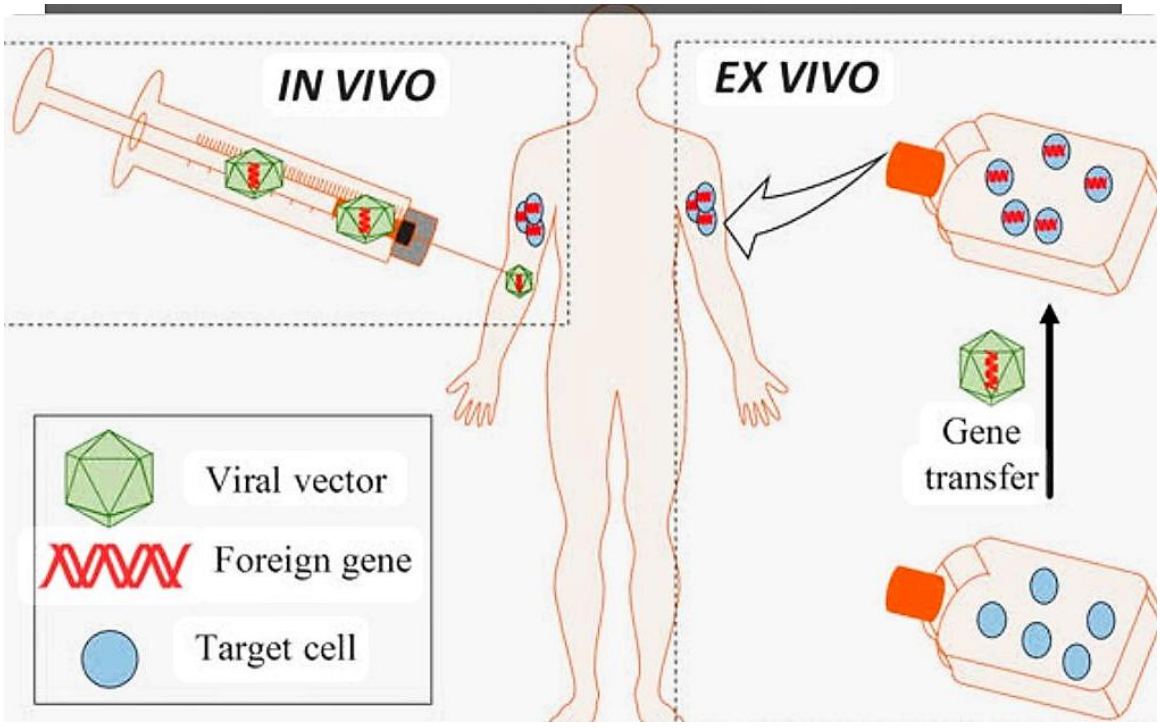

Figure 6. In vivo and ex vivo gene therapy.

Advantages of Gene Therapy

Efficient in focusing on cells that are dividing as well as those that are not. It has a long history of safe use in humans with a large capacity for transgene insertion. Provides stable transgene expression and reduced immunogenicity. Achieves stable transgene expression, although certain methods only target dividing cells [15].

Disadvantages of Gene Therapy

Gene integration poses a risk of insertional mutagenesis. Rapid clearance and low transfection efficiency in vivo. High immunogenicity can be a concern. Cationic liposomes can cause inflammatory toxicity and have low transfection efficiency in vivo [16].

Future of Gene Therapy

In the future, genetic therapies are expected to be developed for both common and rare diseases. Researchers anticipate that, like biologics, gene therapies will experience significant advancements in the coming years [17].

CONCLUSIONS

Gene therapy is transforming the way we think about treating diseases, offering new hope where traditional medicine has fallen short. With advancements in gene-editing tools, like CRISPR-Cas9 and improved delivery methods, scientists are making incredible progress in safely and effectively altering genes to combat a wide range of conditions. When applied to craniofacial regeneration, gene therapy holds the potential to repair damaged tissues in ways we never thought possible, combining genetics with tissue engineering to promote natural healing.

However, challenges remain. Issues, like immune responses, ethical concerns, and ensuring long-term safety, need to be addressed before gene therapy becomes a mainstream treatment. But with ongoing research and clinical trials, the future looks promising. As technology continues to evolve, gene therapy is set to play a key role in personalized medicine, offering tailored treatments that could change lives for generations to come.

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