

Perspective A SCITECHNOL JOURNAL

## Characterization and Symptoms of Gene Therapy

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Received: 03 March, 2023, Manuscript No. JGGT-23-93335;

Editor assigned: 06 March, 2023, PreQC No. JGGT-23-93335 (PQ);

Reviewed: 20 March, 2023, QC No. JGGT-23-93335;

Revised: 27 March, 2023, Manuscript No. JGGT-23-93335 (R);

Published: 06 April, 2023, DOI: 10.4172/Jggt.1000136.

## **Description**

Gene therapy is a medical technique that involves manipulating genes to treat or prevent disease. The technique involves inserting, deleting, or replacing genes in a patient's cells to correct or modify genetic disorders. Gene therapy holds effective potential for treating inherited diseases and genetic disorders that are currently untreatable by using conventional methods. The human body is made up of trillions of cells, each with its own unique set of genes. These genes control the way the body functions and determine the physical traits, such as eye colour and height. When a gene is mutated or missing, it can lead to genetic disorders, such as cystic fibrosis, sickle cell anemia, and Huntington's disease. Gene therapy aims to replace the faulty genes responsible for these disorders.

There are two main types of gene therapy: Somatic and germline. Somatic gene therapy targets the cells of an individual's body, such as skin cells or muscle cells. The goal of somatic gene therapy is to cure or alleviate the symptoms of a particular disease in the individual, without affecting future generations. Germline gene therapy targets the reproductive cells, such as eggs and sperm, and aims to modify the genetic structure of an individual's organism. The Utilization of viral vectors is the most common method of gene therapy. Viral vectors are modified viruses that are used to deliver therapeutic genes to target

cells in the body. The viral vector is designed to enter the target cells and release the therapeutic genes, which either become transformed into the patient's DNA. The modified genes then produce the necessary proteins or correct the defective proteins, providing to the alleviation of the clinical disease.

In the treatment of various genetic disorders, gene therapy has shown significant improvement. gene therapy has been used to treat other disorders, such as hemophilia, Parkinson's disease, and some types of cancer. Despite its potential, gene therapy faces some challenges. One of the major challenges is the risk of unintended effects, such as the activation of oncogenes or the disruption of essential genes. Another challenge is the immune response of the patient's body to the viral vectors used in gene therapy. Additionally, the cost of gene therapy is currently high, making it inaccessible to many patients.

There are different types of gene therapy that are currently being developed and tested for the treatment of various genetic disorders. One approach is called somatic gene therapy, which involves targeting cells in the body that are affected by the genetic disorder. This type of gene therapy is currently being tested for a range of disorders, including cystic fibrosis, sickle cell anemia, and hemophilia. Another type of gene therapy is called germ line gene therapy, which involves modifying the genes that are passed on from one generation to the next generation. This type of gene therapy is more controversial, as it raises ethical concerns about the long-term consequences of modifying the human genome.

In addition to its potential use in the treatment of genetic disorders, gene therapy also has a wide range of other applications. For example, it could be used to enhance athletic performance by introducing genes that enhance muscle growth or endurance. It could also be used to treat non-genetic disorders, such as cancer, by introducing genes that enhance the body's immune response to tumors. Overall, gene therapy is a rapidly developing field with the potentially to transform the treatment of genetic disorders and other diseases. While there are many challenges to overcome, the potential benefits of gene therapy make it a reliable area of research and development for the future.

Citation: Shin M (2023) Characterization and Symptoms of Gene Therapy. J Genetic Gene Ther 7:1.

