Joual of Molecular Pathophysiology, 2022
VOL 11, NO. 5, PAGE 01

COMMENTARY

Open Access

A General Concept of Gene Therapy and Its Cell Types

James Lee*
Department of Neurosurgery, Morehouse School of Medicine, Atlanta, USA

ARTICLE HISTORY
Received: 15-Apr-2022, Manuscript No. JMOLPAT-22-64137;
Editor assigned: 18-Apr-2022, PreQC No: JMOLPAT-22-64137 (PQ);
Reviewed: 03-May-2022, QC No: JMOLPAT-22-64137;
Revised: 09-May-2022, Manuscript No: JMOLPAT-22-64137 (R).
Published: 16-May-2022

Description

Gene therapy is a discipline of medicine that focuses on genetically changing cells for therapeutic reasons or to treat disease by repairing or regenerating damaged genetic material. Gene therapy aims to address a genetic disease at its root. If a mutation in a gene causes the creation of a malfunctioning protein that causes an inherited disease (typically recessively), gene therapy could be used to deliver a copy of the gene that does not include the harmful mutation and thus creates a functional protein. Gene replacement therapy is a treatment approach for inherited retinal disorders. While gene replacement therapy is most typically utilized to treat recessive disorders, novel treatments for diseases with dominant inheritance patterns have been presented.

• CRISPR gene editing has opened new avenues for its application and use in gene therapy, as it allows for the correction of a specific genetic problem rather than just replacing a gene. In the future, therapeutic options for medical challenges such as eradicating latent Human Immunodeficiency Virus (HIV) reservoirs and correcting the sickle cell disease mutation may be available.

• Prosthetic gene therapy tries to allow bodily cells to do functions that they wouldn't normally do. One such example is vision restoration gene therapy, which tries to restore eyesight in people with advanced retinal disorders. Photoreceptors, the retina's basic light-sensitive cells, are irrevocably destroyed in end-stage retinal disorders. Light-sensitive proteins are supplied into the remaining cells of the retina by prosthetic gene therapy, making them light-sensitive and allowing them to send visual information to the brain. Clinical trials are under underway. Not all medical procedures that modify a patient's genetic composition can be classified as gene therapy. Foreign DNA has been identified in individuals after bone marrow transplantation and organ transplantation in general.

Cell types

Somatic: The therapeutic genes are introduced into any cell other than a gamete, germ cell, gametocyte, or undifferentiated stem cell in Somatic Cell Gene Therapy (SCGT). Such changes solely impact the individual sick and are not passed down to descendants. Therapeutic DNA (either integrated in the genome or as a foreign episome or plasmid) is used to cure disease in somatic gene therapy, which is a form of mainstream fundamental and clinical research.

In the United States, there are over 600 clinical trials using SCGT. Immunodeficiencies, haemophilia, thalassemia, and cystic fibrosis are among the most common genetic illnesses studied. Somatic cell therapy is a good option for single gene diseases. It is now impossible to completely repair a genetic condition or replace numerous genes. Only a couple of the trials are nearing completion.

Germline: In Germline Gene Therapy (GGT), functional genes are introduced into the genomes of germ cells (sperm or egg cells). When a germ cell is transformed, the mutated gene is spread throughout the organism's cells. As a result, the alteration is heritable and can be handed down to future generations. GGT is prohibited for use in humans in Australia, Canada, Germany, Israel, Switzerland, and the Netherlands for technical and ethical concerns, including a lack of information regarding potential hazards to future generations and higher risks than SCGT. There are no governmental controls in the United States that expressly address human genetic alteration (beyond FDA regulations for therapies in general).

Contact: Lee J, E-mail: leejames123@gmail.com