Editorial

# Gene Therapy: Introduction

**Timothy Davis**†

## Editorial

DNA makes up genes, which are blueprints for building proteins that keep the body running. In most cases, each individual inherits two copies of each gene from their parents. Hair colour, height, and unique features are all controlled by these genes. However, genes are not always constructed appropriately. A mutation, or tiny change in them, might modify how proteins are expressed (made), affecting the body's capacity to operate effectively. This has the potential to change how a person breathes, walks, and even digests food. Genes can change as a result of hereditary mutations (changes passed down from one generation to the next), as they age, or as a result of chemical or radiation damage.

Gene therapy is intended to be used only once to treat an illness caused by a defective gene. The introduction, removal, or modification of genetic material DNA or RNA into a patient's cells to cure a specific ailment is known as gene therapy. The genetic material given contains instructions for changing the way a protein or a group of proteins is generated by the cell. For some disorders, this entails making adjustments to accommodate for excess, insufficient, or erroneous necessary protein production within cells. A vector is used to introduce new genetic material into the cell, such as a functional gene. Because viruses have evolved to be highly good at getting into cells, they can be utilized as vectors. However, scientists have discovered how to eliminate viral genes and can now use this knowledge to treat or prevent disease. Their goal is to implant the new therapeutic genes into the cell in this scenario. Before being utilized, all viral vectors are thoroughly examined for safety. The vectors can be administered either outside the body (ex-vivo treatment) or injected into the body (in-vivo treatment) (in-vivo treatment).

### Types

Patients with disorders including SMA, some blood malignancies, and an inherited eye disease can presently access FDA-approved gene treatments. The majority of gene therapies, on the other hand, are still in clinical testing. Before a medicine can be made available to the general public, clinical trials are essential to understand how it interacts with the body and if it is safe and effective.

Gene therapy is used to treat rare disorders that are typically fatal or debilitating. The National Institutes of Health defines rare as "any sickness or ailment affecting fewer than 200,000 people in the United States." Currently, there are around 7,000 uncommon diseases that afflict one out of every ten persons. A simple genetic mutation inherited from one or both parents causes many of these rare disorders. Nearly half (45%) of gene therapies up for FDA clearance in the next five years are projected to treat cancer, and nearly four out of ten (38%) are likely to cure rare inherited genetic illnesses. Among the inherited disorders are the following:

- Hematology (blood) disorders like sickle cell disease
- Neurological disorders that affect the brain and spinal cord
- Musculoskeletal (muscle) diseases
- Retinal (eye) disorders
- Oncology (cancers) such as blood cancers

Editorial Office, International Journal of Clinical Skills, London, United Kingdom

†Author for correspondence: Timothy Davis, Editorial Office, International Journal of Clinical Skills, London, United Kingdom, Email: ijclinicalskill@journalres.com