How Gene Therapy Can Cure or Treat Diseases?

- Our health is greatly influenced by the genes in the cells of our body. Indeed, having one or more faulty genes can cause disease.
- Recognizing this, researchers have spent decades figuring out how to alter genes or swap out unhealthy genes with healthy ones in order to treat, eradicate, or prevent illness.
- This study is paying off since modern advances in science and technology are altering how we categories' diseases, create medications, and recommend therapies.
- Numerous gene therapy products have received FDA approval for use in treating cancer and uncommon diseases.

GENES, CELLS, AND THE INTERACTIONS BETWEEN THEM

- Cells and genes have a close relationship.
- Thousands of genes offer the instructions for producing particular proteins that contribute to the structure of the cells in our body.
- The human body is made up of trillions of cells, which are the fundamental building blocks of all living organisms.
- The information that enables different cells to behave differently is provided by the genes.
- The body's tissues and organs, including muscles, bones, and blood, are made up of large cell clusters. All of our body's functions are in turn supported by the tissues and organs.

How Gene Therapy Works?

- Sometimes all or part of the gene is defective or missing from birth. This is often called a genetically inherited mutation.
- In addition, entire genes can change (mutate) during our lifetime. These acquired mutations can result from exposure to the environment. The good news is that most of these genetic changes (mutations) do not cause disease. However, some inherited and acquired mutations can cause developmental disorders, neurological diseases and cancer.
- Depending on what's wrong, gene therapy scientists can do one of the following:
    - They can replace a missing or problem-causing gene.

        - They can add genes to the body to treat diseases.
        - Or they can turn off genes that cause problems.
        - To insert new genes directly into cells, scientists use a vehicle called a "vector".
        - The vectors are genetically modified to deliver the genes needed to treat the disease.
        - Vectors must be able to efficiently deliver genetic material into cells, and there are different types of vectors. Viruses are currently the most widely used vectors in gene therapy because they have a natural ability to deliver genetic material into cells. Before a virus can be used to deliver therapeutic genes into human cells, it is modified so that it cannot cause infectious diseases.
- Gene therapy can be used to modify cells inside or outside the body. When gene therapy is used to modify cells in the body, a doctor will inject the vector that carries the gene directly into the patient.
- When gene therapy is used to modify cells outside the body, doctors take blood, bone marrow, or other tissue and secrete specific cell types in the laboratory. The vector containing the desired gene is introduced into these cells. The cells are subsequently injected into the patient, where the new gene is used to produce the desired effect.

Safety and Efficacy of gene therapy

- Before gene therapy can be marketed for use in humans, the product must undergo clinical studies for safety and efficacy so that FDA scientists can assess whether the risks of the therapy are acceptable given the potential benefits.
- The scientific field of gene therapy products is rapidly changing and heralds a new approach to the treatment of vision loss, cancer and other serious and rare diseases. As scientists continue to make great strides in this therapy, the FDA is committed to accelerating development by engaging with product developers and quickly reviewing breakthrough treatments that have the potential to save lives