EXPLORING GENE THERAPY

WHAT IS GENE THERAPY?

Each person inherits features from his or her parents in the form of genes.

Genes are made up of a molecule called DNA (deoxyribonucleic acid).

When a gene does not work properly, the gene is said to be malfunctioning and may cause a genetic disease.

GENE THERAPY USES FUNCTIONING GENES AS MEDICINE TO HELP CORRECT A GENETIC DEFECT.

HOW DOES GENE THERAPY WORK?

1. Stem cells are collected from the blood.

2. In a lab, a functioning copy of the gene is inserted into the DNA of the stem cells, referred to as transduction, using a modified virus.

3. Chemotherapy is given to make room for the modified stem cells. The modified stem cells are then transplanted back into the body.

4. The goal is for modified stem cells to become a permanent source of blood cells that grow and produce new cells with the functioning copy of the gene.

WHY GENE THERAPY NOW?

In 2012, the first gene therapy was approved in Europe.

Significant advances have been made in gene transfer technology, safety and how the transfer vehicles are manufactured.

Long term safety and efficacy has been observed for more than 5 years and positive data is growing.

For 30 YEARS,

Gene therapy has been studied as a potential treatment for genetic disease.

~2,000

clinical trials have been conducted using different types of gene therapy in cancers, blood diseases, central nervous system disorders and immune system diseases.

THE GOAL

TRANSFORM THE TREATMENT OF GENETIC DISEASES BY PROVIDING FUNCTIONING COPIES OF THE MALFUNCTIONING GENES.

Sources: