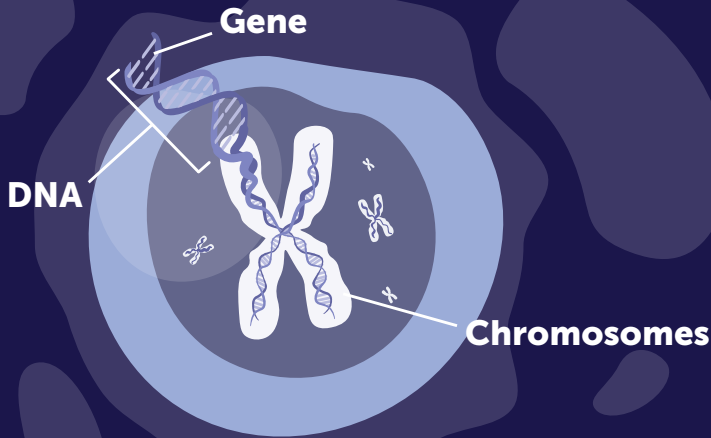


What is Gene Therapy?

Gene therapy is the use of genetic material to treat or prevent diseases.

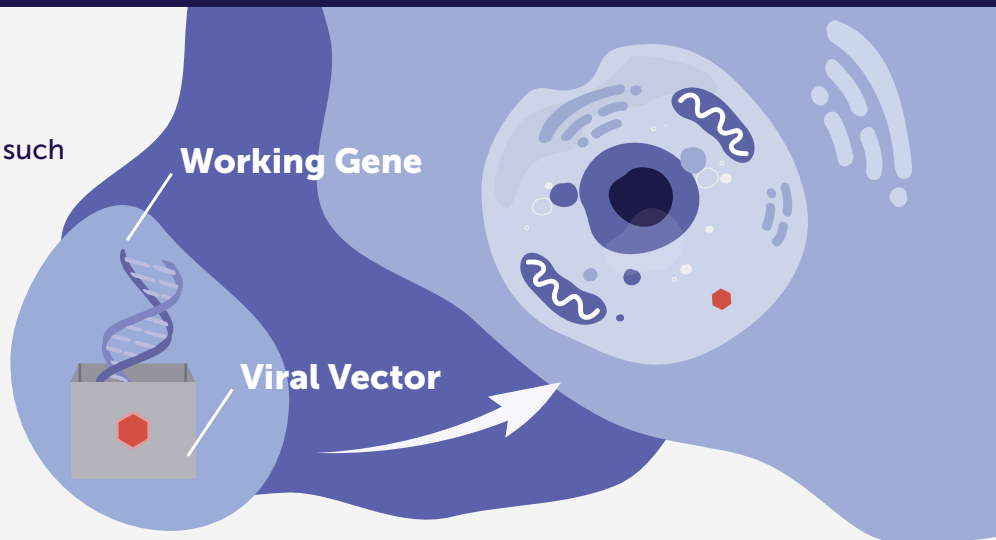


The Importance of Genes

Most of our body's genetic information is stored in 23 paired chromosomes inside the nucleus of our cells. Each chromosome is made up of DNA, and specific sections of DNA are called genes. Genes provide the instructions on how to make proteins for the body. Any change to the DNA within our genes can alter how proteins are built and work. These small changes are referred to as a gene variant or mutation and can have a big impact on the body.

How It Works

Gene therapy delivers genetic material, such as a working gene, to change how a protein is produced by the cell. The genetic material is delivered to the cell using a vector. A vector is like a package with an address label and inside is a specific message for the cells. Typically, viruses are used as vectors because they've evolved to be very good at getting into cells.



How it's Given

Gene therapy can be given two ways. It can be delivered directly into the person (*in vivo*), such as through an injection. Or a person's own cells can be removed, the genetic material is delivered to the cells outside the body (*ex vivo*) and these modified cells are then returned to the body. The right approach depends on the best way to target the disease.

Gene therapy comes with many unique risks and challenges that should be carefully considered, along with great potential benefits. Most gene and cell therapies are being studied in clinical trials. Clinical trials play an important role in finding treatments that are safe and effective.

