GENE THERAPY APPROACHES	This resource offers a summary of gene therapy approaches for your learning journey. **Download to your device or print** to share this information and guide potential conversations with your support system, doctor, and community.

Common types of gene therapy:

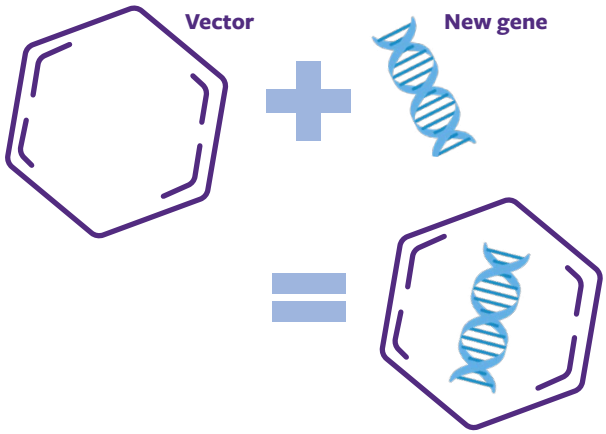
Gene therapy possibilities are changing in exciting ways. There are 2 main types of gene therapy: gene addition and gene editing. You might be wondering how these 2 therapy types differ. The goal is the same: to correct a gene that is not working properly. The main difference is how these 2 therapies accomplish that goal.

Gene addition is a type of gene therapy that targets a specific gene in the body's cells.

This approach adds a working copy of a gene into the cell or adds another gene to bypass the problem. The added gene allows the body to make proteins to potentially manage or treat a genetic disease.

To introduce a new gene through gene addition, a vehicle is needed to deliver the material to the intended target. You can think of this vehicle as an envelope carrying the new gene to its destination. The vehicle is called a vector.

A viral vector is 1 type of vector. They are sometimes used because viruses are naturally very good at entering cells.



Gene addition is thought to work like this:

Scientists create a working copy of the gene in a lab and place it inside the vector.

The vector carries the new gene to the targeted location inside the cells.

The vector unpacks the new gene, like taking a letter out of an envelope.

The new gene tells the body how to build proteins to potentially change the way the body functions.

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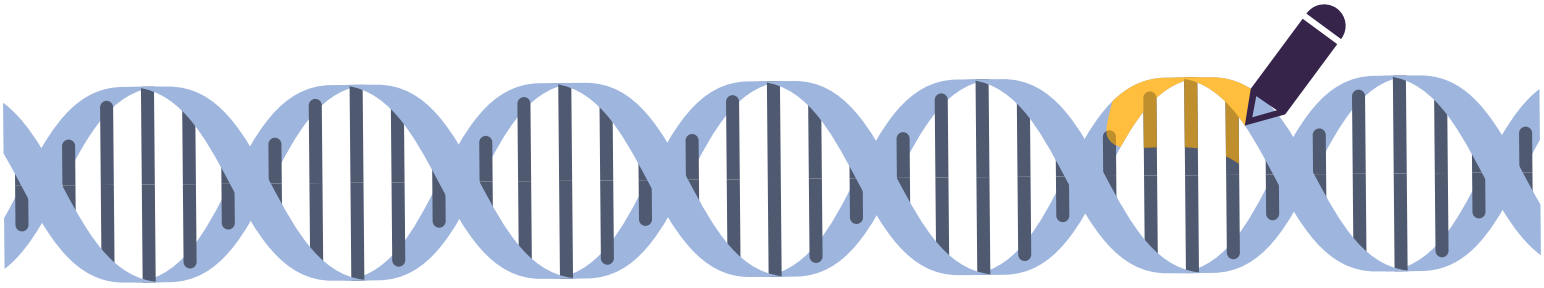
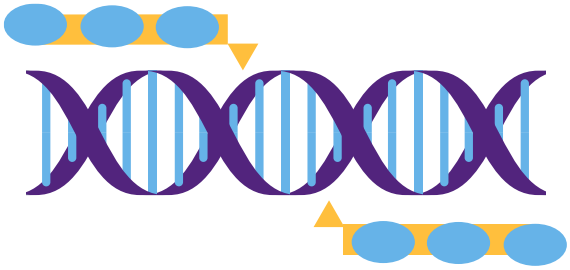
Gene editing is a type of gene therapy.

It is a technique that aims to change the body's genetic material at specific locations in the DNA to manage or treat a disease.



Some of the different types of gene editing are:

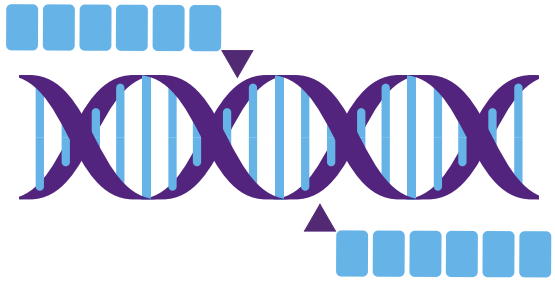


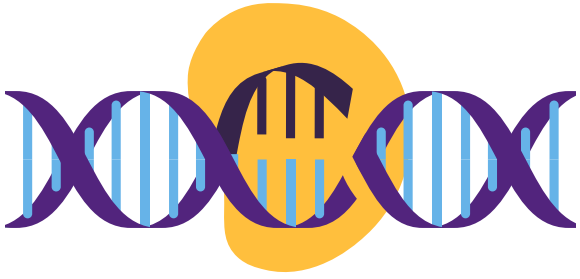
ZFNs: Zinc-finger nucleases

ZFNs are made up of chains of proteins, called "zinc fingers," and tiny DNA repair tools found in bacteria, known as "bacterial nucleases." The zinc fingers and nucleases link up, and together they create a system that targets and edits specific sites in your DNA.

TALENs: Transcription activator-like effector nucleases

TALENs work by combining 2 different types of proteins: enhanced proteins that speed up reactions in the body, and other proteins that bind to DNA to target and edit genes.





CRISPR: Clustered regularly interspaced short palindromic repeats

CRISPR-based gene tools are made up of 2 separate parts: a Cas enzyme and a guide RNA. Together, the CRISPR/Cas system can find specific mutated genes and make the necessary changes to repair them.

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