GENE THERAPY OVERVIEW

This resource offers a summary of gene therapy 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.

Gene therapy is a technique that changes genes. It may help manage or treat a genetic disease.

TARGET

Gene therapy alters a gene at the location of the problem.

CHANGE

The process adds, removes, or edits the gene inside the cells.

CREATE

The newly added or altered gene allows the creation of proteins.

STOP

The proper proteins may change or stop disease in the body.

The goal of gene therapy is to target genetic diseases at the root. It permanently changes a specific gene by adding a new gene or removing or editing the original gene. The body is then able to build proteins to change or stop the disease.

Gene therapy has 2 main approaches:

Gene addition aims to treat a disease by adding a gene where one is not working properly.

This approach could affect each person differently based on their response to gene addition.

However, the goal is to help the cells to create new proteins.

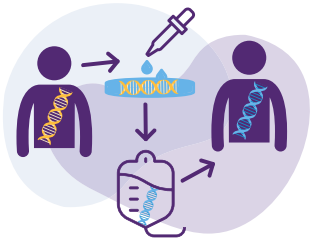
Gene editing aims to change your DNA at the location of the mutation.

Individual results may vary because each person's body is unique and may respond differently to gene editing.

However, the goal is that it potentially creates proteins to treat or manage a genetic disease.

There are 2 ways to deliver gene therapy:

In vivo means that gene therapy is given straight to your cells INSIDE your body, typically through an IV.



Ex vivo means gene therapy is added to your cells OUTSIDE of your body. In this process, certain cells are removed from your body and taken to a lab. Inside the lab, scientists make changes to the genes inside of the cells to treat or correct the mutation. The cells are then returned to your body.

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