

TO THE POINT

GENE THERAPY

National Cooperative 



Did you know the first gene therapy wasn't approved in the US until 2017?

Read on to discover the advancements currently being made and how gene therapy is poised to revolutionize modern medicine.

Gene therapy is a fairly new "experimental technique that uses genes to treat or prevent disease."¹

It works by adding new genes into existing cells to create a protein or make up for a missing or abnormal gene.

Gene Therapy Milestones

- **1990:** A four-year-old patient with severe combined immunodeficiency (SCID) undergoes the first gene therapy procedure.
- **1999:** Jesse Helsingier dies from organ failure in a clinical trial for gene therapy, severely setting back progress in the field.
- **2003:** The world's first gene therapy was approved in China to treat head and neck cancer.
- **2017:** The US Food and Drug Administration approves gene therapies to treat inherited blindness and a rare type of blood cancer.

Methods of Gene Therapy

- Replacing a disease-causing gene with a healthy copy of the gene.
- Inactivating a disease-causing gene that isn't functioning properly.
- Introducing a new or modified gene into the body to help treat a disease.²

Gene Therapy Products

There are various gene therapy products currently being researched which include:

- **Plasmid DNA:** Circular DNA molecules can be genetically engineered to carry therapeutic genes into human cells.
- **Human gene editing technology:** This aims to disrupt harmful genes or repair mutated genes.²

Who Can Receive Gene Therapy?

If you have a medical condition you believe can be treated with gene therapy, talk to your doctor about participating in a clinical trial, or visit clinicaltrials.gov.

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1. "What Is Gene Therapy? - Genetics Home Reference - NIH." U.S. National Library of Medicine, National Institutes of Health, 2018

2. Center for Biologics Evaluation and Research. "Cellular & Gene Therapy Products - What Is Gene Therapy?" U S Food and Drug Administration Home Page, Center for Biologics Evaluation and Research, 2018