

Gene therapy of cancer

Gene therapy is a medical approach that treats or prevents disease by correcting the underlying genetic problem. Gene therapy techniques allow doctors to treat a disorder by altering a person's genetic makeup instead of using drugs or surgery.

The earliest method of gene therapy, often called gene transfer or gene addition, was developed to:

- Introduce a new gene into cells to help fight a disease.
- Introduce a non-faulty copy of a gene to stand in for the altered copy causing disease.

Later studies led to advances in gene therapy techniques. A newer technique, called genome editing (an example of which is CRISPR-Cas9), uses a different approach to correct genetic differences. Instead of introducing new genetic material into cells, genome editing introduces molecular tools to change the existing DNA in the cell. Genome editing is being studied to:

- Fix a genetic alteration underlying a disorder, so the gene can function properly.
- Turn on a gene to help fight a disease.
- Turn off a gene that is functioning improperly.
- Remove a piece of DNA that is impairing gene function and causing disease.

Gene therapies are being used to treat a small number of diseases, including an eye disorder called Leber congenital amaurosis and a muscle disorder called spinal muscular atrophy. Many more gene therapies are undergoing research to make sure that they will be safe and effective. Genome editing is a promising technique also under study that doctors hope to use soon to treat disorders in people.

source:

<https://medlineplus.gov/genetics/understanding/therapy/genetherapy/>