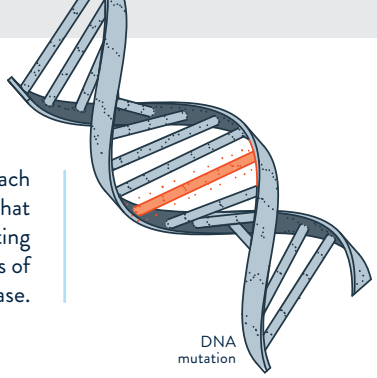
HOW DOES GENE THERAPY WORK?

Gene therapy is a therapeutic approach for a number of genetic diseases that works by adding, deleting, or correcting genetic material in the cells of a person with a disease.



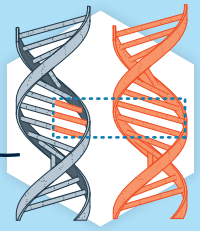
THERE ARE TWO TYPES OF GENE THERAPY BEING STUDIED

GENE ADDITION

GENE ADDITION INVOLVES ADDING FUNCTIONAL COPIES of a gene into a person's cells to do the work of a faulty gene.[1]

GENE EDITING

GENE EDITING INVOLVES THE CREATION OF TARGETED DOUBLE-STRANDED BREAKS IN DNA, with or without repair instructions, to disrupt or correct the function of a gene. It includes different techniques of gene correction or repair and disruption or inactivation.[5,6]

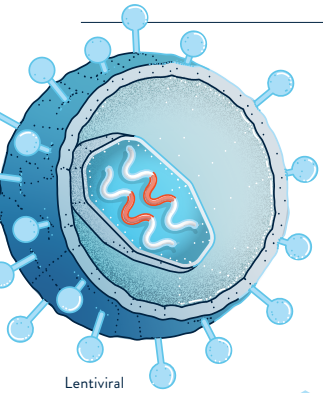


Gene correction is a technique currently in preclinical studies. It is used to recognize and form a break in the DNA, insert new genetic material, and override the faulty gene.[7]

Gene disruption or **inactivation** is when scientists turn off an existing gene to help treat a genetically related health issue. This technique is also known as gene *silencing*, *knockdown*, or *knockout*.[8]

HOW ARE GENES DELIVERED INTO CELLS?

Gene therapy products work by introducing genetic material into the nucleus of a cell with the goal of changing the course of disease. The vehicle that carries this genetic material is known as a **vector**, which is either viral or nonviral[2,3]:

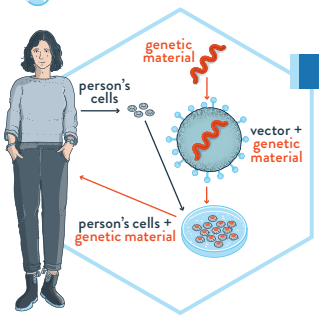


Lentiviral vector

Viral vectors use the blueprint of a virus as a guide to deliver genetic material to the cell. Viral vectors are used in gene therapies due to the virus' natural ability to access cells of the body.[2]

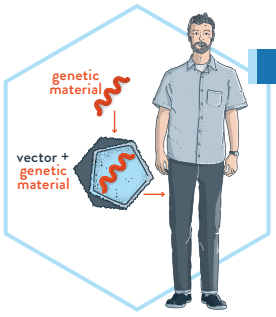
Nonviral vectors introduce genetic material to tissues and cells, either physically or chemically.[3]

Genes can be delivered to cells either inside or outside of the body[4]:



EX VIVO

Ex vivo gene therapy refers to the process of genetically altering a person's cells outside of the body and then transplanting them back in.



IN VIVO

In vivo gene therapy refers to direct administration either intravenously, known as systemic administration, or locally to a specific organ of interest (eg, eye, muscle).

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Learn more about gene therapy techniques at www.thegenehome.com

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References: 1. FDA Commissioner. What is gene therapy? How does it work? US Food and Drug Administration. Accessed May 12, 2021. https://www.fda.gov/consumers/consumer-updates/what-gene-therapy-how-does-it-work. **2.** STAT Reports. The STAT guide to viral vectors, the linchpin of gene therapy. STAT News; 2019. **3.** Al-Dosari MS, Goa X. *AAPS J.* 2009;11(4):671-681. **4.** American Society of Gene & Cell Therapy (ASGCT). Learn about blood cancers and CAR T-cell therapy. Accessed May 12, 2021. https://www.asgct.org/education/more-resources/gene-and-cell-therapy-faqs. **5.** Guha TK et al. *Comput Struct Biotechnol J.* 2017;15:146-160. **6.** Yanik M et al. *Prog Retin Eye Res.* 2017;56:1-18. **7.** Pandey P et al. Chapter 4.5.1: Replacement Therapy. In: Grumezescu A, ed. Drug Targeting and Stimuli Sensitive Drug Delivery Systems. 2018. Accessed May 12, 2021. https://www.sciencedirect.com/science/article/pii/B9780128136898000045. **8.** Khosravi MA et al. *Eur J Pharmacol.* 2019;854:398-405.

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