URL of this page: https://medlineplus.gov/genesandgenetherapy.html

Genes and Gene Therapy

What are genes?

Genes [https://medlineplus.gov/genetics/understanding/basics/gene/] are sections of DNA [https://medlineplus.gov/genetics/understanding/basics/dna/] in your cells that are passed down (inherited) from your parents. You inherit two copies of each gene, one from each parent. Genes carry information that controls what you look like and how your body works.

There are about 20,000 genes that provide instructions for making proteins, which your body needs to work correctly.

Some genes don't make proteins themselves but instead help control how other genes work. They act like switches, turning other genes on or off. This means they help decide when, where, and how much of a protein is made in your body.

What happens when genes change?

Changes in your genes are also called gene variants or mutations. These changes can be inherited, occur as you age, or result from environmental factors such as chemicals or radiation. Often, these changes have no effect, but sometimes, even a small change in the DNA can alter the instructions for making proteins. If genes don't make the right proteins, or don't make them correctly, this can cause a genetic disorder [https://medlineplus.gov/geneticdisorders.html] .

What is gene therapy?

Gene therapy uses genes to treat or prevent disease by correcting genetic problems. It works by changing your genetic material, rather than relying on traditional treatments like medicine or surgery.

New genetic material can be delivered into cells in two main ways:

- ***ex-vivo* treatment**. Cells are removed from your body, modified by adding genetic material, and then placed back into your body.
- ***in-vivo* treatment**. Genetic material is delivered directly into your body, often through an injection.

A common form of gene therapy involves inserting a normal gene to replace an abnormal gene. Other approaches include:

- Repairing an abnormal gene
- Altering the degree to which a gene is turned on or off

Newer techniques offer different ways to correct genetic problems:

- Genome editing [https://medlineplus.gov/genetics/understanding/genomicresearch/genomeediting/] (also called gene editing). Instead of adding new genetic material into cells, this approach changes the DNA that's already in your cells. A well-known example is CRISPR-Cas9. This promising technique is still being studied and may soon be used to treat genetic disorders.
- Cell-based gene therapy [https://medlineplus.gov/genetics/understanding/therapy/othergenetictherapy/] combines gene therapy and cell therapy. An example of this would be CAR T cell therapy. Cells, which are often immune system cells, are genetically altered to help treat a disease and then introduced into the body.

Gene therapies are currently only approved to treat a small number of diseases, such as:

- Leber congenital amaurosis [https://medlineplus.gov/genetics/condition/leber-congenital-amaurosis/] , an inherited eye disorder.
- Spinal muscular atrophy [https://medlineplus.gov/genetics/condition/spinal-muscular-atrophy/] , a genetic muscle disorder.

What are the risks and challenges of gene therapy?

While gene therapy holds great promise, it comes with risks and challenges. Studies are still ongoing to ensure these treatments are safe and effective. Some challenges include:

- **Immune system reactions.** Your body may see the new material as a threat and react to it.
- **Unintended effects.** Changes to DNA could affect other genes in harmful ways.
- **High costs.** Gene therapies are often expensive and are not widely accessible.

Start Here

- Gene Therapy [https://www.mayoclinic.org/tests-procedures/gene-therapy/about/pac-20384619?p=1]
  (Mayo Foundation for Medical Education and Research)
  Also in Spanish [https://www.mayoclinic.org/es/tests-procedures/gene-therapy/about/pac-20384619?p=1]
- Gene Therapy Approaches [https://patienteducation.asgct.org/gene-therapy-101/gene-therapy-approaches]
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- How does gene therapy work?: MedlinePlus Genetics [https://medlineplus.gov/genetics/understanding/therapy/procedures/]
  NIH (National Library of Medicine)
- How Gene Therapy Can Cure or Treat Diseases [https://www.fda.gov/consumers/consumer-updates/how-gene-therapy-can-cure-or-treat-diseases] (Food and Drug Administration)
  Also in Spanish [https://www.fda.gov/consumers/articulos-para-el-consumidor-en-espanol/como-la-terapia-genica-puede-curar-o-tratar-enfermedades]
- What Are Genetic Therapies? [https://www.nhlbi.nih.gov/health/genetic-therapies]
  NIH (National Heart, Lung, and Blood Institute)
  Also in Spanish [https://www.nhlbi.nih.gov/es/salud/terapias-geneticas]
- What is a gene?: MedlinePlus Genetics [https://medlineplus.gov/genetics/understanding/basics/gene/]
  NIH (National Library of Medicine)
- What is gene therapy?: MedlinePlus Genetics [https://medlineplus.gov/genetics/understanding/therapy/genetherapy/]
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Related Issues

- Brief Guide to Genomics [https://www.genome.gov/about-genomics/fact-sheets/A-Brief-Guide-to-Genomics]
  NIH (National Human Genome Research Institute)
  Also in Spanish [https://www.genome.gov/es/about-genomics/fact-sheets/Breve-guia-de-genomica]
- Epigenomics [https://www.genome.gov/about-genomics/fact-sheets/Epigenomics-Fact-Sheet]
  NIH (National Human Genome Research Institute)
  Also in Spanish [https://www.genome.gov/es/about-genomics/fact-sheets/Epigenomica]
- Genetics, Disease Prevention and Treatment FAQ [https://www.genome.gov/FAQ/Genetics-Disease-Prevention-and-Treatment] NIH (National Human Genome Research Institute)
- Genetics: MedlinePlus Genetics [https://medlineplus.gov/genetics/] NIH (National Library of Medicine)
  Also in Spanish [https://medlineplus.gov/spanish/genetica/]
- Pharmacogenomics [https://www.genome.gov/about-genomics/educational-resources/fact-sheets/pharmacogenomics]
  NIH (National Human Genome Research Institute)
- What are whole exome sequencing and whole genome sequencing?: MedlinePlus Genetics [https://medlineplus.gov/genetics/understanding/testing/sequencing] NIH (National Library of Medicine)
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- What is epigenetics?: MedlinePlus Genetics [https://medlineplus.gov/genetics/understanding/howgeneswork/epigenome] NIH (National Library of Medicine)
  Also in Spanish [https://medlineplus.gov/spanish/genetica/entender/comofuncionangenes/epigenetica/]
- What is genetic ancestry testing?: MedlinePlus Genetics [https://medlineplus.gov/genetics/understanding/dtcgenetictesting/ancestrytesting/] NIH (National Library of Medicine)
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- What Is Genetics? [https://biobeat.nigms.nih.gov/2024/04/what-is-genetics/] NIH (National Institute of General Medical Sciences)
- What is Pharmacology? [https://biobeat.nigms.nih.gov/2023/08/what-is-pharmacology/] NIH (National Institute of General Medical Sciences)

Specifics

- Condition Treatments [https://patienteducation.asgct.org/disease-treatments] (American Society of Gene & Cell Therapy)
- What are CAR T cell therapy, RNA therapy, and other genetic therapies?: MedlinePlus Genetics [https://medlineplus.gov/genetics/understanding/therapy/othergenetictherapy/] NIH (National Library of Medicine)
- What are genome editing and CRISPR-Cas9?: MedlinePlus Genetics [https://medlineplus.gov/genetics/understanding/genomicresearch/genomeediting/] NIH (National Library of Medicine)

Statistics and Research

- Gene Circuits Enable More Precise Control of Gene Therapy [https://news.mit.edu/2025/gene-circuits-enable-more-precise-control-gene-therapy-0428] (Massachusetts Institute of Technology)
- Human Genome Project (HGP) [https://www.genome.gov/human-genome-project] NIH (National Human Genome Research Institute)
- Infant with Rare, Incurable Disease Is First to Successfully Receive Personalized Gene Therapy Treatment [https://www.nih.gov/news-events/news-releases/infant-rare-incurable-disease-first-successfully-receive-personalized-gene-therapy-treatment] NIH (National Institutes of Health)

Clinical Trials

- ClinicalTrials.gov: Gene Therapy [https://clinicaltrials.gov/search?intr=%22gene+therapy%22&aggFilters=status:not%20rec] NIH (National Institutes of Health)

Journal Articles

References and abstracts from MEDLINE/PubMed (National Library of Medicine)

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- Article: Sustained Clinical Benefit of AAV Gene Therapy in Severe Hemophilia B. [https://www.ncbi.nlm.nih.gov/pubmed/40499172]
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- Genes and Gene Therapy -- see more articles [https://pubmed.ncbi.nlm.nih.gov/?term=%22Genetic+Therapy%22%5Bmajr%3Anoexp%5D+OR+%22Genes%22%5Bmajr%3Anoexp%5D+AND+humans%5Bmh%5D+AND+english%5Bla%5D+AND+%22last+1+Year%22+%5Bedat%5D+AND+%28patient+education+handout%5Bpt%5D+OR+guideline%5Bpt%5D+OR+clinical+trial%5Bpt%5D%29++NOT+%28letter%5Bpt%5D+OR+case+reports%5Bpt%5D+OR+editorial%5Bpt%5D+OR+comment%5Bpt%5D%29+AND+free+full+text%5Bsb%5D+]

Reference Desk

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- Chromosomes [https://www.genome.gov/about-genomics/fact-sheets/Chromosomes-Fact-Sheet]
  NIH (National Human Genome Research Institute)
  Also in Spanish [https://www.genome.gov/es/about-genomics/fact-sheets/Cromosomas]
- DNA Microarray Technology [https://www.genome.gov/about-genomics/fact-sheets/DNA-Microarray-Technology]
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- Genetic Testing Registry [https://www.ncbi.nlm.nih.gov/gtr/] NIH (National Center for Biotechnology Information)
- Talking Glossary of Genomic and Genetic Terms [https://www.genome.gov/genetics-glossary]
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  Also in Spanish [https://www.genome.gov/es/genetics-glossary]
- Transcriptome [https://www.genome.gov/about-genomics/fact-sheets/Transcriptome-Fact-Sheet]
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  Also in Spanish [https://www.genome.gov/es/about-genomics/fact-sheets/Transcriptoma]

Find an Expert

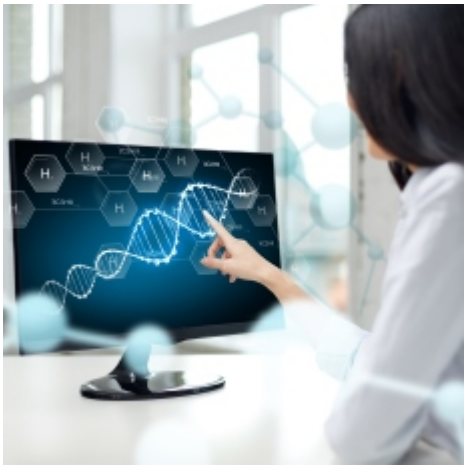
- Gene Therapy Centers [https://patienteducation.asgct.org/patient-journey/gene-therapy-centers]
  (American Society of Gene & Cell Therapy)
- National Human Genome Research Institute [https://www.genome.gov/] NIH

Children

- Gene Changes (Mutations) [https://kidshealth.org/en/parents/gene-mutations.html] (Nemours Foundation)
  Also in Spanish [https://kidshealth.org/es/parents/gene-mutations.html]
- Genetics (For Parents) [https://kidshealth.org/en/parents/about-genetics.html] (Nemours Foundation)
  Also in Spanish [https://kidshealth.org/es/parents/about-genetics.html]
- What Is a Gene? [https://kidshealth.org/en/kids/what-is-gene.html] (Nemours Foundation)
  Also in Spanish [https://kidshealth.org/es/kids/what-is-gene.html]

Teenagers

- Genes and Genetics [https://kidshealth.org/en/teens/genes-genetic-disorders.html] (Nemours Foundation)
  Also in Spanish [https://kidshealth.org/es/teens/genes-genetic-disorders.html]



MEDICAL ENCYCLOPEDIA

Genes [https://medlineplus.gov/ency/article/002371.htm]

Genetics [https://medlineplus.gov/ency/article/002048.htm]

Related Health Topics

Genetic Disorders [https://medlineplus.gov/geneticdisorders.html]

Genetic Testing [https://medlineplus.gov/genetictesting.html]

National Institutes of Health

The primary NIH organization for research on *Genes and Gene Therapy* is the National Human Genome Research Institute [http://www.genome.gov/]

NIH MedlinePlus Magazine

Gene Therapies are a Promising Path to Treating Rare Diseases [https://magazine.medlineplus.gov/article/gene-therapies-are-a-promising-path-to-treating-rare-diseases]

Tesha Samuels Advocates for Others After Living with Sickle Cell Disease [https://magazine.medlineplus.gov/article/tesha-samuels-advocates-for-others-after-living-with-sickle-cell-disease]