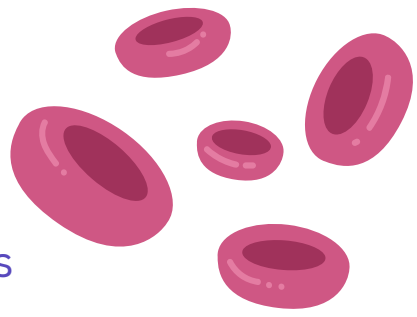
Gene Therapy

What is gene therapy?

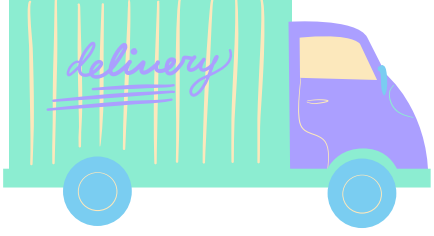
Gene therapy is a technique that uses genetic material to treat or cure a disease. This is often done by inactivating a variant gene that is causing a condition, replacing a variant gene with a typical copy, or bringing in a new or altered gene into the body.

What is cell therapy?

Cell therapy is transferring specific cells into a patient to treat a condition. These cells can come from the affected person or from an unaffected donor, depending on the therapy. Sometimes cell therapies can be combined with gene therapy.

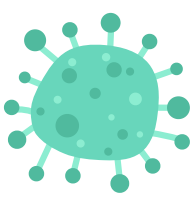


How does gene therapy work?



Therapeutic material is created that can inactivate, replace, or alter the malfunctioning gene. This material is put inside a vector, which is a vehicle that delivers the therapeutic genetic material into the cell. When the material is in the cell, it functions like a set of instructions, telling the cell how to change DNA to get a permanent therapeutic effect.

Viral Vectors



Viral vectors use a virus as the vector. The viral genes are removed so that it's safe to use, and the vector is modified to only deliver the therapeutic material.

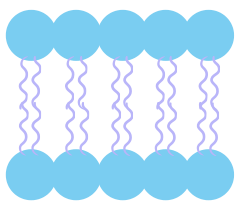
Examples of Viral Vectors:

- Adeno-associated viral (AAV) vectors
- Adenoviral vector
- Lentiviral vector
- Retroviral vector

Pros: Efficient at transferring therapeutic material to the intended cell

Cons: Potential to cause an unwanted immune response in the body or damage cells

Nonviral Vectors



Nonviral vectors use things that are NOT a virus as the vector.

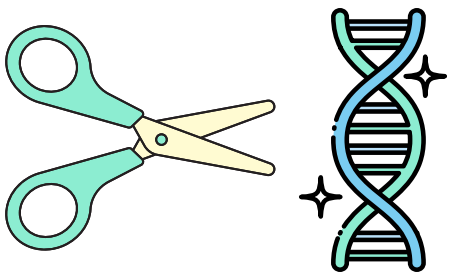
Examples of Nonviral Vectors

- Lipid nanoparticles (LNPs)
- Cationic polymers
- Inorganic nanoparticles
- Exosomes
- Polymer hydrogels

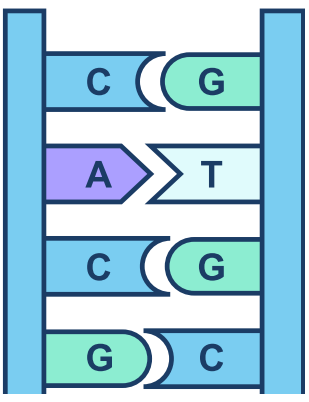
Pros: Less risk for harming the person, reduced toxicity to cells

Cons: Less efficient at transferring therapeutic material to the intended cell

What is gene editing?



Gene editing is a type of gene therapy where genetic material is delivered to the cell, and that material edits pieces of DNA within the cell.



What is CRISPR?

CRISPR is an acronym that stands for Clustered Regularly Interspaced short Palindromic Repeats, and is a tool to edit genetic material. After being brought to the cell with a vector, there is a guide RNA that finds the sequence of DNA that should be edited. Then a protien (like a Cas enzyme) cuts the DNA at the place where the guide RNA says to. CRISPR removes, adds, or changes DNA letters. Then natural DNA repair happens, making this change permanent.

How is gene therapy administered?

Gene therapies are administered differently depending on how genetic information is transferred to the cells.

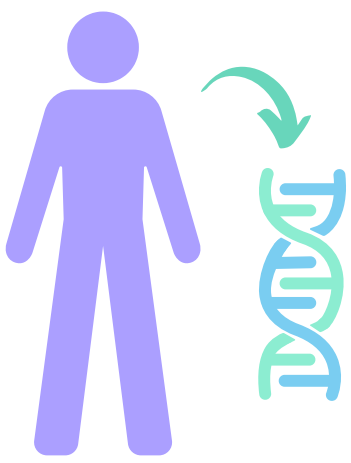
When gene therapy is used to modify genes in the body (*in vivo*), the vectors are injected directly into the patient.

When gene therapy modifies cells outside the body (*ex vivo*), doctors may take blood, bone marrow, or tissue out of the body and introduce the therapeutic product into these cells in the lab. Once modified, the patients' cells are returned to them.

The time it takes to receive a gene therapy varies. Some therapies require a period of conditioning prior to treatment to prepare the body for the therapy. Treatments may also require some length of an in-patient recovery period. These can vary by treatment.

What conditions does gene therapy treat?

Currently, gene therapy products are being studied to treat different conditions including genetic diseases, cancer, and infectious diseases.



How long does gene therapy last?

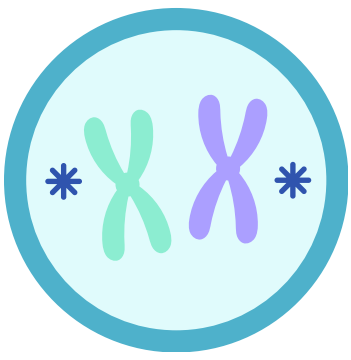
It is not yet known how long different gene therapies will last after treatment. Early research suggests it will last several years, but this is still being studied and may vary by therapy.

Is gene therapy passed onto future generations?

Currently, gene therapy products are being tested in somatic cells, NOT germ cells, which are what passes genetic information to a person's children.

Somatic Cells

Somatic cells are any cell that are NOT germ cells.



Germ Cells

Germ cells, or reproductive cells (sperm and eggs), are what pass on genetic information to a person's child.

Is gene therapy safe?

Every therapy comes with risks. It's advised to talk through any potential therapies with your care teams to learn more about the potential risks and benefits of gene therapies, as well as alternative treatment options, if they exist.

When I look at information about therapies, I see the words ex vivo, in vivo, and in vitro. What do those mean?

In vivo

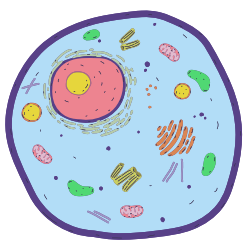
In vivo means it takes place in the body of a living person

In vitro

In vitro means in the laboratory outside of the body, using cells or tissues not taken from the body

Ex vivo

Ex vivo means taking a cell, tissue, or organ from a living person for a treatment or procedure and then returning that to the person



1. https://www.fda.gov/vaccines-blood-biologics/cellular-gene-therapy-products/what-gene-therapy
2. https://patienteducation.asgct.org/gene-therapy-101/vectors-101
3. https://www.ncbi.nlm.nih.gov/pmc/articles/PMC4347098/
4. https://jnanobiotechnology.biomedcentral.com/articles/10.1186/s12951-023-02044-5
5. https://innovativegenomics.org/what-is-crispr/
6. https://patienteducation.asgct.org/gene-therapy-101/gene-therapy-approaches