Understanding Gene Therapy

As research continues to advance, **gene therapy is becoming a treatment option** for many different genetic conditions.

Learn more about what it is, how it works, and how it's different from other treatments.

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What is a genetic condition?

It all starts with DNA.

DNA is a set of instructions that is unique to each person. It comes in sets called **genes.** Genes are the specific instructions that help our bodies grow, develop, and function. They also play a role in how we look because they decide things like hair and eye color.

Genetic variants

Sometimes, small changes can happen to our genes. These changes are often called **genetic variants.**

Normal gene

Gene with variant

We all have these variants, and they are often harmless. However, a **genetic condition** happens when one of those variants causes a problem in someone's body.

You may have heard of genetic conditions like cystic fibrosis, Duchenne muscular dystrophy, or sickle cell disease. Because they are caused by a change to a gene, genetic conditions are usually lifelong.

What is gene therapy?

Gene therapy is a type of treatment designed to treat genetic conditions.

It does this by addressing the problem at the genetic level. This means it either changes a nonworking gene that is in the body, or it gives the body a new, working copy of the gene it needs.

Gene therapy is different from other treatments because it works at the **genetic level** to help address the condition instead of the effects or individual symptoms

- This means it can potentially reduce the need for other therapies that have been used to manage the disease and/or symptoms

Many gene therapies are given as a **one-time treatment.** While each gene therapy and each person are different, these therapies are designed to continue working over time

- Since gene therapies are a newer form of treatment, researchers have not been able to study them long enough to know whether they will be effective for a person's entire lifetime
- The duration of the effects will continue to be studied as people who have received gene therapy grow older

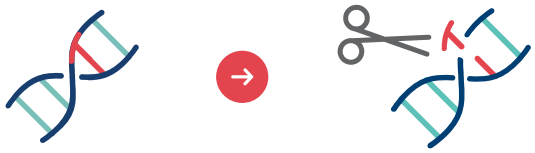
As with any medicine, it is important to remember that gene therapy comes with potential risks and side effects.

Types of gene therapy

While there are several different types of gene therapy, there are typically 3 main categories: gene editing, gene silencing, and gene addition therapy.

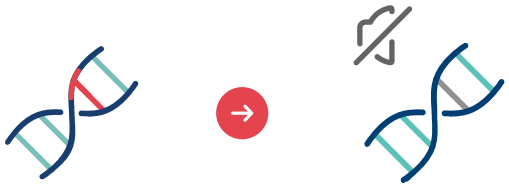
Gene Editing

Uses special tools—like CRISPR—to change (or "edit") a nonworking gene so it works correctly



Gene Silencing

"Turns off" a gene that is not working correctly and causing the genetic condition



Gene Addition Therapy

Delivers a new, working copy of the nonworking gene to certain cells in the body

What is gene addition therapy?

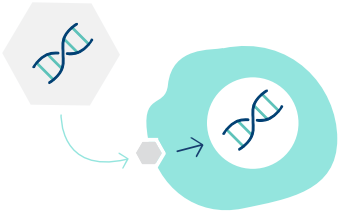
Many gene addition therapies are called adeno-associated virus (AAV) gene therapies.

AAV gene therapy uses a delivery vehicle called a **viral vector** to deliver the new, working genes to specific cells in the body. Viral vectors come from a virus, but they are specially designed so they cannot cause that virus in people.

This is how AAV gene therapy works:

The **viral vector's** DNA is removed. It is replaced with a **working copy of the gene** the body needs

The viral vectors are then put into the body, usually through an **injection or an IV**



The vectors find the right cells in the body and **place the working genes** inside the nucleus

Gene addition therapy is designed to reach the cells that need it in order for them to work better. The new genes help the body work properly, easing symptoms of the genetic condition.

FAQs about gene therapy

Is gene therapy safe?

Before a gene therapy can be prescribed by doctors, it must be tested in clinical studies for safety and effectiveness. These **studies are reviewed by FDA scientists** who make sure that the gene therapy meets strict standards

- To date, the FDA has approved **over 20 gene therapies** for rare and serious conditions, such as spinal muscular atrophy, hemophilia, and sickle cell disease. Some of these therapies are approved for children, including infants, and others are for adults

Expert care teams are specially trained in how to safely give different therapies to patients. These teams often work at hospitals where the gene therapies are studied. People receiving gene therapy will likely have to travel to one of these specific hospitals to receive treatment

Doctors and researchers **continue to study gene therapy** in clinical trials. These trials help them understand how to make treatments safer and more effective

If you want to learn more about gene therapy for yourself or someone you're caring for, talk with your doctor or a genetic specialist. They can help you understand whether gene therapy might be an option and tell you more about what to expect.

How is gene therapy given?

Depending on the condition, gene therapies are usually delivered one of two ways:

Local administration

Given directly to a specific part of the body, like the eye or brain

Intravenous infusion

Given through an IV into the bloodstream

The goal is to get the genes to the specific cells in the body that need them the most. For example, if a gene therapy is meant to treat a condition affecting vision, it may be most effective to deliver the gene therapy through an injection directly to the eyes.

What else do I need to know?

- Like for many other medicines, gene therapy **dosing is determined based on the individual.** This means that things like a person's weight or the physical structure of their body help decide what dosage of gene therapy they will receive
- Some gene therapies **require taking medicines,** like immunosuppressants, before and/or after to help the body tolerate the treatment
- Gene therapy **isn't right for everyone.** Whether a person can receive it depends on things like their genetic condition, their age, other treatments they've had, and certain test results

Visit NSPharma.com to learn more about our pipeline and how gene therapy is changing the treatment landscape for genetic conditions.

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