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Gene Therapy for People with Catecholaminergic Polymorphic Ventricular Tachycardia (CPVT)

Understanding Gene Therapy

What Is Gene Therapy?

Gene therapy is a technique that delivers genetic material directly into cells. This can help compensate for genes that are not working correctly.

Doctors use special noninfectious viruses—called AAVs—to carry the desired genes into your cells.

What Is Needed for Gene Therapy?

AAVs, or adeno-associated viruses, are used to deliver genes into cells

AAV Capsid	Delivery vehicle
Promoter	Tells the gene where to turn on
Transgene	The gene we want to deliver

How Does Gene Therapy Work?

The AAV delivers the desired gene into the cell.

Once inside, the AAV delivers the gene into the cell's nucleus, where it releases the gene.

The gene then forms a new structure that produces a functional protein.

- Viruses are naturally good at delivering genetic material into cells—this is how infections work
- Scientists have adapted viruses to carry therapeutic genes into the cells that need them
- These viruses can also target select tissues (for example, the heart or brain), which allows doctors to introduce a working gene into many cells at once
- The first human gene therapy trials using AAVs were done in 1995,[1] in people with cystic fibrosis; Multiple gene therapies have since been approved around the world[2]

1. Flotte et al., 1996; 2. As of April 2025

Understanding Gene Therapy

Gene Therapy Works Differently Than Traditional Drug Therapy

Understanding the differences between gene therapy and traditional drug therapy may help you **consider whether gene therapy is right for you.**

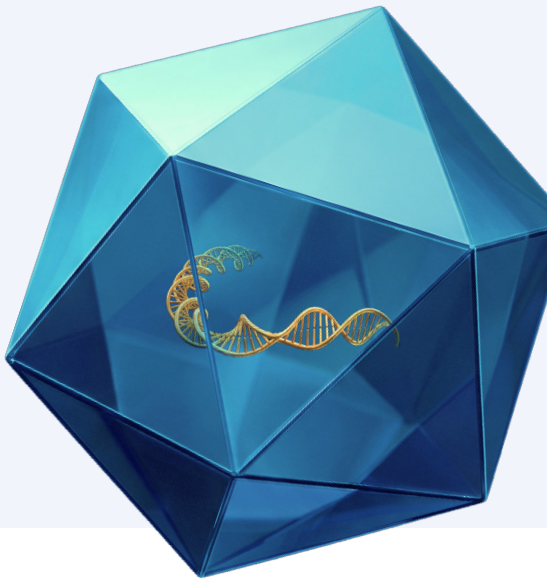
	Gene Therapy*	Traditional Drugs
Goal	Targets the genetic cause of a condition, not just symptoms	Manages a condition and relieves symptoms
Dosing	One-time dose designed to produce a long-lasting effect	Requires ongoing doses to maintain effect
Duration	Non-reversible	Processed and cleared by the body over time
Trial Length	Several years	A few weeks to several years
Impact on Future Treatment Options	May exclude your ability to join future clinical trials or receive another gene therapy	You can usually join other trials after treatment is cleared from your body

Evaluating Gene Therapy

Clinical trials help researchers learn more about a potential gene therapy, including whether it is safe and works as intended. One important area researchers look at is:

Safety

Researchers carefully follow clinical trial participants, often over a long period of time, to better understand any possible side effects or risks



Please talk with your doctor to learn more.

*Adapted from: Friedreich's Ataxia Research Alliance, https://www.curefa.org/research/understanding-clinical-trials/understanding-genetic-therapies-for-friedreichs-ataxia/

Designing and Evaluating Gene Therapy for CPVT

- Improved understanding of the underlying disease process and genetic underpinnings of many cardiac diseases has led to the identification of new therapeutic targets
- Current CPVT treatments do not address the genetic cause
- Genetic changes (mutations) in CPVT alter the movement of calcium within heart cells. This can cause an abnormal release of calcium in heart cells during heart muscle relaxation
- This abnormal release of calcium can trigger dangerous heart rhythm problems (arrhythmias)

For people with CPVT, gene therapy may be used to deliver functioning genes into the cells of the heart to help maintain normal heart function and reduce the risk of irregular heart rhythms.

SGT-501: Solid's Investigational Gene Therapy for CPVT

SGT-501 is the only CPVT therapy in development designed to deliver AAV-based gene therapy to the heart to help maintain normal heart function.

Preclinical Studies Suggest That SGT-501 May Support[1]:

Safe and well-tolerated administration

SGT-501 was well tolerated at studied doses with no adverse events observed.

Decrease in irregular heartbeats

Mice treated with SGT-501 showed improvement in heart function with a reduction in abnormal heartbeat.

Preclinical studies are studies done in the lab and in animals before a treatment is given to people to look at whether a treatment is safe and effective.

To learn more about SGT-501, please visit **www.solidbio.com**.

1. Data on file. Solid Biosciences. 2026.

SGT-501 is an investigational drug that has not been approved by any regulatory body around the world.

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