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Gene Therapy for People with Friedreich's Ataxia (FA)



Kate
Avid biker and fiction lover
Diagnosed with FA at 16

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.**

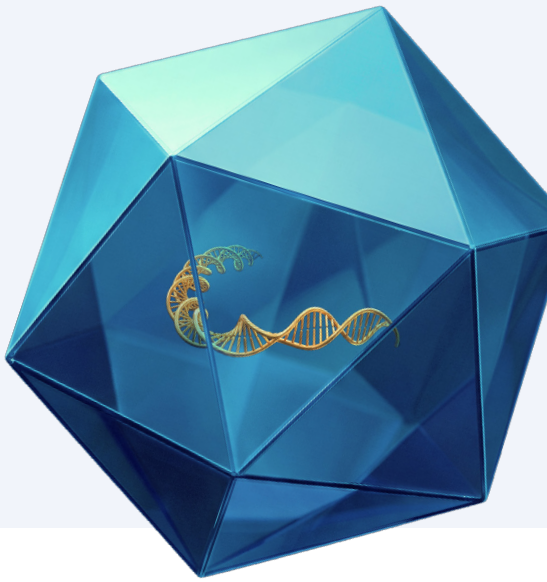
	Gene Therapy*	Traditional Drugs
Goal	Targets the genetic cause of a condition, not just symptoms	Manages a condition and relieves symptoms
Dosing	One-time dose designed to produce a long-lasting effect	Requires ongoing doses to maintain effect
Duration	Non-reversible	Processed and cleared by the body 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 FA

For people with FA, **gene therapy may be used to deliver a functional copy of the frataxin (FXN) gene into cells so they can make frataxin,** a protein the body needs for cells to work correctly. This approach aims to correct the underlying shortage of frataxin that causes FA, rather than only treating symptoms.

Gene Therapy for FA[1]



NEW FUNCTIONAL FXN GENE

MUTATED FXN GENE

SGT-212: Solid's Gene Therapy Candidate for FA

SGT-212 is the only FA therapy in development designed to deliver AAV-based gene therapy to the heart and brain to help restore cardiac and neurologic function.

Preclinical Studies Suggest That SGT-212 May Support[2]:

FXN expression in target tissues



This means that the gene was delivered to the right parts of the body and that it produces frataxin. In FA, the cells don't produce enough frataxin. Some organs, such as the brain and the heart, require a lot of frataxin.

Increased mitochondrial function



The lack of frataxin protein can lead to damage to the parts of the cell that produce energy, called mitochondria. SGT-212 appears to improve mitochondrial function.

Improved heart and nervous system function



Some parts of the body, such as the nervous system and the heart, need a lot of frataxin protein. Preclinical studies suggest that SGT-212 may support improvements in the function of both the heart and nervous system.

Increased survival



Mice that were treated with SGT-212 showed improvement of the heart and the nervous system, which may also help increase survival.

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.

1. Adapted from: Friedreich's Ataxia Research Alliance, https://www.curefa.org/research/understanding-clinical-trials/understanding-genetic-therapies-for-friedreichs-ataxia/
2. Data on file. Solid Biosciences. 2025.

Solid's Approach to Gene Therapy for FA

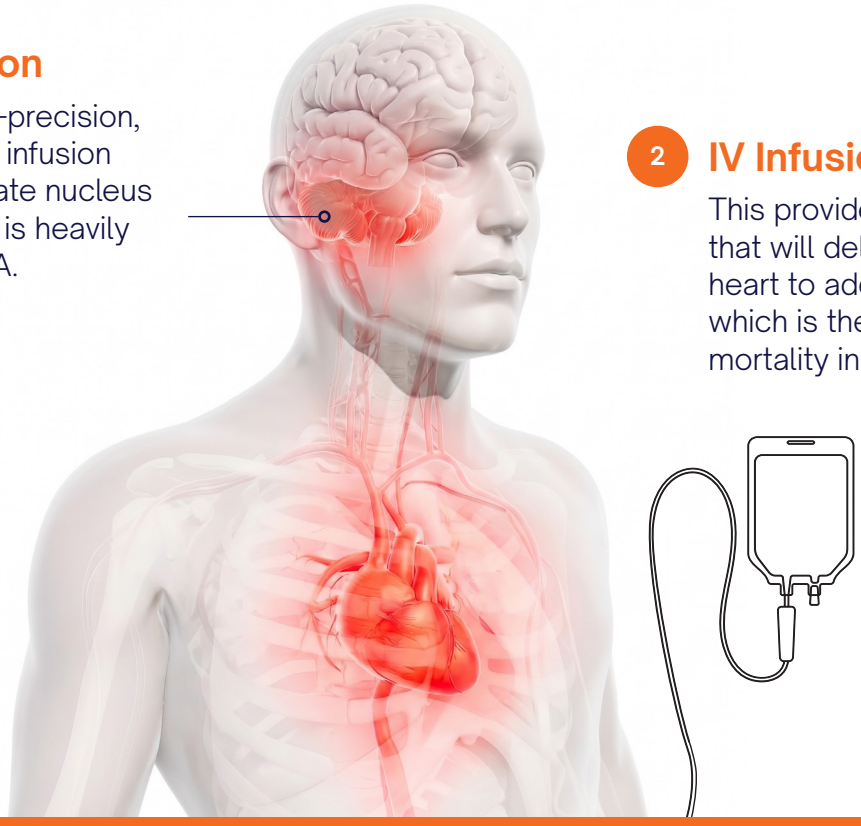
Dual-route Administration Is Designed to Address Patient Needs

SGT-212 will be given by 2 routes: An intradentate nucleus (IDN) infusion into 2 areas of the brain and an intravenous (IV) infusion in the arm

- IDN infusions allow delivery of the therapy directly into the part of the brain most affected by FA
- IV administration addresses the largest cause of mortality in FA, cardiomyopathy, and offers the potential to treat other organ systems that may be affected by FA

1 IDN Infusion

This is a high-precision, robot-guided infusion into the dentate nucleus (DN). The DN is heavily affected in FA.



2 IV Infusion

This provides systemic treatment that will deliver SGT-212 to the heart to address cardiomyopathy, which is the leading cause of mortality in FA[1].

To learn more about SGT-212, please visit **www.solidbio.com**.

Scan for information from the Friedreich's Ataxia Research Alliance (FARA) about gene therapy for people with FA

1. Cook A, Giunti P. British Medical Bulletin. 2017;124:19-30.

SGT-212 is an investigational drug that has not been approved by any regulatory body around the world.

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