



**SOLID**  
BIOSCIENCES

# 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



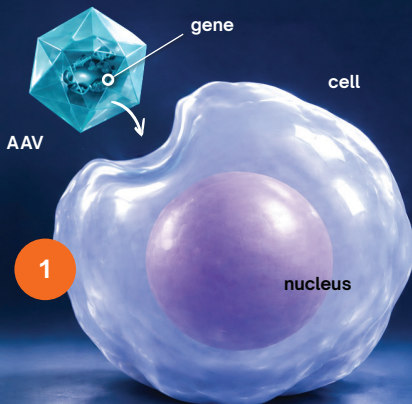
Transgene



The gene we want to deliver

## How Does Gene Therapy Work?

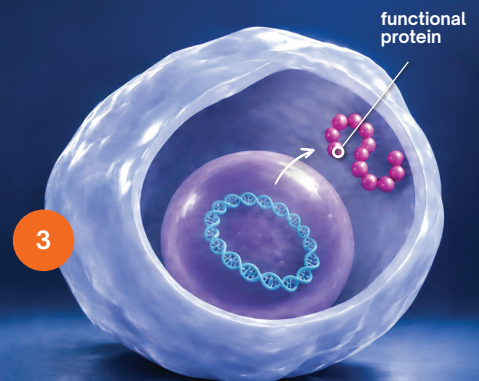
The AAV delivers the desired gene into the cell.



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,<sup>1</sup> in people with cystic fibrosis; Multiple gene therapies have since been approved around the world<sup>2</sup>

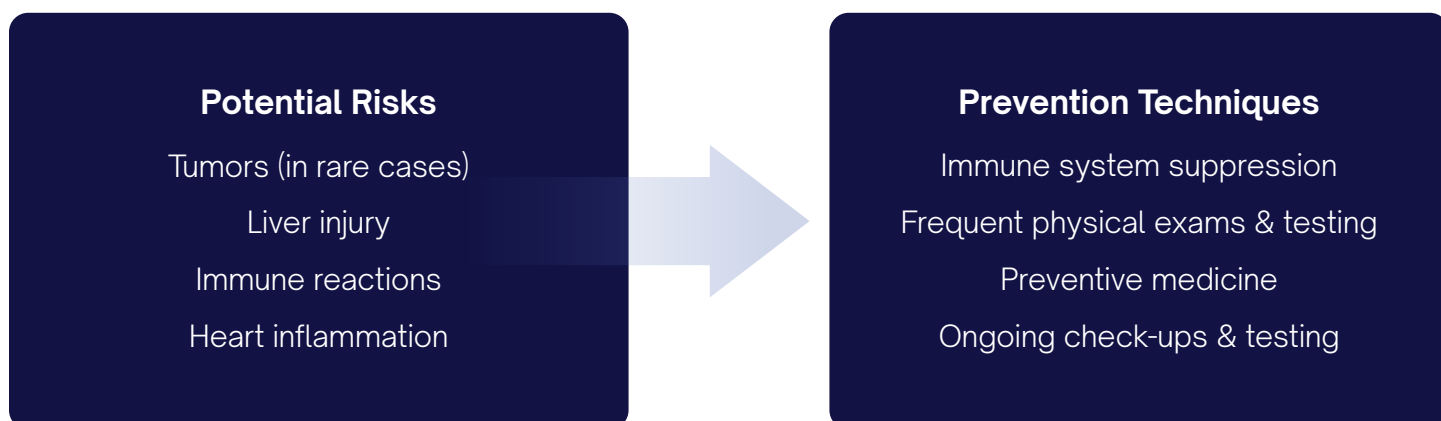
# Understanding Gene Therapy

## 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 |

## What Are the Potential Risks Associated with AAV Gene Therapy?



Careful examination prior to therapy and close monitoring during treatment helps **reduce these risks**

# 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<sup>1</sup>



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<sup>2</sup>:

### 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

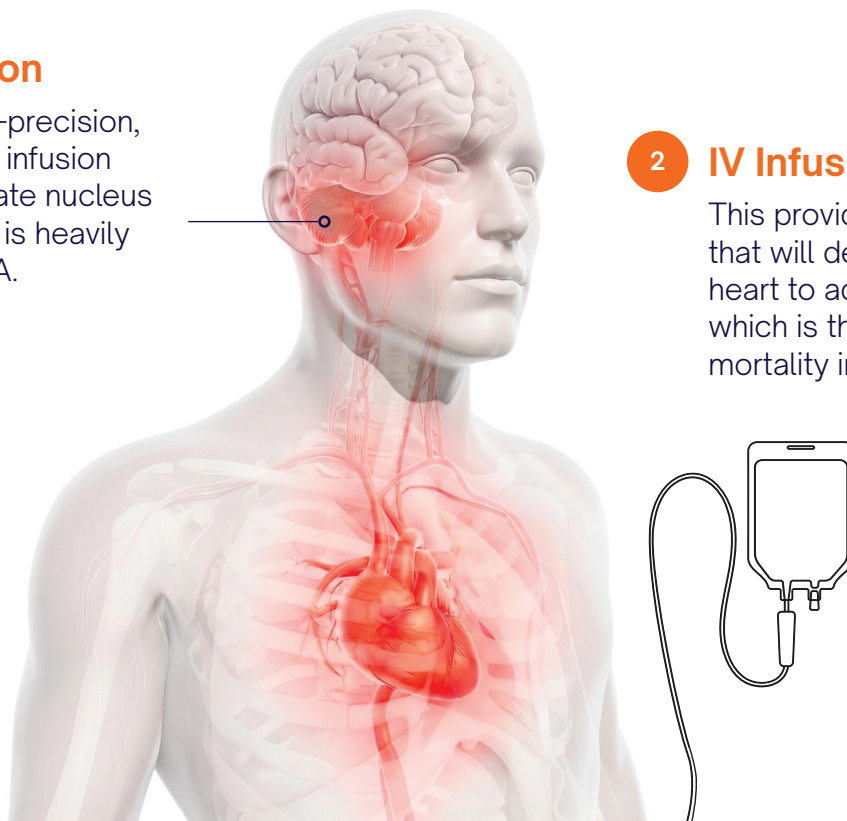
- IDN infusions allow delivery of the therapy directly into the part of the brain most affected by FA
- IV administration addresses the largest cause of mortality in FA, cardiomyopathy, and offers the potential to treat other organ systems that may be affected by FA

### 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<sup>1</sup>.



To learn more about SGT-212, please visit [www.solidbio.com](http://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.