



**American
Heart
Association®**

Your Guide to Gene Editing and Clinical Trials for Heart Health

Clear Answers to Help You Take Control

This guide gives you clear information about heart problems that run in families and innovative treatments like gene editing and clinical trials. It was made to help you make health choices that are right for you.



Intro: About This Guide

By reading this guide you will:

- 1 Understand what gene editing is and why doctors are studying it
- 2 Learn what clinical trials are and how they work
- 3 Think about what matters most to you when choosing treatment
- 4 Get helpful tips for talking to your doctor, family, or caregiver if you want to learn more about joining a trial

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Before your next appointment, highlight parts of the guide you want to discuss with your doctor or questions you'd like to ask. You can also share it with a family member or friend so they can support you.



Scan this QR code to read this guide online or print it. Go to www.heart.org/ATTR-CM to download it and find more helpful information.

1. How This Guide Can Help You

This guide can help you make informed decisions for your treatment

Learning that you or someone you love has a genetic condition that affects the heart can bring up many emotions. You might feel unsure about what it means or what comes next. You're not alone.

This guide is here to help you understand the condition and feel more supported as you explore your options. It gives you clear information about genetic conditions that affect the heart and about advanced treatments being studied in clinical trials, including something called gene editing.



Take notes as you read through this guide.

Take your time—there's no rush.

You don't need to understand everything all at once. Read a little at a time. Stop when you need a break. Talk to your doctor or care team about anything you don't understand or what's most important to you.

Bring this guide to your next appointment!

Use this guide to write down questions, take notes during the visit, and refer to sections you want to review before or during the conversation. See the FAQ section on page 19 for some answers to questions you may already have.

2. What Is a Genetic Condition That Affects the Heart?

Genes affect how your body works

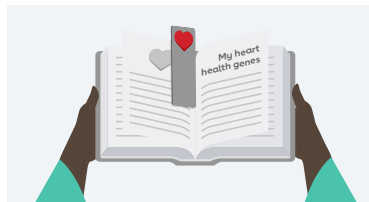
DNA is a tiny chemical structure in all of your cells that carries the instructions your body needs. DNA tells the body how to grow, work, and repair itself. Genes are sections of your DNA. Genes give instructions to your cells to make the proteins that build and maintain your body. Some heart conditions are caused by changes in your genes.

Your DNA comes from your parents. It determines traits like eye color, height, and even your risk for certain diseases. Scientists study DNA to better understand how changes in genes' instructions can affect health and to develop new treatments for genetic conditions.

Your DNA recipe:



Think of DNA as a recipe book. Each gene is like a specific recipe or page in that book.



If one of the pages in your recipe book has a typo, your body might make a protein that doesn't work right.



That can lead to problems, like harmful protein buildup in your heart.

DIGGING DEEPER: Types of genetic conditions that affect the heart

ATTR-CM (transthyretin amyloid cardiomyopathy):

This happens when a protein in your body called transthyretin breaks down and forms deposits in the heart. These buildups, or amyloids, can make it harder for your heart to pump blood properly. There are two types—one that is hereditary and one that is not.

Familial hypercholesterolemia (FH):

This causes very high cholesterol. Cholesterol is a waxy substance in your blood that helps build healthy cells. Too much can cause fatty buildup in your blood vessels, raising your risk of heart disease.

Hypertrophic cardiomyopathy (HCM):

This makes the walls of your heart muscle thicker than normal. It can block blood flow and make your heart work harder than it should.

Long QT syndrome (LQTS):

This affects your heart's rhythm. It can cause your heart to beat abnormally.

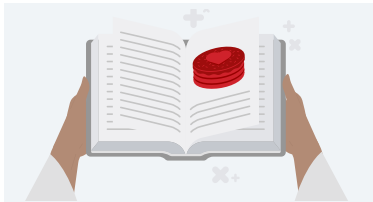
Finding out that you have a heart condition can feel overwhelming. Learning about it is an important first step toward getting the care and support you need.

What is gene editing?

Gene editing is an advanced method scientists are using to fix certain health problems at their source. It focuses on the gene that's causing the problem. Special tools help find the typo in your body's recipe book and correct it. This may help stop your body from making the protein that causes harm.

Gene editing is often considered as a treatment for hereditary diseases, which can be passed down from one or more parents to their children. But gene editing can also treat health conditions that are not hereditary.

How gene editing fixes your DNA recipe:



Imagine your body is baking a cake but the recipe isn't right.



For example, if the recipe calls for a faulty ingredient (eg, 1 wrench), the cake won't turn out right.



Gene editing fixes the recipe, or removes the faulty information entirely, so your body can make the right ingredients.

Go to www.heart.org/ATTR-CM to watch a video explaining how gene editing works.

Gene editing vs gene therapy

Gene therapy and gene editing are two different ways to treat genetic conditions. Gene therapy works by adding a healthy gene to replace or help a faulty one. This gives your body new genetic material to work better. Gene editing, on the other hand, fixes the faulty gene you already have. It uses special tools to correct or turn off the part of the gene causing problems.

Think of gene therapy as adding a new recipe to your cookbook, while gene editing is like fixing a mistake in the recipe you already have.

DIGGING DEEPER: How does gene editing happen inside the body?

Scientists use tools that act like tiny scissors to edit genes. These tools are sent into the body using a delivery system—often a harmless virus or a specially designed particle. The virus is reprogrammed to carry helpful instructions rather than cause infection, while the particle is engineered to safely transport the gene editing tools to their target. Once inside, the tool seeks out the faulty gene and either cuts it out or modifies it to address the problem.

Different treatments for genetic conditions that affect the heart

People with genetic conditions that affect the heart have other treatment options that are part of a standard care plan. Standard care helps manage symptoms and slow the disease. It does not cure it. This standard care treatment plan is often the same as what is used for other heart conditions that are not genetic. Specialized approaches, like gene editing and gene therapy, are being studied to fix or replace the faulty gene and may stop or even reverse the disease in the future.

Standard care includes:



Medicines: Doctors often prescribe drugs to help manage symptoms and slow the disease



Lifestyle changes: Healthy habits like eating nutritious foods, staying active, maintaining a healthy weight, and not smoking can support heart health. These changes are important for all types of heart conditions, whether genetic or not, and can help improve overall well-being



Regular checkups: Seeing a cardiologist (heart doctor) regularly is important to monitor the condition and adjust treatments as needed

Compare treatment in a gene editing trial vs a standard care plan:

Question	Gene Editing Trial	Standard Care Plan
What is the purpose of treatment?	Target the faulty gene causing your genetic condition that affects the heart.	Manage symptoms and slow down the disease.
How often will I be treated?	Treatment can be a one-time infusion, where medicine is given directly into your bloodstream through an IV (a small tube in your vein), or a local injection. Follow-up visits help monitor progress and adjust your care plan.	Daily medicine and regular doctor visits.
What are the potential benefits of treatment?	May halt or reverse the course of the disease.	Slows down the disease but does not stop it.
What are the potential risks of treatment?	May cause side effects during or after treatment.	May cause side effects during or after treatment.
How often will I be monitored during therapy?	You may have more doctor visits during the trial.	You'll have regular check-ups with your doctor.

After reading this section you might want to:

1

Talk to your doctor to better understand your genetic condition that affects the heart

Even if you've been diagnosed, it can help to go deeper. Ask your doctor to explain how your condition works, what it means for your health, and what treatment options—like gene editing—may be available now or in the future.

Try saying: "Can you help me understand more about my genetic condition that affects the heart and what it means for my care?"

2

Talk to your family about your diagnosis

Because genetic conditions can run in families, it may be helpful to share what you've learned with relatives. This can help them get screened or better understand their own health risks.

Try saying: "Since my diagnosis is genetic, it could be helpful for others in our family to get tested, too. I care about your health and wanted to share this with you."

3

Schedule an appointment with a genetic counselor

A genetic counselor can explain what your diagnosis means for you and your family and help clarify whether clinical trials might be a good fit.

Action: Visit [nsgc.org](https://www.nsgc.org) to find a certified genetic counselor near you.

4

Write down any questions you have about gene editing

The content in this section introduces gene editing, but it's normal to have more questions. Bring them to your doctor or care team so they can walk you through what it might mean for your condition.

Example questions:

- "What's the difference between gene editing and gene therapy?"
- "Are both gene editing and gene therapy available to help treat my condition?"
- "Is gene editing being studied for my condition?"
- "What are the risks or benefits I should know about?"

For caregivers

1

Learn more about your loved one's diagnosis

Take time to read up on their specific genetic condition that affects the heart so you feel more confident supporting them in conversations with doctors and in daily care.

Try saying: "Can we review the basics of this condition together so I can better support you?"

2

Encourage family conversations about genetics

Because genetic conditions can affect other relatives, support your loved one in having family discussions—or help facilitate them yourself—especially if others may benefit from screening or genetic counseling.

Try saying: "Would it help if I talked to [family member] about how this might impact them?"

3

Support emotional well-being

A diagnosis of a genetic condition can be overwhelming. Offer a listening ear, help your loved one process what they've learned, and reassure them that they don't have to figure everything out all at once.

Try saying: "This is a lot—I'm here to help you take it one step at a time."






Think about the questions most important to you and ask your doctor during your next visit

3. What Clinical Trials Are and Why They Matter

What is a clinical trial?

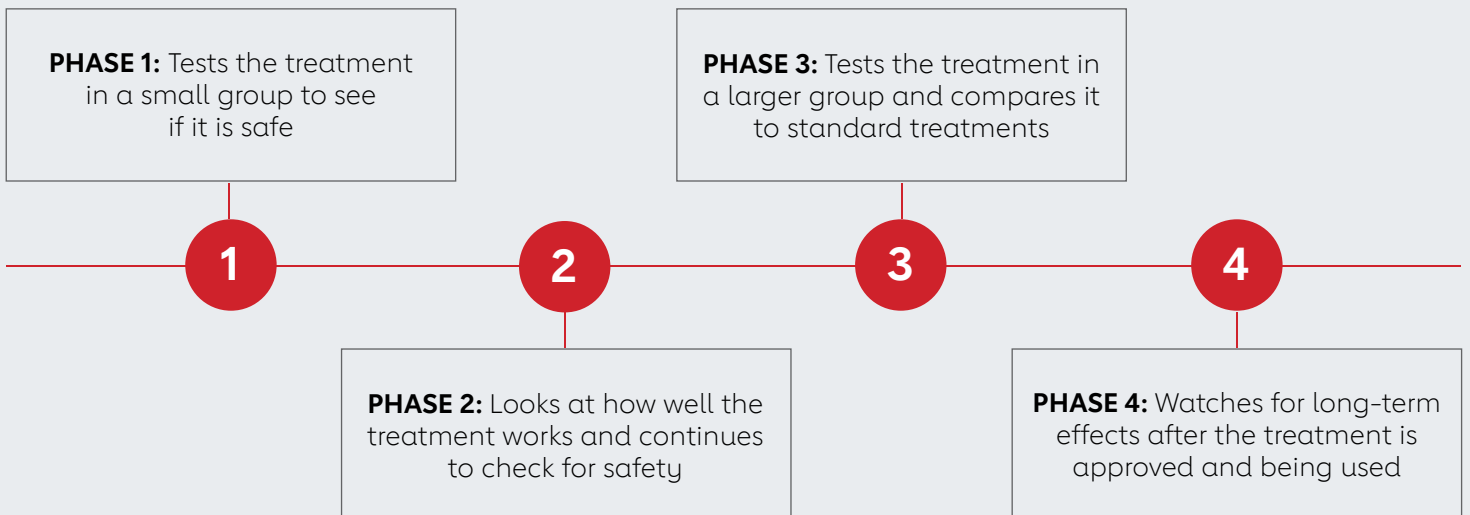
Clinical trials help doctors test treatments to see if they are safe and helpful. If you join a trial, you become part of a team working to improve care for yourself and others.

Key facts about clinical trials:

-  They help researchers learn how a treatment works, what the benefits might be, and if there are any side effects. This can help patients treat their disease.
-  Trials give people the possibility of getting advanced treatments that are not yet available in the market.
-  Joining a trial is your choice. All important facts will be shared with you before you decide. You'll be asked to sign a form that says you understand and agree. You can say no or stop at any time.
-  You will be closely supported and checked by a care team throughout the trial.
-  Groups like the Food and Drug Administration (FDA) and Institutional Review Boards (IRBs) check to make sure everything is done safely and fairly.

DIGGING DEEPER: There are many phases, or steps, of clinical trials

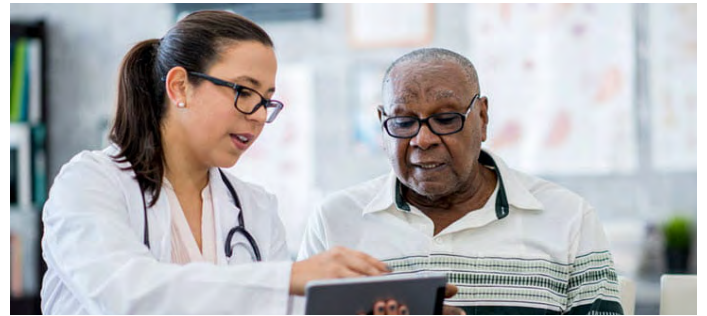
Each phase has a goal:



For a treatment to get FDA approval, the clinical trial must prove it is both safe and effective.

The importance of participating in gene editing trials for people who may qualify

Cardiac gene editing trials are important because they can give hope to people with genetic conditions that affect the heart. These trials focus on fixing the faulty gene that causes the disease, instead of just treating the symptoms. Researchers are studying whether this approach can safely and effectively stop or even reverse the disease. If you qualify, joining a trial means being part of advanced research that could lead to new treatments for conditions like ATTR-CM. This is an exciting step toward better care for you and others with genetic heart diseases.



Talk to your doctor about any concerns you may have.

To join a gene editing trial, a person usually needs to meet certain requirements, such as:

- 1** Having a confirmed diagnosis of a genetic condition that affects the heart, like ATTR-CM
- 2** Having specific health conditions and/or a genetic marker (a tiny part of your genes that can show where something important is located) that match the trial's needs
- 3** Being willing to give informed consent, which means agreeing to join the trial after learning all the details

DIGGING DEEPER: A closer look at gene editing trials

Working with your health care team in a clinical trial usually includes these steps:

- **Screening:** Before you join, the team will ask you questions about your medical history and may do blood tests, physical exams, or other checks to see if the trial is a good fit for you
- **Monitoring:** You will be closely supported during the trial. The team will check how you're doing and look out for any side effects
- **Placebo Possibility:** In some trials, people are placed in a comparison group and may get a placebo—a harmless pill, injection, or IV that doesn't contain the treatment. Even if you don't get the treatment, your part in the study still helps others in the future

You are always in control. You can leave anytime. Being in a trial is your choice. You can stop at any time for any reason.


Things to think about when deciding on participating in a gene editing clinical trial


Gene editing has been approved for other diseases. It is still being studied in clinical trials for genetic heart diseases. You decide if joining is right for you. There is no single right answer. It's a personal choice. What matters most is what feels right for you based on your values, your goals, and what's going on in your life.


You can talk with your doctor, family, or someone you trust to help you think it through.

How will you be supported during the trial?

If you join a clinical trial, you won't be alone. There are many ways people help you along the way:

- 

Medical support: You are cared for by a team of health care professionals who check in often to see how you're doing
- 

Help with appointments: Many trials offer transportation assistance, reminders for appointments, and help for your caregiver, too
- 

Emotional support: You may be able to talk to a counselor or join a support group to share how you're feeling

Want more help? See the Next Steps—Moving Forward With Confidence section on page 22 for support resources.

What to expect in a clinical trial

Every trial differs based on what is being studied. Be sure to ask your doctor about the details of the trial you are thinking about.

Learning about the trial	Screening and consent	Treatment and monitoring	After the trial
<p>Ask your doctor to explain the trial and what it involves. You can also find information in "Next Steps—Moving Forward With Confidence" - the Resources section on page 22.</p>	<p>Doctors will ask about your health and do some tests (like blood work) to see if the trial is right for you.</p>	<p>You may get a treatment or a placebo—it depends on the trial.</p>	<p>Your care team may keep checking in with you to see how you are doing.</p>
<p>If you're thinking about joining, your doctor will check if the trial is a good fit for you.</p>	<p>You'll review a form that explains what the trial is about. It will include any risks and benefits, and you'll decide if you want to join.</p>	<p>You'll have check-ins to see how you're doing and talk through any concerns.</p>	<p>You might hear how the trial helped others or led to a new learning.</p>
<p>You can say no at any point.</p>	<p>The care team will explain how they will keep you safe during the trial.</p>	<p>You can still leave the trial at any time, even during treatment.</p>	<p>You may keep getting care or be connected to other helpful options.</p>

How to ask about clinical trial participation

If you're thinking about joining a gene editing trial, here are the people who can help you understand what it means and what to ask:

- **Your cardiologist** (heart doctor) can tell you if a trial might be right for you and how it fits into your care. You can ask about the risks, possible benefits, and what to expect
- **A genetic counselor** can explain how your genes and family history affect your health. They can also help you understand gene editing in simple words
- **A trial coordinator** can help with questions about signing up, scheduling, and support during the trial. They make sure you know what joining a trial means

Questions to help you talk with your doctor about a clinical trial

What to ask or consider

Notes section

What are the short- and long-term risks I should know about before participating in this trial?	
What kind of support (transport, caregiver help, communication) will I receive?	
Where do I need to go, and how often will I need to go there?	
What will happen during those visits?	
What happens if I miss or stop a treatment?	
What happens when the trial is over? Will I still get care?	
What do I have to pay for during the trial?	
Will I keep taking my regular medicine during the trial?	
Do I need to do anything on my own like keep a journal or fill out surveys or questionnaires?	
Will I need to have internet access or an app on my phone?	

After reading this section, you might want to:

1

Ask your doctor if a clinical trial might be right for you

Bring up clinical trials in your next appointment to learn whether one is available—and appropriate—for your specific condition and health status.

Try saying: “Are there any clinical trials I qualify for, especially ones focused on gene editing for my condition?”

2

Review the phases of a clinical trial with your care team

Understanding the different phases of a trial can help set expectations. Ask where a trial is in the process and what that means for your care.

Try asking: “What phase is this trial in, and what does that mean for safety and results?”

3

Use the suggested questions in this guide—and add your own

Refer to the Questions to Help You Talk With Your Doctor About a Clinical Trial section on page 12 to help guide your conversation. Use it to prepare, write down your own questions, and bring the list to your appointment.

Tip: Add questions that reflect your goals, concerns, or anything you want to better understand.



Prepare a list of questions important to you for your next doctor visit.

For caregivers:

1

Help gather and organize questions for the care team

Offer to write down questions, attend appointments, and make sure your loved one feels supported as they weigh their options.

Try saying: “Would it help if I joined your next appointment to take notes or ask questions?”

2

Look into how the trial might affect your caregiving role

Some trials require more visits or ongoing monitoring. Ask what you might need to help with and what support the trial offers for caregivers.

Try asking: “How might this trial impact me as a caregiver—and what help is available for me?”

Make note of any other thoughts or questions you may have

What other questions do you or your family/caregiver have for your doctor?

4. Weighing Your Options and Values

You choose whether to take part in a clinical trial

Deciding whether to pursue gene editing, or take part in a clinical trial, is a deeply personal choice. This section can help you and your loved ones think about what matters most—your values, your goals, and what kind of treatment feels right for you.

There's no one right answer. What's most important is what feels right to YOU.

Activity

Goal: Help you and your caregivers reflect on what matters most when considering gene editing trials

Instructions: Think about what's most important to you when it comes to your health. Talk with someone you trust and discuss what matters to you. Circle the answer that aligns with what's important to you and write notes to help guide your decision. There are no right or wrong answers, just what feels right for you.

What matters to me

How important is this to me?
(circle one)

Why this matters

What matters to me	How important is this to me? (circle one)	Why this matters	
Staying independent and caring for myself	Very Somewhat Not	Example: "I've always managed my own care, and I want to keep doing that."	_____ _____ _____ _____
Being present for my family	Very Somewhat Not	Example: "Spending time with my grandchildren or supporting my partner means a lot to me. I want to stay well enough to do that."	_____ _____ _____ _____
Making my own care decisions	Very Somewhat Not	Example: "I want to feel confident and informed while making my choice. It matters to me that I understand what's right for me."	_____ _____ _____ _____
Trusting my faith and values	Very Somewhat Not	Example: "My beliefs guide how I make tough choices. I want this decision to feel aligned with what I believe in."	_____ _____ _____ _____

What matters to me

**How important is this to me?
(circle one)**

Why this matters

<p>Helping others in my community</p>	<p>Very Somewhat Not</p>	<p>Example: "If this helps people like me in the future, that matters to me."</p>	<hr/> <hr/> <hr/> <hr/>
<p>Avoiding treatments that feel unnecessary or risky</p>	<p>Very Somewhat Not</p>	<p>Example: "I want to avoid anything that feels too uncertain. I need to feel clear about the risks."</p>	<hr/> <hr/> <hr/> <hr/>
<p>Making the most of today</p>	<p>Very Somewhat Not</p>	<p>Example: "Even if I don't know what tomorrow holds, I want to feel okay and have peace of mind today."</p>	<hr/> <hr/> <hr/> <hr/>
<p>Having support from the people I trust</p>	<p>Very Somewhat Not</p>	<p>Example: "I want to know I'll have help from my care team and my loved ones throughout this."</p>	<hr/> <hr/> <hr/> <hr/>
<p>Not adding stress to my family</p>	<p>Very Somewhat Not</p>	<p>Example: "I don't want my decision to add more stress to my family's life or routine."</p>	<hr/> <hr/> <hr/> <hr/>

After reading this section, you might want to:

1

Reflect on what matters most to you

Take time to consider your personal values, your goals, and what type of care feels right for your life. Use the guide's worksheet to help clarify what's important.

Tip: Use the Activity in this section to get started.

2

Talk with your doctor about how your care aligns with your values

Use your reflections—along with the sample questions in this guide—to have a conversation with your care team. Ask how different treatment options, including gene editing trials, match your personal goals.

Try asking:

“Can we talk about how this treatment fits with my goals?”

“What are the pros and cons for someone like me?”

“What should I consider based on what matters most to me?”

3

Bring a trusted caregiver or loved one into the conversation

You don't have to weigh your options alone. Ask someone you trust to help you think through your decisions, attend appointments, or simply listen.

Tip: Use the notes section in this guide to prepare questions or reflections together in advance.



Talk with someone you trust and discuss what matters to you.

For caregivers:

1

Help your loved one think about what's most important to them

Encourage open conversation about their beliefs and what they hope for from their care. Listen without judgment and help them express those values to the care team.

Try asking: "What's most important to you as you think about your options?"

2

Help start the conversation with your loved one's doctor

If you're a caregiver, here are some ways to start the conversation with the doctor:

Try asking:

"Can we talk about how this care plan fits with what matters most to my loved one?"

"What questions should I help them ask, based on their goals and values?"

"How can I best support them in making this decision?"

"How involved can or should I be in the trial process?"

Make note of any other thoughts or questions you may have

What other questions do you or your family/caregiver have for your doctor?

5. Frequently Asked Questions

It's okay to have questions. This section answers some of the most common ones

If you or someone you care for has a genetic condition that affects the heart, learning about gene editing and clinical trials can feel confusing and overwhelming. You might have questions about safety, side effects, or how trials really work. That's normal.

These questions and answers are here to help you feel more confident, informed, and in control of your choices.

Q: How do I know if I'm eligible for a gene editing trial?

A: You may be able to join if you have a genetic condition that affects the heart and meet certain health requirements. Your doctor can check if you qualify. Trials follow strict safety rules, and you must give informed consent before joining.

Q: If I sign up and am selected, do I get the new therapy?

A: Not always. Some trials include a placebo group. This means you might get a harmless pill, injection, or IV instead of the treatment being studied. This helps researchers see if the treatment really works. Even if you don't get the treatment, being part of the trial helps future patients like yourself. Ask your doctor if there is a placebo group in the trial and how this may impact you if you receive a placebo.

Q: Is gene editing the same as gene therapy?

A: No, they are different. Gene therapy works by adding a healthy gene to help a faulty one. This gives your body new genetic material to work better. Think of your DNA like a recipe for your body. Your genes are the pages in that recipe. Gene therapy adds a brand-new page to the recipe.

Gene editing fixes the faulty gene you already have. It uses special tools to correct or turn off the part of the gene causing problems. If we consider the recipe example again, gene editing fixes typos in the recipe.

Both gene therapy and gene editing aim to help your body work better, but they work in different ways.

Q: Will gene editing change all my DNA?

A: No, it only changes the one gene that's causing your heart condition. It's like fixing a typo in one sentence of a recipe—it doesn't change the whole recipe.

Q: Why should I trust the research?

A: Trusting research can be hard, especially for communities that haven't always been treated fairly in the history of medical research. Today, gene editing trials are carefully planned to keep everyone safe. Doctors, scientists, and patient advocates work together to make sure the trials follow strict rules and are focused on helping people and protecting their rights and privacy. You will get all the facts before joining, and you can leave at any time. Researchers work hard to include people from all backgrounds and to make sure the research is fair. You are a partner in the process, not just a participant.

Q: What if I'm worried about the risks or side effects of gene editing?

A: It's okay to have fears. Before you get the treatment, doctors will run tests to make sure it's a good fit for you. You'll be watched closely during the trial and get follow-up care afterward. You can always ask your doctor to explain more or slow things down.

Q: How does the gene editing treatment get into my body?

A: Gene editing is delivered into your bloodstream by either injection or IV infusion.

Q: What happens if I decide to stop being part of the trial?

A: You are allowed to leave the trial at any time, for any reason. It's always your choice. Your care team will help you find the next step, like another treatment or support option. You may also hear updates in the future based on what the trial learns.

Q: How long do clinical trials typically last?

A: It depends on the trial. Your doctor will explain the full timeline and what to expect.

Q: Do I have to keep track of anything during the trial?

A: You won't have to manage everything alone. A team of doctors, nurses, and coordinators will help with appointments and health checks. There are also digital tools and safety teams that track how things are going, and they will keep you informed and supported the whole time.

Q: What changes will be made to my lifestyle, physical activity, and diet?

A: Not every trial has lifestyle changes, and if they do, your care team will explain everything and help you adjust.

Q: How will I pay for my treatment?

A: Clinical trials are generally free for those who join. You might have some extra costs, like getting to appointments or childcare. You should speak with your insurance provider to see if they may reimburse you for any costs.

Q: What if I'm not eligible for the trial?

A: You're not alone. Many patients don't qualify for trials. Your doctor can help you explore other treatments or discuss future trials. Staying informed about new research and trials can give you more options later.

6. Next Steps—Moving Forward With Confidence

Being informed is the first step, and every step counts

You've just taken a big step by learning about your genetic condition that affects the heart, gene editing, and clinical trials. You and your caregivers may wonder what to do next, but you don't need to make every decision today. Below you will find helpful resources to stay informed, find support, and explore options, even if joining a trial isn't possible right now.

Where to learn more

You and your caregivers can access trusted resources to deepen your understanding and stay updated on new developments:

- **American Heart Association Website:**
Visit for easy-to-understand videos, guides, and answers about genetic conditions that affect the heart, gene editing, and clinical trials. You can also read stories from patients and families.
<https://www.heart.org/clinicaltrials>
- **National Society of Genetic Counselors:**
You can meet and talk with a genetic counselor near you to understand more about your genetic condition that affects the heart and learn about clinical trial opportunities.
<https://www.nsgc.org>
- **Patient Advocacy Groups:**
Connect with groups that support people living with genetic conditions that affect the heart. These groups offer webinars, peer support, and newsletters to keep you informed.
Amyloidosis Research Consortium - <https://arci.org>
Amyloidosis Support Groups - <https://www.amyloidosisupport.org>
American Heart Association Support Network - <https://www.heart.org/SupportNetwork>
- **Clinical Trials.gov:**
Search a global database of clinical studies to learn what trials are happening and where.
<https://clinicaltrials.gov>

Tips for caregivers

- Ask the doctor how you can support your loved one
- Help keep track of appointments or offer rides to visits
- Write down questions together with your loved one before visits
- Remind them they are not alone

You don't have to decide everything today. Learning is a strong first step. Asking questions shows strength. Every step forward counts.

Intellia Therapeutics is proud to support the American Heart Association's Gene Therapy Awareness and Education resources.

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