



Sickle cell disease gene therapy FAQ

Discuss these questions with your [doctor](#) or clinical trial team.

Overview/General Questions

How is eligibility determined for sickle cell disease gene therapy?

Eligibility criteria may be different for each available gene therapies, including clinical trials. However, there are a few general criteria across all therapies, including your age, your sickle disease genotype, other treatments you are taking, your response to other treatments and your general health. To determine your eligibility, you may have to undergo certain tests as part of the screening process.

If I participate in a gene therapy clinical trial, what happens to the treatments I am currently taking for my sickle cell disease (e.g., hydroxyurea, pain medications)?

If you are eligible and decide to receive gene therapy, your clinical team and healthcare providers will make a plan for the treatments you are taking for your sickle cell disease. For example, if you are taking hydroxyurea, you may have to stop taking it two to three months before the stem cell collection process for gene therapy. You may undergo blood transfusions for this period to replace your hydroxyurea or other medications.

What are the steps in the gene therapy process?

Gene therapy involves a series of steps over a long period of time, some of which will include long hospital stays. It is important to obtain specific information about the clinical trial you are considering. These are the general steps across the different clinical trials:

- You will first prepare for stem cell collection.
- Two to three months later you will have your stem cells collected.
- Your stem cells will take seven to 10 weeks to be manufactured and processed in a lab.
- If the modified stem cells meet certain criteria, you will go through chemotherapy for conditioning.
- A few days later, the modified stem cells will be infused back into your body.
- After this, you will remain in the hospital as an inpatient for a long period and monitored closely. This period will approximately last four to six weeks.
- After this inpatient period, you will transition to monthly outpatient visits.

Learn more about each of these steps in the Clinical trial process for sickle cell disease gene therapy [infographic](#).

How will receiving gene therapy clinical trial affect my physical and mental health?

Receiving gene therapy clinical trial can be a difficult process for many reasons, such as the risks of the procedure, difficulty of conditioning process and a long-term hospital stay. It may also be difficult to manage your hopes, expectations and emotions.

After living with sickle cell disease for most of your life, you may struggle with taking on a new identity after the procedure. We created a [fact sheet](#) on mental health and sickle cell disease gene therapy participation to answer some of your questions.

Is gene therapy right for ME?

Gene therapy has the potential to significantly improve the health and quality of life for individuals with sickle cell disease. Talk to your doctor to determine if gene therapy could be right for you.

Where will I be able to receive gene therapy?

Gene therapy should be provided at Qualified Treatment Centers throughout the United States. These institutions should provide multi-disciplinary care. You should ask if the following types of care and services are available:

- Established sickle cell center
- Ability to do apheresis and collect stem cells
- Blood bank
- Fertility preservation
- Mental health program
- Local housing
- Ability to follow patients for at least 15 years

I've heard gene therapy being called "transformative therapy", what does that mean?

You may hear that gene therapy is being called "transformative" or "curative" or even, a "cure". The meanings behind "curative" and "cure" for sickle cell disease can be deeply personal. For now, gene therapy is considered "transformative," meaning people will experience a change.

Researchers will use your data (and the data of others) to determine if gene therapy can be considered "curative" or a "cure" for sickle cell disease.

Risk

What are the risks of sickle cell gene therapy?

The informed consent process will explain the known risks for going through gene therapy. The main risks are from the chemotherapy that is needed for the **conditioning** phase of gene therapy or the gene therapy itself.

The risks from the chemotherapy include infertility, hair loss and mouth ulcers. Chemotherapy also leads to fewer white blood cells, which puts you at a higher risk of serious infection. After the chemotherapy, you will be closely monitored in a protective environment to protect you from the risk of infection while allowing your white blood cells to increase.

There is also the risk that the gene therapy process will not work.

Why is chemotherapy used in gene therapy?

The process used to prepare your body to receive modified stem cells is called conditioning. Chemotherapy helps make room in your bone marrow to receive the modified stem cells. Chemotherapy is given for a few days prior to receiving the modified stem cells. The major side effects of chemotherapy include pain, mouth ulcers, nausea and vomiting, hair loss, infertility, risk for infection and bleeding. Your provider may also tell you about less-common side effects during the consent process.

Is there a risk of cancer from gene therapy?

Gene therapy can increase your risk of cancer. This risk could come from either the chemotherapy process or the gene therapy vector. The gene therapy vector could have an off-target effect and influence the activity of nearby cancer-causing genes, which could lead to the development of cancer. Experts believe that it is a small risk; however, the exact likelihood is still unknown.

Can I have children after I have gene therapy?

Because chemotherapy is used during the conditioning process, gene therapy comes with a risk of infertility. It is important to learn about these risks and discuss them with your provider, and if needed, discuss any options for fertility preservation, such as freezing eggs or sperm. You may be referred to a fertility specialist to address your questions and identify your options.

Can I pass my sickle cell gene to my children if I have gene therapy?

Because gene therapy will not change the DNA in your sperm or egg cells, you will still pass on the sickle cell gene to your child. It is important to know the carrier status of your partner so you can know the risk of your child having sickle cell trait or sickle cell disease. Genetic counselors are trained to help you know the risks of passing the gene to your children. You should ask to speak with a genetic counselor if you need more information.

Will gene therapy cure my sickle cell disease?

Every person's experience may be different. Studies show that people who receive gene therapy for sickle cell have different benefits, such as decreased pain and lower levels of sickled hemoglobin. Any conditions or types of organ damage from sickle cell that happened before receiving the therapy may not improve or change after gene therapy.

Your clinical trial team will create a follow-up plan to monitor your health. Researchers will use your data (and the data of others) to determine if gene therapy can be considered a cure for sickle cell disease.

Clinical trials show that those who participate in gene therapy have less sickled hemoglobin, less hemolysis and little to no vaso-occlusive pain events. Those who participate in gene therapy may also have improved health-related quality of life, including improved emotional and psychosocial wellbeing, general health and less bodily pain.

Benefits

What may be the benefits of sickle cell gene therapy?

Gene therapy is a treatment that may improve the health of people with sickle cell disease. The treatment may stop the sickling of cells and improve the lifespan of red blood cells. It may reduce complications of sickled cells blocking blood flow and depriving tissues of oxygen (called vaso-occlusive crises), pain and tiredness. Researchers do not know how gene therapy will affect people who have pre-existing organ damage in the long term.

What are the outcomes of gene therapy?

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Cost

What are the costs to participate in a sickle cell gene therapy clinical trial?

The clinical trial should cover all your costs for medication, being in the hospital and treatment. However, the clinical trial may not cover additional costs that may result from your participation. You should ask the clinical trial team if the trial covers:

- Procedures to help you have children later (e.g., fertility preservation).

- Outpatient mental health counseling.

- Primary care during and after the gene therapy.

- Costs associated with caregivers who will provide you support (for example, housing close to the clinical trial site).

Will my insurance pay for gene therapy?

If you are participating in an approved gene therapy, costs may be covered by insurance. The Centers for Medicare and Medicaid Services (CMS) developed a cell and gene therapy access model in response to Executive Order 14087 “[Lowering Prescription Drug Costs for Americans](#)”. The model is designed to improve health outcomes, increase access to cell and gene therapies, and lower health care costs. This coverage will depend on what health insurance you have and the agreement between your insurance and the healthcare institution.

What additional costs should I prepare for?

There may still be additional costs (e.g. out-of-pocket costs) that may result from your participation in approved gene therapy or through a clinical trial. You should ask about coverage for the following:

- Procedures to help you have children later (e.g., fertility preservation).

- Outpatient mental health counseling.

- Primary care during and after the gene therapy (including dental and eye vision care).

- Costs associated with caregivers who will provide you with support (e.g., housing close to the qualified treatment center).

Follow up Care

What type of health care will I need after sickle cell gene therapy?

You will be under the care of your clinical trial’s team and physicians for about two to three years. During that time, you will be in the hospital for a period about four to six weeks. After you complete the gene therapy trial, you will continue to have outpatient clinic visits. You should receive regular follow-up care related to your gene therapy from your clinical trial’s team. You should also seek care for your general health from your primary care providers (including your hematologist) after gene therapy. Your research team and general health care providers should work together to care for your health and well-being.

How long will I be followed after I complete my sickle cell gene therapy?

Experts recommend that you are followed for 15 years after your gene therapy treatment as part of the clinical trial. This will allow researchers to study the gene therapy treatment over time and to detect and treat long-term risks. These follow-up clinical studies are intended to help researchers detect all long-term effects over time.

How does the clinical trial team know if the gene therapy worked?

Across the different gene therapy approaches, the goal of gene therapy is to decrease the amount of sickled hemoglobin in every red blood cell. The clinical trial team will know if the gene therapy worked if the amount of sickled hemoglobin has lowered enough at certain checkpoints after the gene-modified stem cells are returned to your body. If the team can lower the sickled hemoglobin enough, the cells will not be fragile, sticky or even sickle. This can decrease the complications and treat or prevent the symptoms of sickle cell disease.

What happens if the gene therapy does not work?

The gene therapy process may not work for a few reasons:

Although there may be several attempts to collect your stem cells, the clinical trial team may fail to collect enough cells to modify for gene therapy.

Once the collected stem cells are gene-modified, the team may find that the cells are unsafe to return to your body.

In both instances, you would be removed from the study.

After the process, gene therapy may not have successfully lowered the amount of sickled hemoglobin in your blood. If this happens, your clinical trial doctor and general provider will talk to you about next steps for your long-term care.

Caregivers

What are some responsibilities that I may have as a caregiver for someone going through gene therapy?

As a caregiver, you may be responsible for financial costs, care coordination (appointments, transportation, lodging), emotional support, physical care, food, maintaining the home environment, and serving as a communication link.

How can I best support my loved one with sickle cell disease?

As a caregiver, it is important that you stay educated on the disease and updated technologies associated with sickle cell disease treatment and therapies. Creating a network of care for your loved one with sickle cell disease so that they can make informed decisions regarding their physical and mental health will also significantly help their quality of life. Visit our [gene therapy education](#) page to stay updated on the newest therapies.

What kind of resources are out there for caregivers?

It is essential for caregivers to take adequate care of themselves while caring for a loved one. In the caregiver community, mental health issues like burnout and depression, as well as physical health issues, may arise. Visit our [resource page](#) for additional information on how to combat common issues in the caregiver community, such as mental health issues like burnout and depression, as well as physical health issues that may arise.

The Democratizing Education Project welcomes your feedback about the sickle cell disease gene therapy resources. Please email your comments or questions to DemocratizingEd@mail.nih.gov.

