

IS IT A CURE?

Gene therapy is a **potentially curative** therapy. This means that it could act as a cure, but it is too new to say for sure. It has been shown to result in a significant decline in pain episodes, but we need to learn more about long-term impacts and side effects. It is also not clear whether this is a “one-and-done” treatment. The FDA currently recommends 15 years of patient follow up after treatment.

HOW DO I LEARN MORE ABOUT GENE THERAPY?

Additional information and resources are available at bit.ly/gene-therapy-FAQs and bit.ly/nhgri_GTed.

WHERE CAN I GET GENE THERAPY?

Only a few centers across the USA offer gene therapy for sickle cell disease. Sites must have both medical expertise and willingness to accept a complex cost structure.



SICKLE CELL DISEASE ASSOCIATION OF AMERICA, INC.

GENE THERAPY FOR SICKLE CELL DISEASE

WHAT YOU NEED TO KNOW



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GENE THERAPY FOR SICKLE CELL DISEASE

In December 2023, two gene therapies (exagamglogene autotemcel and lovetibeglogene autotemcel) were approved by the Food and Drug Administration (FDA) to treat sickle cell disease. Learn more about these new treatment options and what they mean for the sickle cell community.



STEP ONE

Doctors collect stem cells from your body. The blood-forming stem cells make your red blood cells and other blood cells. The DNA in these stem cells create sickled red blood cells. Collecting enough stem cells often needs several sessions.



STEP TWO

The stem cells are taken to a lab. With exagamglogene autotemcel, technicians edit the cells. Lovotibeglogene autotemcel uses a technique called gene addition. After either gene therapy, your stem cells will make red blood cells that don't sickle in your body.



STEP THREE

While your stem cells are having gene therapy in the lab, you will receive chemotherapy to remove the original cells from your bone marrow.



STEP FOUR

Your stem cells that had gene therapy are injected back into your body. While you wait for these stem cells to grow and generate new blood cells, you will need medical support. Plan on at least a month in the hospital and then frequent clinic visits for several months.



STEP FIVE

Follow up visits will be with the gene therapy center for a while, then likely with a sickle cell center for 15 years.

FREQUENTLY ASKED QUESTIONS

Am I eligible?

Gene therapy is approved for people ages 12 and up. Sickle cell disease SS and S-beta-zero-thalassemia are eligible. The FDA indicates that sickle cell disease SC is not included.

Is it safe?

For many people, the benefits of this new treatment outweigh the risks.

What questions should I ask my doctor?

- How long will this take?
- What is the time commitment?
- Where is the nearest treatment center?
- What are my other options?

How much will it cost?

Gene therapy is expensive, and FDA-approved high-cost medications can come with barriers. Initiatives like the Centers for Medicare and Medicaid Services' Cell and Gene Therapy Access Model may help reduce costs for individuals on Medicaid.

Learn more: bit.ly/CMMS_CGT