



Understanding gene therapy approaches

It is important to understand the components of gene therapy, how these components differ and other treatment options for sickle cell disease, such as bone marrow transplant.

Each of these treatment approaches aim to modify the severity of the disease. [Learn more](#) about your red blood cells to help you better understand these various approaches.

Types of treatments

A variety of approaches target the blood stem cells to reduce your symptoms of sickle cell disease. Gene therapies use your own stem cells, while bone marrow transplants use the stem cells of a matched donor.

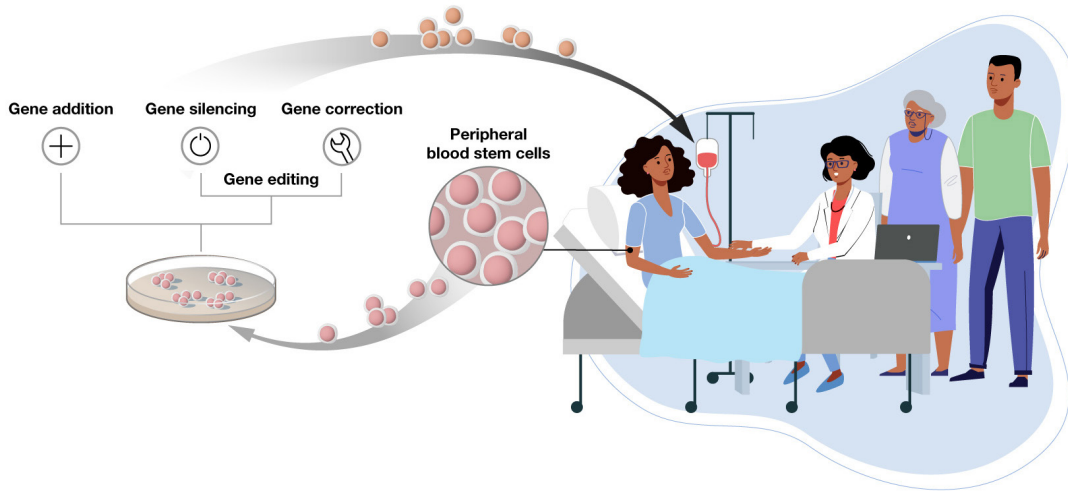
Gene therapy

In **gene therapy**, your stem cells are changed by altering part of your genes. Types of gene therapy include **gene addition** and gene editing. There are two types of gene editing: **gene silencing** and **gene correction**.

While each of these approaches introduces different types of gene-based changes, they start out the same: Your stem cells are collected and taken to a lab for modification.

After modifications are made in a lab, your stem cells are returned to your body. These approaches aim to decrease the amount of hemoglobin S in your red blood cells. If they successfully decrease the hemoglobin S, your cells will not be fragile or even sickle. As a result, these approaches can decrease the complications, treat or prevent symptoms of sickle cell disease. However, this type of gene therapy will neither reverse some complications (such as avascular necrosis) if they are present at the time of the gene therapy nor alter the genes that you pass on to your children.

Gene therapy

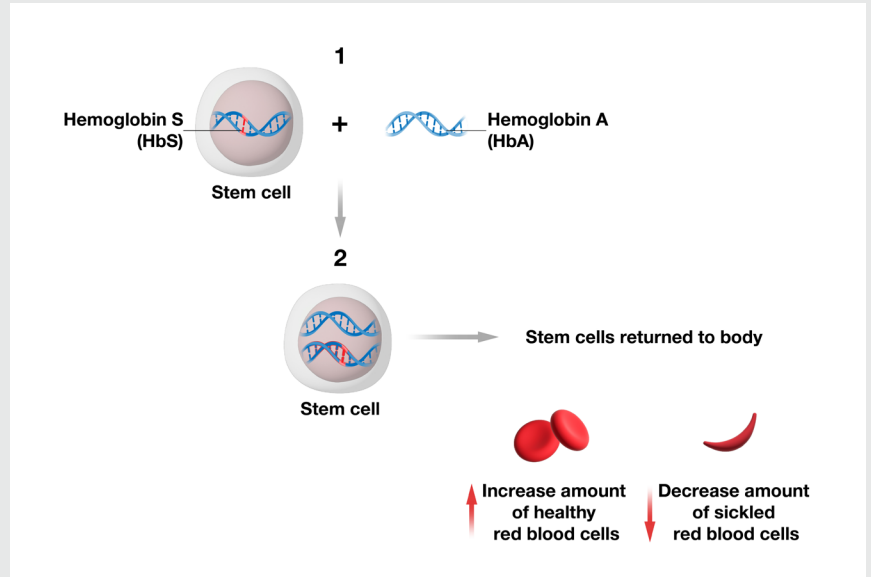


Different gene therapy approaches used in treatment

Gene addition

Patient stem cells are collected and taken to a lab for modification.

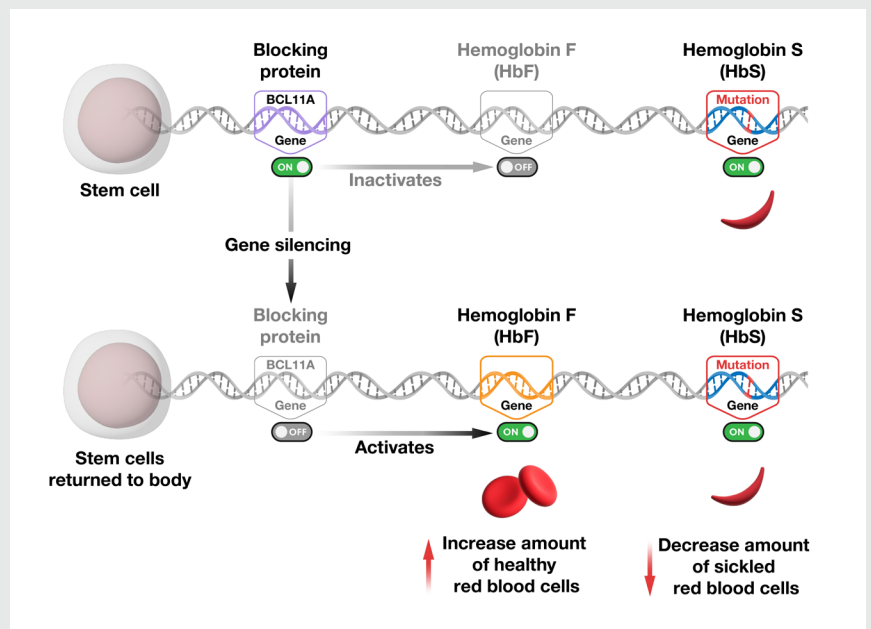
An extra copy of a hemoglobin A gene (without the variant) is added to the stem cell, which allows your cells to produce hemoglobin A (non-sickling hemoglobin).



Gene silencing

Patient stem cells are collected and taken to a lab for modification.

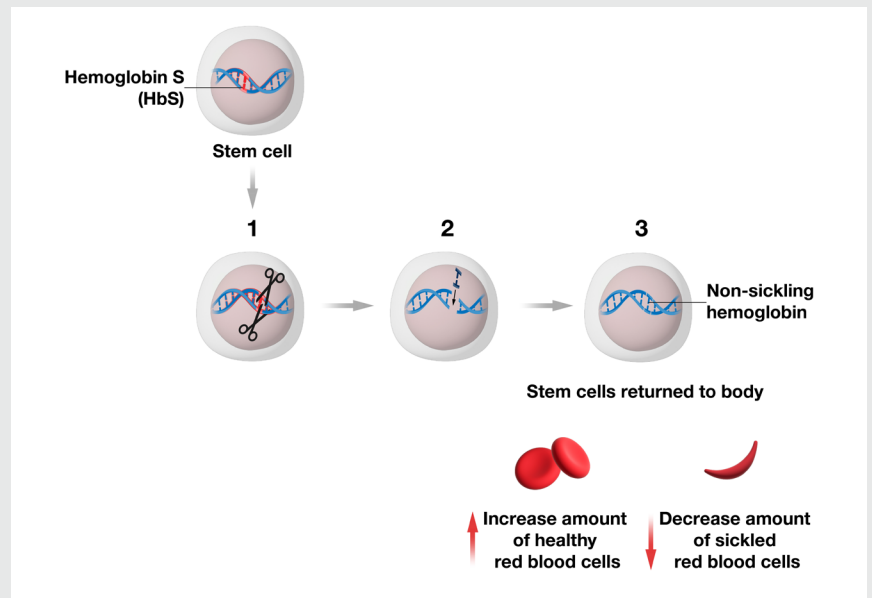
The gene that produces the BCL11A-blocking protein, which inactivates hemoglobin F, is silenced. By silencing this gene, the gene that makes hemoglobin F can be activated, which allows your cells to produce hemoglobin F (non-sickling).



Gene correction

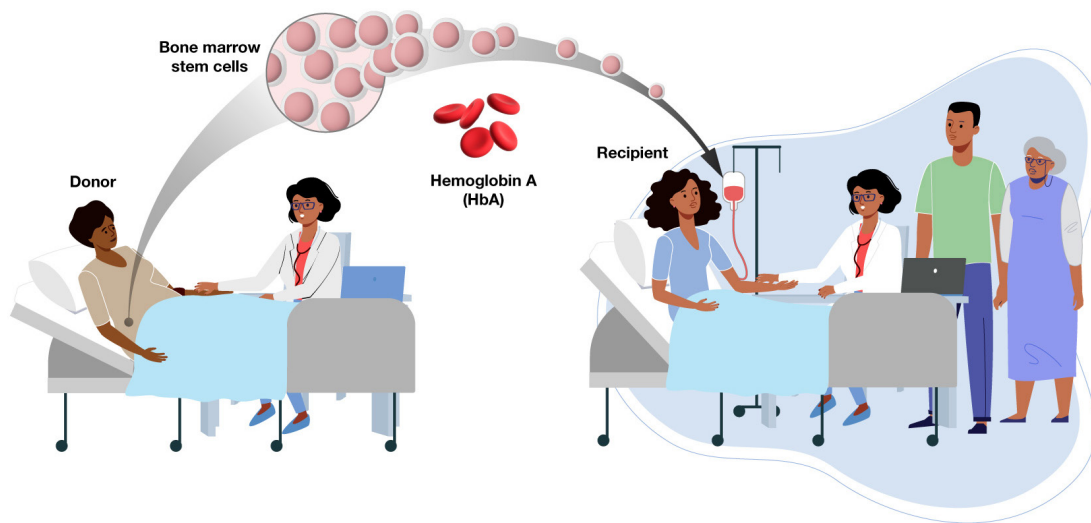
Patient stem cells are collected and taken to a lab for modification.

The variant in the gene that causes sickle cell disease is corrected so that it codes for a non-sickling hemoglobin.



Bone marrow transplant

In a **bone marrow transplant**, also called a stem cell transplant, your stem cells are replaced with new stem cells that have genes that do not code for sickle cell disease. These new stem cells are usually from another person (an allo-transplant). Ideally, this donor should be a full match (allogeneic transplant) to the recipient, however, in sickle cell disease, it can be hard to find someone who is a full match. Today, donors who are only half-matched (haploidentical transplant) are able to donate their stem cells to use in a bone marrow transplant.



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.

