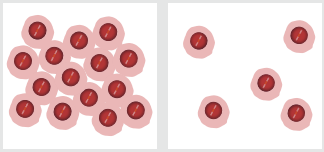
BETA THALASSEMIA, AN INHERITED BLOOD DISORDER

Beta thalassemia is a rare inherited blood disorder caused by a defect in the adult type of hemoglobin, the oxygen-carrying part of the blood. It results in decreased production of healthy red blood cells.



Blood; Normal vs Decreased Production of RBCs

People with the most severe forms of beta thalassemia experience life-threatening anemia. Because they need frequent transfusions of blood, their condition is called transfusion-dependent beta thalassemia. Other symptoms include osteoporosis, which results in weak bones that fracture easily; and enlarged organs, such as the heart, liver, and spleen.

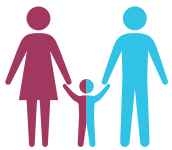
Current Treatment is Not Adequate

- Most people need frequent blood transfusions and medical checkups every few weeks.
- Iron overload from frequent blood transfusions can cause organ damage to the liver, heart, and endocrine system (the glands that secrete hormones into the blood). While iron chelation can help to reduce this overload, it is not fully effective.
- Blood stem cell transplant, also called bone marrow transplant from a related, or unrelated, healthy donor is the current standard of care. However, it is often difficult to find a good donor match. Plus, donor transplants carry such risks as graft-vs-host disease, where the donor cells attack the recipient's body.
- Even with treatment, many patients have a shortened life expectancy.

Who is Affected

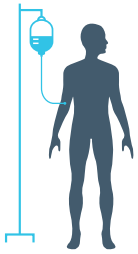
The World Health Organization estimates that 25.000 children are born with TDT (transfusion-dependent beta thalassemia) each year.

It primarily affects people of Mediterranean, Middle Eastern, African, and Southeast Asian descent.



Beta thalassemia has an autosomal recessive inheritance pattern, meaning a child must inherit the mutation from each parent to be affected.

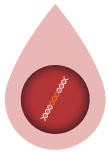
ST-400-01 CLINICAL STUDY

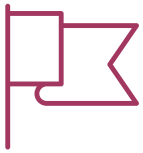


ST-400-01 is a **Phase 1/2** single-arm clinical trial of a one-time infusion of a person's own genetically edited CD34+ blood stem cells, called ST-400.

All study participants will receive the ST-400 treatment.

Researchers want to see if increasing the production of fetal hemoglobin can produce enough oxygen-carrying red blood cells to **reduce or eliminate the need for blood transfusions, and improve health.**





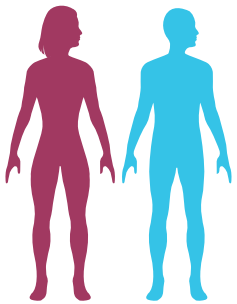
The goal of this clinical study is to test the safety, tolerability, and efficacy of ST-400.

If effective, this trial could reduce the time people living with beta thalassemia spend dealing with the medical system.

Talk to your doctor about potential risks and benefits of participating in this study.

You May be Eligible if You:

- Are 18-40 years old
- Have transfusion-dependent beta thalassemia
- Have been dependent upon red blood cell transfusions over the past two years
- Are in good general health



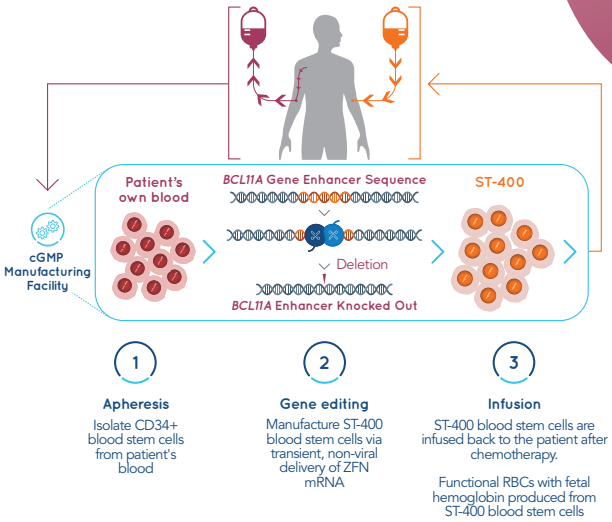
The study will continue to monitor the 6 people in the trial.

CELL THERAPY FOR BETA THALASSEMIA USING GENOME EDITING

Cell therapy using genome editing is being studied to find out if it is safe, tolerable, and effective in treating people with transfusion-dependent beta thalassemia. Instead of using donor blood stem cells, as in the current treatment standard in bone marrow transplantation, the use of a patient's own cells could lower or eliminate such complications as graft failure and graft-vs-host disease.

If effective, the therapy will reduce the need of frequent blood transfusions.

ST-400 Treatment:



ST-400 Treatment

1. A person's own CD34+ blood stem cells are removed.
2. These blood stem cells are then genetically edited in the lab and infused back into the person's body.
3. The goal is to increase production of fetal hemoglobin, to improve the oxygen-carrying capacity of red blood cells, and to decrease dependence on blood transfusions.
4. Unlike other studies, ST-400-01 edits DNA within cells without the use of a virus.

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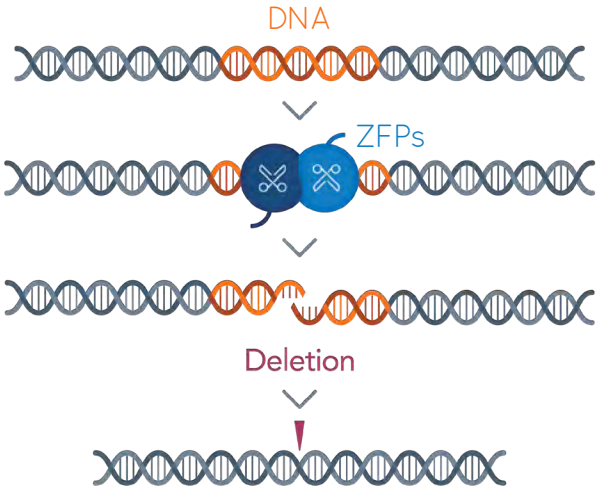
USING ZINC FINGER PROTEINS IN GENOME EDITING



Zinc finger proteins (ZFPs) are naturally found throughout the body and can be specifically engineered for genome editing. When combined with a nuclease, a cutting tool, zinc finger nucleases (ZFNs) can be used therapeutically. In this study, ZFNs are being used to modify the person's blood stem cells by altering a regulatory DNA sequence in the genome to boost production of fetal hemoglobin, giving the body more of the oxygen-carrying red blood cells it needs.

Sangamo™ THERAPEUTICS

Thales

An Investigational Study for the Treatment of Beta Thalassemia

PIONEERING GENETIC CURES™

For Sangamo Therapeutics, science is a means to develop new medicines with the potential to transform the lives of patients living with serious genetic diseases. Over two decades, our scientists have advanced the most precise, efficient and specific genome editing technologies for therapeutic applications. In that time, we have also acquired technical capabilities and expertise across multiple genomic technology platforms which allow us to develop and deliver the best possible medicines to meet the needs of the patients we are trying to serve.

TAKE THE LEAD

Ask your doctor if you qualify for a cell therapy study for people living with beta thalassemia

Contact Us

To learn more about Sangamo Therapeutics, visit our website at www.sangamo.com

clinicaltrials@sangamo.com
thalesclinicalstudy.com