What is a clinical research study?

Clinical research studies help scientists and doctors explore whether a medical strategy, therapy, or device is safe and effective for humans. Before a new therapy or device can be prescribed for a specific condition in the United States, it must go through several rounds of clinical research and be approved by the U.S. Food and Drug Administration (FDA). Participation in clinical research studies is voluntary, and participants may stop at any time.

For more information or questions, contact the Restore study team.

RestoreStudySupport@kamautx.com

Restore

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Are you living with severe Sickle Cell Disease (SCD)?

The Restore study is researching a possible new gene therapy for people with severe SCD.

Learn more today.

Restore

What is the Restore study?

The Restore study is researching a possible new gene therapy for people with severe SCD. Currently, a common treatment option for SCD is a bone marrow transplant from a donor. However, the donor needs to be very closely matched, like a sibling, and many people do not have a match for this kind of procedure. When a well-matched donor is unavailable, the risks of performing a bone marrow transplant can be very high.

If you have severe SCD (see eligibility criteria below) and do not have a well-matched bone marrow donor, the Restore study may be an option for you. This study is researching a possible way to help people with SCD using their own cells. This means that participants do not need a bone marrow donor and may have fewer risks than performing a transplant with a bone marrow donor that is not well-matched.

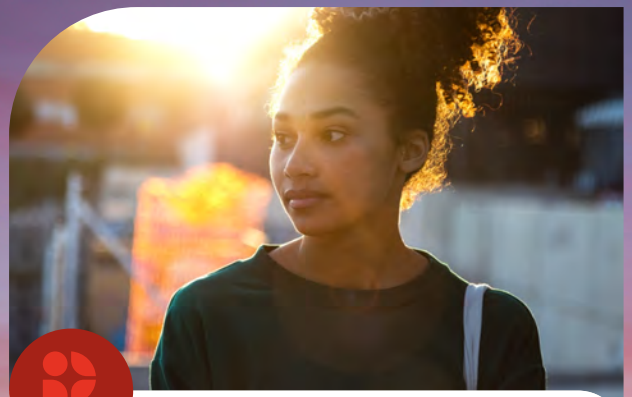
WHO CAN JOIN THE STUDY?

You may be eligible to join the study if you meet the following requirements:

- Are 12-40 years old
- Have a diagnosis of SCD, hemoglobin SS
- In the last 2 years, have sought treatment at a medical facility for either:
  - + 4 episodes of vaso-occlusive crisis (VOC), also known as pain crises
  - + 2 episodes of acute chest syndrome (ACS)

  Note: If you have been on chronic transfusions and experienced severe SCD symptoms prior to starting a transfusion regimen, you may be eligible to participate
- Do not have an available matched sibling bone marrow donor
- Have never had a bone marrow transplant or gene therapy

Note: Other requirements apply.



What is the study drug product?

The study drug product is called nula-cel, and it is a type of gene therapy called gene correction which is performed on stem cells. It is different than a standard bone marrow transplant, because your own stem cells are removed from your blood and sent to a lab for the gene correction procedure, so you do not need a donor's stem cells.

In the lab, the faulty gene that causes SCD is taken out and replaced with a copy of the gene that creates normal red blood cells. The corrected cells are frozen until you are ready to have them infused back into your body. Once returned to you, the study drug product is designed to make normal red blood cells.

PATIENT/CAREGIVER SUPPORT INCLUDES:

- Fertility Preservation
- Travel and Accommodation Assistance

Contact study team to learn about additional support provided:

RestoreStudySupport@kamautx.com

What can study participants expect?

The Restore study will be conducted in 6 parts:

1. **Screening (up to 90 days) –** The study team will perform tests to confirm you qualify for the study and it is safe for you to participate. These include heart, liver, and lung tests; bone marrow tests; and blood tests.
2. **Cell collection preparation (3-4 months) –** You will receive regular blood transfusions for at least 12 weeks. This is to help get your body ready for the cell collection procedure.
3. **Stem cell collection (1 week) –** You will be given a drug called plerixafor to help your stem cells leave your bone marrow and enter your bloodstream. Your stem cells will then be collected from your blood using a machine in a process called apheresis. You will be in the hospital for this procedure and you may have multiple days of stem cell collection. Depending on how many cells are collected, you may need to come back to repeat this step up to 3 times.
4. **Conditioning & Infusion (1 week) –** Once the study drug product is made, you will be checked back into the hospital to make sure you are still healthy and to receive chemotherapy over 4 days. After the chemotherapy, you will rest for at least 2 days before receiving the study drug product infusion.
5. **Post-Infusion (4-6 weeks) -** You will stay in the hospital for 4-6 weeks, so the study team can monitor your health after study drug product infusion and make sure the corrected cells go back into your bone marrow. While recovery is expected to take no more than 4-6 weeks, you may be required to stay longer than 6 weeks to monitor your health if needed.
6. **Long-term Follow-up (up to 15 years) -** After you leave the hospital, you will return to have additional follow-up study visits over 24 months. After that, you will be invited to participate in a long-term study of 13 years. This will give the study team more information about the longterm safety of the study drug product.