



RESEARCH STUDY INFORMATION

City of Hope, located in Duarte near Los Angeles, California, is seeking adult volunteers with sickle cell disease to take part in a clinical trial (research study) on blood stem cells, which are the cells responsible for the formation of all blood cells in your body.

WHY IS THIS STUDY BEING DONE?

In the future, patients' blood stem cells could be genetically corrected in a lab and given back as a treatment for sickle cell disease. For this purpose, doctors need to find a way to collect enough of these cells in the blood of patients. Researchers need your help to determine if up to two injections of a drug called plerixafor can be used to increase the number of stem cells in the blood of sickle cell patients.

WHAT WILL HAPPEN DURING THE STUDY?

- You will have up to eight visits at City of Hope, for which you will not be hospitalized, but you will have to stay nearby for four to five nights.
- If you are taking hydroxyurea, you will have to stop for approximately 10 days.
- Prior to the treatment, you will have an exchange blood transfusion to decrease the number of sickled blood cells.
- You will receive one or two injections of plerixafor underthe skin.
- You will have a few blood draws over two to three days.
- When done, you will be checked by phone to make sure you are doing well.

WHY SHOULD YOU PARTICIPATE?

Cell Mobilization With Plerixafor as Part of a Gene Therapy Strategy in Sickle Cell Disease

In this study, you will not receive genetically corrected blood cells, but you will help doctors find a way to develop gene therapy and contribute to medical knowledge. You may remain eligible for future gene therapy trials.

WHO CAN PARTICIPATE?

To qualify, you must:

(PISMO Study: Plerixafor-Induced Stem Cell Mobilization)

- Be between the ages of 18 and 40
- Not have serious heart, kidney, or liver diseaseor infections
- · Have severe sickle cell disease, defined as at least two vaso-occlusive pain crises, OR one stroke, OR one episode of acute chest syndrome OR regular transfusion therapy in the past two years

COMPENSATION

You will be compensated for your participation and studyrelated expenses (such as travel, food and lodging).

YOU WANT TO LEARN MORE. WHAT'S NEXT?

If you think you might qualify, or want to learn more about this trial, please contact Lisa Pullens, R.N., at 626-218-1231 or lpullens@coh.org.

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