Blood Stem Cell Transplant from Half-matched Family Member to Cure Sickle Cell Disease in Children and Young Adults

COH 17136: A Pilot Study of Pre-Transplant Immunosuppressive Therapy for Haploidentical Transplants in Patients with Sickle Cell Disease.

RESEARCH STUDY INFORMATION

City of Hope, a medical center located in Duarte, California, is seeking children and young adults with sickle cell disease to participate in a clinical trial (research study) on blood stem cell transplants from a half-matched family donor.

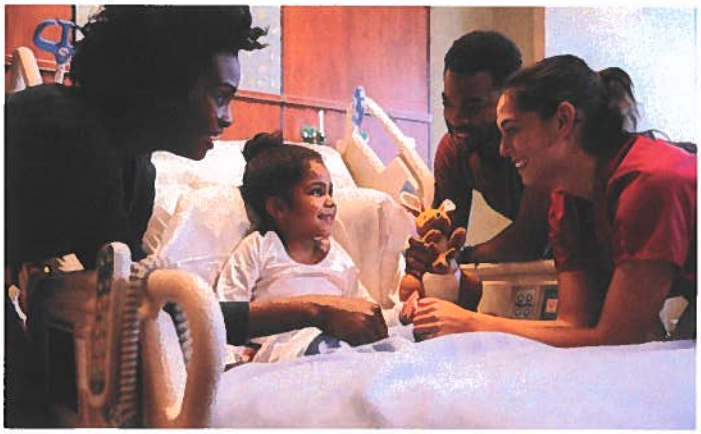
WHY IS THIS STUDY DONE?

Many patients with sickle cell anemia don't have a fully-matched donor to give his or her blood stem cells for a transplant. We have designed a transplant study that uses blood stem cells from half-matched family members, making it easier to find a donor. The combination of medications used during the transplant could prevent transplant failure, result in fewer complications and offer a potential cure for sickle cell disease.

WHO CAN PARTICIPATE?

Patients who:

- Have complications related to sickle cell disease and could benefit from blood stem cell transplant
- Are between 1-30 years of age
- Do not have a fully matched donor
- Have a half-matched family member



WHAT IS INVOLVED?

Patients who participate will receive five days of immune system-suppressing medication 70 and 40 days before their blood stem cell transplant. Patients will then be treated with drugs that destroy his or her bone marrow cells so that healthy donated stem cells can be introduced by injecting them into the patient's veins. The donors' bone marrow will then start to replace the patients' bone marrow, restoring the immune system and creating normal red blood cells.

During recovery, doctors watch closely for any signs of complications.

The patients will be monitored for two years to assess the success of the transplant.

CONTACT INFORMATION

If you think you might qualify or want to learn more about this clinical trial, please contact **Anna Pawlowska, M.D.**, at **626-218-8442** or email **apawlowska@coh.org**.

For more details about the study, visit **ClinicalTrials.gov** and enter "NCT03279094" in the 'other terms' field.

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