More Information....

How long is the study?

The average hospitalization is 6-8 weeks. After leaving the hospital, you/your child will be followed in the outpatient clinic for two years after the transplant. Follow-up with the transplant doctor is very important.

What are the risks?

There are risks associated with the study medicine, Defibrotide, and with the transplant. Some of the risks are listed below, but there are others not described here. Side effects and risks of Defibrotide may include (but are not limited to):

- Bleeding, low blood pressure and coagulopathy (disturbance of blood clotting).

Side effects and risks of the transplant may include (but are not limited to):

- Skin rash, fever, vomiting, diarrhea, Infection, bleeding, mouth sores, nausea, hair loss, liver problems, trouble breathing
- Graft versus Host Disease (GVHD): a condition that occurs when donor cells attack the patient's cells because they are different. This condition can range from mild to severe and from temporary to chronic. GVHD can become life threatening.

What is the cost?

Your insurance company will be asked upfront to pay for standard transplant costs including hospital stay, medications, doctor visits, and related donor costs. The study drug, Defibrotide, is being provided free of charge from Jazz Pharmaceuticals. There is no charge for research tests. You or your child will not be paid to be in this study.

Financial Sponsors

The research parts of this clinical protocol are supported in part by:

- **Jazz Pharmaceuticals**
- **Pediatric Cancer Research Foundation**

NYMC IRB Approval

11/16/17 TO 11/15/18

Joining a research study is an important decision. Please discuss with your doctor. Not everyone who is interested may be able to join, and there is no guarantee that transplantation will cure you/your child's sickle cell disease.

For more information talk with your doctor or contact:
Mitchell Cairo, MD
Phone: 914-594-2150
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Valhalla, NY 10595

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Haplo SCD Consortium

A Clinical Trial:

The Safety and Efficacy of Prophylactic Defibrotide Administration in Children, Adolescents, and Young Adults with Sickle Cell Disease Following Myeloablative Conditioning (MAC) and Haploidentical Stem Cell Transplantation Utilizing CD34 Enrichment and T-Cell (CD3) Addback (IND 127812)

For Patients and Families

The Haplo SCD Consortium

was developed to provide resources to families, patients, and physicians pursuing Familial Haploidentical (Parent-to-Child; Haplo) Transplantation for sickle cell disease.

We have developed a consortium of four clinical transplant sites and multiple core centers in a variety of laboratory, biostatistics, neuroimaging, quality of life, hepatic imaging and late effects/survivorship. We've also partnered with the Sickle Cell Disease Association of Illinois (SCDA) to help educate patients and their families regarding sickle cell disease and familial haploidentical stem cell transplant.

The Collaborating Centers have joined efforts on the protocol The Safety and Efficacy of Prophylactic Defibrotide Administration in Children, Adolescents, and Young Adults with Sickle Cell Disease Following Myeloablative Conditioning (MAC) and Haploidentical Stem Cell Transplantation Utilizing CD34 Enrichment and T-Cell (CD3) Addback (IND 127812)

Please talk with your doctor for more information about this research opportunity.

Tufts Medical Center | Medical College of Wisconsin | Washington University in St. Louis School of Medicine | UCLA | Harvard Medical School | BCM Baylor College of Medicine | Johns Hopkins School of Medicine

What is a Clinical Trial?

A Clinical Trial (study) is a carefully controlled way to study the effectiveness of new treatments or new combinations of therapies. You do not have to agree to participate.

What is this study about?

This study uses half-matched (Haplo) family members as bone marrow donors to try and cure sickle cell disease in children & in young adults up to age 35 years. A serious complication of using the chemotherapy for the bone marrow transplant is called veno-occlusive disease (VOD). VOD affects you/your childs liver and can be life-threatening. This study is being done to evaluate how safe and effective the study drug, Defibrotide, is at preventing VOD. The study is also testing to see whether one of the conditioning drugs (cyclophosphamide) can be lowered by 50% compared to the previous study.

Who can join?

To join the study, you or your child must:

- Have Sickle Cell disease, Sickle Cell/Beta Thalassemia or Hemoglobin SC Disease
- Be at least 2 years old and under 21 years old
- Patients 2 years to under 21 years old must have at least 1 or more disease complications:
  - Neurologic event or deficit (stroke)
  - Severe chest syndrome more than once
  - Multiple severe pain problems
  - Changes in blood flow in the brain

  OR
- Be at least 21 years to under 35 years old and have at least 2 or more disease complications:
  - White blood cell count (WBC) greater than 13,500
  - Heart function problems
  - Require monthly transfusions
  - History of a severe life threatening infection

Study Details

Patient

Two months before transplant, you/your child will start taking additional medications to prepare for transplant. About 15 days before the transplant, the you/your child will begin a combination of medications, chemotherapies, and radiation (one day) that will suppress the immune system and prepare the bone marrow for the new donor cells. Starting 10 days before the transplant you/your child will begin taking the study medication, Defibrotide, until approximately 30 days after the transplant.

Due to the decreased immune system, there is a risk for infection and you/your child will need to remain in the hospital during this time. On the day of transplant, the new donor cells will be given to you/your child much like a blood transfusion.
After the transplant, you/your child will have to take additional medications to try and prevent problems or complications from the transplant.

Donor

All potential donors will have testing done to determine if they are healthy enough to be a donor. The donor will receive injections of a growth factor that stimulates bone marrow cells to circulate in the blood. The circulating blood cells will be separated and collected using an apheresis machine, which draws blood from a donor's vein and separates it by specific cells. You will have a needle placed in your arm during the collection process. There is no surgery required to donate. Your bone marrow cells will be collected and stored frozen so that they can be given to the patient at a later date.

Additional tests will be done to determine if you/ your child can be in the study.

NYMC IRB Approval

11/16/17 TO 11/15/18