A Cure!

Bone marrow transplant can cure sickle cell disease (SCD).

Here's how it works

Blood is made of three main types of cells, each with different jobs:

- White blood cells fight infection
- Platelets help clot blood
- Red blood cells carry oxygen to the body

In sickle cell disease, the red blood cells are sickle-shaped instead of the normal circular shape. This can stop the red blood cells from moving well through your small blood vessels and harm your body throughout your entire life.

All blood cells are made in the bone marrow. Bone marrow transplant cures a patient with sickle cell disease by replacing their bone marrow, which produces sickle-shaped red blood cells, with bone marrow from a donor that produces healthy circular-shaped red blood cells.

Children's of Alabama

Committed to a Cure

Alabama Center for Childhood Cancer and Blood Disorders

UAB MEDICINE PEDIATRICS

SICKLE CELL DISEASE BONE MARROW TRANSPLANT

Bone Marrow Transplant: A Cure for Sickle Cell Disease

For more information, visit www.ChildrensAL.org/sickle-cell-disease

Things to Consider About Bone Marrow Transplant

Potential Benefits of Bone Marrow Transplant

- **Cure**
  The problems from sickle cell disease such as acute chest syndrome and new pain crises will go away after a successful bone marrow transplant.
- **Improved Quality of Life**
  Patients will have more energy and feel better.
- **Stopping Further Organ Damage**
  The organ damage from sickle cell disease such as stroke, kidney disease and lung injury will lessen.

Risks of Bone Marrow Transplant

Early After Transplant

- Nausea and vomiting
- Hair loss (usually temporary)
- Mouth sores
- Need for transfusion of blood and platelets
- Increased risk of infection
- Infinfertility (there are options to preserve fertility)
- Problems with organs such as heart, liver, lungs or kidneys

Later After Transplant

- Graft failure
  This happens when the donor bone marrow does not grow in your child's body. It is rare. Depending on the strength of the medications used in the preparative regimen, graft failure can result in reoccurrence of sickle cell disease or the need for a second transplant.
- Graft versus host disease (GVHD)
  This occurs when the donor's white blood cells (infection-fighting cells) attack your child's body. This can cause rash, diarrhea, liver problems, joint and muscle problems, dry eyes and many other possible effects. Most cases of GVHD are mild and eventually go away, but some cases are severe and can be life-threatening.

The Bone Marrow Transplant Process

Before the Bone Marrow Transplant

- We need to find your child a bone marrow donor. A blood test called HLA typing is done to help match your child with a possible donor. Possible donors include:
  Matched Sibling Each full sibling has a 25 % chance of being your child's HLA match. Survival after bone marrow transplant with a matched sibling is excellent at about 95 %.
  Matched Unrelated Donor An HLA matched donor that is not a family member can be found by searching a bone marrow donor registry such as the National Marrow Donor Program (NMDP).
  Haploidentical Donor A family member, such as a parent or sibling, who is not a match, can be a partial HLA match. Research studies are beginning to use these donors for patients with sickle cell disease.
- Your child will undergo a pre-transplant evaluation to make sure your child is a good candidate for bone marrow transplant. This evaluation includes CT scans, blood work and studies of the kidneys and lungs.

Hospital Admission and Preparative Regimen

- The preparative regimen is a combination of drugs that prepares your child to receive the donor's bone marrow. Your child will be admitted to the hospital during the preparative regimen. Medicines are given to weaken your child's immune system to create space for the donor's healthy bone marrow. A special IV called a central line will be placed to allow these medications to be given and for blood to be drawn.
- The medicines in the preparative regimen will be given for 7 to 21 days prior to your bone marrow transplant.
- The medicines in the preparative regimen will make your child's white blood cell count very low and cause your child to be at risk for infections.

Transplant Day

- The donor's bone marrow is given to your child through a central line. This looks just like a blood transfusion.
- This can take anywhere from a few minutes to several hours.
- Your child is awake and in his/her own hospital room during the bone marrow transplant.
- The donor bone marrow moves from the blood vessels directly into your child's bone marrow space and begins to grow.

After the Transplant

- Your child may require blood and platelet transfusions while waiting for the new bone marrow to grow.
- Your child's blood counts begin to improve 12-28 days after the transplant.
- Your child recovers from any side effects from the preparative medications.
- If your child has no major complications, a typical hospital stay is 5-6 weeks.

After Hospital Discharge

- Your child will still need to take many medications after the transplant and be seen in Bone Marrow Transplant (BMT) clinic 1-3 times weekly.
- Your child will need to stay in the Birmingham area until cleared by the transplant physician (usually about 3 months after transplant).

Before, During and After Transplant

A Cure!

Is bone marrow transplant
an option for your child?

Who should consider bone marrow transplant for sickle cell disease?

- Patients who have matched sibling donors and have problems from sickle cell disease.
- Patients with severe sickle cell disease (prior stroke, acute chest syndrome admission or multiple pain crises each year).
- Patients who have an unrelated donor or a haploidentical donor.
- Patients who are eligible for a clinical trial.

Whom should I contact to explore this option for my child?

- Call the Pre-Transplant Coordinator, Pediatric Blood and Marrow Transplantation Program, Children's of Alabama, at 205.638.5435.

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