University Hospitals
Seidman Cancer Center

Rainbow
Babies & Children's

PATIENT INFORMATION: Bone Marrow (Stem Cell) Transplantation

What is a bone marrow transplantation and how is it used for sickle cell disease?

Bone marrow transplants have been used for the treatment and cure of a variety of cancers, immune system diseases and blood diseases for many years. Doctors in the United States and other countries have developed studies to treat children who have sickle cell disease with bone marrow transplants.

- In a person with sickle cell disease, the bone marrow produces red blood cells that contain sickle hemoglobin (hemoglobin S). This leads to the complications of sickle cell disease.
- To prepare for a bone marrow transplant, strong medicines, called chemotherapy, are used to weaken or destroy the patient's own bone marrow, stem cells and immune system. This is done so the patient does not reject the new marrow or stem cells coming from the donor.
- The patient's bone marrow is replaced with blood-forming stem cells from a donor who does not have sickle cell disease. This can be a donor with normal hemoglobin or sickle cell trait. The transplant is given like a blood transfusion through an IV tube.
- The new bone marrow then produces healthy red blood cells, blood that does not have sickle hemoglobin.

Who can be a bone marrow donor?

There are three main types of bone marrow or stem cell donors:

1. **Matched related** – a brother or a sister who has the same bone marrow type and the same mother and father. Brothers and sisters are matched through special blood tests called HLA typing.
2. **Matched unrelated** – volunteers who have the same bone marrow type as the patient. These donors are found through national organizations that match donors and patients who have the same type of bone marrow.
3. **Haploidentical** – "half" matched family members (usually a mother or father). This type of donation is still considered experimental and should only be performed as part of a research study.

Stem cells can be obtained from the donor's bone marrow or peripheral blood (blood in the veins). In some cases, stem cells are collected from the umbilical cord at the time of birth.

What is required for a transplant?

Two major requirements must be met for a transplant to proceed:

1. The first is to identify the person who is the best donor. Blood tests will determine if the donor is a good match for the patient.
2. After the best donor is chosen, both the donor and the patient will have pretransplant testing of the heart, lungs, kidney, etc. Meetings with a psychologist and a social worker are also an important part of the process.

What are the benefits?

Bone marrow transplantation is the only treatment available today that can cure sickle cell disease.

What are the risks?

- **Infection**: Chemotherapy lowers the white blood cells which normally fight and prevent infections. This puts a patient at high risk for infections. Medicines are given to help prevent infections but infections can be very serious and, if a patient does not respond to treatment, infection can lead to death in 5 – 10 percent of patients.
- **Graft-versus-host disease (GVHD)**: This problem occurs when the immune cells of the donor (graft cells) sense that the cells of the patient (host cells) are different and attack them. This can be a serious side effect of transplant. GVHD occurs in up to 10 percent of patients who undergo matched-related transplants. It can be higher in transplants from other donors. GVHD can cause damage to the skin, liver and intestinal tract of the patient. Drugs are given to help prevent GVHD. These drugs can increase the risk of infection. GVHD that does not respond to treatment can lead to organ damage or death.
- Other risks can include nutrition problems, low blood counts, social and emotional concerns, and infertility.

Rainbow Sickle Cell Anemia Center

If your child has signs or symptoms of illness, please call 216-844-3345 and follow the prompts.

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