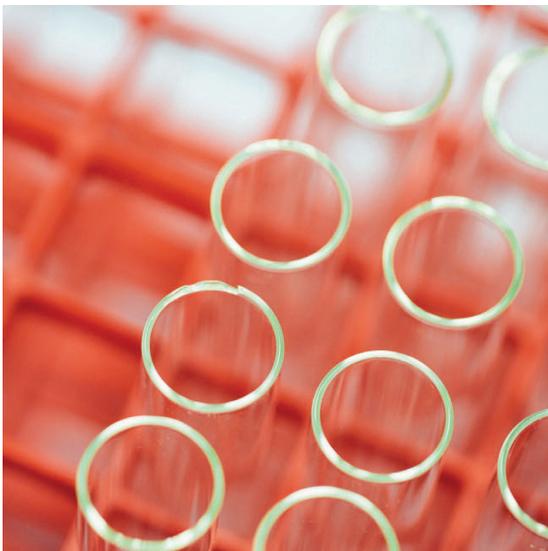


## What is BMT?

A bone marrow transplant (BMT) is the only known cure for sickle cell disease. The transplant offers children the chance to live a relatively normal life and the possibility of living longer.

## What is Bone Marrow?

Bone marrow is the center space inside the bone where blood cells are made. The outside of the bone is hard, providing strength and support. Most of the bone marrow is found inside the hip bones, breastbone, and back bones. Bone marrow is the spongy inside of the bones, and is a red liquid when drawn out. The bone marrow produces the most important type of blood cell, a stem cell. Stem cells are young, immature cells, that grow to become red blood cells, white blood cells, or platelets. As they grow, they move into the blood stream where they travel through the body to perform their special functions.



**Children's National™**

111 Michigan Ave NW  
Washington, DC 20010-2970  
Phone: 202-476-5000  
Fax: 202-476-2976  
ChildrensNational.org



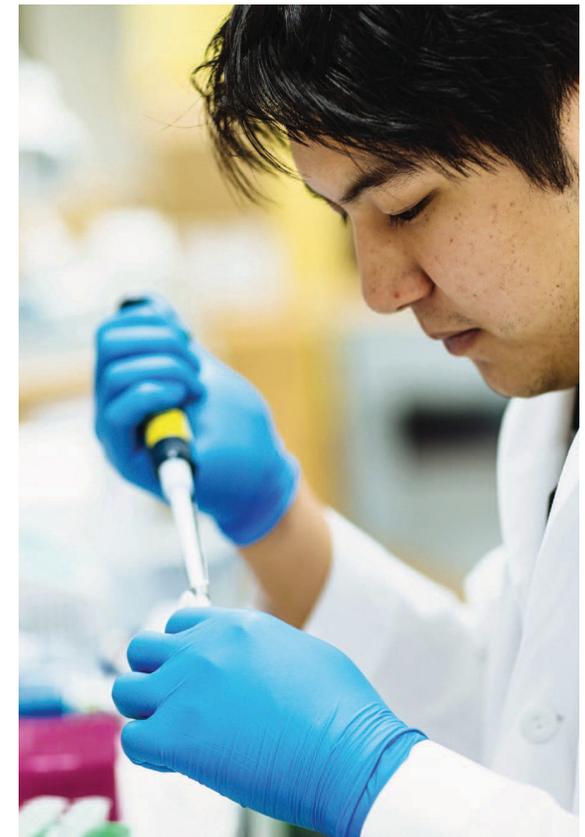
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### Sickle Cell Disease Program

## Bone Marrow Transplant for Sickle Cell Disease

### Graft-Versus-Host Disease

Children who have had an allogeneic blood or marrow transplant may or may not develop graft-versus-host disease (GVHD). GVHD is the response of the new marrow (graft) recognizing the new "home" (or host) as different. These symptoms may be mild, moderate, or severe. The greater the difference in matching between the donor and the recipient, the greater the risk of GVHD. The risk for GVHD increases for older patients and patients receiving unrelated donor transplants.





## How Does Transplant Work for Sickle Cell Disease?

Transplantation provides new bone marrow that makes healthy cells instead of sickle cells. These new, healthy red blood cells can live longer and no longer cause damage.

In order for the transplant to be successful, the whole blood system including the white bloods, platelets, and red blood cells, must be replaced.

## Is Bone Marrow Transplant a Surgical Procedure?

No, the bone marrow transplant is just like a blood transfusion.

## What is the BMT Process?

Think of your bone marrow as a house. The house is made up of white blood cells, red blood cells and platelets. Patients with sickle cell disease produce enough of these cells to fully occupy their house, even though their red blood cells are abnormal. In order to make room for the new donor cells in the house, the patient will receive chemotherapy. Enough cells are removed by chemotherapy to allow for the new bone marrow system to be transplanted. This process requires the patient to stay in the hospital for at least four to six weeks.

## What Happens in a BMT?

- Chemotherapy
- Transplantation
- Recovery



## What Are the Major Risks of This Procedure?

**Mortality:** In order to replace the whole blood system, the patient is at risk for infection and other complications. There is always the risk these complications could be so severe that they lead to death. The risk is small, but it is still a risk of which every family should be aware.

**Rejection:** The body's refusal to accept the transplanted marrow.

**Chemotherapy:** All chemotherapy agents have undesirable side effects and can be toxic to healthy cells and organs, as well as diseased cells. The most common effects during and soon after chemotherapy treatment include:

- Nausea, vomiting and diarrhea
- Hair loss
- Mouth sores
- Organ damage, specifically to the liver and/or kidneys

Other medications will be used simultaneously to lessen the severity of the expected side effects of nausea and vomiting, and to control the pain of mouth sores. Our doctors will be watching very carefully so that organ damage can be caught early. The patient may experience the following side effects long after the chemotherapy treatment:

- Short stature, infertility and thyroid disease
- Development of cancer
- Graft-versus-Host Disease (GVHD)