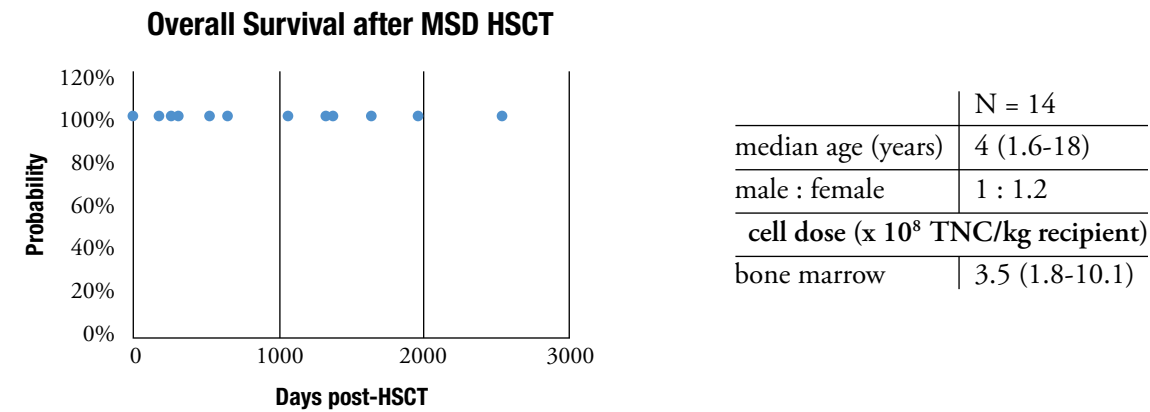


Curing Sickle Cell Disease with Bone Marrow Transplant

BMT from a matched sibling donor (MSD) or unrelated donor remains the best option of treatment for severe sickle cell disease. In the last three years, an increasing number of transplants have been performed at Nationwide Children's, and the results are promising.



HSCT = hematopoietic stem cell transplantation

- All children are doing well with no evidence of graft versus host disease.
- We have initiated new trials using unrelated donors for BMT in children who lack sibling donors and have initiated efforts to minimize the acute toxicity of the procedure.
- Event-free survival after MSD BMT is 93 percent.

Initial Consultation

An initial consult will be done by BMT physicians to determine if a child fits the criteria for BMT. During this process, a complete history of problems with sickle cell disease will be reviewed, medication compliance and response analyzed, and details of the procedure, including risks and benefits, explained to families.

BMT Resources for Families

The Division of Hematology/Oncology and Bone Marrow Transplant provides families with multiple resources during the extended stays required for the BMT procedure. Those services include:

1. Social services
2. Ronald McDonald House
3. Housing and transportation
4. Interpreters
5. Sibling care
6. Pastoral care

We will also place potential candidates in touch with families and children who have already gone through BMT to better understand the process from a patient's perspective.



Sickle Cell Disease and Bone Marrow Transplant



For an urgent consultation, call the Physician Direct Connect Line at (614) 355-0221 or (877) 355-0221. To make a referral, call (614) 722-3563 or visit NationwideChildrens.org/Hematology-Oncology-BMT.





Our Sickle Cell Program

The Comprehensive Sickle Cell and Thalassemia Program at Nationwide Children's Hospital currently cares for 370 patients with sickle cell disease, ranging from birth to age 21. Comprehensive clinics are located at the Nationwide Children's main campus and at the East Broad *Close To Home*SM Center. A separate Adolescent Sickle Cell Clinic is also located at the main campus. Our comprehensive, expert services include social work, psychology, educational testing, genetic counseling, newborn screening, pastoral care and apheresis.

Severity of Sickle Cell Disease

The symptoms of sickle cell disease are highly variable in affected patients. Some children with sickle cell disease feel good in general, but have occasional, painful episodes each year. Other children have severe disease with multiple complications.

Sickle cell disease is categorized as SEVERE when a person:

- Requires hospitalization three or more times per year for pain
- Has a history of acute chest syndrome
- Has neurologic complications (stroke—overt or silent; abnormal transcranial Doppler)
- Has pulmonary hypertension

The majority of these patients are on chronic transfusions for control of their symptoms.

Complications of Sickle Cell Disease

- Vaso-occlusive crisis/“pain crisis” – most common
- Acute chest syndrome - most life threatening
- Stroke – most disabling
- Infection
- Dactylitis
- Retinopathy
- Delayed growth and puberty
- Leg ulcers
- Gallstones
- Necrosis of joints
- Priapism
- Splenic sequestration
- Pulmonary hypertension
- Renal dysfunction

Sickle Cell Disease and Bone Marrow Transplant (BMT)

When should I refer a patient with Sickle Cell Disease for BMT?

Any child or young adult with severe sickle cell disease (genotype Hb SS or Sβ° thalassemia) who has one or more of the following clinical complications related to sickle cell disease is a candidate for BMT:

1. Previous clinically significant neurologic event (stroke) or any neurologic defect lasting >24 hours that is accompanied by an infarct or cerebral vasculopathy on cerebral MRI
2. Asymptomatic cerebrovascular disease, as evidenced by abnormal transcranial Doppler or MRI/MRA scanning (silent stroke), and the child is on chronic transfusion therapy
3. Minimum of two episodes of acute chest syndrome (≥ 2 in a lifetime), despite adequate supportive care measures (examples: asthma therapy, hydroxyurea)
4. History of severe pain episodes, defined as three or more severe pain events per year in the last two years (≥ 3 per year for two years), despite adequate supportive care measures
5. Recurrent priapism
6. Osteonecrosis of multiple joints
7. Evidence of pulmonary hypertension, as evidenced by tricuspid regurgitation jet velocity (TRV) > 2.5 m/s on echocardiogram
8. Red-cell alloimmunization (≥ 2 antibodies) during long-term transfusion therapy

What are the advantages of Bone Marrow Transplant (BMT)?

1. It is curative.
2. It prevents and stops organ damage associated with sickle cell disease.
3. It avoids the iron overload associated with chronic transfusions.
4. Studies show that it may also lead to a reversal of neurological damage and recovery of splenic function.
5. If done early, it may improve overall life expectancy.
6. It is actually more cost-effective than the standard of care.