

Transplant and Hurler Syndrome

Learning more about your child's treatment options can help you make informed medical decisions.

This fact sheet **tells you:**

- What Hurler syndrome is
- What the transplant steps are
- When to see a transplant doctor
- Questions to ask your doctor

What is Hurler syndrome?

Hurler syndrome is an inherited disease. It affects how the body breaks down food into energy. In Hurler syndrome, the body is missing an important protein to break down certain sugars in the body. When the sugars aren't broken down, they build up inside the body's cells and can damage the brain, heart and other organs.

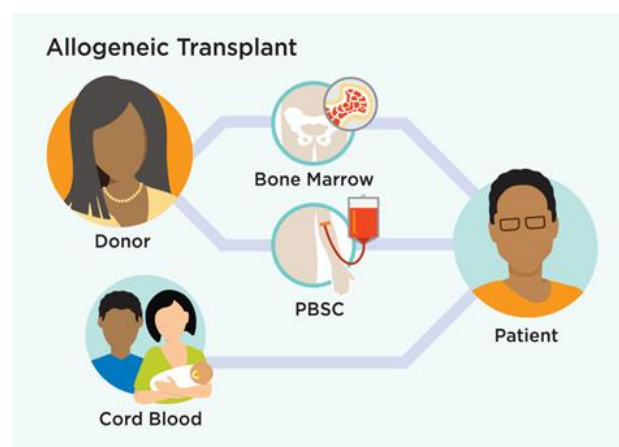
Sometimes parents and doctors see signs of the disease at birth. Other times, children will start showing signs when they're between 2 and 8 years old. Children with the disease may grow more slowly and have problems hearing, learning and moving.

What is a blood or marrow transplant (BMT)?

BMT, also called bone marrow transplant, replaces unhealthy blood-forming cells with healthy ones. The new cells have the right proteins so the body can break down sugars. Transplant prevents more damage to the body. But it can't fix any damage that has already happened.

The type of BMT that treats Hurler syndrome is called an **allogeneic transplant**. It uses healthy

blood-forming cells donated by someone else to replace the unhealthy ones. The healthy cells can come from a family member, an unrelated donor, or umbilical cord blood.



Allogeneic transplant steps

1. First, your doctor searches for your child's donor. This can take weeks to months.
2. Then, your child will get chemotherapy, and possibly radiation, to kill the unhealthy cells. This can take up to 2 weeks.
3. On transplant day, the donated cells are given to your child through an intravenous (IV) infusion. The new cells travel to the inside of their bones.
4. After a few weeks, the donated cells begin to make healthy blood cells.
5. Then, your child will need many months to recover. Your child will spend some of this time in or near the hospital.

Doctors, advanced practice providers, nurses, dietitians, and social workers will closely care for your child during the entire process to prevent and treat any side effects or

When to **see a transplant doctor**

Your child should see a transplant doctor **as soon as they're diagnosed**. Early treatment can help your child live longer and reduce serious symptoms.

Your first appointment with a transplant doctor

The transplant doctor will:

- Review your child's medical history
- Talk with you about treatment options and their risks and benefits
- Recommend the best time for your child to get a transplant and prepare for treatment
- Start searching for a donor

Questions to ask your doctor

- What are the chances transplant will stop the Hurler syndrome from causing more damage?

- What are the possible side effects of transplant? How can they be reduced?

- How might my child's quality of life change over time, with or without transplant?



AT EVERY STEP, WE'RE **HERE TO HELP**

Be The Match has a team dedicated to providing information and support to you before, during, and after transplant. You can contact our Patient Support Center to ask questions you may have about transplant, request professional or peer support, or receive free patient education materials.

CALL: **1 (888) 999-6743** | EMAIL: **patientinfo@nmdp.org** | WEB: **BeTheMatch.org/one-on-one**



Every individual's medical situation, transplant experience, and recovery is unique. You should always consult with your own transplant team or family doctor regarding your situation. This information is not intended to replace, and should not replace, a doctor's medical judgment or advice.