

Transplant and Metachromatic Leukodystrophy (MLD)

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

This fact sheet tells you:

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

What is MLD?

MLD is an inherited disease. It affects how the body breaks down food into energy. In MLD, the body is missing an important protein to break down certain fatty substances, or fats. When the fats aren't broken down, they build up and damage the brain, nervous system, bones and other tissues.

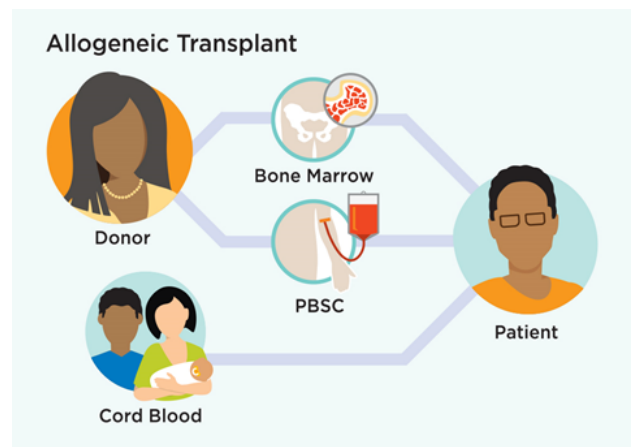
There are 3 types of MLD:

1. **Late-infantile MLD** – It typically affects infants between 6 and 24 months old. Children may have trouble moving, walking, talking and learning.
2. **Juvenile MLD** – It typically affects children between 4 and 12 years old. Symptoms are similar to late-infantile MLD.
3. **Adult MLD** – It can affect teens and adults of any age. The first symptoms are often changes in personality and difficulty at work or school.

What is a blood or marrow transplant (BMT)?

BMT, also called bone marrow transplant, replaces unhealthy blood-forming cells with healthy ones. With healthy blood-forming cells, the body is able to break down fat-based substances normally. A transplant will keep the brain, nervous system, bones and other tissues from more damage, but will not fix any damage that has already happened.

The type of BMT that treats MLD 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, 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 complications.

When to see a transplant doctor

You should see a transplant doctor as soon as possible after a diagnosis of MLD. BMT can stop the disease from progressing but it can't fix damage that has already taken place.

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 a donor search, if you and your child's doctor decide BMT is the best option
- Schedule appointments with other medical specialists

Questions to ask your doctor

What are the chances transplant will stop the MLD 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

NMDPSM 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](https://www.BetheMatch.org/one-on-one)

Resources for you

- Talk to someone who can help
 - Call: 1 (888) 999-6743
 - Email: patientinfo@nmdp.org
- Find a clinical trial for MLD
 - Call: 1 (888) 814-8610
 - Email: contact@ctsearchsupport.org
- Order free resources
 - Visit: [BeTheMatch.org/request](https://www.BetheMatch.org/request)