

Transplant and Adrenoleukodystrophy (ALD)

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

This fact sheet **tells you:**

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

What is **ALD**?

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

The 2 most common types of ALD are:

1. **Childhood cerebral form**

This is a severe type of ALD. It mostly affects boys between 4 and 10 years old. It may cause behavior changes, muscle cramps, difficulty walking, difficulty swallowing, hearing loss, or seizures.

2. **Adrenomyelopathy (AMN)**

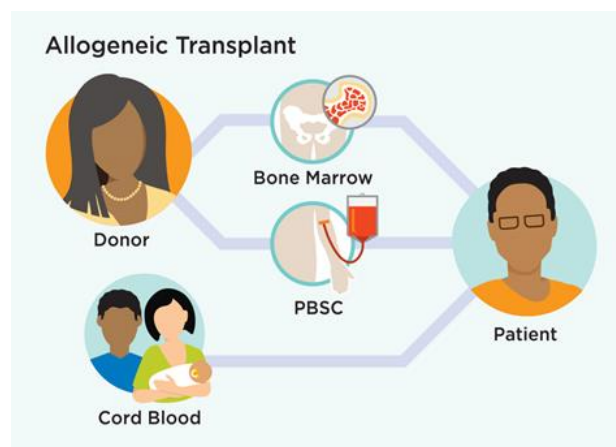
This type is less severe. Problems usually start between ages 21 and 35. People with this type of ALD may have stiffness and weakness that gets worse and changes in their ability to walk.

What is a **blood or marrow transplant (BMT)**?

BMT, also called bone marrow transplant, is a treatment for ALD. It replaces unhealthy blood-forming cells with healthy ones. The new cells have the right proteins so the body can break down fat. Transplant prevents more damage to

the brain, spinal cord and nervous system. But it can't fix any damage that has already happened.

The type of BMT that treats ALD 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 donor. This can take weeks to months.
2. Then, you 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 you through an intravenous (IV) infusion. The new cells travel to the inside of your bones.
4. After a few weeks, the donated cells begin to make healthy blood cells.
5. Then, you'll need many months to recover. You'll 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 ALD**. 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 medical history
- Talk with you about treatment options and their risks and benefits
- Recommend the best time for you to get a

transplant and prepare for treatment

- Start a donor search, if you and your doctor decide BMT is the best option
- Schedule appointments with other doctors, like a neurologist

Resources for you

Talk to someone who can help:

- Call: 1 (888) 999-6743
- Email: patientinfo@nmdp.org

For help finding a clinical trial:

- Call: 1 (888) 814-8610
- Email: clinical_trials@jcctp.org

Order free resources to learn more. To see a full list, visit [BeTheMatch.org/request](https://www.bethematch.org/request).

Questions to ask your doctor

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

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

- How might my 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](https://www.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.