



Aplastic Anemia

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What is aplastic anemia?

- All of your blood cells (red blood cells, white blood cells and platelets) are made in the bone marrow. Red blood cells carry oxygen throughout our body. White blood cells fight infection. Platelets help us form a clot when we bleed.
- Aplastic anemia occurs when the bone marrow does not produce enough of these cells.

What are the symptoms of aplastic anemia?

- Looking tired or pale (due to low red blood cells, or anemia)
- Easy bruising or nosebleeds (due to having low platelets)
- Frequent infections or mouth sores (due to having low white blood cells).

What causes aplastic anemia in children?

- Medication
- Exposure to a toxin
- A recent infection. Some infections that could cause aplastic anemia are due to viruses that cause the common cold, hepatitis A or B, Epstein-Barr virus (EBV), or cytomegalovirus (CMV).
- The immune system becomes confused and attacks the patient's own bone marrow, causing it to be destroyed and become "aplastic".
- Many cases are "idiopathic", meaning there is no known cause.

How is childhood aplastic anemia diagnosed?

- Your doctor may order blood tests that show that your blood has low numbers of red blood cells, white blood cells and platelets.
- A bone marrow aspiration and biopsy is needed to help your doctor learn if your bone marrow is making all of these cells as it should. In aplastic anemia, the bone marrow test may show that some of these cells are not being made in the bone marrow. This test is performed at Children's of Alabama Hospital under sedation, so no pain will be felt during the procedure.

How is aplastic anemia treated in children?

- Patients are treated with a bone marrow transplant or medications that suppress the immune system. The treatment often depends on how severe (grade) the aplastic anemia is.
 - **Bone Marrow Transplant** - This treatment may offer the best chance for cure and rapid recovery for severe aplastic anemia.
 1. Patient is given drugs, or chemotherapy, that destroy the patient's immune system.
 2. New bone marrow from a healthy donor is given to the patient through an IV, and the bone marrow cells. The new cells move to the correct location in the body, where they can make normal red blood cells, white blood cells, and platelets.
 3. After a bone marrow transplant, medicines have to be given for several months to prevent complications.
 - **How do I find a Bone Marrow Donor?**
 - Our doctors can work to identify whether there is a good bone marrow donor by doing blood tests.
 - The best match is always a brother or sister
 - There is a 1 in 4 chance that a sibling will be a perfect match.
 - If there is a sibling matched donor, this is the best treatment for aplastic anemia. The procedure should be done as fast as possible, usually within 1-2 months of diagnosis
 - If there is no sibling or if the sibling does not match, then doctors will look for other donors that are enrolled in a large registry to find as close of a match as possible.
 - Doctors will discuss other options if there is not a match, including unrelated donor transplant or immune suppression drugs.
 - **Immunosuppressive Therapy** - This is one of the possible treatments for aplastic anemia.
 - Based on the theory that aplastic anemia is caused by the immune system destroying your bone marrow.
 - Starting medications that suppress your immune system may allow the bone marrow to start working again.
 - Medications include anti-thymocyte globulin (ATG), cyclosporine (CsA), and eltrombopag.
 - It may take three to six months for the treatment to begin to work.
 - Your child will need frequent doctor visits and may need red blood cell or platelet transfusions and antibiotics.

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What are the outcomes for treating aplastic anemia??

- A few decades ago, aplastic anemia was a fatal disease. Today, our therapies have dramatically improved outcomes.
- Bone marrow transplant can cure most children who have a matched sibling.
- For children without a match, immune suppressive medications can make blood counts return to normal for 2/3 of patients.
- The disease may not completely go away but patients can live a long, healthy life.
- If aplastic anemia recurs, the best option is a bone marrow transplant from an unrelated donor, though your doctor may try other medications.