

Aplastic Anemia :

Overview

Aplastic anemia is a rare but serious condition that occurs when a child's bone marrow produces too few of the three types of blood cells: red cells that carry oxygen, white cells that fight infection and platelets that prevent bleeding. This occurs because the child's bone marrow fails to produce enough stem cells, the basic cells that give rise to the three blood cell types.

The low number of red cells causes a drop in hemoglobin, the protein in red blood cells that carries oxygen to tissues of the body. A low number of white cells makes a child very susceptible to infections. And a low number of platelets causes bruising and bleeding because the blood will not clot normally.

Causes of Aplastic Anemia

Aplastic anemia in children may have many causes, such as a previous illness or infection. But in 50 percent to 75 percent of cases, the reason for the condition isn't known. Causes may include:

- A history of specific infectious diseases such as hepatitis, Epstein-Barr virus, cytomegalovirus, parvovirus B19 or human immunodeficiency virus (HIV)
- A history of taking certain medications
- Exposure to certain toxins such as heavy metals
- Exposure to radiation
- History of an autoimmune disease such as lupus

Children also may inherit a disorder that increases their risk of developing aplastic anemia. Some disorders known to increase the risk of developing the condition include:

- **Fanconi Anemia** — One of several inherited anemias, this anemia leads to bone marrow failure.
- **Dyskeratosis Congenita** — This is a rare disorder in which three types of symptoms occur: darkening or unusual absence of skin color;

progressive nail degeneration; and slowly changing characteristics of mucous membranes in the anus, urethra, lips, mouth and eye.

- **Schwachman-Diamond Syndrome** — This is a rare disease that mainly involves the pancreas, bone marrow and skeleton.
- **Amegakaryocytic Thrombocytopenia** — Another rare condition, this syndrome results in bruising and bleeding due to low levels of platelets.

Signs & symptoms

Aplastic anemia is suspected when test results indicate that all three blood cell levels are extremely low, but the cells themselves have a relatively normal appearance. It remains a rare disease, **striking only two to six out of every one million people annually in the United States and Europe.**

In addition to low blood cell counts, aplastic anemia is characterized by other physical symptoms of anemia such as pale skin and fingernails, rapid pulse, heart murmur and fatigue. Children also may exhibit abnormal bleeding including multiple bruises, nosebleeds, bleeding gums and small hemorrhages under the skin as well as infection, especially fever. However, each child may experience symptoms differently. Other possible symptoms include:

- Headache
- Dizziness
- Nausea
- Shortness of breath
- Blood in stool
- Sinus tenderness
- Enlarged liver or spleen
- Oral thrush, which produces white patches on a red, moist, swollen surface anywhere in the mouth

Diagnosis

The symptoms of aplastic anemia may resemble other blood disorders or conditions, so in addition to a complete medical history and a thorough physical examination, the following diagnostic tests will be performed:

- **Blood Tests** — These tests determine the extent of the problem and the possible cause.
- **Bone Marrow Aspiration and Biopsy** — Bone marrow fluid may be drawn by aspiration or suction. In addition, a needle biopsy may be performed under general anesthesia. In the aspiration phase of the test, a fluid specimen of bone marrow is removed from the hipbone for examination under a microscope. In a needle biopsy, a small piece of the bone marrow is taken by inserting a sturdy needle into the large pelvic bone just below the waist on either side of the spine. This sample is examined in the laboratory for low levels of stem cells and other microscopic changes.

These tests usually are performed together. A pediatric hematologist, a doctor who specializes in blood disorders in children, usually will confirm the diagnosis based on the results.

Treatment

Specific treatments for aplastic anemia will be determined by your child's doctor based on the following:

- Your child's age, overall health and medical history
- Extent of your child's anemia
- Cause of the anemia
- Your child's tolerance for specific medications, procedures or therapies
- Expectations for the course of the anemia
- Your opinion or preference

Treatment for aplastic anemia usually depends on the underlying cause. For certain causes, recovery can be expected after treatment. However, relapses can occur. If all treatments fail, aplastic anemia can be fatal.

Supportive Therapy

To treat the low blood counts, initial treatment is supportive, meaning it is necessary to treat the symptoms but it doesn't cure the disease. Supportive therapy may include:

- Blood transfusion for both red blood cells and platelets

- Preventive antibiotic therapy
- Meticulous hand washing
- Special care for food preparation, such as only eating cooked foods
- Avoiding construction sites that may be a source of certain fungi

The main treatments for aplastic anemia are:

Bone Marrow Transplant

Bone marrow transplants are performed to replace diseased marrow with healthy marrow from a well-matched donor. This treatment, with a good marrow match, can be highly successful, preventing recurrence in about 80 percent of young patients and about 40 to 70 percent of older patients. There is a chance, however, that your child may reject the transplant, leading to life-threatening complications. Not everyone will have a suitable donor.

Immunosuppressive Therapy

Immunosuppressive therapy uses drugs to stimulate blood cell production. Aplastic anemia may be due to an autoimmune disorder that causes your child's immune system to attack and damage cells in his or her bone marrow. To prevent this, doctors sometimes prescribe drugs that suppress immune cells that are damaging bone marrow cells.

In addition, a synthetic version of the male hormone androgen is being studied as a treatment for aplastic anemia. The hormone stimulates blood cell production.

Some treatments must be performed in the hospital or our outpatient Pediatric Treatment Center. But most of your child's medications will be taken at home. Your child will need to have frequent blood tests to monitor progress.