CONDITION

Idiopathic aplastic anemia

Idiopathic aplastic anemia is bone marrow failure that reduces blood cell production without an identifiable cause.

WHAT IT IS
Bone marrow failure without an identifiable cause

BODY SYSTEM
Blood and bone marrow

MAIN SYMPTOMS
Fatigue, infections, easy bruising, bleeding

WHAT CAUSES IT
Usually immune attack with an unknown trigger

HOW IT IS DIAGNOSED
Blood counts and bone marrow biopsy

MAIN TREATMENT
Stem cell transplant or immune-suppressing treatment

CAN IT BE CURED?
Sometimes - stem cell transplant can cure

IS IT AN EMERGENCY?
Sometimes - fever or serious bleeding

LONG-TERM OUTLOOK
Often treatable, though relapse and late complications can occur

Overview

Idiopathic aplastic anemia is a rare condition in which the bone marrow does not make enough blood cells. Bone marrow is the soft tissue inside certain bones where new blood cells are produced.

"Aplastic" means that the marrow is not producing cells normally. "Anemia" usually refers to too few red blood cells, but aplastic anemia can affect all three main types of blood cell:

Having low levels of all three is called pancytopenia .

"Idiopathic" means that testing has not found a specific cause. In many cases, the immune system appears to attack the blood-forming stem cells in the marrow, but it is not clear what started that reaction.

Idiopathic aplastic anemia is not the same as iron-deficiency anemia. Taking iron does not correct marrow failure unless you also have a separate iron deficiency. The condition is also not itself a cancer, although it can sometimes be associated with later bone marrow disorders.

The seriousness of aplastic anemia is based mainly on blood cell counts, symptoms, and how well the marrow is working. Severe cases need prompt specialist treatment because infection or bleeding can become dangerous.

- Red blood cells, which carry oxygen
- White blood cells, which help fight infection
- Platelets, which help stop bleeding

Symptoms

Symptoms develop because the body has too few functioning blood cells. They may appear gradually or over a shorter period.

Too few red blood cells can cause:

Too few white blood cells, particularly infection-fighting cells called neutrophils, can lead to frequent or serious infections. Fever may be the only early sign of an infection when neutrophil levels are very low.

Too few platelets can cause:

Aplastic anemia usually does not cause enlarged lymph nodes or an enlarged spleen. Findings such as these may lead the medical team to investigate other explanations for low blood counts.

- Tiredness or weakness
- Shortness of breath, especially during activity
- Dizziness or headaches
- Pale skin
- A fast or noticeable heartbeat
- Easy bruising
- Tiny red or purple spots under the skin, called petechiae
- Nosebleeds or bleeding gums
- Heavy menstrual bleeding

- Bleeding that takes longer than expected to stop
- Blood in urine, vomit, or bowel movements

Causes and risk factors

In idiopathic aplastic anemia, no outside cause or inherited condition is found after a careful evaluation. It is usually considered an acquired condition, meaning it develops during a person's lifetime.

The main suspected process is an abnormal immune response. Certain white blood cells may attack the stem cells that should develop into red cells, white cells, and platelets. The trigger for this immune response often remains unknown.

Other forms of aplastic anemia can be linked to medicines, toxic chemicals, radiation, viral infections, pregnancy, or inherited bone marrow failure conditions. Doctors check for these possibilities before using the term idiopathic.

Idiopathic aplastic anemia is not contagious. It is not passed between people through blood, physical contact, food, or air.

The condition itself is not usually inherited. However, inherited marrow failure conditions can sometimes first become apparent in adults. Genetic and specialized cell testing may therefore be important, particularly in younger people, those with a suggestive family history, or those being considered for a stem cell transplant.

Diagnosis

A complete blood count, or CBC, measures red blood cells, white blood cells, and platelets. In aplastic anemia, more than one cell type is usually low. A reticulocyte count measures young red blood cells and helps show whether the marrow is producing new cells properly.

A bone marrow aspiration and biopsy are needed to confirm the diagnosis. During this procedure, a small amount of liquid marrow and a tiny core of bone are usually taken from the back of the hip. Local anesthetic is used to numb the area.

In aplastic anemia, the marrow typically contains far fewer blood-forming cells than expected and more fatty tissue. This is called hypocellular marrow .

Further tests help rule out other causes of low blood counts, including leukemia, myelodysplastic syndromes, vitamin deficiencies, infections, medication effects, and inherited marrow conditions. Testing may include:

The medical team also grades the condition as non-severe, severe, or very severe. This classification helps guide how quickly treatment is needed and which options may be suitable.

- Examination of blood and marrow cells
- Chromosome and genetic tests
- Liver, kidney, vitamin, and infection tests
- Tests for paroxysmal nocturnal hemoglobinuria, or PNH
- Telomere or chromosome-breakage tests when inherited conditions are possible

Treatment and management

Treatment is managed by a hematologist, a doctor who specializes in blood conditions. The plan is based on the severity of the marrow failure, age, general health, donor availability, symptoms, and previous treatment.

Some people with stable, non-severe aplastic anemia may initially have close monitoring. Treatment is more likely when blood counts are falling, symptoms are significant, transfusions are needed, or the condition is severe.

A stem cell transplant replaces the poorly functioning marrow with blood-forming stem cells from a donor. It offers the clearest possibility of a cure. The medical team carefully matches potential donors and weighs this benefit against risks such as serious infection, organ complications, infertility, or graft-versus-host disease. In graft-versus-host disease, donated immune cells attack the recipient's tissues.

Immune-suppressing treatment reduces the immune attack on the marrow so that remaining stem cells can recover. Treatment for severe aplastic anemia commonly involves antithymocyte globulin, or ATG, with ciclosporin. Eltrombopag, a medicine that encourages blood cell production, may also be included. These treatments require regular blood tests and monitoring for side effects.

Immune-suppressing treatment can produce a lasting recovery, but it is not always a cure. Some people do not respond fully, and others relapse after an initial response. Further immune treatment or a stem cell transplant may then be considered.

Supportive care protects you while the marrow recovers. It may include:

Repeated transfusions can lead to excess iron or immune reactions against donated cells. Blood banks and treatment teams use specific transfusion practices to reduce these risks.

- Red blood cell transfusions for significant anemia

- Platelet transfusions to prevent or control bleeding
- Antibiotic or antifungal treatment for infection
- Medicines to reduce treatment-related side effects

Complications

Very low white blood cell counts increase the risk of severe infection. Because the immune response is reduced, an infection may not cause much redness, swelling, or pus. Fever or feeling suddenly unwell may be the main warning signs.

Low platelets can cause serious bleeding. Bleeding into the digestive system or brain is uncommon but can be life-threatening and needs emergency care.

Longer-term complications can include:

These later marrow conditions do not occur in everyone. Regular blood counts and specialist follow-up help identify changes early.

- Iron overload after many red blood cell transfusions
- Relapse after successful immune-suppressing treatment
- Kidney, liver, or blood pressure problems from treatment
- Graft-versus-host disease after a donor transplant
- Development of PNH, myelodysplastic syndrome, or acute myeloid leukemia

Prevention

Idiopathic aplastic anemia generally cannot be prevented because its trigger is unknown. There is no screening test that reliably predicts who will develop it.

Avoiding unnecessary exposure to benzene and other marrow-toxic chemicals may reduce the risk of some forms of acquired marrow failure, but it does not specifically prevent idiopathic aplastic anemia.

Do not stop a prescribed medicine because of concern about aplastic anemia without medical advice. Many medicines associated with marrow problems are essential treatments, and serious reactions are uncommon.

For someone who already has aplastic anemia, infection and bleeding precautions may reduce complications. The precautions needed depend on current blood counts. They may include

careful hand hygiene, avoiding close contact with people who are unwell, and avoiding activities likely to cause injury when platelet levels are very low.

Living with it and what to expect

The course differs from person to person. Blood counts may remain stable, improve with treatment, decline, or recover and later fall again.

A response to immune-suppressing treatment is usually gradual and can take several months. During this period, regular blood tests and sometimes transfusions are needed. Medicines such as ciclosporin may need to continue for an extended time and are usually reduced only under specialist supervision.

Recovery after a stem cell transplant also takes time. Blood cell production may begin within weeks, but immune recovery and return to usual activities can take many months or longer. Follow-up focuses on infection, medication effects, graft-versus-host disease, and the health of the new marrow.

Many people who achieve a stable recovery return to work, education, exercise, and family life. Others continue to have lower blood counts, fatigue, treatment effects, or repeated appointments. The safest level of activity can change as blood counts change.

Ask the treatment team before taking aspirin, anti-inflammatory pain medicines, supplements, or herbal products. Some can increase bleeding or interact with immune-suppressing medicines. Dental work, vaccinations, travel, pregnancy planning, and fertility preservation may also require advance discussion.

Long-term follow-up remains important even when blood counts are normal. It allows the team to look for relapse, late treatment effects, and changes suggesting another marrow condition.

When to get medical help

Contact your hematology team urgently if you develop a temperature of 38°C or 100.4°F or higher, unless your team has given you a different threshold. Also seek urgent advice for chills, sudden illness, a new cough, painful urination, or other possible signs of infection.

Get urgent medical help for new or increasing bruising, persistent nosebleeds, bleeding gums, blood in urine, black bowel movements, or menstrual bleeding that is much heavier than usual.

Call emergency services for:

These symptoms can indicate serious bleeding, infection, or severe anemia.

For routine follow-up, tell your medical team about steadily increasing fatigue, breathlessness during normal activity, more frequent infections, medication side effects, or difficulty managing appointments and daily activities. Do not wait for the next planned visit if symptoms are clearly worsening.

- Bleeding that is heavy or will not stop
- Vomiting blood or passing a large amount of blood
- A sudden severe headache, confusion, weakness, or difficulty speaking
- Severe shortness of breath, chest pain, collapse, or fainting

Common questions

Is idiopathic aplastic anemia cancer?

No. It is a bone marrow failure condition, not a cancer. However, a small number of people later develop related marrow conditions such as myelodysplastic syndrome or acute myeloid leukemia, so long-term monitoring is important.

Can idiopathic aplastic anemia be cured?

Sometimes. A successful donor stem cell transplant can cure the condition. Immune-suppressing treatment can lead to long-lasting recovery, but relapse or incomplete recovery remains possible.

Is idiopathic aplastic anemia an autoimmune condition?

It is usually considered immune-mediated. This means the immune system appears to attack blood-forming stem cells. It is not always possible to identify why this happens, and it may not behave like more familiar autoimmune conditions.

How long does treatment take to work?

Stem cell production does not recover immediately. After immune-suppressing treatment, improvement often takes several months. Transfusions and infection protection may be needed while waiting for a response.

Can aplastic anemia turn into leukemia?

It can happen, but it is not inevitable. Some people develop abnormal groups of marrow cells over time, a process called clonal evolution. Regular blood counts and, when needed, repeat marrow testing help monitor for this.

Can I live a normal life with aplastic anemia?

Many people return to usual activities after a lasting treatment response. Others need ongoing

medicines, monitoring, or transfusions. What is safe at a given time is strongly influenced by blood counts, treatment effects, and infection or bleeding risk.

Sources

- National Heart, Lung, and Blood Institute: Aplastic Anemia , including causes, diagnosis, symptoms, and treatment
- British Society for Haematology: Guidelines for the diagnosis and management of adult aplastic anaemia , 2024
- The EBMT Handbook: Aplastic Anemia , European Society for Blood and Marrow Transplantation, 2024
- Young NS. Aplastic Anemia , New England Journal of Medicine , 2018;379:1643-1656