

Aplastic Anemia

A plain-English guide for you and your family

WHAT IS HAPPENING

Your bone marrow is soft, spongy tissue inside your bones that works like a cell factory. Every day it makes billions of red blood cells (to carry oxygen), white blood cells (to fight germs), and platelets (to stop bleeding). The factory runs on master cells called stem cells—they can turn into any blood cell your body needs. In aplastic anemia, something damages or kills these stem cells, so the whole factory goes quiet and stops making new blood cells. The most common reason (about 7 out of 10 cases) is your own immune system attacking the stem cells by mistake—special immune cells called T cells release chemicals that tell the stem cells to die. Other triggers include certain medicines (the antibiotic chloramphenicol, sulfa drugs, some pain pills like NSAIDs), viruses (hepatitis viruses, the virus that causes fifth disease in kids), chemicals like benzene (found in gasoline and industrial solvents), radiation, and rare inherited conditions (such as Fanconi anemia or a disease called dyskeratosis congenita that makes cells age too fast). Because all three blood cell types come from the same broken factory, you see three problems at once: anemia (low red cells make you tired and pale), low white cells (so infections break through easily), and low platelets (so you bruise and bleed easily—tiny red dots on your skin, nosebleeds, bleeding gums). One important clue: your spleen stays normal size, because the problem is not too many abnormal cells being made (like in leukemia)—it's that hardly any cells are being made at all. Every symptom you see or feel traces back upstream to that one broken factory inside your bones.

What you might feel

- Fatigue and getting tired quickly during activity
- Pale skin, pale inside of eyelids, and pale nail beds
- Easy bruising and tiny red or purple dots on the skin (petechiae)
- Bleeding gums, nosebleeds, or very heavy menstrual periods
- Getting infections over and over (like skin infections, pneumonia, or mouth sores)
- Fever with no clear cause
- Shortness of breath during activity or even at rest

- Severe bleeding from the stomach, intestines, bladder, or inside the skull
- Dizziness or feeling lightheaded
- Fast or pounding heartbeat (palpitations)

Call the doctor right away if...

- Fever (temperature 100.4°F or 38°C or higher) when your absolute neutrophil count is below 500/ μ L—this is neutropenic fever and means you could have a life-threatening bloodstream infection (sepsis). You need emergency IV antibiotics right away.
- Platelet count below 10,000/ μ L—very high risk of bleeding inside your skull (intracranial bleeding (hemorrhage)) or other organs without any warning. You may need an urgent platelet transfusion.
- Sudden severe headache, confusion, slurred speech, one-sided weakness, or vision changes—these can mean bleeding inside your brain from low platelets. This is a medical emergency.
- Blood counts dropping fast over days to weeks—this suggests very severe aplastic anemia or the bone marrow disease is changing into something worse like myelodysplastic syndrome or leukemia. Needs urgent specialist evaluation.
- Chest pain, extreme shortness of breath, or confusion—these mean your heart or brain is starving for oxygen because of severe anemia, and you need immediate care and possibly a blood transfusion.

What the care team will do

- Monitor temperature every 4 hours and immediately report any reading of 100.4°F (38°C) or higher to the provider, then prepare for stat blood cultures from two sites and rapid start of broad-spectrum IV antibiotics within 1 hour.
- Check platelet count daily and inspect skin, gums, and all body fluids for signs of bleeding every shift. Hold all injections, rectal temperatures, and suppositories if platelets are below 20,000/ μ L. Keep the bed in low position and pad side rails if platelets drop below 10,000/ μ L.
- Use strict neutropenic precautions: private room, all visitors and staff wash hands with soap for 20 seconds or alcohol gel before entering, no fresh flowers or plants, no raw fruits or vegetables, and anyone with cold symptoms stays out.
- Transfuse red blood cells when hemoglobin drops below 7 g/dL or sooner if the patient has chest pain, shortness of breath at rest, or heart rate above 110 while lying down. Pre-medicate with acetaminophen and diphenhydramine (an antihistamine) 30 minutes before transfusion, and use leukoreduced (white-blood-cell-filtered) and irradiated blood products.

- Administer immunosuppressive drugs (anti-thymocyte globulin, cyclosporine, eltrombopag) exactly on schedule, monitor for infusion reactions during ATG (fever, chills, low blood pressure, rash), and check cyclosporine trough levels as ordered to keep levels in therapeutic range.
- Teach and enforce meticulous mouth care with soft toothbrush or foam swabs four times daily, rinse with salt water or baking soda solution, and avoid mouthwashes with alcohol. Inspect the mouth every shift for sores, white patches, or bleeding.
- Monitor ferritin level every 4 to 6 weeks in patients receiving regular transfusions, and prepare to give iron chelation therapy (deferasirox by mouth or deferoxamine by IV or under the skin) if ferritin rises above 1000 ng/mL.
- Coordinate care with the transplant team if the patient is a transplant candidate: ensure human white blood cell (leukocyte) antigen (HLA) typing is complete for patient and family members, avoid transfusions from family members (to prevent sensitization), and minimize total transfusions when possible.

Medicines you may be given — and why

Anti-thymocyte globulin (ATG)

ATG is made from the blood of horses or rabbits that were exposed to human T-cells (a type of white blood cell). It contains many different antibodies that bind to and destroy T-cells that are attacking the bone marrow stem cells. In aplastic anemia, your own T-cells mistakenly kill the stem cells that make blood. ATG removes those attacking T-cells, giving the stem cells a chance to survive and grow again.

Cyclosporine

Cyclosporine blocks a protein inside T-cells called calcineurin (think of it as a switch that turns on T-cell activity). When calcineurin is blocked, T-cells cannot make the chemical signals (cytokines like interleukin-2) they need to multiply and attack. This stops the T-cells from destroying bone marrow stem cells. Cyclosporine works hand-in-hand with ATG: ATG removes the attacking T-cells already present, and cyclosporine prevents new ones from activating.

Eltrombopag

Eltrombopag mimics a natural hormone called thrombopoietin, which tells stem cells in the bone marrow to wake up and make more blood cells (especially platelets, but also red and white cells). In aplastic anemia, eltrombopag helps the few surviving stem cells work harder. It also appears to have a mild immune-calming effect, though scientists are still studying exactly how. Eltrombopag is always used together with ATG and cyclosporine—it boosts the stem cells while the other drugs stop the immune attack.

Packed red blood cells (pRBC transfusion)

Transfusion of packed red blood cells directly replaces the red cells your marrow cannot make. Red cells carry oxygen from your lungs to every tissue in your body. In aplastic anemia, you do not have enough red cells (anemia), so your heart has to pump harder to deliver oxygen and you feel tired, weak, dizzy, and short of breath. Transfusing red cells quickly raises your oxygen-carrying capacity, relieving symptoms and preventing heart strain while you wait for immunosuppressive therapy or transplant to rebuild your marrow.

Platelet transfusion

Platelet transfusion directly replaces the platelets your marrow cannot make. Platelets are tiny cell fragments that clump together to form clots and stop bleeding. In aplastic anemia, dangerously low platelet counts (severe a low platelet count (thrombocytopenia)) put you at risk for spontaneous bleeding—nosebleeds, gum bleeding, bruising, and most dangerously, bleeding in the brain (intracranial bleeding (hemorrhage)). Transfusing platelets temporarily raises your count and reduces bleeding risk while the marrow recovers or until transplant.

Filgrastim (G-CSF)

Filgrastim is a man-made copy of a natural hormone called G-CSF (granulocyte colony-stimulating factor). G-CSF tells the bone marrow to make more neutrophils (a type of white blood cell that fights bacteria and fungi). In aplastic anemia, dangerously low neutrophil counts (severe dangerously low infection-fighting cells (neutropenia)) leave you open to life-threatening infections. Filgrastim boosts neutrophil production, raising your count and helping you fight infections while you wait for immunosuppressive therapy or transplant to rebuild the marrow.

Antibiotics (broad-spectrum, e.g. piperacillin-tazobactam, meropenem, fluoroquinolones)

In severe aplastic anemia, very low neutrophil counts (neutropenia, ANC <500/ μ L) mean you have almost no soldiers to fight bacteria and fungi. Even normal skin and gut bacteria can invade the bloodstream and cause life-threatening infections (sepsis, pneumonia). Broad-spectrum antibiotics kill a wide range of bacteria (both gram-positive like Staph and gram-negative like E. coli and Pseudomonas), protecting you until your neutrophil count recovers. Preventive (Prophylactic) antibiotics (given to prevent infection before it starts) are used when ANC is very low; therapeutic antibiotics are started immediately if you develop fever (febrile dangerously low infection-fighting cells (neutropenia)) because any delay can be fatal.

Antifungals (fluconazole, voriconazole, or posaconazole for prevention (prophylaxis); amphotericin B or echinocandins for treatment)

In severe dangerously low infection-fighting cells (neutropenia) (ANC <500/ μ L), you cannot fight fungal infections that healthy people easily control. Fungi like *Candida* (yeast) and *Aspergillus* (mold) invade the lungs, sinuses, bloodstream, and organs, causing pneumonia, brain abscesses, and sepsis that can be fatal within days. Antifungals kill or stop fungi from growing. Preventive (Prophylactic) antifungals (fluconazole, posaconazole) are given daily to prevent infection when dangerously low infection-fighting cells is prolonged. If you develop fever despite antibiotics or have signs of fungal infection (persistent fever, lung nodules on CT, sinus pain), stronger antifungals (voriconazole, amphotericin B) are started immediately.

Where this information comes from

- American Society of Hematology (ASH) guidelines on aplastic anemia
- British Society for Haematology (BSH) 2016 guidelines for diagnosis and management of aplastic anemia
- National Institutes of Health (NIH) consensus on acquired aplastic anemia
- Aplastic Anemia and MDS International Foundation evidence-based standards

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