CONDITION

Acute myeloid leukemia (AML) - children

Learn about childhood acute myeloid leukemia, including symptoms, testing, treatment, complications, and follow-up care.

WHAT IT IS
Cancer of blood-forming bone marrow cells

BODY SYSTEM
Blood and bone marrow

MAIN SYMPTOMS
Tiredness, infections, easy bruising, bleeding

WHAT CAUSES IT
Usually unknown genetic changes

HOW IT IS DIAGNOSED
Bone marrow aspiration and biopsy with cell and genetic testing

MAIN TREATMENT
Intensive chemotherapy, sometimes targeted therapy or stem cell transplant

CAN IT BE CURED?
Sometimes - AML can return after remission

IS IT AN EMERGENCY?
Sometimes - with uncontrolled bleeding

LONG-TERM OUTLOOK
Remission is possible, but relapse and late effects can occur

Overview

Acute myeloid leukemia, or AML, is a cancer of blood-forming cells in the bone marrow, the soft tissue inside certain bones where blood cells are made.

In AML, harmful genetic changes cause immature myeloid cells, called leukemia cells or myeloblasts, to multiply quickly without maturing normally. These cells crowd the bone marrow and interfere with production of healthy red blood cells, white blood cells, and platelets. This can cause tiredness, infections, bruising, or bleeding. Leukemia cells may also enter the bloodstream or collect in the skin, gums, brain and spinal cord, or other organs.

AML differs from acute lymphoblastic leukemia, or ALL, which begins in another type of developing blood cell. Tests that identify the leukemia cell type and genetic features are essential for planning treatment.

Childhood AML requires prompt care at a pediatric cancer center. Treatment is intensive and depends on the AML subtype, genetic findings, response to therapy, general health, and previous medical conditions.

Acute promyelocytic leukemia, or APL, is a distinct AML subtype that can cause dangerous clotting and bleeding problems. It requires urgent recognition and is treated differently from most other forms of AML.

Symptoms

Symptoms often develop over days or weeks. They can also occur with common childhood illnesses, so having one or more does not necessarily mean a child has leukemia.

Low red blood cell levels, called anemia, may cause:

Low levels of normal infection-fighting white blood cells may cause frequent, persistent, or severe infections. Fever may be the only obvious sign.

Low platelet levels can cause:

Other symptoms may include bone or joint pain, a swollen abdomen, poor appetite, or unexplained weight loss. Some children develop swollen gums, firm skin spots, or a lump of leukemia cells called a myeloid sarcoma.

Less commonly, involvement of the brain or spinal cord may cause persistent headache, vomiting, vision changes, weakness, seizures, or changes in alertness.

Contact a healthcare professional promptly about persistent fever, unusual bruising or bleeding, marked paleness, worsening tiredness, or symptoms that do not improve.

Seek emergency care for severe breathing difficulty, uncontrolled bleeding, a seizure, fainting, confusion, extreme sleepiness, or difficulty waking the child.

- Unusual tiredness or weakness
- Pale skin
- Shortness of breath with normal activity
- Dizziness, headache, or a fast heartbeat
- Easy bruising
- Nosebleeds or bleeding gums
- Tiny red or purple spots under the skin, called petechiae
- Bleeding that lasts longer than expected

- Blood in urine, stool, or vomit
- Unusually heavy menstrual bleeding

Causes and risk factors

AML develops when genetic changes disrupt the growth and maturation of an early blood-forming cell. These changes are usually limited to leukemia cells and are not necessarily inherited. In most children, there is no clear reason AML developed. It is not caused by diet, routine activities, minor injuries, or something a parent did.

Factors that can increase risk include:

Most children with these risk factors do not develop AML. Myeloid leukemia associated with Down syndrome has distinct biological features and may require different treatment.

A family history, physical findings, or leukemia test results may suggest an inherited predisposition. Genetic counseling and testing may then be recommended because the results can affect treatment, relatives, or potential stem cell donors.

- Inherited conditions such as Down syndrome, Fanconi anemia, and certain bone marrow failure or cancer-predisposition syndromes
- Previous treatment with particular chemotherapy medicines or radiation
- Myelodysplastic syndromes or other blood-production disorders
- Transient abnormal myelopoiesis in some infants with Down syndrome

Diagnosis

Evaluation usually includes a medical history, physical examination, complete blood count, or CBC, and a blood smear. Results may show anemia, low platelets, an unusual white blood cell count, or myeloblasts. Blood tests alone cannot fully classify AML.

A bone marrow aspiration and biopsy are usually required. Aspiration removes liquid marrow through a needle, often from the back of the hip bone; biopsy removes a small piece of marrow tissue. Children receive pain control and often sedation or anesthesia.

Laboratory testing may include:

AML has traditionally been diagnosed when myeloblasts make up at least 20% of blood or marrow cells. Some AML types can be diagnosed at a lower percentage when specific defining genetic changes are present.

A lumbar puncture, or spinal tap, may be performed to check the fluid around the brain and spinal cord. Treatment may be placed into the fluid during the procedure. Timing depends on the child's condition and bleeding risk.

Other tests assess blood clotting and kidney, liver, and heart function. HLA tissue typing may be performed in case stem cell transplant is considered. After treatment begins, repeat marrow, molecular, and measurable residual disease, or MRD, tests look for small numbers of leukemia cells and measure response.

- Morphology: examination of cell appearance
- Flow cytometry: identification of proteins on or inside cells
- Cytogenetic testing: detection of chromosome changes
- Molecular testing: detection of gene changes that may guide risk assessment or treatment

Treatment and management

Treatment is usually provided at a specialized pediatric cancer center. Plans depend on AML subtype, chromosome and gene findings, MRD results, organ function, and whether AML is newly diagnosed or has returned. A clinical trial may be offered.

Chemotherapy

Most children receive intensive chemotherapy in several cycles.

Induction treatment aims to remove visible leukemia from the blood and marrow and restore normal blood production. More than one induction cycle may be needed.

Post-remission or intensification treatment aims to destroy leukemia cells remaining after remission. Remission means standard tests no longer show leukemia and blood production has recovered, but it does not guarantee that every leukemia cell is gone.

Unlike childhood ALL treatment, AML treatment usually does not include a long maintenance phase. Medicines and cycle numbers vary by protocol.

Treatment directed at specific AML features

Some children receive targeted medicines when testing identifies a matching protein or gene change.

APL is commonly treated with medicines that help abnormal promyelocytes mature, including all-trans retinoic acid and arsenic trioxide, sometimes with chemotherapy or other medicines. Because APL can cause life-threatening bleeding or clotting, APL-directed care may begin

quickly when the condition is strongly suspected.

Stem cell transplant

An allogeneic hematopoietic stem cell transplant replaces the child's blood-forming system with donor stem cells. It may be considered during first remission for certain higher-risk forms of AML or when AML does not respond fully or returns.

Transplant is not appropriate for every child. The team considers relapse risk, transplant risks, donor availability, MRD, genetic findings, and overall health.

Supportive care

Treatment temporarily lowers healthy blood cell counts. Children may need red blood cell or platelet transfusions and medicines to prevent or treat infections, nausea, pain, or high uric acid caused by rapid leukemia cell breakdown.

Support may also include nutrition, mouth and central-line care, physical activity, emotional care, school planning, and fertility counseling. Check with the oncology team before giving vitamins, herbal products, over-the-counter medicines, or vaccines. Some may interfere with treatment or be unsafe during immune suppression.

Complications

Short-term complications include serious infection, anemia, bleeding, and reactions to medicines or transfusions. Tumor lysis syndrome occurs when many leukemia cells break down quickly, releasing substances that can damage the kidneys and affect heart rhythm or muscle function.

Depending on the medicines used, treatment may affect the heart, liver, kidneys, nerves, skin, or digestive system. Mouth sores, nausea, diarrhea, appetite changes, and hair loss may occur.

APL-related clotting and bleeding, especially near diagnosis, require urgent treatment.

Stem cell transplant can cause infection, organ injury, infertility, and graft-versus-host disease, in which donor immune cells attack the recipient's tissues.

AML that does not respond fully is called refractory AML. AML that returns after remission is relapsed AML. Further care may include different chemotherapy, targeted treatment, a clinical-trial immunotherapy, or stem cell transplant.

Possible late effects include heart problems, altered growth or hormone function, learning or attention difficulties, reduced fertility, and second cancers. Risk depends on treatment exposures and other health factors, and not every child develops these problems.

Follow up

During treatment, follow-up includes examinations, blood counts, chemistry tests, and scheduled marrow or molecular testing. These results track recovery, complications, and treatment response.

A fever during AML treatment requires urgent contact with the oncology team because infection can worsen quickly when white blood cell counts are low. Follow the team's temperature threshold and instructions rather than giving fever medicine and waiting for improvement.

Seek urgent medical advice for new bleeding, persistent vomiting or diarrhea, reduced urination, worsening pain, unusual sleepiness, central-line redness or drainage, or known exposure to certain contagious infections.

Seek emergency care for severe breathing difficulty, uncontrolled bleeding, a seizure, collapse, confusion, or difficulty waking the child. If possible, alert the oncology team while arranging emergency help.

After treatment, visits become less frequent over time. Follow-up may include blood tests, relapse monitoring, growth and development assessments, heart testing when appropriate, and review of learning, emotional health, and fertility concerns.

Survivorship care should include a written treatment summary and late-effect screening plan. Vaccines may need to be delayed or repeated after chemotherapy or transplant; follow the oncology team's schedule.

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