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About Acute Myeloid Leukemia (AML)

Overview of AML

If you have been diagnosed with acute myeloid leukemia or are worried about it, you likely have a lot of questions. Learning some basics is a good place to start.

- [What Is Acute Myeloid Leukemia \(AML\)?](#)

Research and Statistics

See the latest estimates for new cases of acute myeloid leukemia and deaths in the US and what research is currently being done.

- [Key Statistics for Acute Myeloid Leukemia \(AML\)](#)
- [What's New in Acute Myeloid Leukemia \(AML\) Research?](#)

What Is Acute Myeloid Leukemia (AML)?

Cancer starts when cells in a part of the body begin to grow out of control. There are many kinds of cancer. Cells in nearly any part of the body can become cancer. To learn more about cancer and how it starts and grows, see [What Is Cancer?](#)¹

Leukemias are cancers that start in cells that would normally develop into different types of blood cells. Most often, leukemia starts in early forms of white blood cells, but some leukemias start in other blood cell types. There are several types of leukemia,

which are divided based mainly on whether the leukemia is acute (fast growing) or chronic (slower growing), and whether it starts in myeloid cells or lymphoid cells.

Acute myeloid leukemia (AML) starts in the bone marrow (the soft inner part of certain bones, where new blood cells are made), but most often it quickly moves into the blood, as well. It can sometimes spread to other parts of the body including the lymph nodes, liver, spleen, central nervous system (brain and spinal cord), and testicles.

Most often, AML develops from cells that would turn into white blood cells (other than lymphocytes), but sometimes AML develops in other types of blood-forming cells. The different types of AML are discussed in [Acute Myeloid Leukemia \(AML\) Subtypes and Prognostic Factors²](#).

Acute myeloid leukemia (AML) has many other names, including acute myelocytic leukemia, acute myelogenous leukemia, acute granulocytic leukemia, and acute non-lymphocytic leukemia.

Normal bone marrow, blood, and lymph tissue

To understand leukemia, it helps to know about the blood and lymph systems.

Bone marrow

Bone marrow is the soft inner part of certain bones. It is made up of blood-forming cells, fat cells, and supporting tissues. A small fraction of the blood-forming cells are **blood stem cells**.

Inside the bone marrow, blood stem cells develop into new blood cells. During this process, the cells become either lymphocytes (a kind of white blood cell) or other blood-forming cells, which are types of **myeloid cells**. Myeloid cells can develop into red blood cells, white blood cells (other than lymphocytes), or platelets. These myeloid cells are the ones that are abnormal in AML.

Types of blood cells

There are 3 main types of blood cells:

- **Red blood cells (RBCs)** carry oxygen from the lungs to all other tissues in the body, and take carbon dioxide back to the lungs to be removed.
- **Platelets** are actually cell fragments made by a type of bone marrow cell called the

megakaryocyte. Platelets are important in stopping bleeding. They help plug up holes in blood vessels caused by cuts or bruises.

- **White blood cells (WBCs)** help the body fight infections.

There are different types of WBCs:

- **Granulocytes** are mature WBCs that develop from myeloblasts, a type of blood-forming cell in the bone marrow. Granulocytes have granules that show up as spots under the microscope. These granules contain enzymes and other substances that can destroy germs, such as bacteria. The 3 types of granulocytes – **neutrophils**, **basophils**, and **eosinophils** – are distinguished by the size and color of their granules.
- **Monocytes** are WBCs that develop from blood-forming monoblasts in the bone marrow. After circulating in the bloodstream for about a day, monocytes enter body tissues to become **macrophages**, which can destroy some germs by surrounding and digesting them. Macrophages also help lymphocytes recognize germs and make antibodies to fight them.
- **Lymphocytes** are mature WBCs that develop from lymphoblasts in the bone marrow. Lymphocytes are the main cells that make up lymph tissue, a major part of the immune system. Lymph tissue is found in lymph nodes, the thymus (a small organ behind the breast bone), the spleen, the tonsils and adenoids, and is scattered throughout the digestive and respiratory systems and the bone marrow. The 2 main types of lymphocytes are B cell and T cells.

Hyperlinks

1. www.cancer.org/cancer/cancer-basics/what-is-cancer.html
2. www.cancer.org/cancer/acute-myeloid-leukemia/detection-diagnosis-staging/how-classified.html

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Key Statistics for Acute Myeloid Leukemia (AML)

The American Cancer Society's estimates for leukemia in the United States for 2020 are:

- About 60,530 new cases of leukemia (all kinds) and 23,100 deaths from leukemia (all kinds)
- About 19,940 new cases of acute myeloid leukemia (AML). Most will be in adults.
- About 11,180 deaths from AML. Almost all will be in adults.

AML is one of the most common types of leukemia in adults. Still, AML is fairly rare overall, accounting for only about 1% of all cancers.

AML is generally a disease of older people and is uncommon before the age of 45. The average age of people when they are first diagnosed with AML is about 68. But [AML can occur in children](#)¹ as well.

AML is slightly more common among men than women, but the average lifetime risk of getting AML in both sexes is about ½ of 1%.

Information on treatment success rates for AML in adults can be found in [Treatment Response Rates for Acute Myeloid Leukemia](#)².

Visit the [American Cancer Society's Cancer Statistics Center](#)³ for more key statistics.

Hyperlinks

1. www.cancer.org/cancer/leukemia-in-children.html
2. www.cancer.org/cancer/acute-myeloid-leukemia/treating/response-rates.html
3. <https://cancerstatisticscenter.cancer.org/>

References

American Cancer Society. *Cancer Facts & Figures 2020*. Atlanta, Ga: American Cancer Society; 2020.

National Cancer Institute. SEER Cancer Stat Facts: Acute Myeloid Leukemia. Accessed at <https://seer.cancer.gov/statfacts/html/amyl.html> on June 12, 2018.

Last Medical Review: August 21, 2018 Last Revised: January 8, 2020

What's New in Acute Myeloid Leukemia (AML) Research?

Researchers are now studying the causes, diagnosis, and treatment of acute myeloid leukemia (AML) at many medical centers, university hospitals, and other institutions.

Genetics of AML

There has been great progress in understanding how changes in the DNA (genes) inside normal bone marrow cells can cause them to develop into leukemia cells. A greater understanding of the gene changes that often occur in AML is providing insight into why these cells become abnormal. As researchers have found more of these changes, it is becoming clear that there are many types of AML. Each might have different gene changes that affect how the leukemia will progress and which treatments might be most helpful. Doctors are now learning how to use these changes to help them determine a person's outlook and if they should receive more or less intensive treatment.

Perhaps even more important, this knowledge is now being used to help develop newer targeted therapies against AML (see below).

Detecting minimal residual disease

In recent years, highly sensitive tests have been developed to detect even the smallest amount of leukemia left after treatment (known as minimal residual disease, or MRD), even when there are so few leukemia cells left that they can't be found by routine bone marrow tests.

For example, the polymerase chain reaction (PCR) test can identify even very small numbers of AML cells in a sample, based on their gene changes. A PCR test can be useful in determining how completely the treatment has destroyed the AML cells.

Doctors are now trying to determine what effect minimal residual disease has on a patient's outlook, and how this might affect the need for further or more intensive treatment.

Improving treatment

Treatment for AML can be very effective for some people, but it doesn't cure everyone, and it can often cause serious or even life-threatening side effects. Many studies are being done to find more effective and safer treatments for AML.

Chemotherapy

[Chemotherapy](#)¹ (chemo) is still the main treatment for most types of AML. Researchers are looking for the most effective combination of chemotherapy (chemo) drugs that will avoid unwanted side effects. This is especially important for older patients, who are less likely to benefit from current treatments.

Researchers are studying many new chemo drugs for AML, including:

- **Sapacitabine**, a drug that has shown promise as a treatment option for older patients with AML
- **Laromustine**, a drug also being tested as an option for older adults with AML
- **Guadecitabine**, a drug which has also shown promise in early studies.

The effectiveness of chemo may be limited in some cases because the leukemia cells become resistant to it over time. Researchers are now looking at ways to prevent or

reverse this resistance by using other drugs along with chemo. They are also looking at combining chemo with a number of newer types of drugs to see if this might work better.

Stem cell transplants

Researchers continue to refine [stem cell transplants](#)² to try to increase their effectiveness, reduce complications, and determine which patients are likely to be helped by this treatment. Many studies are trying to determine exactly when autologous, allogeneic, and mini-transplants might best be used.

Targeted therapy drugs

Chemo drugs can help many people with AML, but these drugs don't always cure the disease. Newer [targeted drugs](#)³ that specifically attack some of the gene changes seen in AML cells are now becoming an important part of treatment for some people with AML. These drugs don't work the same as standard chemotherapy drugs. Some examples include:

- **FLT3 inhibitors.** In some people with AML, the leukemia cells have a mutation in the *FLT3* gene. Newer drugs called FLT3 inhibitors target cells with this gene change. Midostaurin (Rydapt) and gilteritinib (Xospata) are now approved to treat people whose AML has an *FLT3* mutation. Other FLT3 inhibitors, such as crenolanib, have also shown activity against AML in early studies, and are now being studied further.
- **IDH inhibitors.** In some people with AML, the leukemia cells have a mutation in the *IDH1* or *IDH2* gene, which stops the cells from maturing properly. IDH inhibitors can help the leukemia cells mature into normal blood cells. Some of these drugs, such as enasidenib (Idhifa) and ivosidenib (Tibsovo), are now approved to treat AML with certain *IDH* gene mutations. Several other IDH inhibitors are now being studied as well.
- **Histone deacetylase (HDAC) inhibitors**, such as vorinostat (Zolinza) and panobinostat (Farydak)
- **BCL-2 inhibitors**, such as venetoclax (Venclexta)
- **Polo-like kinase (Plk) inhibitors**, such as alisertib

Immunotherapy

Immunotherapy works to boost the body's immune system to help fight off or destroy

cancer cells.

Monoclonal antibodies: These are man-made versions of immune system proteins (antibodies) that are designed to attach to specific targets, such as substances on the surface of cancer cells. Some work by boosting the body's immune response against the cancer cells. Others have radioactive chemicals or cell poisons attached to them. When they are injected into the patient, these antibodies act like a homing device, bringing the radioactivity or poison directly to the cancer cells, which kills them.

[Gemtuzumab ozogamicin \(Mylotarg\)⁴](#) is a monoclonal antibody with a cell poison attached to it. It is now approved to treat AML in some patients. Several other monoclonal antibodies are now being studied as well.

Immune checkpoint inhibitors: An important part of the immune system is its ability to keep itself from attacking other normal cells in the body. To do this, it uses “checkpoints” – molecules on immune cells that need to be turned on (or off) to start an immune response. Cancer cells sometimes use these checkpoints to avoid being attacked by the immune system. But newer drugs that target these checkpoints hold a lot of promise as treatments. Some of these drugs are already being used to treat other types of cancer, and they are now being studied for use in AML as well.

CAR T-cell therapy: This is a promising new way to get the patient's immune system to fight leukemia. For this technique, immune cells called T cells are removed from the patient's blood and altered in the lab so they have specific substances (called chimeric antigen receptors, or CARs) that will help them attach to leukemia cells. The T cells are then grown in the lab and infused back into the patient's blood, where they can now seek out the leukemia cells and attack them.

This technique has shown very promising results in early clinical trials against some other types of advanced, hard-to-treat leukemias. Although it's not yet clear if it will work against AML, clinical trials are now in progress to find out. One concern with this treatment is that some people have had very serious side effects, including very high fevers and dangerously low blood pressure in the days after it's given. Doctors are learning how to manage these side effects.

Hyperlinks

1. www.cancer.org/cancer/acute-myeloid-leukemia/treating/chemotherapy.html
2. www.cancer.org/cancer/acute-myeloid-leukemia/treating/bone-marrow-stem-cell-transplant.html
3. www.cancer.org/cancer/acute-myeloid-leukemia/treating/targeted-therapy.html

4. www.cancer.org/cancer/acute-myeloid-leukemia/treating/targeted-therapy.html

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