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About Acute Myeloid Leukemia (AML)

Get an overview of acute myeloid leukemia and the latest key statistics in the US.

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)?

Normal bone marrow, blood, and lymph tissue

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 <u>Acute Myeloid Leukemia (AML) Subtypes and Prognostic Factors</u>².

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 bloodforming 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/understanding-cancer/what-is-cancer.html
- 2. <u>www.cancer.org/cancer/types/acute-myeloid-leukemia/detection-diagnosis-staging/how-classified.html</u>
- 3. www.cancer.org/cancer/types/leukemia-in-children.html

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Last Revised: August 21, 2018

Key Statistics for Acute Myeloid Leukemia (AML)

The American Cancer Society's estimates for acute myeloid leukemia (AML) in the United States for 2024 are:

- About 20,800 people will be diagnosed with AML. Most cases will be in adults.
- About 11,220 people will die from AML. Again, most of these deaths will be in adults.

AML accounts for about 1 out of 3 leukemias in adults. Still, AML isn't common, accounting for about 1% of all cancers overall.

AML can occur in children¹, but it is uncommon in people under the age of 45. The average age of people when they are first diagnosed with AML is about 69.

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

Information on treatment success rates for AML in adults can be found in <u>Treatment</u> Response Rates for Acute Myeloid Leukemia².

Visit the American Cancer Society's Cancer Statistics Center³ for more key statistics.

Hyperlinks

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

References

American Cancer Society. *Cancer Facts & Figures 2024*. Atlanta : American Cancer Society; 2024.

National Cancer Institute. SEER Cancer Stat Facts: Acute Myeloid Leukemia (AML). 2024. Accessed at https://seer.cancer.gov/statfacts/html/amyl.html on June 3, 2024.

Last Revised: June 5, 2024

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

Researchers continue to study the causes, diagnosis, and treatment of acute myeloid leukemia (AML) at many medical centers, university hospitals, and other institutions around the world.

- Genetics of acute myeloid leukemia (AML)
- Improving treatment for acute myeloid leukemia (AML)

Genetics of acute myeloid leukemia (AML)

Researchers continue to make progress in understanding how normal bone marrow cells can develop into leukemia cells. It has become clear that there are many types of AML. Each type of AML might have different DNA (gene) changes that affect how it will progress and which treatments might be most helpful. Researchers continue to study how DNA changes specific to different AML types can help us understand how to best treat each person's AML.

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.

Multiparameter flow-cytometry (MFC), quantitative polymerase chain reaction (qPCR), and next-generation sequencing (NGS) are tests that can be used to identify even very small numbers of AML cells in a biopsy sample. These tests are useful in determining how completely the treatment has destroyed the AML cells.

Studies are looking at how to best use the information from these tests. The presence of MRD affects a person's outlook, as well as if they will need further or more intensive treatment.

Improving treatment for acute myeloid leukemia (AML)

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. Studies are looking for more effective and safer treatments for AML. Doctors are studying how best to sequence and combine treatments for AML to best fight the disease.

Chemotherapy

Chemotherapy¹ (chemo) is still the main treatment for most types of AML.

Researchers are looking for the most effective combination of chemo drugs that will also

limit unwanted side effects. This is especially important for older people who might not be able to tolerate the side effects of current treatments for AML.

The effectiveness of chemo may be limited in some cases because the leukemia cells can become resistant to it over time. Research is 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 newer types of drugs to see if this might work better.

Stem cell transplants

Researchers continue to refine <u>stem cell transplants</u>² to try to increase their effectiveness, reduce complications, and determine which people are most 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 <u>targeted drugs</u>³ that specifically attack some of the gene changes seen in AML cells have become an important part of treatment for some people. These drugs don't work the same way as standard chemo drugs. Some examples include:

FLT3 inhibitors: In some people with AML, the leukemia cells have a change (mutation) in the *FLT3* gene. Drugs called FLT3 inhibitors target AML cells with this gene change. FLT3 inhibitors such as midostaurin (Rydapt), quizartinib (Vanflyta), and gilteritinib (Xospata) are now approved to treat people whose AML cells have an *FLT3* mutation. Several other FLT3 inhibitors are now being studied as well.

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 leukemia cells mature into normal blood cells. Some of these drugs, such as enasidenib (Idhifa), olutasidenib (Rezlidhia), and ivosidenib (Tibsovo), are now approved to treat AML with certain *IDH* gene mutations. Several other IDH inhibitors are now being studied as well.

BCL-2 inhibitors: Some people with AML have leukemia cells that make too much of a protein called BCL-2. Leukemia cells that have too much BCL-2 tend to be harder to kill with chemo drugs. BCL-2 inhibitors prevent the BCL-2 protein from working in cancer cells. Venetoclax (Venclexta) is a BCL-2 inhibitor that has been approved to treat AML with too much BCL-2 protein. Several other BCL-2 inhibitors are being studied as well.

Researchers are also looking at newer types of targeted drugs to treat AML.

Immunotherapy drugs

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

Bispecific antibodies: A bispecific antibody consists of two antibodies that each attach to a different target. Once inside the body, this type of drug can act as a link to bring two types of cells close together. One antibody is usually designed to attach to a target on the leukemia cell, while the other is designed to attach to a target on immune cells (for example, T cells). When the bispecific antibody brings the leukemia cell and immune cell together, the immune system is alerted and starts to fight the leukemia cell. Several bispecific antibodies are now being studied for use against AML.

Antibody-drug conjugates (ADC): An ADC is a drug with two parts: an antibody designed to attach to a surface protein on cancer cells and a toxin meant to kill the cancer cells. When ADCs are injected into the body, they act like a homing device, bringing the toxin directly to the cancer cells, which kills them. ADCs are already used to treat some types of cancer, and some ADCs are now being studied for use against AML.

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 'checkpoint' proteins 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. Drugs called **immune checkpoint inhibitors** (ICIs) target these checkpoint proteins. They are already used in many other cancers, and they are now being studied for use in AML, especially combined with chemo or targeted therapy drugs.

Chimeric antigen receptor (CAR) T-cell therapy: For this treatment, immune cells called T cells are removed from the person'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 person's blood, where they can now seek out the leukemia cells and attack them.

This therapy has been shown to work in other types of blood cancers, although it's not yet clear if it will work against AML. Researchers are continuing to study how this therapy might be used to treat AML.

Hyperlinks

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

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Last Revised: June 5, 2024

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