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LYMPHOMA
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The AML Guide

Information for Patients and Caregivers

Acute Myeloid Leukemia



Emily, AML survivor

Support for this publication
provided by



Revised 2015

The Leukemia & Lymphoma Society wants you to have the most up-to-date information about blood cancer treatment. See below for important new information that was not available at the time this publication was printed.

- In April 2017, the Food and Drug Administration (FDA) approved midostaurin (Rydapt®) for the treatment of adult patients with:
 - Newly diagnosed acute myeloid leukemia (AML) that is FLT3 mutation positive as detected by an FDA-approved test, in combination with standard cytarabine and daunorubicin induction and cytarabine consolidation.

Limitations of Use:

Rydapt is not indicated as a single-agent induction therapy for the treatment of patients with AML.

- Aggressive systemic mastocytosis (ASM), systemic mastocytosis with associated hematological neoplasm (SM-AHN), or mast cell leukemia (MCL).

For more information, contact an Information Specialist at (800) 955-4572 or infocenter@lls.org.

A Message From Louis J. DeGennaro, PhD

President and CEO of The Leukemia & Lymphoma Society

The Leukemia & Lymphoma Society (LLS) is the world's largest voluntary health organization dedicated to finding cures for blood cancer patients. Since 1954, we have invested more than \$1 billion in research specifically targeting blood cancers to advance therapies and save lives. We will continue to invest in research for cures, programs and services to improve the quality of life for people with acute myeloid leukemia (AML).

We know that understanding AML can be tough.

We are here to help and are committed to providing you with the most up-to-date information about AML, your treatment and your support options. We know how important it is for you to understand your health information. We want you to be able to use that information, along with the help of members of your healthcare team, on your pathway toward good health, remission and recovery.

Our vision is that one day all people with AML will be cured or be able to manage their disease and have a good quality of life.

Until then, we trust the information in this *Guide* will help you along your journey.

We wish you well.

A handwritten signature in black ink, appearing to read "Louis J. DeGennaro". The signature is fluid and cursive, with a long horizontal stroke extending to the right.

Louis J. DeGennaro, PhD

*President and Chief Executive Officer
The Leukemia & Lymphoma Society*

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This LLS Guide about AML is for information only. LLS does not give medical advice or provide medical services.

Introduction

Acute myeloid leukemia (AML) is a type of blood cancer. Another name for AML is “acute myelogenous leukemia.” AML is the most common acute leukemia affecting adults.

Remission rates for people who have AML are improving. Remission means that no AML cells are found in either the blood or marrow and blood cell counts are back to normal. But more work needs to be done. New treatment approaches are under study. There are new treatment studies (called “clinical trials”) for patients

- Who have been diagnosed with any type of AML
- Of all ages and in all stages of treatment.

More information about clinical trials begins on page 25.

About 18,860 people in the United States were expected to be diagnosed with AML in 2014.

About 37,726 people in the United States are either living with, or are in remission from, AML.

People who have AML need to see special blood cancer doctors, called “hematologist-oncologists” to help them. Please use this *Guide*

- As a resource to help you understand AML
- To find good doctors and other healthcare providers
- To understand complicated healthcare terms
- To access and use our Information Specialists, healthcare information, booklets and other resources.

This *Guide* includes

- Information on how to contact our Information Specialists: call (800) 955-4572

- Links to important free LLS disease and treatment information: www.LLS.org/booklets
- Information about AML, diagnosis, treatment and care
- Lists of suggested questions to ask about selecting a doctor and about treatment (see page 36)
- Simplified glossary of healthcare terms (see page 32).

We are here to help.

Want more information?



You can view, print or order the free LLS booklet *Acute Myeloid Leukemia* at www.LLS.org/booklets or contact our Information Specialists for a copy.

Resources and Information

LLS offers free information and services for patients and families affected by blood cancers. This section of the *Guide* lists various resources available to you. Use this information to learn more, to ask questions and to make the most of your healthcare team.

For Help and Information

Consult with an Information Specialist. Information Specialists are master's level oncology social workers, nurses and health educators. They offer up-to-date information about disease, treatment and support. Language services are available. For more information, please

- Call: (800) 955-4572 (Monday through Friday, 9 a.m. to 9 p.m. ET)
- Email: infocenter@LLS.org
- Live chat: www.LLS.org/information specialists.

Free Information Booklets. LLS offers free education and support booklets that can either be read online or ordered. For more information, please visit www.LLS.org/booklets.

Telephone/Web Education Programs. LLS offers free telephone/Web education programs for patients, caregivers and healthcare professionals. For more information, please visit www.LLS.org/programs.

Community Resources and Networking

Online Blood Cancer Discussion Boards and Chats. Online discussion boards and moderated online chats can help cancer patients reach out, share information and provide and receive support. For more information, please visit www.LLS.org/disussionboard and www.LLS.org/chat.

LLS Chapters. LLS offers community support and services in the United States and Canada including the *Patti Robinson Kaufmann First Connection Program* (a peer-to-peer support program), in-person support groups and other great resources. For more information about these programs or to contact your chapter, please

- Call: (800) 955-4572
- Visit: www.LLS.org/chapterfind.

Other Helpful Organizations. LLS offers an extensive list of resources for patients and families. There are resources that provide help with financial assistance, counseling, transportation, locating summer camps and other needs. For more information, please visit www.LLS.org/resourcedirectory.

Clinical Trials (Research Studies). New treatments for patients are under way. Many are part of clinical trials. Patients can learn about clinical trials and how to access them. For more information, please

- Call: (800) 955-4572 to speak with an LLS Information Specialist who can help conduct a clinical-trial search
- Visit: www.LLS.org/clinicaltrials.

Advocacy. The LLS Office of Public Policy (OPP) enlists volunteers to advocate for policies and laws to speed new treatments and improve

access to quality medical care. For more information, please

- Call: (800) 955-4572
- Visit: www.LLS.org/advocacy.

Additional Help for Specific Populations

Información en Español (LLS information in Spanish). For more information, please visit www.LLS.org/espanol.

Language Services. Let your doctor know if you need a language interpreter or other resource, such as a sign language interpreter. Often, these services are free.

World Trade Center Survivors. People involved in the aftermath of the 9/11 attacks and subsequently diagnosed with a blood cancer may be able to get help from the World Trade Center (WTC) Health Program. People eligible for help include

- Responders
- Workers and volunteers who helped with rescue, recovery and cleanup at the WTC-related sites in New York City (NYC)
- Survivors who were in the NYC disaster area and those who lived, worked or were in school in that area
- Responders to the Pentagon and the Shanksville, PA crashes.

For more information, please

- Call: WTC Health Program at (888) 982-4748
- Visit: www.cdc.gov/wtc/faq.html.

Depression. Treating depression has benefits for cancer patients. Seek medical advice if your mood does not improve over time, for example, if you feel depressed every day for a two-week period. For more information, please

- Call: National Institute of Mental Health (NIMH) at (866) 615-6464
- Visit: NIMH at www.nimh.nih.gov, enter “depression” in the search box.

Feedback. To provide your opinion of this *Guide*.

- Visit: www.LLS.org/publicationfeedback.

Part 1

Understanding AML

Leukemia is the general term for some different types of blood cancer. AML is one of four main types of leukemia.

About Blood

Blood is the red liquid that circulates in our bodies. It is created inside the bones, in a spongy place called the “marrow.”

Blood is made up of plasma and blood cells.

Plasma. This is the liquid part of the blood. It is mostly water. It also has some vitamins, minerals, proteins, hormones and other natural chemicals.

Blood cells. Each blood cell starts as a stem cell. Then it turns into a specific kind of blood cell. There are three types of blood cells, they are

- Blood platelets (allow blood to clot)
- White blood cells (fight infection)
- Red blood cells (carry oxygen).

Normal Blood Cell Count Fast Facts

The ranges of blood cell counts below are for adults. These may be a little different from lab to lab and the ranges may be different for children and teens.

Red blood cell (RBC) count

- Men: 4.5 to 6 million red cells per microliter of blood
- Women: 4 to 5 million red cells per microliter of blood

Hematocrit (HCT) (the part of the blood made up of red cells)

- Men: 42% to 50% of the total volume of blood
- Women: 36% to 45% of the total volume of blood

Hemoglobin (Hgb) (amount of the red cell pigment that carries oxygen)

- Men: 14 to 17 grams per deciliter of blood
- Women: 12 to 15 grams per deciliter of blood

Platelet count (PLAT C)

- 150,000 to 450,000 platelets per microliter of blood

White blood cell (WBC) count

- 4,500 to 11,000 white cells per microliter of blood

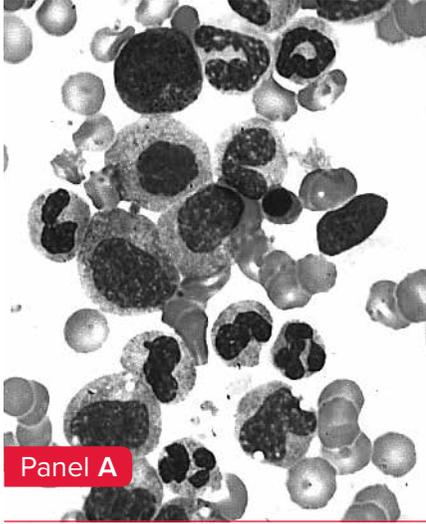
Differential (also called diff)

- Shows the different types of white cells and provides the percentage of each type present in the blood.
- Types of white cells counted are neutrophils, lymphocytes, monocytes, eosinophils and basophils.
- Adults usually have about 60% neutrophils, 30% lymphocytes, 5% monocytes, 4% eosinophils and less than 1% basophils in their blood.

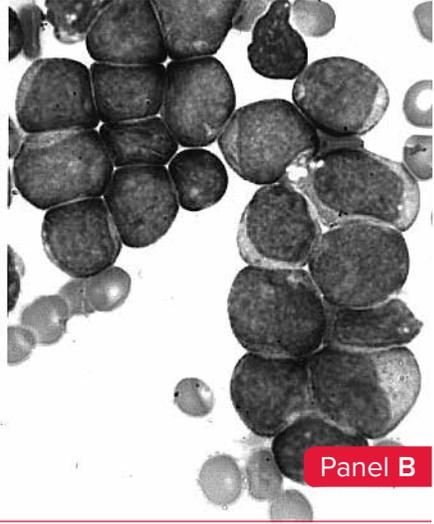
What is AML?

AML is a type of cancer that begins in the bone marrow. The chance of getting AML increases with age. But a person can get AML at any age. About 8 in 10 adults with acute leukemia have AML. About 1 in 6 children with leukemia has AML.

Normal Marrow Cells and AML Blast Cells



Panel A



Panel B

The cells in panels A and B are shown much larger than actual size. The cells are also stained with a special dye so that they can be seen more clearly.

Panel A shows different types of normal marrow cells seen through a microscope. These normal cells are in various stages of development.

Panel B shows AML blast cells seen through a microscope. These cells have stopped developing.

Causes and Risk Factors of AML. AML starts with a change to a single cell in the bone marrow. Doctors do not know what causes most cases of AML. There is no way to prevent AML. You can't catch AML from someone else.

Certain things may increase the risk of getting AML, such as

- Some types of chemotherapy

- Radiation therapy used to treat cancer
- Genetic disorders such as Fanconi anemia, Shwachman syndrome, Diamond-Blackfan syndrome and Down syndrome
- Tobacco smoke
- Repeated contact with the chemical benzene.

Benzene can harm normal marrow cells. Most benzene in the environment comes from petroleum products; however, half of the personal exposure to benzene comes from cigarette smoke. The average smoker is exposed to about 10 times the daily intake of benzene compared to that of nonsmokers. Benzene is also found in some work settings. But, strict rules have led to lowered amounts of benzene in the workplace.

Most people with these risks do not get AML.

Signs and Symptoms

Many of the signs and symptoms of AML are also caused by other types of illness. Most people with these signs and symptoms do not have AML.

A “sign” is a change in the body that the doctor sees in an exam or a note on a test result.

A “symptom” is a change in the body that the patient can see or feel.

Some Signs and Symptoms of AML

Not feeling well	Having fewer normal bone marrow cells may cause aches in the legs, arms or hips. Patients may have a mild fever, enlarged lymph nodes or swollen gums.
Tiring more easily, shortness of breath, pale skin color	People who have fewer healthy red cells may have lower energy levels. They may also feel short of breath while doing day-to-day activities. Some people with fewer red cells may have a pale skin color.
Weight loss	Some people with AML lose weight because they eat less and/or they use more energy.
Black-and-blue marks or pinhead-sized red spots	A low platelet count may cause patients to bruise more easily or to have tiny red spots called “petechiae” on the skin.
Bleeding for a long time from minor cuts	A low platelet count may lead to a longer bleeding time or slower healing of cuts.

Diagnosing AML

It is important for patients to receive the right diagnosis. AML is diagnosed on the basis of blood and bone marrow test results.

Blood Cell Counts. The doctor orders a test called a “CBC” (complete blood count) to count the numbers of red cells, white cells and platelets. Usually, patients with AML have lower-than-expected numbers of red blood cells and platelets.

Blood Cell Examination. The cells are stained (dyed) and looked at through a microscope. This test is also called a “blood smear.” A person with AML usually has too many leukemic blast cells in the blood. These cells do not function like normal cells.

Bone Marrow Tests. Your doctor will also do other tests to confirm the diagnosis of AML. Tests called “bone marrow aspiration” and “bone marrow biopsy” are done to find out the percentage of AML cells in the marrow. Usually, at least 20 percent of the cells in the bone marrow of a person with AML are AML cells.

Flow Cytometry. Your doctor will order a test called “flow cytometry” to identify the subtype of AML you have.

Cytogenetic Analysis. Other tests are done to look for genetic changes in your AML cells. This examination of AML cells is called “cytogenetic analysis.” The results help your doctor to plan your treatment.

Tracking Your AML Tests

The following tips may help you to save time and to know more about the status of your health:

- Ask your doctor why certain tests are being done and what to expect.
- Discuss test results with your doctor.
- Ask for and keep copies of lab reports in a file folder or three-ring binder. Organize test reports in date order.
- Find out if and when follow-up tests are needed.
- Mark appointments that are coming up on your calendar.

Want more information?



You can view, print or order the free LLS booklet *Understanding Lab and Imaging Tests* at www.LLS.org/booklets to learn more about lab tests and what to expect or contact our Information Specialists for a copy.

Subtypes of AML

Knowing the patient's AML subtype helps the doctor to plan treatment. Originally the AML subtypes were defined by the French, American, British (FAB) classification system. Most people with AML have one of the eight AML subtypes: M0, M1, M2, M3, M4, M5, M6 or M7. Most subtypes of AML are treated in the same way. But patients with subtype M3 (acute promyelocytic leukemia, or APL for short) need a different treatment plan. More information about APL treatment is on page 22.

The World Health Organization (WHO) developed a new classification system in 1999. The AML subtypes in this system include

- AML with recurrent genetic abnormalities
- AML (megakaryoblastic) with a translocation between chromosomes 1 and 22
- AML with myelodysplasia-related changes
- AML related to previous chemotherapy or radiation
- AML not otherwise categorized (does not fall into above categories—similar to FAB classification)
- Myeloid sarcoma (also known as “granulocytic sarcoma,” “chloroma” or “extramedullary myeloblastoma”)
- Undifferentiated and biphenotypic acute leukemias (also known as mixed “phenotype acute leukemias”).

For more information about the FAB and WHO classification system, please see the free LLS booklet *Acute Myeloid Leukemia* at www.LLS.org/booklets or call an Information Specialist.

Treating AML

Finding the Right Doctor

Patients with AML are treated by doctors called “hematologist-oncologists” who are specially trained in treatment of both blood disorders and cancer.

Finding the right doctor, one who you feel comfortable with, is important. The following resources can help you find the right cancer specialist:

- Your primary care doctor
- The community cancer center
- Your health plan and its referral services
- LLS resources such as our Information Specialists who can be reached from Monday to Friday 9 a.m. to 9 p.m. at (800) 955-4572
- Online doctor-finder resources, such as
 - The American Medical Association’s (AMA) “DoctorFinder”
 - The American Society of Hematology’s (ASH) “Find a Hematologist.”

Once you find a doctor to treat AML, he or she will help you understand the disease and create a treatment plan. When you meet with your doctor

- Ask questions
 - Use the two question guides on pages 36 and 38 to help
 - Read and print LLS “What to Ask” question guides at www.LLS.org/whattoask
- Take notes or bring an audio recorder to record your visits.
- Bring a friend or family member who can listen to the doctor as well and even take notes.

- Make sure you understand what the doctor is saying. If you don't, ask the doctor to explain so you can understand.
- If you need more information, consider getting another opinion (a second opinion from another qualified doctor).

Want more information?



You can view, print or order the free LLS booklet *Choosing a Blood Cancer Specialist or Treatment Center* at www.LLS.org/booklets or contact our Information Specialists for a copy.

Treatment Planning

The goal of treatment for AML is to cure the disease.

- Almost half of children with AML are cured.
- Patients with acute promyelocytic leukemia (APL) have higher cure rates overall compared to adults with other AML subtypes.
- Some adults with other subtypes of AML may be cured or have long periods of remission.
- There has been improvement in treatment outcomes for people with all subtypes of AML.

Some things that may affect the outcome of your AML treatment are

- Your subtype of AML
- The results of your lab tests
- Your age and general health
- Your medical history, including whether you
 - Were treated with chemotherapy before for another type of cancer
 - Had a myelodysplastic syndrome (MDS)

- Whether you have
 - A serious infection at the time of diagnosis
 - AML in your central nervous system
 - AML that has not responded to treatment or has relapsed.

It is important to talk to your doctor about the results of your bone marrow examination. This test provides information about the marrow cells that is needed for treatment planning. It is also important to talk to your doctor about molecular and genetic tests.

Want more information?



For lists of questions to ask about finding a doctor and about your treatment, see the question guides on pages 36 and 38. You can also access and print “What to Ask” question guides about second opinions and other topics at www.LLS.org/whattoask or contact our Information Specialists for copies.

About AML Treatments

Treatment for patients who have AML (or relapsed AML) may include

- Chemotherapy
- Stem cell transplantation
- New approaches under study (clinical trials).

A patient may receive different drugs from those described in this *Guide* and still be receiving proper treatment.

Speak to your doctor to find out what treatment is best for you. Our Information Specialists can help you plan questions to ask your doctor about treatment.

Treatment Related to Chromosome and Gene Changes. Your bone marrow will be examined to see if there are changes to your

chromosomes and genes. This information helps your doctor to assess risk and it is an important part of treatment planning. About 60 percent of people with AML have abnormal chromosomes. Talk to your doctor about additional tests that may need to be done to figure out the best treatment plan for you.

Induction Therapy. Induction therapy is the first part of treatment using chemotherapy. Most AML patients need to start induction chemotherapy right away. Induction therapy is done in the hospital. Patients are often in the hospital for 4 to 6 weeks for this first part of treatment.

The aim of induction therapy is to

- Kill as many AML cells as possible
- Get blood counts back to normal
- Get rid of all signs of the disease for an extended period of time.

Chemotherapy and other drugs. Chemotherapy drugs and other types of drugs kill or damage cancer cells. Several types of drugs are used to kill AML cells. Each drug type works in a different way. Combining the drugs can make the treatment work better.

The first round of chemotherapy usually does not get rid of all the AML cells. Most patients will need more rounds of therapy. Usually the same drugs are used for the additional rounds.

When no AML cells are found in either the blood or marrow, the disease is said to be “in remission.”

Want more information?



You can view, print or order the free LLS booklet *Acute Myeloid Leukemia* at www.LLS.org/booklets or contact our Information Specialists for a copy.

Some drugs are given by mouth. Other drugs are given through a port into a central line, or “peripherally inserted central catheter” (PIC or PICC). Medications, nutrition and blood products can all be administered via central lines or PICCs. Blood for lab tests can also be withdrawn via the ports for these lines. Ports, central lines and PICCs can stay in place for weeks or months. You can talk to your doctor about which type of line will work best for you or your child.

Central Lines, Ports and PICCs

Central Line

This is a thin tube that is put under the skin and into a large vein in the chest. The central line stays firmly in place. “Catheter” is another word for central line.

Port

This is a small device that is used to access a central line. The port is placed under the skin of the chest and attached to the end of the catheter. After the site heals, no dressings are needed and no special home care is needed. To give medicines or nutrition or to take blood samples, the doctor or nurse puts a needle through the skin into the port. A numbing cream can be put on the skin before the port is used.

PICC or PIC Line

“PICC” or “PIC line” is short for percutaneously inserted central venous catheter. A PICC is inserted through a vein in the arm.

Some of the drugs used to treat AML are listed on page 18. These are some of the standard drugs used and some of the drugs under study in AML clinical trials.

Some Drugs Used to Treat AML or in Clinical Trials

Anthracyclines (Antitumor Antibiotics)

daunorubicin (Cerubidine®)
doxorubicin (Adriamycin®)
idarubicin (Idamycin®)
mitoxantrone (Novantrone®)

Antimetabolites

cladribine (2-CdA; Leustatin®)
clofarabine (Clolar®)
cytarabine (cytosine arabinoside,
ara-C; Cytosar-U®)
fludarabine (Fludara®)
methotrexate
6-mercaptopurine (Purinethol®)
6-thioguanine (Thioguanine
Tabloid®)

Topoisomerase Inhibitors

etoposide (VP-16; VePesid®,
Etopophos®)
topotecan (Hycamtin®)

DNA Damaging (Alkylating) Agents

cyclophosphamide (Cytoxan®)
carboplatin (Paraplatin®)
temozolomide (Temodar®)

Cell-Maturing Agents

all-*trans* retinoic acid (ATRA,
tretinoin; Vesanoid®)
arsenic trioxide (Trisenox®)

Hypomethylating Agents

azacitidine (Vidaza®)
decitabine (Dacogen®)

Immunomodulator

Lenalidomide (Revlimid®)

Histone deacetylase inhibitors

panobinostat (Farydak®)
vorinostat (Zolinza®)

Antibody conjugate

gemtuzumab ozogamicin
(Mylotarg®)

FLT3 inhibitors

quizartinib (AC-220)
sorafenib
midostaurin

This table lists some drugs used in standard treatment for AML and some drugs under study in clinical trials. A patient may be treated with drugs that are not listed in this table and still be receiving appropriate and effective treatment.

Want more information?



You can view, print or order the free LLS booklet *Understanding Side Effects of Drug Therapy* at www.LLS.org/booklets or contact our Information Specialists for a copy.

Radiation Therapy. Sometimes radiation therapy may be used to treat a large mass of AML cells in the spine or brain called a “chloroma.”

Postremission Therapy. More treatment is usually needed even after an AML patient is in remission because some AML cells may remain that are not found by common blood or marrow tests. This part of AML treatment is called “postremission therapy” or “consolidation therapy.” Chemotherapy is part of postremission therapy for AML. Stem cell transplantation may be part of postremission therapy for some AML patients.

The treatment setting (hospital or outpatient) and the length of hospital stay depend on the postremission treatment. The length of time in the hospital also depends on any side effects of treatment. Patients are often in the hospital for 4 to 6 weeks. Some patients may need to be in the hospital longer.

Stem Cell Transplantation

Your doctor will talk with you about whether stem cell transplantation is a treatment option for you. Information about different types of transplants follows.

Allogeneic Stem Cell Transplantation. An allogeneic transplant uses stem cells from a donor. The donor may be a brother or sister. Or, the donor can be an unrelated person with stem cells that “match” the patient’s. Stem cells may also come from a cord blood unit (the blood in the umbilical cord after a baby’s birth).

The goals of an allogeneic transplant are to

- Restore the body’s ability to make normal blood cells after high-dose chemotherapy
- Cure the patient of his or her AML by killing any remaining AML cells.

Allogeneic transplants may be done in the hospital. First, the patient is given high-dose chemotherapy and/or radiation therapy. Stem cells are collected from a donor. The donor stem cells are given to the patient through an IV (intravenous) line or central line. The donor stem cells go from the patient's blood to the marrow and help start a new supply of red cells, white cells and platelets.

Your doctor will talk with you about whether an allogeneic transplant is a treatment option for you.

Allogeneic stem cell transplantation is a high-risk procedure. Doctors are working to make allogeneic transplants safer. An allogeneic transplant may be a choice for an AML patient if

- He or she has a subtype of AML that is hard to treat.
- The expected benefits of an allogeneic transplant exceed the risks.
- There is a stem cell donor.

The upper age limit for an allogeneic transplant depends on the treatment center. Many centers use age 60 or 65 years as the upper age limit for an allogeneic transplant.

Reduced-Intensity Allogeneic Stem Cell Transplantation. Your doctor will talk to you about whether a reduced-intensity allogeneic stem cell transplant is a treatment option for you. The goal of a reduced-intensity transplant is to cure the patient of his or her AML. Lower doses of chemotherapy are used for a reduced-intensity allogeneic transplant than are used in a standard allogeneic transplant. Some older and sicker patients may be helped by this treatment. Many centers use 70 years or older as the upper age limit for a reduced-intensity allogeneic transplant.

A reduced-intensity allogeneic transplant may be a choice for an AML patient if

- He or she has a type of AML that is hard to treat.

- A standard allogeneic transplant is not a choice because of the patient's age or overall health.
- The expected benefits of a reduced-intensity allogeneic transplant exceed the risks.
- There is a stem cell donor.

Autologous Stem Cell Transplantation. An autologous transplant uses the patient's own stem cells.

The goal of an autologous transplant is to restore the body's ability to make normal blood cells after high-dose chemotherapy. The patient's own blood or marrow stem cells are stored before chemotherapy begins. They are infused back into the patient's blood after chemotherapy ends. The stem cells are given back to the patient through an IV line or central line. The stem cells go from the patient's blood to the marrow and help start a new supply of red cells, white cells and platelets.

Your doctor will talk with you about whether an autologous transplant is a treatment option for you. Patients who have not responded well to treatment and do not have a matched donor for an allogeneic transplant may be given very high doses of chemotherapy and an autologous transplant.

Want more information?



You can view, print or order the free LLS booklets *Blood and Marrow Stem Cell Transplantation* and *Cord Blood Stem Cell Transplantation* at www.LLS.org/booklets or contact our Information Specialists for copies.

Treatment for Relapsed or Refractory AML

Some patients have a remission after treatment, but then AML cells return later (a relapse). Patients may still have AML cells in the marrow even after treatment (refractory AML).

Patients who relapse may be treated with the same drugs as newly diagnosed patients—or different drugs may be given. Patients who have a matched donor may be given an allogeneic stem cell transplant. Information about allogeneic stem cell transplantation begins on page 19.

With refractory AML, drugs that were not used in the first round of treatment may be given. An allogeneic transplant may also be a part of treatment.

Acute Promyelocytic Leukemia (APL) Treatment

APL is the M3 subtype of acute myeloid leukemia (AML). It is the most curable subtype of AML. Patients with APL need different treatment than patients with other AML subtypes.

APL patients are treated with the drug all-*trans* retinoic acid (ATRA). ATRA is also called tretinoin (Vesanoid®). Until recently, APL treatment combined ATRA with anthracycline-based chemotherapy during both the induction and consolidation phases of treatment. Recent research has shown that the addition of arsenic trioxide (ATO) in consolidation therapy may provide equal or even better results than the standard therapy alone. Other studies indicate that the combination of ATRA and ATO, without chemotherapy, may be sufficient treatment for patients who are considered favorable- or low-risk.

ATO is also the recommended therapy for patients who do not achieve a molecular remission at the end of consolidation or who relapse later on in the treatment. Allogeneic stem cell transplantation may be also be advised, if a human leukocyte antigen (HLA) matched donor is available. Patients who do not have a donor, or cannot have an allogeneic stem cell transplant for other reasons, may be candidates for an autologous stem cell transplantation. Stem cell transplant information is on pages 19 to 21.

All patients treated for AML, including patients with APL, need long-term follow-up care. Some patients may require further therapy.

Supportive Care for APL. Specific supportive care may be necessary when treating patients with APL. A patient who is being treated for APL may experience fluid retention, labored breathing, fluid accumulation around the heart or lungs, and episodes of low blood pressure. This group of symptoms is known as “differentiation syndrome.” Patients must be closely monitored for the development of these symptoms since differentiation syndrome, along with hemorrhage, are the leading causes of death during induction therapy. Finding these symptoms early and starting corticosteroid therapy right away, are essential to managing this potential complication.

Blood clots are a common concern for patients who have APL. There are specific blood tests to check newly diagnosed patients for this problem and it is important to have those tests done.

Acute Monocytic Leukemia Treatment

Acute monocytic leukemia is the M5 subtype of acute myeloid leukemia (AML). AML cells can invade the lining of the spinal canal or brain. The risk for this happening is greater for patients who have acute monocytic leukemia.

A common medical procedure called a “lumbar puncture” (also called a “spinal tap”) is used to check the spinal fluid for AML cells. This procedure is done while the patient is either sedated or has been given local anesthesia. A needle is placed into the spinal canal. Then, the spinal fluid is removed and checked for AML cells.

The spinal cord and brain are parts of the body that aren’t easily reached with IV chemotherapy. So, if AML cells are found there, fluid that contains drugs, usually cytarabine or methotrexate, is injected into the spinal canal to kill the AML cells.

After treatment, lumbar punctures are repeated from time to time so your doctor can check whether the AML cells are being killed.

AML Treatment in Children

There are about 4,100 new cases of leukemia each year in the United States in children, adolescents and young adults younger than 20 years.

Induction therapy for children with AML starts with two or three drugs. More treatment is needed after a child with AML is in remission (postremission therapy). It is given because some AML cells may remain after induction therapy. These AML cells do not show up in standard blood or marrow tests. Postremission therapy in children includes a number of chemotherapy drugs.

AML treatment is less likely to bring about a remission or cure in children who

- Have very high white cell counts
- Are younger than 2 years old
- Have certain chromosome changes in their AML cells.

Allogeneic stem cell transplantation may be used in children who are not doing well or whose AML returns after high-dose chemotherapy. Doctors will discuss the benefits and risks of allogeneic transplantation with parents and older children.

Children who receive intensive chemotherapy, including anthracyclines, should be monitored by their doctor since these drugs may affect the heart. Exams of kidney function and hearing are also recommended.

Want more information?



You can view, print or order the free LLS booklets *Coping With Childhood Leukemia and Lymphoma*; *Learning & Living With Cancer—Advocating for your child's educational needs*; *Pictures of My Journey*; and *The Stem Cell Transplant Coloring Book* at www.LLS.org/booklets or contact our Information Specialists for copies.

AML Treatment in Older Patients

AML is more common in older patients. At least half of patients are over 65 years old when their disease is diagnosed.

Today, remissions are possible for some older people with AML, including those who may have other serious health problems. But treatment results in adults are not as good as treatment results in children.

Some healthy older patients can be treated with the same doses of chemotherapy as younger adults. Sometimes older patients have other medical problems, such as heart disease, kidney or lung disease or diabetes. The doctor takes these other medical problems into account to decide which drugs to use and in what dosage.

The doctor will also consider

- The type of AML
- The patient's physical ability to handle the treatment
- The patient's feelings about the treatment approach.

Some older patients may undergo a reduced-intensity allogeneic stem cell transplant. See page 20 for more information.

Part 3

About Clinical Trials

Doctors may recommend that a patient join a clinical trial. Clinical trials are careful studies done by doctors to test new drugs or treatments, or to find new uses for approved drugs or treatments. For example, changing an amount or dose of a drug or giving a drug along with another type of treatment might be more effective. Some clinical trials combine drugs for AML in new sequences or dosages.

There are clinical trials for

- Newly diagnosed AML patients
- Patients who do not have a good response to treatment
- Patients who relapse after treatment.

A carefully conducted clinical trial may provide the best available therapy. Ask your doctor if treatment in a clinical trial is right for you. Drugs being studied in clinical trials are listed in *Some Drugs Used to Treat AML* on page 18. You can also call our Information Specialists who will give you information about clinical trials, or you can use our free clinical-trial service at www.LLS.org/clinicaltrials.

Want more information?



You can view, print or order the free LLS booklets *Understanding Clinical Trials for Blood Cancers* and *Knowing All Your Treatment Options* at www.LLS.org/booklets or contact our Information Specialists for copies.

Part 4

Side Effects and Follow-Up Care

Side Effects of AML Treatment

The term “side effect” is used to describe the way that treatment affects healthy cells.

The aim of treatment for AML is to kill AML cells. Treatment for AML also affects healthy cells. Side effects of AML treatment may be severe, but they usually go away once treatment ends. Ask your doctor about the side effects to expect from your treatment.

AML treatment may affect your blood cell counts in the following ways:

- The number of red blood cells may decrease (anemia). Red cell transfusions (red cells that are taken from a donor and given to the patient) may be needed to increase red cell counts.
- There may be a drop in the number of platelets. A platelet transfusion may be needed to prevent bleeding if a patient's platelet count is very low.
- A big drop in the number of white blood cells may lead to an infection. These infections are usually treated with antibiotics.

Fever or chills may be the only signs or symptoms of infection. Patients with an infection may also have

- Coughing
- Sore throat
- Pain when urinating
- Frequent, loose bowel movements.

Growth factors are sometimes given to increase the number of white blood cells. G-CSF (Neupogen®) and GM-CSF (Leukine®) are drugs that increase white cell counts.

Growth factors are only given to children in certain cases. Researchers are studying which children with AML are most likely to be helped by treatment with growth factors to prevent infection.

To lower the risk of infection

- The patient, the patient's visitors and medical staff need to wash their hands well.
- The patient's central line must be kept clean.
- Patients should take good care of their teeth and gums.

The doctor may talk about the "absolute neutrophil count" or the

“ANC.” This is the number of neutrophils (a type of white blood cell) that a person has circulating in his or her bloodstream. Neutrophils fight off infection.

Other Treatment Side Effects. Chemotherapy affects the parts of the body where new cells form quickly. This includes the inside of the mouth and bowel and the skin and hair. The following side effects are common during chemotherapy:

- Mouth sores
- Diarrhea
- Hair loss
- Rashes
- Nausea
- Vomiting.

Not all patients have these side effects. Treatment to prevent or manage nausea, vomiting, diarrhea and other side effects can help patients feel more comfortable.

Chemotherapy may cause the amount of uric acid to increase in the blood of some AML patients. (Some patients also have a buildup of uric acid from the disease itself.) Uric acid is a chemical made in the body. A high level of uric acid can cause kidney stones.

Patients with high uric acid levels may be given allopurinol (Aloprim[®], Zyloprim[®]) by mouth. Another drug used to treat high uric acid levels, rasburicase (Elitek[®]), is given IV.

Want more information?



You can view, print or order the free LLS booklet *Understanding Side Effects of Drug Therapy* at www.LLS.org/booklets or contact our Information Specialists for a copy.

Long-Term and Late Effects

Long-term effects are medical problems that last for months or years after treatment ends. Fatigue is an example.

Late effects are medical problems that do not show up until years after treatment ends. Heart disease is an example.

Children and adults who have been treated for AML need to see the doctor for follow-up care.

Children who are treated for AML may have

- Growth problems
- Fertility problems (ability to have children later on)
- Bone problems
- Heart problems
- Learning problems.

Adults who are treated for AML may have

- Fertility problems
- Thyroid problems
- Problems concentrating
- Persistent fatigue.

Patients should talk with their doctors about any long-term or late effects that may be related to their treatment. Parents should talk to the doctor about when their child's learning skills should be checked.

Want more information?



You can view, print or order the free LLS booklets *Long-term and Late Effects of Treatment for Childhood Leukemia or Lymphoma*; *Long-term and Late Effects of Treatment in Adults*; or *Fertility Facts* at www.LLS.org/booklets or contact our Information Specialists for copies.

Follow-up Care

Medical follow-up is important for every AML patient. Children and adults who have been treated for AML should see their primary care doctor and an oncologist (cancer specialist) for follow-up care. At follow-up visits, the doctor will check the patient carefully to see if more treatment is needed.

Follow-up care includes physical exams and blood tests. Sometimes marrow tests are also needed. Patients should talk to the doctor about how often to have follow-up visits. They can ask what tests they will need—and find out how often to have the tests.

The doctor may advise longer periods of time between follow-up visits if a patient

- Continues to be free of signs of AML
- Does not need medical care for any long-term or late effects.

Ongoing Care

It is important that you take good care of yourself. Review the following tips to keep yourself healthy:

- Keep all appointments with the doctor.
- Talk about how you feel with the doctor at each visit.
- Ask any questions you may have about side effects.
- People with AML may have more infections. Follow the doctor's advice for preventing infection.
- Eat healthy food each day. It is okay to eat four or five small meals instead of three big ones.
- Contact the doctor about tiredness, fever or any other symptoms.
- Do not smoke. Patients who smoke should get help to quit.

- Get enough rest.
- Exercise, but first talk with your doctor before starting an exercise program.
- Keep a healthcare file with copies of lab reports and treatment records.
- Have regular cancer screenings for cancers other than AML.
- See your primary care doctor to keep up with other healthcare needs.
- Talk with family and friends about how you feel. When family and friends know about AML and its treatment, they may worry less.
- Seek medical advice if you feel “down” or “blue” or don’t want to do anything and your mood does not improve over time. For example, if you feel sad or depressed every day for two weeks, seek help. Depression is an illness. It should be treated even when a person is being treated for AML. Treatment for depression has benefits for people living with cancer.

Health Terms

Absolute Neutrophil Count (ANC). The number of neutrophils (a type of white cell) that a person has to fight infection. It is calculated by multiplying the total number of white blood cells by the percent of neutrophils.

Anemia. A decrease in the levels of hemoglobin in the blood.

Antibiotics. Drugs that are used to treat infections caused by bacteria and fungi. Penicillin is one type of antibiotic.

Basophil. A type of white blood cell that plays a part in allergies.

Blast cells. Early bone marrow cells. About 1 to 5 percent of normal marrow cells are blast cells.

Bone marrow aspiration. A procedure to remove and examine marrow cells to see if they are normal. A liquid sample containing cells is taken from the marrow and then the cells are looked at under a microscope.

Bone marrow biopsy. A procedure to remove and examine marrow cells to see if they are normal. A very small amount of bone filled with marrow cells is taken from the body, and the cells are looked at under a microscope.

Central line. Special tubing the doctor puts into a large vein in the upper chest to prepare a patient for chemotherapy treatment. The central line is used to administer chemotherapy drugs, infuse blood cells and remove blood samples. Also called an “indwelling catheter.”

Chemotherapy or drug therapy. Treatment with chemical agents to treat AML and other cancers.

Chloroma. A large mass of AML cells, which may be treated with radiation.

Chromosomes. Any of the 23 pairs of certain basic structures in human cells. Chromosomes are made up of genes. Genes give the instructions that tell each cell what to do. The number or shape of chromosomes may be changed in blood cancer cells.

Clinical trials. Careful studies done by doctors for new drugs or treatments or new uses for approved drugs or treatments. The goal of clinical trials for blood cancers is to improve treatment and quality of life and to find cures.

Combination chemotherapy or drug therapy. The use of two or more drugs together to treat AML and other cancers.

Consolidation therapy. Added treatment given to a cancer patient after the disease is in remission. It usually includes chemotherapy drugs not used during induction treatment. Also called “intensification therapy.”

Cytogenetic analysis. The examination of the chromosomes of AML cells to give doctors information about how to treat patients. Cell samples can be taken from blood or marrow.

Diagnose. To detect a disease from a person’s signs, symptoms and test results. The doctor diagnoses a patient.

Drug resistance. When a drug used to treat a patient’s disease does not work or stops working.

Eosinophil. A type of white cell that plays a part in allergies.

FDA. The short name for the US Food and Drug Administration. Part of the FDA’s job is to assure the safety and security of drugs, medical devices and the United States food supply.

Flow cytometry. This test is a method of measuring the number of cells in a sample, the percent of cells in a sample, the size and shape of the cell, and the presence of markers on the cell surface.

Genes. Parts of cells that give instructions for making proteins. Proteins help cells to do their jobs.

Hematologist. A doctor who treats blood cell diseases.

Hemoglobin. The part of the red cell that carries oxygen.

Immune response. The reaction of the body to foreign material. Examples of foreign material are an infection-causing microorganism, a vaccine or the cells of another person when those cells are used for an allogeneic stem cell transplant.

Immune system. Cells and proteins in the body that defend it against infection.

Immunoglobulins. Proteins that fight infection.

Immunophenotyping. A lab test that can be used to identify the type of AML cells.

Immunotherapy. The treatments that are used to boost the body's immune system.

Leukemia. A cancer of the marrow and blood.

Lymph nodes. Small bean-shaped organs around the body that are part of the body's immune system.

Marrow. The spongy material in the center of bones where blood cells are made.

Oncologist. A doctor who treats patients who have cancer.

Pathologist. A doctor who identifies disease by studying cells and tissues under a microscope.

Plasma. The liquid part of the blood.

Platelet. A type of blood cell that helps prevent bleeding. Platelets cause plugs to form in the blood vessels at the site of an injury.

Postremission therapy. The treatment given to AML patients after induction therapy. Postremission therapy may have two parts: consolidation (or intensification) and maintenance.

Radiation therapy. Treatment with x-rays or other high-energy rays.

Refractory AML. AML that has not responded to initial treatment. Refractory disease may be disease that is getting worse or staying the same (stable disease).

Relapsed AML. AML that responded to treatment but then returned.

Remission. No sign of the disease and/or a period of time when the disease is not causing any health problems.

Resistance. See Drug resistance.

Signs and symptoms. Changes in the body that show the presence of disease. A sign is a change that the doctor sees in an exam or a lab test result. A symptom is a change that a patient can see or feel.

Stem cell. A type of cell found in marrow that makes red cells, white cells and platelets.



Questions to Help You Choose a Specialist

Asking questions will help you take an active role in managing your (or your child's) care. If you do not understand any part of the information your healthcare provider gives you, ask him or her to explain it in another way.

Doctor's name _____

Date of appointment or call _____

- 1.** What is your board certification and licensing? Are you a member of any professional societies?

- 2.** How much experience do you have treating patients who have my disease?

- 3.** Is your hospital, university, center or clinic accredited and experienced in treating blood cancers?

- 4.** How long would I usually have to wait for appointments or return of my phone calls?

5. Will there be nurses, social workers and case managers available to help me with support needs and quality-of-life concerns?

6. Do you know of other oncologists who specialize in treating blood cancers? Would you recommend that I speak to any of them?

7. What types of things should I call you about? What types of things should I call my family doctor about?

8. How should I contact you when I have questions?

9. How do I contact you at night? On weekends? On holidays?

To print additional copies of this question guide, or to print copies of question guides on other topics, go to www.LLS.org/whattoask. You may also request that copies be sent to you by contacting our Information Specialists at (800) 955-4572.



Asking your healthcare provider questions at any phase of your treatment will help you take an active role in managing your (or your child's) care. If you do not understand any part of the information your healthcare provider gives you, ask him or her to explain it in another way.

Doctor's name _____

Date of appointment or call _____

- 1.** What are my (my child's) treatment options? What is the goal of the treatment?

- 2.** What are the FDA-approved treatments, and are there treatments being studied in clinical trials (study treatments), for my (my child's) diagnosis?*

- 3.** What are the benefits and risks of the treatment(s) available to me (my child)?
What are the expected side effects?

4. Is there one treatment option (FDA-approved or study treatment) that you recommend over the others? Please explain.

5. If I (my child) enroll(s) in a clinical trial, who will be in charge of my (my child's) treatment?

6. When do you think I (my child) will need to begin treatment?

7. How long will I (my child) be treated and how many treatments will be needed?

8. Will I (my child) need to be hospitalized for all or part of the treatment?

9. What kind of testing will be done to monitor my (my child's) disease and treatment? How often will testing be needed?

10. If I am treated at an out-patient clinic or at the doctor's office, will I be able to drive/get myself home after treatments or will I need someone to assist me?

11. What are the risks if I don't (my child doesn't) get treatment?

12. How will I know if the treatment is effective? What options are available if the treatment is not effective?

13. How do I find out if my insurance will cover the costs of my (my child's) treatment or the study treatment? Who can help answer any medical questions my insurance company or health plan asks?

14. If I do not have insurance coverage, how can the healthcare team help me (my child) get treatment? Is there someone I need to speak to for assistance?

15. If I'm (my child is) getting a study treatment, will I be responsible for paying any treatment-related costs, such as tests, travel or the clinical trial drug(s)?

16. Will the healthcare team continue to check on me (my child) after the treatment is over? If so, for what period of time?

17. I (My child) would like to continue some type of lifelong follow-up care in order to be monitored for long-term and late effects of treatment. Can I (my child) follow up with you?

*For definitions of an FDA-approved treatment and a clinical trial (study treatment), visit www.LLS.org or contact an Information Specialist.

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Our Mission:

Cure leukemia, lymphoma, Hodgkin's disease and myeloma, and improve the quality of life of patients and their families.

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