Treating Acute Myeloid Leukemia

General treatment information about acute myeloid leukemia

As noted earlier, adult acute myeloid leukemia (AML) is not a single disease. It is really a group of related diseases, and patients with different subtypes of AML can have different outlooks and responses to treatment.

Once AML has been diagnosed, your cancer care team will discuss your treatment options with you. Your options may be affected by the AML subtype and lab tests of the leukemia cells, as well as certain other prognostic factors (described in How is acute myeloid leukemia classified?), as well as your overall state of health.

Several types of treatment may be used for people with AML. The main treatment for AML is chemotherapy, sometimes along with a targeted therapy drug. This might be followed by a stem cell transplant. Other drugs (besides standard chemotherapy drugs) may also be used to treat people with acute promyelocytic leukemia (APL). Surgery and radiation therapy may be used in special circumstances.

The typical treatment approach for AML is different from the treatment approach for acute promyelocytic leukemia (APL).

It's important to discuss all of your treatment options and their possible side effects with your doctors to help make the decision that best fits your needs. It's also very important to ask questions if there is anything you're not sure about. You can find some good questions to ask in What should you ask your doctor about acute myeloid leukemia?

In most cases AML can progress rapidly, so it is important to start treatment as soon as possible after the diagnosis is made.

Thinking about taking part in a clinical trial
Clinical trials are carefully controlled research studies that are done to get a closer look at promising new treatments or procedures. Clinical trials are one way to get state-of-the-art cancer treatment. In some cases they may be the only way to get access to newer treatments. They are also the best way for doctors to learn better methods to treat cancer. Still, they are not right for everyone.

If you would like to learn more about clinical trials that might be right for you, start by asking your doctor if your clinic or hospital conducts clinical trials. See Clinical Trials to learn more.

**Considering complementary and alternative methods**

You may hear about alternative or complementary methods that your doctor hasn’t mentioned to treat your cancer or relieve symptoms. These methods can include vitamins, herbs, and special diets, or other methods such as acupuncture or massage, to name a few.

Complementary methods refer to treatments that are used along with your regular medical care. Alternative treatments are used instead of a doctor’s medical treatment. Although some of these methods might be helpful in relieving symptoms or helping you feel better, many have not been proven to work. Some might even be dangerous.

Be sure to talk to your cancer care team about any method you are thinking about using. They can help you learn what is known (or not known) about the method, which can help you make an informed decision. See the Complementary and Alternative Medicine section to learn more.

**Help getting through cancer treatment**

Your cancer care team will be your first source of information and support, but there are other resources for help when you need it. Hospital- or clinic-based support services are an important part of your care. These might include nursing or social work services, financial aid, nutritional advice, rehab, or spiritual help.

The American Cancer Society also has programs and services – including rides to treatment, lodging, support groups, and more – to help you get through treatment. Call our National Cancer Information Center at 1-800-227-2345 and speak with one of our trained specialists.

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Chemotherapy for Acute Myeloid Leukemia

Chemotherapy (chemo) is the use of anti-cancer drugs that are injected into a vein, under the skin, or into the cerebrospinal fluid (CSF), or drugs that are taken by mouth to destroy or control cancer cells. Except when given into the CSF, these drugs enter the bloodstream and reach all areas of the body, making this treatment useful for cancers such as leukemia that spread throughout the body.

Chemotherapy is the main treatment for most people with acute myeloid leukemia (AML). Doctors give chemo in cycles, with each period of treatment followed by a rest period to allow the body time to recover. Chemo is often not recommended for patients in poor health, but advanced age by itself is not a barrier to getting chemo.

Treatment of AML is usually divided into 2 phases:

- **Induction** is the first phase of treatment. The goal is to clear the blood of leukemia cells (blasts) and to reduce the number of blasts in the bone marrow to normal.
- **Consolidation** is chemo given after the patient has recovered from induction. It is meant to kill the small number of leukemia cells that are still around but can’t be seen (because there are so few of them).

A third phase called **maintenance** involves giving a low dose of chemo for months or years after consolidation is finished. This is often used for the M3 subtype of AML (also known as *acute promyelocytic leukemia*, or APL), but it is rarely used for other types of AML.

The chemo drugs used most often to treat AML are cytarabine (cytosine arabinoside or ara-C) and the anthracycline drugs (such as daunorubicin (daunomycin), idarubicin, and mitoxantrone).

Some of the other chemo drugs that may be used to treat AML include:

- Cladribine (Leustatin®, 2-CdA)
Fludarabine (Fludara®)  
Topotecan  
Etoposide (VP-16)  
6-thioguanine (6-TG)  
Hydroxyurea (Hydrea®)  
Corticosteroid drugs, such as prednisone or dexamethasone (Decadron®)  
Methotrexate (MTX)  
6-mercaptopurine (6-MP)  
Azacitidine (Vidaza®)  
Decitabine (Dacogen®)

Possible side effects

Chemo drugs attack cells that are dividing quickly, which is why they work against cancer cells. But other cells in the body, such as those in the bone marrow (where new blood cells are made), the lining of the mouth and intestines, and the hair follicles, also divide quickly. These cells are also likely to be affected by chemo, which can lead to side effects.

The side effects of chemo depend on the type and dose of drugs given and how long they are taken. These side effects can include:

- Hair loss
- Mouth sores
- Loss of appetite
- Nausea and vomiting
- Diarrhea or constipation

Chemo drugs also affect the normal cells in bone marrow, which can cause lowering of blood cell counts in AML patients. This can lead to:

- Increased risk of infections (from having too few normal white blood cells)
- Easy bruising or bleeding (from having too few blood platelets)
- Fatigue (from having too few red blood cells)

Most side effects last a short time and go away once treatment is finished. Low blood cell counts can last weeks, but then should return to normal. There are often ways to lessen these side effects. For example, drugs can be given to help prevent or reduce nausea and vomiting. Be sure to ask about medicines to help reduce side effects, and let your doctor or nurse know when you do have side effects so they can be managed effectively.
Drugs known as *growth factors*, such as G-CSF (filgrastim, Neupogen®) and GM-CSF (sargramostim, Leukine®), are sometimes given to increase the white blood cell counts after chemo, to reduce the chance of infection. However, it’s not clear if they have an effect on treatment success.

If your white blood cell counts are very low during treatment, you can help reduce your risk of *infection* by carefully avoiding exposure to germs. During this time, your doctor may tell you to:

- Wash your hands often.
- Avoid fresh, uncooked fruits and vegetables and other foods that might carry germs.
- Avoid fresh flowers and plants because they may carry mold.
- Make sure other people wash their hands before they come in contact with you.
- Avoid large crowds and people who are sick.

You may get antibiotics before there are signs of infection or at the earliest sign that an infection may be developing (such as a fever). You may also get drugs that help prevent viral and fungal infections.

Some of the most serious side effects of chemo are caused by low white blood cell counts. Decisions about when a patient can leave the hospital are often influenced by his or her blood counts. Some people find it helpful to keep track of their counts. If you are interested in this, ask your doctor or nurse about your blood cell counts and what these numbers mean.

If your platelet counts are low, you may be given drugs or platelet transfusions to help prevent bleeding. Likewise, shortness of breath and extreme fatigue caused by low red blood cell counts may be treated with drugs or with *red blood cell transfusions*.

Certain drugs have some specific possible side effects. For example, when used at high doses, cytarabine can cause certain problems, including dryness in the eyes and effects on certain parts of the brain, which can lead to problems with coordination or balance. The drug dose may need to be reduced or stopped altogether if these side effects appear.

Anthracyclines can damage the heart, so they might not be used in someone who already has heart problems.

Other organs that could be damaged by chemo drugs include the kidneys, liver, testicles, ovaries, and lungs. Doctors and nurses carefully monitor treatment to limit the risk of these side effects as much as possible.
If serious side effects occur, the chemo may have to be reduced or stopped, at least for a short time. Careful monitoring and adjustment of drug doses are important because some side effects can be permanent.

**Tumor lysis syndrome** is another possible side effect of chemo. This can occur in patients who have large numbers of leukemia cells in the body, so it mainly occurs in patients during the induction phase of treatment. When chemo kills these cells, they break open and release their contents into the bloodstream. This can overwhelm the kidneys, which aren’t able to get rid of all of these substances at once. Excess amounts of certain minerals can also affect the heart and nervous system. This can be prevented by giving extra fluids during treatment and by giving certain drugs, such as bicarbonate, allopurinol, and rasburicase, which help the body get rid of these substances.

For more about chemo and its side effects, see the [Chemotherapy](#) section of our website.

- **References**
  See all references for Acute Myeloid Leukemia

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**Targeted Therapy for Acute Myeloid Leukemia**

In recent years, new drugs that target specific parts of cancer cells have been developed. These targeted drugs work differently than standard chemotherapy (chemo) drugs. They can sometimes be helpful even when chemo isn’t, or they can be used along with chemo to help it work better. These drugs tend to have different side effects from chemo.

Some of these drugs can be useful in certain people with acute myeloid leukemia (AML).
**Midostaurin (Rydapt)**

In some people with AML, the leukemia cells have a mutation in the *FLT3* gene. This gene helps the cells make a protein (also called FLT3) that helps the cells grow. Researchers are now developing drugs that target the FLT3 protein.

**Midostaurin** is a drug that works by blocking FLT3 and several other proteins on cancer cells that can help the cells grow. This drug can be used along with certain chemotherapy drugs to treat newly diagnosed adults whose leukemia cells have an *FLT3* gene mutation. Your doctor can test your blood to see if you have this mutation.

This drug is taken as pills, twice a day.

Common side effects can include low levels of white blood cells (with increased risk of infection), fever, nausea, vomiting, redness or sores in the mouth, headache, muscle or bone pain, bruising, nosebleeds, high blood sugar levels, and upper respiratory infections.

Less often, this drug can cause serious lung problems, which might show up as a cough, chest pain, or shortness of breath. Tell your doctor or nurse right away if you have any of these symptoms.

**Enasidenib (Idhifa)**

In some people with AML, the leukemia cells have a mutation in the *IDH2* gene. This gene helps the cells make a protein (also called IDH2) that helps the cells grow. Mutations in the *IDH2* gene can stop blood cells from maturing the way they normally would.

**Enasidenib** is a drug that works by blocking the IDH2 protein on leukemia cells. It seems to work by helping the leukemia cells mature (differentiate) into more normal cells. Because of this, it is sometimes referred to as a *differentiation agent*.

This drug can be used to treat AML that comes back after treatment or is no longer responding to other treatments, and in which the leukemia cells have an *IDH2* gene mutation. Your doctor can test your blood to see if you have this mutation.

This drug is taken as pills, once a day.

Common side effects can include nausea, vomiting, diarrhea, increased levels of
bilirubin (a substance found in bile), and loss of appetite.

An important possible side effect of this drug is known as *differentiation syndrome*. This occurs when the leukemia cells release certain chemicals into the blood. It is most often seen during the first cycle of treatment. Symptoms can include fever, breathing problems from fluid buildup in the lungs and around the heart, low blood pressure, liver or kidney damage, and severe fluid buildup elsewhere in the body. It can often be treated by stopping the drugs for a while and giving a steroid such as dexamethasone.

**Gemtuzumab ozogamicin (Mylotarg)**

This is a targeted therapy that consists of a monoclonal antibody (a manmade immune protein) linked to a chemotherapy drug. The antibody attaches to protein called CD33, which is found on most AML cells. The antibody acts like a homing signal, bringing the chemo drug to the leukemia cells, where it enters the cells and kills them when they try to divide into new cells.

This drug can be used along with chemotherapy as part of the initial treatment of AML. It can also be used by itself, either as the first treatment (especially in people who might not be healthy enough for intense chemo), or if other treatments are no longer working. It is given as an infusion into a vein (IV).

The most common side effects are fever, nausea and vomiting, low levels of blood cells (with increased risks of infection, bleeding, and fatigue), swelling and sores in the mouth, constipation, rash, and headaches.

Less common but more serious side effects can include:

- Severe liver damage, including veno-occlusive disease (blockage of veins in the liver)
- Reactions during the infusion (similar to an allergic reaction). You will likely be given medicines before each infusion to help prevent this.
- Serious or life-threatening infections, especially in people who have already had a stem cell transplant
- Changes in the rhythm of the heart

To learn more about targeted therapy drugs as a treatment for cancer, see [Targeted Cancer Therapy](#).

- **References**
- See all references for Acute Myeloid Leukemia
Non-Chemo Drugs for Acute Promyelocytic Leukemia

Chemotherapy is the main treatment for most types of acute myeloid leukemia (AML). But acute promyelocytic leukemia (APL or AML M3) is different from other types of AML in some important ways.

The leukemia cells (or blasts) in APL contain proteins that when released into the bloodstream can cause the blood to clot in an out-of-control way. This can lead to problems not only with blood clots, but also with severe bleeding. In the past, when regular chemotherapy (chemo) drugs were used alone to kill these cells, these proteins were released into the bloodstream. Patients sometimes died from complications from the out-of-control clotting or bleeding.

Experts realized that the leukemia cells in APL have a specific gene change that makes them sensitive to certain drugs that aren't like regular chemo drugs. These drugs signal the blasts to transform into mature myeloid cells. This process is known as **differentiation**, and these drugs are called **differentiation agents**. Since the blasts don’t die, they don’t release the harmful proteins into the blood, which helps keep the clotting process from getting out of control.

There are 2 drugs that are used for this in APL: all-trans-retinoic acid (ATRA, tretinoin, or Vesanoïd®) and arsenic trioxide (ATO, Trisenox®).

**ATRA**

ATRA is a form of vitamin A that is often part of the initial (induction) treatment of APL. It is often given along with chemo. It can also be given with arsenic trioxide for the initial treatment of APL, in which case no regular chemo drugs are given. If ATRA is part of the initial treatment for APL, it is often used for some time after to help keep the leukemia from coming back. For the consolidation phase of treatment, it may be used with chemo, with arsenic trioxide, or with both chemo and arsenic trioxide. For longer-
term maintenance, ATRA might be used by itself or along with chemo.

ATRA can have side effects similar to those seen if you take too much vitamin A. Symptoms can include headache, fever, dry skin and mouth, skin rash, swollen feet, sores in the mouth or throat, itching, and irritated eyes. It can also cause blood lipid levels (like cholesterol and triglycerides) to go up. Often blood liver tests become abnormal. These side effects often go away when the drug is stopped.

**Arsenic trioxide**

Arsenic trioxide (ATO) is a form of arsenic, which can be a poison if given in high doses. But doctors found that it can act in a way similar to ATRA in patients with APL. It can be given with ATRA as the first treatment, but it is also helpful in treating patients whose APL comes back after treatment with ATRA plus chemo. In these patients, ATO is given without chemo.

Most side effects of ATO are mild and can include fatigue (tiredness), nausea, vomiting, diarrhea, abdominal (belly) pain, and nerve damage (called neuropathy) leading to numbness and tingling in the hands and feet. ATO can also cause problems with heart rhythm, which can be serious. Your doctor may check your heart rhythm with an EKG often (even daily) while you are getting this drug.

**Differentiation syndrome**

The most important side effect of either of these drugs is known as differentiation syndrome (previously called retinoic acid syndrome). This occurs when the leukemia cells release certain chemicals into the blood. It is most often seen during the first cycle of treatment.

Symptoms can include fever, breathing problems due to fluid buildup in the lungs and around the heart, low blood pressure, kidney damage, and severe fluid buildup elsewhere in the body. It can often be treated by stopping the drugs for a while and giving a steroid such as dexamethasone.

- References
  See all references for Acute Myeloid Leukemia

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Surgery for Acute Myeloid Leukemia

Surgery has a very limited role in the treatment of acute myeloid leukemia (AML). Because leukemia cells are spread widely throughout the bone marrow and blood, it’s not possible to cure this type of cancer with surgery. Surgery rarely has any role even in the diagnosis of AML, since this can usually be done with a bone marrow aspirate and biopsy. On rare occasions, an isolated tumor of leukemia cells (known as a granulocytic sarcoma or a chloroma) may be treated with surgery.

Often before chemotherapy is about to start, a minor type of surgery is used to place a small flexible tube, called a central venous catheter (CVC) or venous access device (VAD), into a large vein in the chest. This may be done by a surgeon in the operating room, or by a special type of radiologist. The end of the tube stays just under the skin or sticks out in the chest area or upper arm. The VAD is left in place during treatment to give intravenous (IV) drugs, such as chemotherapy, and to take blood samples for tests. This lowers the number of needle sticks needed during treatment. If you have a VAD, it is very important to learn how to care for it to keep it from getting infected.

* References

See all references for Acute Myeloid Leukemia

Radiation Therapy for Acute Myeloid Leukemia

Radiation therapy uses high-energy radiation to kill cancer cells. It is usually not part of the main treatment for people with acute myeloid leukemia (AML), but there are a few instances in which it may be used:
Radiation is sometimes used to treat leukemia that has spread to the brain and spinal fluid or to the testicles.

- Radiation to the whole body is often an important part of treatment before a stem cell transplant (see Stem cell transplant for acute myeloid leukemia).
- It is used (rarely) to help shrink a tumor if it is pressing on the trachea (windpipe) and causing breathing problems. But chemotherapy is often used instead, as it often works more quickly.
- Radiation can be used to reduce pain in an area of bone that is invaded by leukemia, if chemotherapy hasn’t helped.

Before your treatment starts, the radiation team will take careful measurements to determine the correct angles for aiming the radiation beams and the proper dose of radiation. The type of radiation therapy used to treat AML is called external beam radiation. The treatment is much like getting an x-ray, but the radiation is much stronger. The procedure itself is painless. The number of treatments you get depends on the reason radiation therapy is being used. Each treatment lasts only a few minutes, although the setup time getting you into place for treatment – usually takes longer.

The possible side effects of radiation therapy depend on where the radiation is aimed. Sunburn-like skin changes in the treated area are possible. Radiation to the head and neck area can lead to mouth sores and trouble swallowing. Radiation to the abdomen can cause nausea, vomiting, or diarrhea. Radiation can lower blood counts, leading to fatigue (from low red blood cell counts), bleeding or bruising (from low platelet counts), and an increased risk of infection (from low white blood cell counts).

To learn more about radiation therapy, see the Radiation Therapy section of our website.

- References
See all references for Acute Myeloid Leukemia

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Stem Cell Transplant for Acute Myeloid
Leukemia

The doses of chemotherapy drugs that doctors can give are limited by the serious side effects they can cause. Even though higher doses of these drugs might kill more cancer cells, they can't be given because they could severely damage the bone marrow, which is where new blood cells are formed. This could lead to life-threatening infections, bleeding, and other problems due to low blood cell counts.

Doctors can sometimes use a stem cell transplant (SCT) to give higher doses of chemotherapy (sometimes combined with radiation therapy) than could normally be given. After the treatment is finished, the patient gets an infusion of blood-forming stem cells to restore their bone marrow.

The blood-forming stem cells used for a transplant can come either from the blood or from the bone marrow. Sometimes stem cells from a baby's umbilical cord blood are used.

Types of transplants

The 2 main types of stem cell transplants differ based on whom the blood-forming stem cells come from.

Allogeneic stem cell transplant

This is the most common type of SCT used to treat acute myeloid leukemia (AML). In an allogeneic SCT, the stem cells come from someone other than the patient – usually a donor whose tissue type (also known as the HLA type) closely matches the patient’s. Tissue type is based on certain substances on the surface of cells in the body. These substances can cause the immune system to react against the cells. Therefore, the closer a tissue “match” is between the donor and the recipient, the better the chance the transplanted cells will “take” and begin making new blood cells.

The best donor is often a close relative, such as a brother or sister, if they are a good match. If no close relatives match, stem cells might be available from a matched unrelated donor (MUD), an unrelated volunteer whose tissue type matches that of the patient. But the use of stem cells from a MUD is linked to more complications. Sometimes umbilical cord stem cells are used. These stem cells come from blood drained from the umbilical cord and placenta after a baby is born and the umbilical cord is cut.
In AML, using an allogeneic SCT is preferred over an autologous SCT (see below) because leukemia is a disease of the blood and bone marrow, so giving the patient his or her own cells back may mean giving them back some leukemia cells as well. Donor cells are also helpful because of the “graft-versus-leukemia” effect. When the donor immune cells are infused into the body, they may recognize any remaining leukemia cells as being foreign to them and attack them. This effect doesn’t happen with autologous stem cell transplants.

Allogeneic transplants can have serious risks and side effects, so patients typically need to be younger and relatively healthy to be good candidates. Another challenge is that it can sometimes be difficult to find a matched donor.

One of the most serious complications of allogeneic SCTs is known as *graft-versus-host disease* (GVHD). It happens when the patient’s immune system is taken over by that of the donor. When this happens, the donor immune system may consider the patient’s own body tissues to be foreign and attacks them.

Symptoms can include severe skin rashes, itching, mouth sores (which can affect eating), nausea, and severe diarrhea. Liver damage can cause yellowing of the skin and eyes (jaundice). The lungs can also be damaged. The patient may also become easily fatigued and develop muscle aches. Sometimes GVHD can become disabling, and if it is severe enough, it can be life-threatening. Drugs that affect the immune system may be given to try to control it.

**Non-myeloablative transplant (mini-transplant):** Many older people can’t tolerate a standard allogeneic transplant that uses high doses of chemo. Some may still be able to get a non-myeloablative transplant (also known as a *mini-transplant* or *reduced-intensity transplant*), where they get lower doses of chemo and radiation that don’t completely destroy the cells in their bone marrow. They then get the allogeneic (donor) stem cells. These cells enter the body and establish a new immune system, which sees the leukemia cells as foreign and attacks them (a “graft-versus-leukemia” effect).

A non-myeloablative transplant can still sometimes work with much less toxicity. In fact, a patient can receive the transplant as an outpatient. The major complication is graft-versus-host disease (described below).

Many doctors still consider this an experimental procedure for AML, and studies are under way to determine how useful it may be.

**Autologous stem cell transplant**

In an autologous transplant, a patient’s own stem cells are removed from his or her
bone marrow or blood. They are frozen and stored while the person gets treatment (high-dose chemotherapy and/or radiation). A process called purging may be used to try to remove any leukemia cells in the samples. The stem cells are then put back (reinfused) into the patient’s blood after treatment.

Autologous transplants are sometimes used for people with AML who are in remission after initial treatment and who don’t have a matched donor for an allogeneic transplant. Some doctors feel that it is better than standard “consolidation” chemotherapy (see Typical treatment of acute myeloid leukemia) for these people, but not all doctors agree with this.

Autologous transplants are generally easier to tolerate than allogeneic transplants, because the patient is getting his or her own cells back, which lowers the risk of some complications. But the high-dose chemo can still cause major side effects. This type of transplant can be done in any otherwise healthy person, although very old patients might not be suitable.

One problem with autologous transplants is that it’s hard to separate normal stem cells from leukemia cells in the bone marrow or blood samples. Even after purging (treating the stem cells in the lab to try to kill or remove any remaining leukemia cells), there is the risk of returning some leukemia cells with the stem cell transplant.

**The transplant procedure**

Blood-forming stem cells from the bone marrow or blood are collected, frozen, and stored. The patient gets high-dose chemo and sometimes also radiation treatment to the entire body. (Radiation shields are used to protect the lungs, heart, and kidneys from damage during radiation therapy.)

These treatments are meant to destroy any cancer cells in the body. They also kill the normal cells of the bone marrow and the immune system. After these treatments, the frozen stem cells are thawed and given as a blood transfusion. The stem cells settle into the patient’s bone marrow over the next several days and start to grow and make new blood cells.

In an allogeneic SCT, the person getting the transplant is given drugs to keep the new immune system in check. For the next few weeks the patient will get regular blood tests and supportive therapies as needed, which might include antibiotics, red blood cell or platelet transfusions, other medicines, and help with nutrition.

Usually within a couple of weeks after the stem cells have been infused, they begin
making new white blood cells. This is followed by new platelets and, several weeks later, new red blood cells.

Patients need to stay in the hospital until their neutrophil count (often called the ANC) rises to a safer level (at least 500, but sometimes 1,500 is the target). Other factors also affect how long a person needs to stay in the hospital, like the type of transplant, the presence of an infection or other complications, and the ability of the patient to be followed-up in the outpatient clinic. After discharge from the hospital, the patient is seen in the outpatient clinic for several weeks, often daily. Because platelet counts take longer to return to a safe level, patients may get platelet transfusions as an outpatient.

**Practical points**

A stem cell transplant is a complex treatment that can sometimes cause life-threatening side effects. If the doctors think you might benefit from a transplant, it should be done at a hospital where the staff has experience with the procedure and with managing the recovery phase. Some stem cell transplant programs might not have experience in certain types of transplants, especially transplants from unrelated donors.

SCT is very expensive (costing well over $100,000) and often requires a lengthy hospital stay. Because some types of SCT may be viewed as experimental by insurance companies, they may not pay for the procedure. It is important to find out what your insurer will cover before deciding on a transplant to get an idea of what you might have to pay.

**Possible side effects**

Side effects from SCT are generally divided into early and long-term effects.

**Early or short-term effects:** The early complications and side effects are basically the same as those caused by any other type of chemotherapy (see Chemotherapy for acute myeloid leukemia), although they tend to be more severe. They can include low blood cell counts (with fatigue and an increased risk of infection and bleeding), nausea, vomiting, loss of appetite, mouth sores, and hair loss.

One of the most common and serious short-term effects is the increased risk of infection. Antibiotics are often given to try to prevent this from happening. Other side effects, like low red blood cell and platelet counts, may require blood product transfusions or other treatments.

A possible serious side effect of allogeneic transplants is graft-versus-host disease,
which is described above.

**Long-term side effects:** Some complications and side effects can remain for a long time or might not occur until months or years after the transplant. These include:

- Chronic graft-versus-host disease (only in allogeneic transplants)
- Loss of fertility
- Damage to the lungs, causing shortness of breath
- Damage to the thyroid gland, causing problems with metabolism
- Cataracts (damage to the lens of the eye that can affect vision)
- Bone damage called *aseptic necrosis* (where the bone dies because of poor blood supply). If damage is severe, the patient might need to have part of the bone and the joint replaced.
- Development of another cancer years later

For more on stem cell transplants, see [Stem Cell Transplant (Peripheral Blood, Bone Marrow, and Cord Blood Transplants)](#).

- **References**

  See all references for Acute Myeloid Leukemia

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**Typical Treatment of Most Types of Acute Myeloid Leukemia (Except Acute Promyelocytic M3)**

Treatment of most cases of acute myeloid leukemia (AML) is usually divided into 2 chemotherapy (chemo) phases:

- Remission induction (often just called *induction*)
- Consolidation (post-remission therapy)

Treatment usually needs to start as quickly as possible after the diagnosis because
AML can progress very quickly. Sometimes another type of treatment needs to be started even before the chemo has had a chance to work.

**Treating leukostasis**

Some people with AML have very high numbers of leukemia cells in their blood when they are diagnosed, which can cause problems with normal circulation. This is called *leukostasis* and was discussed in *Signs and Symptoms of Acute Myeloid Leukemia*. Chemo can take a few days to lower the number of leukemia cells in the blood. In the meantime, *leukapheresis* (sometimes just called *pheresis*) might be used before chemo.

For this procedure, the patient’s blood is passed through a special machine that removes white blood cells (including leukemia cells) and returns the rest of the blood to the patient. Two intravenous (IV) lines are required – the blood is removed through one IV, goes through the machine, and then is returned to the patient through the other IV. Sometimes, a single large catheter is placed in a vein in the neck or under the collar bone for the pheresis – instead of using IV lines in both arms. This type of catheter is called a *central line* and has both IVs built in.

This treatment lowers blood counts right away. The effect is only for a short time, but it may help until the chemo has a chance to work.

**Induction**

This first part of treatment is aimed at getting rid of as many leukemia cells as possible. How intense the treatment is can depend on a person’s age and health. Doctors often give the most intensive chemo to people under the age of 60. Some older patients in good health may benefit from similar or slightly less intensive treatment.

People who are much older or are in poor health might not do well with intensive chemo. Treatment of these patients is discussed below.

Age, health, and other factors clearly need to be taken into account when considering treatment options. Doctors are also trying to determine whether people with certain gene or chromosome changes are more likely to benefit from more intensive treatment.

In younger patients, such as those under 60, induction often involves treatment with 2 chemo drugs, cytarabine (ara-C) and an anthracycline drug such as daunorubicin (daunomycin) or idarubicin. Sometimes a third drug, cladribine (Leustatin, 2-CdA), is given as well. The chemo is usually given in the hospital and lasts about a week.
For patients whose leukemia cells have an \textit{FLT3} gene mutation, the targeted therapy drug midostaurin might be given along with chemo. This drug is taken twice daily as a pill.

Patients with poor heart function can’t be treated with anthracyclines, so they may be treated with another chemo drug, such as fludarabine (Fludara) or topotecan.

In rare cases where the leukemia has spread to the brain or spinal cord, chemo may also be given into the cerebrospinal fluid (CSF). Radiation therapy might be used as well.

Induction destroys most of the normal bone marrow cells as well as the leukemia cells. Most patients develop dangerously low blood counts at this time, and may be very ill. Most patients need antibiotics and blood product transfusions. Drugs to raise white blood cell counts may also be used. Blood counts tend to stay low for a few weeks. Usually, the patient stays in the hospital during this time.

About 1 or 2 weeks after chemo is done, the doctor will check a bone marrow biopsy. It should show few bone marrow cells (\textit{hypocellular} bone marrow) and only a small portion of blasts. If the biopsy shows that there are still leukemia cells in the bone marrow, more chemo may be given. Sometimes a stem cell transplant is recommended at this point. If it isn’t clear on the bone marrow biopsy whether the leukemia is still there, another bone marrow biopsy may be done again in about a week.

Over the next few weeks, normal bone marrow cells will return and start making new blood cells. The doctor may check other bone marrow biopsies during this time. When the blood cell counts recover, the doctor will again check cells in a bone marrow sample to see if the leukemia is in remission (blasts make up no more than 5% of the bone marrow).

Remission induction usually does not destroy all the leukemia cells, and a small number often remain. Without consolidation treatment, the leukemia is likely to return within several months.

**Consolidation (post-remission therapy)**

Induction is considered successful if remission is achieved. Further treatment is then given to try to destroy any remaining leukemia cells and help prevent a relapse. This is called \textit{consolidation}.

For younger patients, the main options for consolidation therapy are:
Several cycles of high-dose cytarabine (ara-C) chemo (sometimes known as HiDAC)
- Allogeneic (donor) stem cell transplant
- Autologous stem cell transplant

Consolidation chemo differs from induction therapy in that usually only cytarabine is used. The drug is given at very high doses, typically over 5 days. This is repeated about every 4 weeks, usually for a total of 3 or 4 cycles. For people who got the targeted drug midostaurin during induction, this is typically continued during consolidation.

Another approach after successful induction therapy is to give very high doses of chemo followed by either an allogeneic (from a donor) or autologous (patient’s own) stem cell transplant. Stem cell transplants have been found to reduce the risk of leukemia coming back more than standard chemo, but they are also more likely to have serious complications, including an increased risk of death from treatment.

Older patients or those in poor health may not be able to tolerate such intensive consolidation treatment. Often, giving them more intensive therapy raises the risk of serious side effects (including treatment-related death) without providing much more of a benefit. These patients may be treated with:

- 1 or 2 cycles of higher dose cytarabine (usually not quite as high as in younger patients)
- 1 or 2 cycles of standard dose cytarabine, possibly along with idarubicin, daunorubicin, or mitoxantrone
- Non-myeloablative stem cell transplant (mini-transplant)

It is not always clear which treatment option is best for consolidation. Each has pros and cons. Doctors look at several different factors when recommending what type of therapy a patient should get. These include:

- **How many courses (cycles) of chemo it took to bring about a remission.** If it took more than one course, some doctors recommend that the patient get a more intensive program, which might include a stem cell transplant.
- **The availability of a brother, sister, or an unrelated donor who matches the patient’s tissue type.** If a close enough tissue match is found, an allogeneic (donor) stem cell transplant may be an option, especially for younger patients.
- **The potential of collecting leukemia-free bone marrow cells from the patient.** If lab tests show that a patient is in remission, collecting stem cells from the patient’s bone marrow or blood for an autologous stem cell transplant may be an option. Stem cells collected from the patient would be purged (treated in the lab to
try to remove or kill any remaining leukemia cells) to lower the chances of relapse.

- The presence of one or more adverse prognostic factors, such as certain gene or chromosome changes, a very high initial white blood cell count, AML that develops from a previous blood disorder or after treatment for an earlier cancer, or spread to the central nervous system. These factors might lead doctors to recommend more aggressive therapy, such as a stem cell transplant. On the other hand, for people with good prognostic factors, such as favorable gene or chromosome changes, many doctors might advise holding off on a stem cell transplant unless the disease recurs.

- The patient’s age. Older patients may not be able to tolerate some of the severe side effects that can occur with high-dose chemo or stem cell transplants.

- The patient’s wishes. There are many issues that revolve around quality of life that must be discussed. An important issue is the higher chance of early death from high-dose chemo or a stem cell transplant. This and other issues must be discussed between the patient and the doctor.

Stem cell transplants are intensive treatments with real risks of serious complications, including death, and their exact role in treating AML is not always clear. Some doctors feel that if the patient is healthy enough to withstand the procedure and a compatible donor is available, an allogeneic transplant offers the best chance for long-term survival. Others feel that studies have not yet shown this conclusively, and that in some cases a transplant should be reserved in case the leukemia comes back after standard treatment. Still others feel that stem cell transplants should be given if the leukemia is likely to come back based on certain gene or chromosome changes. Research in this area continues to see which AML patients get the most benefit from stem cell transplant and what is the best transplant procedure.

**Treating frail, older adults**

Treatment of AML in people under 60 is fairly standard. It involves cycles of intensive chemo, sometimes along with a stem cell transplant (as discussed above). Many patients older than 60 are healthy enough to be treated in the same way, although sometimes the chemo may be less intense.

People who are much older or are in poor health may not be able to tolerate this intense treatment. In fact, intense chemo could actually shorten their lives.

In some cases, doctors may recommend low-intensity chemo with a low dose of cytarabine given in cycles. Sometimes, these patients may be treated with other chemo drugs like azacitidine (Vidaza) or decitabine (Dacogen). These drugs aren’t approved to treat AML, but still may be helpful. In some cases, this may induce remission. In others,
it may control the leukemia for a time. Treatment of these patients is often not divided into induction and consolidation phases, but it may be given every so often as long as it seems helpful.

Another option might be the targeted drug gemtuzumab ozogamicin (Mylotarg).

Some patients decide against chemo and other drugs and instead choose supportive care. This focuses on treating any symptoms or complications that arise and keeping the person as comfortable as possible.

- References

See all references for Acute Myeloid Leukemia

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**Treatment of Acute Promyelocytic (M3) Leukemia**

Early diagnosis and treatment of acute promyelocytic leukemia (APL), the M3 subtype of acute myeloid leukemia (AML), is important because patients with APL can develop serious blood-clotting or bleeding problems. This is less often a problem now that treatment includes differentiating drugs like all-trans-retinoic acid (ATRA). Other treatments might include chemotherapy and transfusions of platelets or other blood products.

**Induction**

The treatment of most cases of APL differs from usual AML treatment. Initial treatment includes the non-chemotherapy drug all-trans-retinoic acid (ATRA), which is most often combined with an anthracycline chemotherapy (chemo) drug (daunorubicin or idarubicin), sometimes also with the chemo drug cytarabine (ara-c).

Another option is to give ATRA plus another differentiating drug called arsenic trioxide (Trisenox). This is often used in patients who can’t tolerate an anthracycline drug, but
it’s an option for other patients as well.

**Consolidation**

As with other subtypes of AML, patients with APL then receive post-remission treatment. What drugs are used depends on what was given for induction. Some of the options include:

- An anthracycline along with ATRA for a few cycles (sometimes different anthracyclines are used in different cycles)
- An anthracycline plus cytarabine for at least 2 cycles
- Arsenic trioxide for 2 cycles (over about 2½ months), then ATRA plus an anthracycline for 2 cycles
- ATRA plus arsenic trioxide for several cycles

**Maintenance**

For some patients, consolidation may be followed by maintenance therapy with ATRA for at least a year. Sometimes low doses of the chemo drugs 6-mercaptopurine (6-MP) and methotrexate are given as well.

- References

[See all references for Acute Myeloid Leukemia]

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chemotherapy (chemo) with daunorubicin and cytarabine go into remission. This usually means the bone marrow contains fewer than 5% blast cells, the blood cell counts are within normal limits, and there are no signs or symptoms of the disease. The actual chance of remission depends to a large part on a person’s specific prognostic factors, such as age or the presence of certain gene or chromosome changes.

If remission is achieved, patients may then get more chemo (consolidation). Up to half of patients that get this go into long-term remission (and may be cured). But this number is also affected by prognostic factors, such as a person’s age and whether the leukemia cells have certain gene or chromosome changes. Using an allogeneic stem cell transplant as consolidation has a higher success rate, but it also has a higher risk of death as a complication.

Older patients generally don’t do as well as those younger than 60. They often have trouble tolerating intensive treatment and often have chromosome changes in their leukemia cells that are linked to a poorer outlook. About half of these patients go into remission after initial treatment.

For acute promyelocytic leukemia (APL)

More than 90% of patients with APL go into remission with standard induction treatment. With consolidation and maintenance, about 70% to 90% of patients with APL are successfully treated long-term.

- References
See all references for Acute Myeloid Leukemia

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What If Acute Myeloid Leukemia Doesn’t Respond or Comes Back After Treatment?
For most types of acute myeloid leukemia

If acute myeloid leukemia (AML) doesn’t go away with the first treatment, newer drugs or more intensive doses of chemotherapy (chemo) drugs may be tried, if they can be tolerated. A stem cell transplant may be tried in younger patients if a suitable stem cell donor can be found. Clinical trials of new treatment approaches may also be an option.

If the leukemia went away and has now come back, the treatment options depend on the patient’s age and health, and on how long the leukemia was in remission. AML most often recurs in the bone marrow and blood. The brain or cerebrospinal fluid (CSF) rarely will be the first place it recurs, but if it does, it is often treated with chemo given directly into the CSF (during a lumbar puncture/spinal tap).

For those whose remission lasted longer than 12 months, it is sometimes possible to put the leukemia into remission again with more chemo, although this is not likely to be long-lasting. For younger patients (generally those younger than 60), most doctors would then advise a stem cell transplant if a suitable donor can be found. Clinical trials of new treatment approaches may also be considered.

If AML comes back sooner than 12 months, most doctors will advise a stem cell transplant for younger patients, if possible. Taking part in a clinical trial is another option.

Another option for AML that doesn’t go away or comes back after treatment might be the targeted drug gemtuzumab ozogamicin (Mylotarg).

If the leukemia keeps coming back or doesn’t go away, further chemo treatment will probably not be very helpful. If a stem cell transplant is not an option, a patient may want to consider taking part in a clinical trial of newer treatments.

For AML with an IDH2 gene mutation

If the leukemia cells have an IDH2 gene mutation, one option if the leukemia doesn’t go away or if it comes back later might be treatment with a targeted drug such as enasidenib (Idhifa). Other options might include chemo or a stem cell transplant.

For acute promyelocytic leukemia

For patients with acute promyelocytic leukemia (APL) who don’t respond to initial treatment with chemo plus ATRA or who relapse, arsenic trioxide (Trisenox) is often very effective. A stem cell transplant may be another option if a donor can be found.
If treatment with arsenic trioxide achieves a remission, further courses of this drug may be given. An autologous stem cell transplant may also be an option. If a second remission is not achieved, treatment options may include an allogeneic stem cell transplant or taking part in a clinical trial.

**Palliative treatment**

If further treatment or a clinical trial is not an option, the focus of treatment may shift to controlling symptoms caused by the leukemia, rather than trying to cure the leukemia. This is called palliative treatment or supportive care. For example, the doctor may advise less intensive chemo to try to slow the leukemia growth instead of trying to cure it.

As the leukemia grows in the bone marrow it may cause pain. It’s important that you be as comfortable as possible. Treatments that may be helpful include radiation therapy and appropriate pain-relieving medicines. If medicines such as aspirin and ibuprofen don’t help with the pain, stronger opioid medicines such as morphine are likely to be helpful. Some people may worry about taking stronger drugs for fear of being sleepy all the time or becoming addicted to them. But many people get very effective pain relief from these medicines without serious side effects. It’s very important to let your cancer care team know if you are having pain so that it can be treated.

Other common symptoms from leukemia are low blood counts and fatigue. Medicines or blood transfusions may be needed to help correct these problems. Nausea and loss of appetite can be treated with medicines and high-calorie food supplements. Infections that occur may be treated with antibiotics.

- References

See all references for Acute Myeloid Leukemia

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