



1. What is acute myeloid leukemia?

Acute myeloid leukemia (AML) is a type of cancer of the blood. It affects a group of white blood cells called myeloid cells. Normally, myeloid and other blood cells are produced by the bone marrow (the spongy area in the middle of bones) in a controlled fashion. In someone with AML, this production process is abnormal and large numbers of immature cells are produced and released into the blood stream.

2. How effects acute myeloid leukemia other, healthy bone marrow cells?

The overproduction of myeloid cells prevents the bone marrow from producing other important blood cells, including red blood cells, other types of white blood cells, and platelets. This results in a variety of systemic symptoms, anemia, bleeding, and an increased risk of infection.

3. How is acute myeloid leukemia diagnosed?

Most commonly, patients present with symptoms related to impaired bone marrow function: anemia may cause weakness, fatigue and shortness of breath; low white blood cell counts may trigger infections; low platelet counts may present with bleeding or bruising. Sometimes routine laboratory studies reveal an abnormal blood count. Appropriate testing, including a bone marrow biopsy which entails a number of additional tests (immuno diagnostics, genetic analysis of the bone marrow cells) will yield the diagnosis.

4. How is acute myeloid leukemia treated?

A number of chemotherapy medications are effective against AML. Studies are in progress to find the best medicines, doses, and treatment schedules for patients with AML. Researchers have discovered that the genetic makeup of the leukemia cells can vary, which affects how a particular patient responds to treatment. Alterations in treatment can be made depending upon careful analysis of this genetic material. Leukemia research centers are constantly investigating new treatment regimens to improve outcomes.

5. What is the chance of survival with acute myeloid leukemia?

Treatment of AML also depends upon the age of the patient. Regimens that tend to work well in young patients may not work as well or have dangerous side effects in patients over age 60 ([see "Treatment in older patients" below](#)).



6. What are the side effects of chemotherapy for AML?

Side effects of treatment depend on the dose, schedule, and type of medication used. Many of the chemotherapy medicines used to treat AML cause loss of hair (which is temporary), nausea and vomiting, mouth sores, and an increased risk of infections and bleeding. Treatment to minimize these side effects is available and is generally quite effective.

7. What is induction chemotherapy?

The usual treatment of AML is divided into two phases: induction of remission and postremission therapy.

The most common remission induction regimen includes [cytarabine](#) (cytosine arabinoside, Ara-C, or Cytosar), given continuously for seven days through an intravenous (IV) line. [Daunorubicin](#) (Daunomycin, Cerubidine, or Rubidomycin) is given in a single IV dose for the first three days of treatment. This is sometimes known as the "7+3" regimen. In some medical centers a variation of this regimen is used, in which daunorubicin is replaced by [idarubicin](#), or cytarabine is given alone in much higher doses. In some cases, other medicines such as [etoposide](#) or 6-thioguanine may be added to the "7+3" regimen.

8. How long does induction chemotherapy take?

This phase of treatment takes about four weeks and is almost always performed while the patient remains in the hospital. The induction phase usually consists of one or two cycles. A cycle of chemotherapy refers to the time it takes to give the drugs and the time required for the body to recover.

9. How does induction chemotherapy work?

Chemotherapy drugs work by interfering with the ability of rapidly growing cells (like cancer cells) to divide or reproduce themselves. Because most of an adult's normal cells are not actively growing, they are less affected by chemotherapy, with the exception of bone marrow (where the blood cells are produced), the hair, and the lining of the gastrointestinal tract. Effects of chemotherapy on these and other normal tissues cause side effects during treatment, including anemia (lowered red blood cell count), susceptibility to infection (lowered white blood cell count) and bleeding (lowered platelet count).



10. What happens at the end of induction chemotherapy?

Induction of remission frequently results in a complete remission of the AML, meaning that there are no detectable leukemic cells in the blood or bone marrow and that the bone marrow is functioning normally. However, such remissions are usually short-lived unless additional, postremission chemotherapy is given.

11. What is postremission chemotherapy?

There are two basic treatment choices for postremission therapy: additional chemotherapy, and stem cell transplantation from a donor.

a. Additional chemotherapy — The same chemotherapy regimen used for remission induction can be repeated for one or more cycles, referred to as consolidation chemotherapy. Genetic analysis of the leukemia (done before the initial induction of remission treatment) is useful in deciding which chemotherapy regimen is best. In some cases, other chemotherapy agents may be used. High dose [cytarabine](#) is sometimes given to younger patients. Some regimens call for periodic chemotherapy cycles given for up to three years, which is known as remission "maintenance" therapy. Side effects and potential toxicities vary depending on the medications used.

b. Stem cell transplantation — Stem cell transplantation, also called bone marrow transplantation or hematopoietic cell transplantation, is a treatment in which the patient is given very high doses of chemotherapy or radiation. This is intended to kill cancer cells, but it also destroys all normal cells developing in the bone marrow. This means that the body's normal source of critical blood components (ie, the bone marrow) is no longer functional. After the treatment, the patient must have a healthy supply of very young blood cells (called stem cells) reintroduced, or transplanted. The transplanted cells then reestablish the blood cell production process in the bone marrow.

Stem cell transplantation is not recommended for all patients with AML. Complications are higher than those seen with chemotherapy. In certain groups of patients, there is no clear benefit of stem cell transplantation over chemotherapy. However, transplantation may be appropriate in some patients, such as those with more aggressive forms of AML, those who have a relapse following remission, or those patients who fail to achieve a remission following initial induction therapy.

12. What are the two different types of stem cell transplantation?

There are two main types of stem cell transplantation: allogeneic and autologous.

a. Allogeneic transplantation uses stem cells from a donor other than the patient, ideally a sibling with a similar genetic makeup (called a matched related donor). If the patient does not have a sibling with similar genetic characteristics, an unrelated person with a similar genetic makeup may be used (called a matched unrelated donor). One other



possibility is to use a sibling with partially similar genetic characteristics, although this is not as well studied. Allogeneic transplantation treats AML in two ways. First, high doses of chemotherapy or radiation are given immediately before the transplant, which aggressively attacks and kills the leukemia cells present in the blood and bone marrow. Second, when cells from another person are injected, the donor stem cells undergo an immune response that helps destroy any remaining leukemia cells. This is called the "graft versus leukemia" or "graft versus tumor" effect. Unfortunately, this response is closely associated with a complication called "graft versus host disease", in which the immune response includes an attack on some of the patient's own organs. Symptoms can include severe skin rash, diarrhea, liver damage, and other problems. Still, allogeneic transplant is preferred over autologous transplantation in patients with AML.

b. In an autologous transplant, the patient's own normal stem cells are removed while he/she is in remission, before the high dose chemotherapy or radiation is given. In some cases, the cells are treated in order to remove any lingering leukemia cells that may be present, then they are frozen for later use. After the patient's chemotherapy or radiation is complete, the harvested cells are thawed and returned to the patient by transfusion.

Because the transplanted stem cells do not come from another person, there is no "graft versus host" disease. This helps reduce some of the side effects of treatment, but in general it also makes autologous transplantation somewhat less effective than allogeneic transplantation in fighting the leukemia, because of the lack of a "graft versus leukemia" effect.

13. Chemotherapy and stem cell transplantation sounds very toxic. How about leukemia patients that cannot tolerate intense treatment, such as older patients?

In general, patients over 60 years old do not respond as well to treatment for AML. This is related to the following factors:

- Adverse characteristics of the leukemia cells may be more common in older people
- An increased prevalence of previous blood disorders (such as polycythemia vera or myelodysplasia) makes AML more difficult to treat
- The presence of other disorders, such as diabetes, kidney, lung, or heart disease, increase the risk of treatment related complications

Treatment decisions for older patients with AML are best made on a case by case basis. Sometimes, induction of remission is a reasonable goal. In an otherwise healthy older patient who does not have high-risk genetic findings, administration of standard chemotherapy induction regimens may be advised. In other patients, the expected benefit in terms of long-term outcome may not be worth the anticipated discomfort, hospitalization, and toxicity of chemotherapy or other treatments.



In some patients, supportive care can provide benefits that are equivalent to chemotherapy with a lower risk of complications or toxicity. There are cases in which the AML does not progress quickly, and these patients may do better with an approach that treats AML related problems, such as infection or anemia, as they occur rather than trying to cure the disease. Transfusions and antibiotics can be given as needed in place of more aggressive forms of therapy.

Patients and families should get information from their healthcare provider about the type of AML, expected benefits of various treatments, possible side effects and toxicities, and long term outlook. These discussions are critical in determining the best course of action for the individual patient.

14. What are the options if the disease comes back after successful induction and consolidation chemotherapy?

When patients fail to respond or relapse after initial chemotherapy, management is more difficult:

- Approximately 50 percent of patients with long first remissions (greater than one year) have a second induction of remission with daunorubicin and cytarabine or high-dose cytarabine (HDAC), but the duration of the second remission is usually shorter than the first. Because of this, hematopoietic cell transplantation should be considered for any patient who relapses after their initial treatment.
- Patients who relapse within 12 months of initial diagnosis usually have significant drug resistance and a lower rates of a second complete remission. Medicines specifically approved for use in patients with relapsed AML (eg, Mylotarg) or experimental agents may be useful in this setting, with hematopoietic cell transplantation considered for responding patients.

15. When do we consider a patient with acute myeloid leukemia cured?

Patients in complete remission need long term monitoring so that any reemergence (relapse) of the disease can be detected and treated. Typically, patients undergo examination of the bone marrow every three to six months for at least two years following remission. Patients with AML who maintain complete, continuous remission for three to five years are considered cured and no longer need routine bone marrow.