



[cancer.org](https://www.cancer.org) | 1.800.227.2345

## Treating Childhood Leukemia

If your child has been diagnosed with leukemia, your child's treatment team will discuss the options with you. It's important to weigh the benefits of each treatment option against the possible risks and side effects.

### How is childhood leukemia treated?

The main treatment for most childhood leukemias is chemotherapy. For some children with higher risk leukemias, high-dose chemotherapy may be given along with a stem cell transplant. Other treatments might also be used in special circumstances.

- [Surgery for Childhood Leukemia](#)
- [Radiation Therapy for Childhood Leukemia](#)
- [Chemotherapy for Childhood Leukemia](#)
- [Targeted Therapy Drugs for Childhood Leukemia](#)
- [Immunotherapy for Childhood Leukemia](#)
- [High-dose Chemotherapy and Stem Cell Transplant for Childhood Leukemia](#)

### Common treatment approaches

After leukemia is diagnosed and [tests](#)<sup>1</sup> have been done to determine its [type](#)<sup>2</sup> and [subtype](#)<sup>3</sup>, your child's cancer care team will discuss the treatment options with you. The most important factor in choosing a treatment is the type of leukemia, but [other factors](#)<sup>4</sup> also play a role.

Treatment of acute forms of childhood leukemia (ALL or AML) is usually very intensive, so it's important that it takes place in a center that specializes in treating childhood cancers.

- [Immediate Treatment for Childhood Leukemia](#)

- [Treatment of Children With Acute Lymphocytic Leukemia \(ALL\)](#)
- [Treatment of Children With Acute Myeloid Leukemia \(AML\)](#)
- [Treatment of Children With Acute Promyelocytic Leukemia \(APL\)](#)
- [Treatment of Children With Juvenile Myelomonocytic Leukemia \(JMML\)](#)
- [Treatment of Children With Chronic Myeloid Leukemia \(CML\)](#)

## Who treats leukemia in children?

Children and teens with leukemia and their families have special needs. These needs can be met best by cancer centers for children and teens, working closely with the child's primary care doctor. These centers offer the advantage of being treated by teams of specialists who know the differences between cancers in adults and those in children and teens, as well as the unique needs of younger people with cancer.

For childhood leukemias, this team is typically led by a **pediatric oncologist**, a doctor who treats children's cancers. Many other health professionals may be involved in your child's care as well, including other doctors, nurses, nurse practitioners (NPs), physician assistants (PAs), psychologists, social workers, rehabilitation specialists, and others.

- [How to Find the Best Cancer Treatment for Your Child<sup>5</sup>](#)
- [Navigating the Health Care System When Your Child Has Cancer<sup>6</sup>](#)

## Making treatment decisions

After leukemia is diagnosed and tests have been done to determine its type, your child's cancer care team will discuss the treatment options with you.

It's important to discuss your child's treatment options as well as their possible side effects with the treatment team to help make the decision that's the best fit for your child. If there is anything you don't understand, ask to have it explained.

If time allows, getting a second opinion from another doctor experienced with your child's type of cancer is often a good idea. This can give you more information and help you feel more confident about the treatment plan you choose. If you aren't sure where to go for a second opinion, ask your doctor for help.

- [Questions to Ask About Childhood Leukemia<sup>7</sup>](#)
- [How to Talk to Your Child's Cancer Care Team<sup>8</sup>](#)
- [Seeking a Second Opinion<sup>9</sup>](#)

## Thinking about taking part in a clinical trial

Today, most children and teens with cancer are treated at specialized children's cancer centers. These centers offer the most up-to-date-treatment by conducting clinical trials (studies of promising new therapies). Children's cancer centers often conduct many clinical trials at any one time, and in fact most children treated at these centers take part in a clinical trial as part of their treatment.

Clinical trials are one way to get state-of-the art cancer treatment. Sometimes they may be the only way to get access to newer treatments (although there is no guarantee that newer treatments will be better). They are also the best way for doctors to learn better methods to treat these cancers. Still, they might not be right for everyone.

If you would like to learn more about clinical trials that might be right for your child, start by asking the treatment team if your clinic or hospital conducts clinical trials.

- [Clinical Trials](#)<sup>10</sup>

## Considering complementary and alternative methods

You may hear about alternative or complementary methods that your doctor hasn't mentioned to treat your child's tumor 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* standard medical treatment. Although some of these methods might be helpful in relieving symptoms or helping people feel better, many have not been proven to work. Some might even be harmful.

Be sure to talk to your child's 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.

- [Complementary and Alternative Medicine](#)<sup>11</sup>

## Preparing for treatment

Before treatment, the doctors and other members of the team will help you, as a parent, understand the tests that will need to be done. The team's social worker will also counsel you about the problems you and your child might have during and after

treatments such as surgery, and might be able to help you find housing and financial aid if needed.

- [When Your Child Has Cancer](#)<sup>12</sup>

### Help getting through cancer treatment

Your child's 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 can also be an important part of your care. These might include nursing or social work services, financial aid, nutritional advice, rehab, or spiritual help. For children and teens with cancer and their families, other specialists can be an important part of care as well.

The American Cancer Society also has programs and services – including rides to treatment, lodging, 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.

- [Finding Help and Support When Your Child Has Cancer](#)<sup>13</sup>
- [Find Support Programs and Services in Your Area](#)<sup>14</sup>

*The treatment information given here is not official policy of the American Cancer Society and is not intended as medical advice to replace the expertise and judgment of your cancer care team. It is intended to help you and your family make informed decisions, together with your doctor. Your doctor may have reasons for suggesting a treatment plan different from these general treatment options. Don't hesitate to ask him or her questions about your treatment options.*

---

## Immediate Treatment for Childhood Leukemia

Some children are critically ill when they are first diagnosed with leukemia. For example:

- They might have a shortage of normal white blood cells, which might lead to very

serious [infections](#)<sup>1</sup>.

- They might have low levels of platelets or clotting factors in the blood, which can cause severe bleeding.
- They might not have enough red blood cells, which can lower the amount of oxygen getting to body tissues and put a tremendous strain on the heart.
- If they have too many (leukemic) white blood cells in the blood, it can slow down the circulation (known as **leukostasis**). This can lead to serious problems in the brain, heart, or lungs. It might also cause bleeding or blood clotting inside the body. This is not common, but when it happens it needs to be treated right away.

These problems must often be addressed before treatment of the leukemia can begin. Antibiotics, blood growth factors, and [transfusions of platelets and red blood cells](#),<sup>2</sup> or procedures to lower white blood cell counts (for leukostasis) might be needed to treat or help prevent some of these conditions.

## Hyperlinks

1. [www.cancer.org/treatment/treatments-and-side-effects/physical-side-effects/infections/infections-in-people-with-cancer.html](http://www.cancer.org/treatment/treatments-and-side-effects/physical-side-effects/infections/infections-in-people-with-cancer.html)
2. [www.cancer.org/treatment/treatments-and-side-effects/treatment-types/blood-transfusion-and-donation.html](http://www.cancer.org/treatment/treatments-and-side-effects/treatment-types/blood-transfusion-and-donation.html)

## References

Horton TM, Steuber CP. Overview of the treatment of acute lymphoblastic leukemia in children and adolescents. UpToDate. 2018. Accessed at [www.uptodate.com/contents/overview-of-the-treatment-of-acute-lymphoblastic-leukemia-in-children-and-adolescents](http://www.uptodate.com/contents/overview-of-the-treatment-of-acute-lymphoblastic-leukemia-in-children-and-adolescents) on December 29, 2018.

Tarlock K, Cooper TM. Acute myeloid leukemia in children and adolescents. UpToDate. 2018. Accessed at [www.uptodate.com/contents/acute-myeloid-leukemia-in-children-and-adolescents](http://www.uptodate.com/contents/acute-myeloid-leukemia-in-children-and-adolescents) on December 29, 2018.

Last Medical Review: February 12, 2019 Last Revised: February 12, 2019

# Surgery for Childhood Leukemia

Surgery has a very limited role in treating childhood leukemia. Because leukemia cells spread widely throughout the bone marrow and blood, it's not possible to cure this type of cancer with surgery. Aside from a possible [lymph node biopsy](#)<sup>1</sup>, surgery rarely has any role even in diagnosing leukemia, since this is usually done with a bone marrow aspirate and biopsy can usually diagnose leukemia.

## Placing a central venous catheter

Often before [chemotherapy](#)<sup>2</sup> is about to start, surgery is needed to insert a small plastic tube, called a [central venous catheter \(CVC\) or venous access<sup>3</sup>device \(VAD\)](#)<sup>4</sup>, into a large blood vessel. The end of the tube stays just under the skin or sticks out in the chest area or upper arm.

The CVC is left in place during treatment (often for many months) to give intravenous (IV) drugs such as chemotherapy and to take blood samples. This lowers the number of needle sticks needed during treatment. It's very important for parents to learn how to care for the catheter to keep it from getting infected.

For more information on surgery as a treatment for cancer, see [Cancer Surgery](#)<sup>5</sup>.

## Hyperlinks

1. [www.cancer.org/cancer/leukemia-in-children/detection-diagnosis-staging/how-diagnosed.html](http://www.cancer.org/cancer/leukemia-in-children/detection-diagnosis-staging/how-diagnosed.html)
2. [www.cancer.org/cancer/leukemia-in-children/treating/chemotherapy.html](http://www.cancer.org/cancer/leukemia-in-children/treating/chemotherapy.html)
3. [www.cancer.org/treatment/treatments-and-side-effects/central-venous-catheters.html](http://www.cancer.org/treatment/treatments-and-side-effects/central-venous-catheters.html)
4. [www.cancer.org/treatment/treatments-and-side-effects/central-venous-catheters.html](http://www.cancer.org/treatment/treatments-and-side-effects/central-venous-catheters.html)
5. [www.cancer.org/treatment/treatments-and-side-effects/treatment-types/surgery.html](http://www.cancer.org/treatment/treatments-and-side-effects/treatment-types/surgery.html)

## References

Horton TM, Steuber CP. Overview of the treatment of acute lymphoblastic leukemia in children and adolescents. UpToDate. 2018. Accessed at

www.uptodate.com/contents/overview-of-the-treatment-of-acute-lymphoblastic-leukemia-in-children-and-adolescents on December 29, 2018.

National Cancer Institute. Childhood Acute Lymphoblastic Leukemia Treatment (PDQ®)–Health Professional Version. Accessed at <https://www.cancer.gov/types/leukemia/hp/child-all-treatment-pdq> on December 29, 2018.

National Cancer Institute. Childhood Acute Myeloid Leukemia/Other Myeloid Malignancies Treatment (PDQ®)–Health Professional Version. Accessed at <https://www.cancer.gov/types/leukemia/hp/child-aml-treatment-pdq> on December 29, 2018.

Tarlock K, Cooper TM. Acute myeloid leukemia in children and adolescents. UpToDate. 2018. Accessed at [www.uptodate.com/contents/acute-myeloid-leukemia-in-children-and-adolescents](http://www.uptodate.com/contents/acute-myeloid-leukemia-in-children-and-adolescents) on December 29, 2018.

Last Medical Review: February 12, 2019 Last Revised: February 12, 2019

---

## Radiation Therapy for Childhood Leukemia

Radiation therapy uses high-energy radiation to kill cancer cells.

Radiation is not always needed to treat leukemia, but it can be used in certain situations:

- It is sometimes used to try to prevent or treat the spread of leukemia to the brain or treat the testicles in boys if the leukemia has reached them. But [chemotherapy](#)<sup>1</sup> is often used in these situations instead.
- It can be used (rarely) to treat a tumor that is pressing on the trachea (windpipe). But chemotherapy is often used instead, as it may work more quickly.
- Radiation to the whole body is often an important part of treatment before a stem cell transplant (see [High-Dose Chemotherapy and Stem Cell Transplant](#)<sup>2</sup>).

## How is radiation therapy given?

Before treatment starts, the radiation team will take careful body measurements to determine the correct angles for aiming the radiation beams and the proper dose of radiation. This planning session, called simulation, usually includes getting [imaging tests](#)<sup>3</sup> such as CT or MRI scans.

The treatment itself is much like getting an x-ray, but the radiation is much stronger. It is painless, but some younger children may need to be sedated to make sure they don't move during the treatment. Each treatment lasts only a few minutes, although the setup time – getting your child into place for treatment – usually takes longer.

## Possible side effects of radiation

The possible **short-term** [side effects](#)<sup>4</sup> depend on where the radiation is aimed, and can include:

- Sunburn-like skin changes
- Hair loss in the treated area
- Nausea, vomiting, or diarrhea (from radiation to the abdomen)
- Fatigue
- Increased risk of infection

**Longer-term side effects** are also possible and are described in [Living as a Childhood Leukemia Survivor](#)<sup>5</sup>.

More information on radiation therapy can be found in [Radiation Therapy](#)<sup>6</sup>.

## Hyperlinks

1. [www.cancer.org/cancer/leukemia-in-children/treating/chemotherapy.html](http://www.cancer.org/cancer/leukemia-in-children/treating/chemotherapy.html)
2. [www.cancer.org/cancer/leukemia-in-children/treating/bone-marrow.html](http://www.cancer.org/cancer/leukemia-in-children/treating/bone-marrow.html)
3. [www.cancer.org/cancer/leukemia-in-children/detection-diagnosis-staging/how-diagnosed.html](http://www.cancer.org/cancer/leukemia-in-children/detection-diagnosis-staging/how-diagnosed.html)
4. [www.cancer.org/treatment/treatments-and-side-effects/physical-side-effects.html](http://www.cancer.org/treatment/treatments-and-side-effects/physical-side-effects.html)
5. [www.cancer.org/cancer/leukemia-in-children/after-treatment/follow-up.html](http://www.cancer.org/cancer/leukemia-in-children/after-treatment/follow-up.html)
6. [www.cancer.org/treatment/treatments-and-side-effects/treatment-types/radiation.html](http://www.cancer.org/treatment/treatments-and-side-effects/treatment-types/radiation.html)

## References

Horton TM, Steuber CP. Overview of the treatment of acute lymphoblastic leukemia in children and adolescents. UpToDate. 2018. Accessed at [www.uptodate.com/contents/overview-of-the-treatment-of-acute-lymphoblastic-leukemia-in-children-and-adolescents](http://www.uptodate.com/contents/overview-of-the-treatment-of-acute-lymphoblastic-leukemia-in-children-and-adolescents) on December 29, 2018.

National Cancer Institute. Childhood Acute Lymphoblastic Leukemia Treatment (PDQ®)—Health Professional Version. Accessed at <https://www.cancer.gov/types/leukemia/hp/child-all-treatment-pdq> on December 29, 2018.

National Cancer Institute. Childhood Acute Myeloid Leukemia/Other Myeloid Malignancies Treatment (PDQ®)—Health Professional Version. Accessed at <https://www.cancer.gov/types/leukemia/hp/child-aml-treatment-pdq> on December 29, 2018.

Tarlock K, Cooper TM. Acute myeloid leukemia in children and adolescents. UpToDate. 2018. Accessed at [www.uptodate.com/contents/acute-myeloid-leukemia-in-children-and-adolescents](http://www.uptodate.com/contents/acute-myeloid-leukemia-in-children-and-adolescents) on December 29, 2018.

Last Medical Review: February 12, 2019 Last Revised: February 12, 2019

---

# Chemotherapy for Childhood Leukemia

Chemotherapy (chemo) is the main treatment for most childhood leukemias. This is treatment with anti-cancer drugs that are given in a vein (IV), in a muscle, in the cerebrospinal fluid (CSF) around the brain and spinal cord, or are taken by mouth. Except when given in the CSF, chemo drugs enter the bloodstream and reach all areas of the body, making this treatment very useful for cancers such as leukemia.

Leukemia is treated with combinations of several chemo drugs. Doctors give chemo in cycles, with each period of treatment followed by a rest period to give the body time to recover.

In general, [treatment for acute myeloid leukemia \(AML\)](#)<sup>1</sup> uses higher doses of chemo over a shorter period of time (usually less than a year), and [treatment for acute](#)

[lymphocytic leukemia \(ALL\)](#)<sup>2</sup> uses lower doses of chemo over a longer period of time (usually 2 to 3 years).

Some of the chemo drugs used to treat childhood leukemia include:

- Vincristine
- Daunorubicin, (daunomycin)
- Doxorubicin (Adriamycin)
- Idarubicin
- Cytarabine (cytosine arabinoside or ara-C)
- L-asparaginase, PEG-L-asparaginase (pegaspargase)
- Etoposide
- 6-mercaptopurine (6-MP)
- 6-thioguanine (6-TG)
- Methotrexate
- Mitoxantrone
- Cyclophosphamide
- Corticosteroid drugs such as prednisone, prednisolone, dexamethasone, or hydrocortisone

Children will probably get several of these drugs at different times during the course of treatment, but they do not get all of them.

### **Possible side effects of chemo**

Chemo drugs can affect some normal cells in the body, which can lead to side effects.

The [side effects of chemo](#)<sup>3</sup> depend on the type and dose of drugs given and the length of treatment. These side effects can include:

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

Chemo drugs also affect the normal cells in bone marrow, which can lower blood cell counts. This can lead to:

- Increased risk of infections (from having too few normal white blood cells)
- Bruising and bleeding easily (from having too few blood platelets)
- Fatigue (from having too few red blood cells)

The problems with blood cell counts are often caused by the leukemia itself at first. They might get worse during the first part of treatment because of the chemo, but they will probably improve as the leukemia cells are killed off and the normal cells in the bone marrow recover.

Most side effects usually go away when treatment is finished. There are often ways to reduce these side effects. For instance, drugs can be given to help prevent or reduce nausea and vomiting. Other drugs known as **growth factors** can be given to help keep the blood cell counts higher.

**Tumor lysis syndrome:** This side effect of chemo can happen in children who had large numbers of leukemia cells in the body before 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. Too much of certain minerals can also affect the heart and nervous system. This problem can be prevented by making sure the child gets lots of fluids during treatment and certain drugs, such as bicarbonate, allopurinol, and rasburicase, which help the body get rid of these substances.

Some chemo drugs can also have other specific side effects. For example:

- Vincristine can damage nerves, which can lead to numbness, tingling, or weakness in hands or feet (known as [peripheral neuropathy](#)<sup>4</sup>).
- L-asparaginase and PEG-L-asparaginase can increase the risk of blood clots.

Some chemo drugs can also cause **late or long-term side effects**, such as effects on growth and development, effects on fertility later in life, or an increased risk of getting a second cancer (often AML). For more on this, see [Living as a Childhood Leukemia Survivor](#)<sup>5</sup>.

Be sure to ask your child's doctor or nurse about any specific side effects you should watch for and about what you can do to help reduce these side effects.

Chemo given directly into the cerebrospinal fluid (CSF) around the brain and spinal cord (known as **intrathecal chemotherapy**) can have its own side effects, although these are not common. Intrathecal chemo may cause trouble thinking or even seizures in

some children.

For more information on chemo, see [Chemotherapy](#)<sup>6</sup>.

## Hyperlinks

1. [www.cancer.org/cancer/leukemia-in-children/treating/children-with-aml.html](http://www.cancer.org/cancer/leukemia-in-children/treating/children-with-aml.html)
2. [www.cancer.org/cancer/leukemia-in-children/treating/children-with-all.html](http://www.cancer.org/cancer/leukemia-in-children/treating/children-with-all.html)
3. [www.cancer.org/treatment/treatments-and-side-effects/physical-side-effects.html](http://www.cancer.org/treatment/treatments-and-side-effects/physical-side-effects.html)
4. [www.cancer.org/treatment/treatments-and-side-effects/physical-side-effects/peripheral-neuropathy.html](http://www.cancer.org/treatment/treatments-and-side-effects/physical-side-effects/peripheral-neuropathy.html)
5. [www.cancer.org/cancer/leukemia-in-children/after-treatment/follow-up.html](http://www.cancer.org/cancer/leukemia-in-children/after-treatment/follow-up.html)
6. [www.cancer.org/treatment/treatments-and-side-effects/treatment-types/chemotherapy.html](http://www.cancer.org/treatment/treatments-and-side-effects/treatment-types/chemotherapy.html)

## References

Horton TM, Steuber CP. Overview of the treatment of acute lymphoblastic leukemia in children and adolescents. UpToDate. 2018. Accessed at [www.uptodate.com/contents/overview-of-the-treatment-of-acute-lymphoblastic-leukemia-in-children-and-adolescents](http://www.uptodate.com/contents/overview-of-the-treatment-of-acute-lymphoblastic-leukemia-in-children-and-adolescents) on December 29, 2018.

National Cancer Institute. Childhood Acute Lymphoblastic Leukemia Treatment (PDQ®)—Health Professional Version. Accessed at <https://www.cancer.gov/types/leukemia/hp/child-all-treatment-pdq> on December 29, 2018.

National Cancer Institute. Childhood Acute Myeloid Leukemia/Other Myeloid Malignancies Treatment (PDQ®)—Health Professional Version. Accessed at <https://www.cancer.gov/types/leukemia/hp/child-aml-treatment-pdq> on December 29, 2018.

Tarlock K, Cooper TM. Acute myeloid leukemia in children and adolescents. UpToDate. 2018. Accessed at [www.uptodate.com/contents/acute-myeloid-leukemia-in-children-and-adolescents](http://www.uptodate.com/contents/acute-myeloid-leukemia-in-children-and-adolescents) on December 29, 2018.

Last Medical Review: February 12, 2019 Last Revised: February 12, 2019

# Targeted Therapy Drugs for Childhood Leukemia

In recent years, new drugs that target specific parts of cancer cells have been developed. These targeted drugs work differently from standard [chemotherapy](#)<sup>1</sup> drugs. They can be used instead of or along with chemo in some situations, and they have side effects that are different from those of chemo. Some targeted drugs can be useful in certain childhood leukemias.

## BCR-ABL inhibitors for CML (and some cases of ALL)

Nearly all children with [chronic myeloid leukemia \(CML\)](#)<sup>2</sup> have an abnormal chromosome in their leukemia cells known as the [Philadelphia chromosome](#)<sup>3</sup>. These chromosomes have a specific gene mutation known as *BCR-ABL*, which helps the leukemia cells grow.

Targeted drugs known as tyrosine kinase inhibitors (TKIs), such as **imatinib (Gleevec)**, **dasatinib (Sprycel)**, and **nilotinib (Tasigna)** attack cells that have the *BCR-ABL* gene mutation. These drugs are very effective at controlling the leukemia for long periods of time in most children, although it's not yet clear if the drugs can help cure CML. These drugs are taken daily as pills.

A small number of children with [acute lymphocytic leukemia \(ALL\)](#)<sup>4</sup> also have the Philadelphia chromosome in their leukemia cells. Studies have shown that their outcome is improved when these drugs are given along with chemotherapy.

Possible side effects include diarrhea, nausea, muscle pain, fatigue, and skin rashes. These are generally mild. A common side effect is swelling around the eyes or in the hands or feet, which may be caused by the drugs' effects on the heart. Other possible side effects include lower red blood cell and platelet counts when treatment starts. These drugs might also slow a child's growth, especially if used before puberty.

## Gemtuzumab ozogamicin (Mylotarg) for AML

This targeted therapy is a monoclonal antibody (a man-made immune protein) linked to a chemotherapy drug. 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 to treat some children with [acute myeloid leukemia \(AML\)](#)<sup>5</sup> that has come back after treatment or is no longer responding to treatment. It is given as an infusion in a vein (IV), typically for 3 doses, with 3 days in between each dose.

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). Your child likely will 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 heart rhythm

Many other targeted drugs are now being used to treat AML in adults, and some of these are now being tested in [clinical trials](#)<sup>6</sup> for use in children as well. (See [What's New in Childhood Leukemia Research?](#)<sup>7</sup>)

## Differentiation agents for APL

Acute promyelocytic leukemia (APL) is different from other subtypes of AML in some important ways. The leukemia cells in APL (called blasts), have certain gene changes that stop them from maturing into normal white blood cells. Drugs called **differentiation agents** can help the blasts mature (differentiate) into normal white blood cells. Two of these drugs can be used to [treat APL](#)<sup>8</sup>:

- All-trans-retinoic acid (ATRA, tretinoin)
- Arsenic trioxide (ATO)

**ATRA** is a form of vitamin A that is typically part of the initial treatment of APL. It is given either along with chemo or along with ATO. It can also be used during later phases of treatment. Side effects can include:

- Headache
- Fever
- Dry skin and mouth

- Skin rash
- Swollen feet
- Sores in the mouth or throat
- Itching
- Irritated eyes

It can also raise blood lipid levels (like cholesterol and triglycerides). Often blood liver test results become abnormal. These side effects often go away when the drug is stopped.

**Arsenic trioxide (ATO)** can act in a way similar to ATRA in patients with APL. It can be given with ATRA in the initial treatment of APL, but it is also helpful in treating APL that comes back after treatment with ATRA plus chemo. Most side effects are mild and can include:

- Feeling tired
- Nausea
- Vomiting
- Diarrhea
- Belly pain
- Nerve damage (neuropathy), leading to numbness and tingling in the hands and feet

ATO can also cause problems with heart rhythm, which can sometimes be serious.

Both of these drugs can cause a serious side effect 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 couple of weeks of treatment, and in patients with a high white blood cell count.

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. While differentiation syndrome can be serious, it can often be treated by stopping the drugs for a while and giving a steroid such as dexamethasone.

For more general information on targeted drugs, see [Targeted Therapy](#)<sup>9</sup>.

## Hyperlinks

1. [www.cancer.org/cancer/leukemia-in-children/treating/chemotherapy.html](http://www.cancer.org/cancer/leukemia-in-children/treating/chemotherapy.html)
2. [www.cancer.org/cancer/leukemia-in-children/treating/children-with-cml.html](http://www.cancer.org/cancer/leukemia-in-children/treating/children-with-cml.html)
3. [www.cancer.org/cancer/leukemia-in-children/causes-risks-prevention/what-causes.html](http://www.cancer.org/cancer/leukemia-in-children/causes-risks-prevention/what-causes.html)
4. [www.cancer.org/cancer/leukemia-in-children/treating/children-with-all.html](http://www.cancer.org/cancer/leukemia-in-children/treating/children-with-all.html)
5. [www.cancer.org/cancer/leukemia-in-children/treating/children-with-aml.html](http://www.cancer.org/cancer/leukemia-in-children/treating/children-with-aml.html)
6. [www.cancer.org/treatment/treatments-and-side-effects/clinical-trials.html](http://www.cancer.org/treatment/treatments-and-side-effects/clinical-trials.html)
7. [www.cancer.org/cancer/leukemia-in-children/about/new-research.html](http://www.cancer.org/cancer/leukemia-in-children/about/new-research.html)
8. [www.cancer.org/cancer/leukemia-in-children/treating/children-with-apl.html](http://www.cancer.org/cancer/leukemia-in-children/treating/children-with-apl.html)
9. [www.cancer.org/treatment/treatments-and-side-effects/treatment-types/targeted-therapy.html](http://www.cancer.org/treatment/treatments-and-side-effects/treatment-types/targeted-therapy.html)

## References

Horton TM, Steuber CP. Overview of the treatment of acute lymphoblastic leukemia in children and adolescents. UpToDate. 2018. Accessed at [www.uptodate.com/contents/overview-of-the-treatment-of-acute-lymphoblastic-leukemia-in-children-and-adolescents](http://www.uptodate.com/contents/overview-of-the-treatment-of-acute-lymphoblastic-leukemia-in-children-and-adolescents) on December 29, 2018.

National Cancer Institute. Childhood Acute Lymphoblastic Leukemia Treatment (PDQ®)—Health Professional Version. Accessed at <https://www.cancer.gov/types/leukemia/hp/child-all-treatment-pdq> on December 29, 2018.

National Cancer Institute. Childhood Acute Myeloid Leukemia/Other Myeloid Malignancies Treatment (PDQ®)—Health Professional Version. Accessed at <https://www.cancer.gov/types/leukemia/hp/child-aml-treatment-pdq> on December 29, 2018.

Tarlock K, Cooper TM. Acute myeloid leukemia in children and adolescents. UpToDate. 2018. Accessed at [www.uptodate.com/contents/acute-myeloid-leukemia-in-children-and-adolescents](http://www.uptodate.com/contents/acute-myeloid-leukemia-in-children-and-adolescents) on December 29, 2018.

Last Medical Review: February 12, 2019 Last Revised: February 12, 2019

# Immunotherapy for Childhood Leukemia

Immunotherapy is the use of medicines to help a patient's own immune system recognize and destroy cancer cells. Several types of immunotherapy are being studied for use against childhood leukemia, and some are now coming into use.

## Chimeric antigen receptor (CAR) T-cell therapy

For this treatment, immune cells called **T cells** are removed from the child's blood and genetically altered in the lab to have specific receptors (called **chimeric antigen receptors**, or CARs) on their surface. These receptors can attach to proteins on leukemia cells. The T cells are then multiplied in the lab and given back into the child's blood, where they can seek out the leukemia cells and attack them.

### Tisagenlecleucel (Kymriah)

This is a type of CAR T-cell therapy that targets the CD19 protein on certain leukemia cells. It can be used to treat childhood acute lymphoblastic leukemia (ALL) that has come back after treatment or that is no longer responding to treatment.

To make this treatment, T cells are removed from the child's blood during a process called **leukapheresis**. Blood is removed through an IV line and goes into a machine that removes the T cells. The remaining blood then goes back into the body. This typically takes a few hours, and it might need to be repeated. The cells are then frozen and sent to a lab, where they are turned into CAR T cells and are multiplied. This process can take a few weeks.

For the treatment itself, the child typically gets chemotherapy for a few days to help prepare the body. Then the CAR T cells are infused into a vein.

In most children who have had this treatment, the leukemia could no longer be detected within a few months of treatment, although it's not yet clear if this means that they have been cured.

### Possible side effects

This treatment can have serious or even life-threatening side effects, which is why it needs to be given in a medical center that is specially trained in its use.

**Cytokine release syndrome (CRS):** CRS happens when T cells release chemicals

(cytokines) that ramp up the immune system. This can happen within a few days to weeks after treatment, and can be life-threatening. Symptoms can include:

- High fever and chills
- Trouble breathing
- Severe nausea, vomiting, and/or diarrhea
- Severe muscle or joint pain
- Feeling dizzy or lightheaded

**Nervous system problems:** This drug can have serious effects on the nervous system, which can result in symptoms such as:

- Headaches
- Changes in consciousness
- Confusion or agitation
- Seizures
- Trouble speaking and understanding
- Loss of balance

**Other serious side effects:** Other possible side effects can include:

- Serious infections
- Low blood cell counts, which can increase the risk of infections, fatigue, and bruising or bleeding

It's very important to report any side effects to the health care team right away, as there are often medicines that can help treat them.

## Monoclonal antibodies

Antibodies are proteins made by the body's immune system to help fight infections. Man-made versions of these proteins, called **monoclonal antibodies**, can be designed to attack a specific target, such as a protein on the surface of leukemia cells.

### Blinatumomab (Blincyto)

Blinatumomab is a special kind of monoclonal antibody because it can attach to 2 different proteins at the same time. One part of blinatumomab attaches to the CD19

protein, which is found on B cells. Another part attaches to CD3, a protein found on immune cells called T cells. By binding to both of these proteins, this drug brings the cancer cells and immune cells together, which helps the immune system attack the cancer cells.

This drug is used to treat some types of B-cell ALL, typically after chemotherapy has been used. It is given into a vein (IV) as a continuous infusion over 28 days. This may be repeated after 2 weeks off. Because of certain serious side effects that occur more often during the first few times it is given, the child usually needs stay in the hospital for the first few days of at least the first 2 cycles.

The most common **side effects** are fever, headache, swelling of the feet and hands, nausea, tremor, rash, constipation, and low blood potassium levels. It can also cause low white blood cell counts, which increase the risk of serious infection.

This drug can also cause nervous system problems, such as seizures, trouble speaking or slurred speech, passing out, confusion, and loss of balance.

Some children might have serious reactions during the infusion (similar to an allergic reaction). Symptoms can include feeling lightheaded or dizzy (due to low blood pressure), headache, nausea, fever or chills, shortness of breath, and/or wheezing. Your child will be given medicines before each infusion to help prevent this.

### **Gemtuzumab ozogamicin (Mylotarg)**

This monoclonal antibody, which can be used to treat acute myeloid leukemia (AML), works in a different way. It is described in [Targeted Therapy Drugs for Childhood Leukemia<sup>1</sup>](#).

Other types of immunotherapy are also being studied for use against leukemia.

## **Hyperlinks**

1. [www.cancer.org/cancer/leukemia-in-children/treating/targeted-therapy.html](http://www.cancer.org/cancer/leukemia-in-children/treating/targeted-therapy.html)

## **References**

Horton TM, Steuber CP. Overview of the treatment of acute lymphoblastic leukemia in children and adolescents. UpToDate. 2018. Accessed at [www.uptodate.com/contents/overview-of-the-treatment-of-acute-lymphoblastic-](http://www.uptodate.com/contents/overview-of-the-treatment-of-acute-lymphoblastic-)

leukemia-in-children-and-adolescents on December 29, 2018.

National Cancer Institute. Childhood Acute Lymphoblastic Leukemia Treatment (PDQ®)—Health Professional Version. Accessed at <https://www.cancer.gov/types/leukemia/hp/child-all-treatment-pdq> on December 29, 2018.

National Cancer Institute. Childhood Acute Myeloid Leukemia/Other Myeloid Malignancies Treatment (PDQ®)—Health Professional Version. Accessed at <https://www.cancer.gov/types/leukemia/hp/child-aml-treatment-pdq> on December 29, 2018.

Tarlock K, Cooper TM. Acute myeloid leukemia in children and adolescents. UpToDate. 2018. Accessed at [www.uptodate.com/contents/acute-myeloid-leukemia-in-children-and-adolescents](http://www.uptodate.com/contents/acute-myeloid-leukemia-in-children-and-adolescents) on December 29, 2018.

Last Medical Review: February 12, 2019 Last Revised: February 12, 2019

---

## High-dose Chemotherapy and Stem Cell Transplant for Childhood Leukemia

A stem cell transplant (SCT) (also known as a **bone marrow transplant**) can sometimes be used to help improve the chances of curing childhood leukemia. SCT lets doctors use even higher doses of [chemotherapy](#)<sup>1</sup> than a child could normally tolerate.

High-dose chemotherapy destroys the bone marrow, which is where leukemia starts, but it's also where new blood cells are formed. This could lead to life-threatening infections, bleeding, and other problems caused by low blood cell counts. A stem cell transplant is given after the chemo to restore the blood-forming stem cells in the 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.

### Allogeneic stem cell transplant

For childhood leukemias, the type of transplant used is known as an allogeneic stem cell transplant. In this type of transplant, the blood-forming stem cells are donated from another person.

The donor's tissue type (also known as the **HLA type**) should match the patient's tissue type as closely as possible to help prevent the risk of major problems with the transplant. Tissue type is based on certain substances on the surface of cells in the body. The closer the tissue match between the donor and the recipient, the better the chance the transplanted cells will "take" and begin making new blood cells.

The donor is usually a brother or sister with the same tissue type as the patient. Rarely, it can be an HLA-matched, unrelated donor – a stranger who has volunteered to donate blood-forming stem cells. 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. (This blood is rich in stem cells.) Whatever their source, the stem cells are then frozen and stored until they are needed for the transplant.

To learn about how a stem cell transplant is done, see [Stem Cell Transplant for Cancer<sup>2</sup>](#).

### When a stem cell transplant might be used

**Acute lymphocytic leukemia (ALL):** In ALL, SCT might be used in children in some [high-risk groups<sup>3</sup>](#), whose leukemia is more likely to come back after the [initial \(induction\) chemo<sup>4</sup>](#). In this case, the transplant is done after the induction chemo puts the leukemia into remission.

SCT might also be an option if the leukemia doesn't respond well to initial treatment, or if it relapses (comes back) soon after going into remission. It's less clear if SCT should be used for children whose ALL relapses later (such as more than 6 months or a year) after finishing the initial chemo. These children will often do well with another round of standard dose chemo.

SCT may also be recommended for children with some less common forms of ALL, such as those whose leukemias have the Philadelphia chromosome or those with T-cell ALL that don't respond well to initial treatment.

**Acute myelogenous leukemia (AML):** Because AML relapses more often than ALL, SCT might be recommended right after the AML has gone into remission (after the [initial chemo treatment<sup>5</sup>](#)), if the child has a brother or sister with the same tissue type who can donate stem cells for the transplant. This is especially true if there is a very high risk of relapse (as with some subtypes of AML or when there are certain gene or chromosome

changes in the leukemia cells). There is still some debate about which children with AML need this type of intensive treatment.

If a child with AML relapses after his or her first round of standard chemo, most doctors will recommend SCT as soon as the child goes into remission again.

In either case, it is important that the leukemia is in remission before getting a stem cell transplant. Otherwise, the leukemia is more likely to return.

**Other leukemias:** SCT might also offer the best chance to cure some less common types of childhood leukemia, such as [juvenile myelomonocytic leukemia \(JMML\)](#)<sup>6</sup> and [chronic myelogenous leukemia \(CML\)](#)<sup>7</sup>. For CML, newer [targeted therapy drugs](#)<sup>8</sup> are likely to be used first for most children, but a transplant might still be needed at some point.

## Practical points

A stem cell transplant is a complex treatment that can cause life-threatening [side effects](#)<sup>9</sup>. If the doctors think your child can benefit from a transplant, the best place to have this done is at a cancer center where the staff has experience with the procedure and with managing the recovery period.

A stem cell transplant often requires a long hospital stay and can be very expensive. Even if the transplant is covered by your insurance, your co-pays or other costs could easily amount to many thousands of dollars. It's important to find out what your insurer will cover before the transplant to get an idea of what you might have to pay.

Be sure to talk to your child's doctor before the transplant to learn about possible long-term side effects your child might have. More information on long-term effects can be found in [Living as a Childhood Leukemia Survivor](#)<sup>10</sup>.

To learn more about the details of stem cell transplants, including how they're done and the possible risks and side effects, see [Stem Cell Transplant for Cancer](#)<sup>11</sup>.

## Hyperlinks

1. [www.cancer.org/cancer/leukemia-in-children/treating/chemotherapy.html](http://www.cancer.org/cancer/leukemia-in-children/treating/chemotherapy.html)
2. [www.cancer.org/treatment/treatments-and-side-effects/treatment-types/stem-cell-transplant.html](http://www.cancer.org/treatment/treatments-and-side-effects/treatment-types/stem-cell-transplant.html)
3. [www.cancer.org/cancer/leukemia-in-children/detection-diagnosis-staging/prognostic-factors.html](http://www.cancer.org/cancer/leukemia-in-children/detection-diagnosis-staging/prognostic-factors.html)

4. [www.cancer.org/cancer/leukemia-in-children/treating/children-with-all.html](http://www.cancer.org/cancer/leukemia-in-children/treating/children-with-all.html)
5. [www.cancer.org/cancer/leukemia-in-children/treating/children-with-aml.html](http://www.cancer.org/cancer/leukemia-in-children/treating/children-with-aml.html)
6. [www.cancer.org/cancer/leukemia-in-children/treating/children-with-jmml.html](http://www.cancer.org/cancer/leukemia-in-children/treating/children-with-jmml.html)
7. [www.cancer.org/cancer/leukemia-in-children/treating/children-with-cml.html](http://www.cancer.org/cancer/leukemia-in-children/treating/children-with-cml.html)
8. [www.cancer.org/cancer/leukemia-in-children/treating/targeted-therapy.html](http://www.cancer.org/cancer/leukemia-in-children/treating/targeted-therapy.html)
9. [www.cancer.org/treatment/treatments-and-side-effects/treatment-types/stem-cell-transplant/transplant-side-effects.html](http://www.cancer.org/treatment/treatments-and-side-effects/treatment-types/stem-cell-transplant/transplant-side-effects.html)
10. [www.cancer.org/cancer/leukemia-in-children/after-treatment/follow-up.html](http://www.cancer.org/cancer/leukemia-in-children/after-treatment/follow-up.html)
11. [www.cancer.org/treatment/treatments-and-side-effects/treatment-types/stem-cell-transplant.html](http://www.cancer.org/treatment/treatments-and-side-effects/treatment-types/stem-cell-transplant.html)

## References

Horton TM, Steuber CP. Overview of the treatment of acute lymphoblastic leukemia in children and adolescents. UpToDate. 2018. Accessed at [www.uptodate.com/contents/overview-of-the-treatment-of-acute-lymphoblastic-leukemia-in-children-and-adolescents](http://www.uptodate.com/contents/overview-of-the-treatment-of-acute-lymphoblastic-leukemia-in-children-and-adolescents) on December 29, 2018.

National Cancer Institute. Childhood Acute Lymphoblastic Leukemia Treatment (PDQ®)—Health Professional Version. Accessed at <https://www.cancer.gov/types/leukemia/hp/child-all-treatment-pdq> on December 29, 2018.

National Cancer Institute. Childhood Acute Myeloid Leukemia/Other Myeloid Malignancies Treatment (PDQ®)—Health Professional Version. Accessed at <https://www.cancer.gov/types/leukemia/hp/child-aml-treatment-pdq> on December 29, 2018.

Tarlock K, Cooper TM. Acute myeloid leukemia in children and adolescents. UpToDate. 2018. Accessed at [www.uptodate.com/contents/acute-myeloid-leukemia-in-children-and-adolescents](http://www.uptodate.com/contents/acute-myeloid-leukemia-in-children-and-adolescents) on December 29, 2018.

Last Medical Review: February 12, 2019 Last Revised: February 12, 2019

---

# Treatment of Children With Acute

# Lymphocytic Leukemia (ALL)

The main treatment for children with acute lymphocytic (lymphoblastic) leukemia (ALL) is [chemotherapy](#)<sup>1</sup>, which is usually given in 3 main phases:

- Induction
- Consolidation (also called intensification)
- Maintenance

The entire length of treatment is typically about 2 to 3 years, with the most intense treatment in the first few months.

Children with ALL are typically classified by [risk group](#)<sup>2</sup> to make sure that the correct types and doses of drugs are given. Treatment may be more or less intense, depending on the risk group.

## Induction

The goal of induction chemotherapy is to achieve a **remission**. This means that leukemia cells are no longer found in bone marrow samples, the normal marrow cells return, and the blood counts become normal. (A remission is not necessarily a cure.) More than 95% of children with ALL enter remission after 1 month of induction treatment.

This first month is intense and requires prolonged hospital stays for treatment and frequent visits to the doctor. Your child may spend some or much of this time in the hospital, because serious [infections](#)<sup>3</sup> or other complications can occur. It is very important to take all medicines as prescribed. Sometimes complications can be serious enough to be life-threatening, but in recent years, advances in supportive care (nursing care, nutrition, antibiotics, red blood cell and platelet transfusions as needed, etc.) have made these much less common than in the past.

Children with standard-risk ALL often receive 3 drugs for the first month of treatment. These include the chemotherapy drugs L-asparaginase and vincristine, and a steroid drug (such as dexamethasone). For children in high-risk groups, a fourth chemo drug in the anthracycline class (most often daunorubicin) is typically added. Other drugs that may be given early are methotrexate and/or 6-mercaptopurine.

Children with Philadelphia chromosome-positive ALL may benefit from the addition of a [targeted drug](#)<sup>4</sup> such as imatinib (Gleevec).

**Intrathecal chemotherapy:** All children also get chemo into the cerebrospinal fluid (CSF) to kill any leukemia cells that might have spread to the brain and spinal cord. This treatment, known as intrathecal chemotherapy, is given through a [lumbar puncture \(spinal tap\)](#)<sup>5</sup>. It is usually given twice (or more if the leukemia is high risk or leukemia cells have been found in the CSF) during the first month and several times during the next 1 or 2 months. It is then repeated less often during the rest of treatment.

Usually, methotrexate is the drug used for intrathecal chemo. Hydrocortisone (a steroid) and cytarabine (ara-C) may be added, particularly in high-risk children.

Along with intrathecal chemo, some high-risk patients (for example, those with T-cell ALL) and those with many leukemia cells in their CSF when the leukemia is diagnosed may be given [radiation therapy](#)<sup>6</sup> to the brain. This was more common in the past, but recent studies have found that many children even with high-risk ALL may not need radiation therapy if they are given more intensive chemo. Doctors try to avoid giving radiation to the brain if possible, especially in younger children, because no matter how low the dose is kept, it can cause problems with thinking, growth, and development.

A possible side effect of intrathecal chemo is seizures during treatment, which happen in a small percentage of children. Children who develop seizures are treated with drugs to prevent them.

### **Consolidation (intensification)**

The next, and usually more intense, consolidation phase of chemo starts once the leukemia is in remission and typically lasts for several months. This phase further reduces the number of leukemia cells still in the body. Several chemo drugs are combined to help prevent the remaining leukemia cells from developing resistance. Intrathecal chemo (as described above) is continued at this time.

Children with standard-risk ALL are usually treated with drugs such as methotrexate, 6-mercaptopurine (6-MP), vincristine, L-asparaginase, and/or prednisone, but regimens differ among cancer centers.

Children with high-risk leukemia (because of gene or chromosome changes in the leukemia cells, for example, or because there is still minimal residual disease after induction) generally get more intense chemo. Extra drugs such as L-asparaginase, doxorubicin (Adriamycin), etoposide, cyclophosphamide, and cytarabine (ara-C) are often used, and dexamethasone is substituted for prednisone.

There may be a second round of intense chemotherapy as part of consolidation. (This is known as **delayed intensification**.)

Children with Philadelphia chromosome-positive ALL may benefit from the addition of a [targeted drug](#)<sup>7</sup> such as imatinib (Gleevec).

For some children in high-risk groups, a [stem cell transplant](#)<sup>8</sup> might be an option at this time once the leukemia is in remission.

## Maintenance

If the leukemia remains in remission after induction and consolidation, maintenance therapy can begin. Most treatment plans use daily 6-mercaptopurine (6-MP) and weekly methotrexate, given as pills, often along with vincristine, which is given into a vein (IV), and a steroid (prednisone or dexamethasone). These latter 2 drugs are given for brief periods every 4 to 8 weeks. Other drugs may be added depending on the type of ALL and the risk of recurrence.

Some children at higher risk may get more intense maintenance chemo and intrathecal therapy.

## Treatment of residual disease

The treatment plans may change if the leukemia doesn't go into remission during induction or consolidation. The doctor will probably check the child's bone marrow soon after treatment starts to see if the leukemia is going away. If not, treatment might need to be more intense or prolonged.

If standard lab tests show the leukemia seems to have gone away, the doctor may use more [sensitive tests](#)<sup>9</sup> to look for even small numbers of remaining leukemia cells (known as **minimal residual disease**, or MRD). If any are found, chemotherapy again might need to be intensified or prolonged.

## Treatment of recurrent ALL

If the ALL recurs (comes back) during or after treatment, the child will most likely be treated again with [chemotherapy](#)<sup>10</sup>. Much of the treatment strategy depends on how soon the leukemia returns after the first treatment. If the relapse occurs after a long time, the same drugs might still be effective, so the same or similar treatment may be used to try to get the leukemia into a second remission.

If it comes back after a shorter time interval, more aggressive chemo with other drugs may be needed. The most commonly used chemo drugs are vincristine, L-asparaginase, anthracyclines (doxorubicin, daunorubicin, or mitoxantrone),

cyclophosphamide, cytarabine (ara-C), and either etoposide or teniposide. The child will also receive a steroid (prednisone or dexamethasone). Intrathecal chemo will also be given.

For children whose leukemia comes back sooner after starting treatment, or for children with T-cell ALL who relapse, a [stem cell transplant](#)<sup>11</sup> may be considered, especially if the child has a brother or sister who is a good tissue type match. Stem cell transplants may also be used for children who relapse after a second course of chemotherapy.

Some children have an **extramedullary relapse**, meaning that leukemia cells are found in one part of the body (such as the cerebrospinal fluid [CSF] or the testicles) but are not detectable in the bone marrow. In addition to intensive chemotherapy as described above, children with spread to the CSF may get more intense intrathecal chemotherapy, sometimes with [radiation](#)<sup>12</sup> to the brain and spinal cord (if that area had not been already treated with radiation). Boys with relapse in a testicle may get radiation to the area.

If ALL doesn't go away completely or if it comes back after a stem cell transplant, it can be very hard to treat. For some children, newer types of [immunotherapy](#)<sup>13</sup>, such as CAR T-cell therapy or blinatumomab (a monoclonal antibody) might be helpful.

### Philadelphia chromosome-type ALL

For children with certain types of ALL, such as those with the Philadelphia chromosome, standard chemotherapy for ALL (as outlined above) might not be as effective. A [stem cell transplant](#)<sup>14</sup> may be advised if induction treatment puts the leukemia in remission and a suitable stem cell donor is available.

Newer, [targeted drugs](#)<sup>15</sup> such as imatinib (Gleevec) and dasatinib (Sprycel) are designed to kill leukemia cells that have the Philadelphia chromosome. These drugs are taken as pills. Adding these drugs to chemotherapy throughout treatment seems to help improve outcomes, according to studies done so far.

### Hyperlinks

1. [www.cancer.org/cancer/leukemia-in-children/treating/chemotherapy.html](http://www.cancer.org/cancer/leukemia-in-children/treating/chemotherapy.html)
2. [www.cancer.org/cancer/leukemia-in-children/detection-diagnosis-staging/prognostic-factors.html](http://www.cancer.org/cancer/leukemia-in-children/detection-diagnosis-staging/prognostic-factors.html)
3. [www.cancer.org/treatment/treatments-and-side-effects/physical-side-effects/infections/infections-in-people-with-cancer.html](http://www.cancer.org/treatment/treatments-and-side-effects/physical-side-effects/infections/infections-in-people-with-cancer.html)
4. [www.cancer.org/cancer/leukemia-in-children/treating/targeted-therapy.html](http://www.cancer.org/cancer/leukemia-in-children/treating/targeted-therapy.html)

5. [www.cancer.org/cancer/leukemia-in-children/detection-diagnosis-staging/how-diagnosed.html](http://www.cancer.org/cancer/leukemia-in-children/detection-diagnosis-staging/how-diagnosed.html)
6. [www.cancer.org/cancer/leukemia-in-children/treating/radiation.html](http://www.cancer.org/cancer/leukemia-in-children/treating/radiation.html)
7. [www.cancer.org/cancer/leukemia-in-children/treating/targeted-therapy.html](http://www.cancer.org/cancer/leukemia-in-children/treating/targeted-therapy.html)
8. [www.cancer.org/cancer/leukemia-in-children/treating/bone-marrow.html](http://www.cancer.org/cancer/leukemia-in-children/treating/bone-marrow.html)
9. [www.cancer.org/cancer/leukemia-in-children/detection-diagnosis-staging/how-diagnosed.html](http://www.cancer.org/cancer/leukemia-in-children/detection-diagnosis-staging/how-diagnosed.html)
10. [www.cancer.org/cancer/leukemia-in-children/treating/chemotherapy.html](http://www.cancer.org/cancer/leukemia-in-children/treating/chemotherapy.html)
11. [www.cancer.org/cancer/leukemia-in-children/treating/bone-marrow.html](http://www.cancer.org/cancer/leukemia-in-children/treating/bone-marrow.html)
12. [/content/cancer/en/cancer/leukemia-in-children/detailed-guide/childhood-leukemia-treating-radiation.html](http://content.cancer/en/cancer/leukemia-in-children/detailed-guide/childhood-leukemia-treating-radiation.html)
13. [www.cancer.org/cancer/leukemia-in-children/treating/immunotherapy.html](http://www.cancer.org/cancer/leukemia-in-children/treating/immunotherapy.html)
14. [www.cancer.org/cancer/leukemia-in-children/treating/bone-marrow.html](http://www.cancer.org/cancer/leukemia-in-children/treating/bone-marrow.html)
15. [www.cancer.org/cancer/leukemia-in-children/treating/targeted-therapy.html](http://www.cancer.org/cancer/leukemia-in-children/treating/targeted-therapy.html)

Last Medical Review: February 12, 2019 Last Revised: February 12, 2019

---

## Treatment of Children With Acute Myeloid Leukemia (AML)

Treatment of most children with acute myeloid leukemia (AML) is divided into 2 main phases of [chemotherapy](#)<sup>1</sup>:

- Induction
- Consolidation (intensification)

Because of the intensity of treatment and the risk of serious complications, children with AML need to be treated in [cancer centers](#)<sup>2</sup> or hospitals that have experience with this disease.

### Induction

The chemo drugs most often used to treat AML are daunorubicin (daunomycin) and cytarabine (ara-C), which are each given for several days in a row. The treatment schedule may be repeated in 10 days or 2 weeks, depending on how intense doctors

want the treatment to be. A shorter time between treatments can be more effective in killing leukemia cells, but it can also cause more severe side effects.

If the doctors think that the leukemia might not respond to just 2 drugs alone, they may add etoposide and/or 6-thioguanine. Children with very high numbers of white blood cells or whose leukemia cells have certain chromosome abnormalities may fall into this group.

Treatment with these drugs is repeated until the bone marrow shows no more leukemia cells. This usually occurs after 2 or 3 cycles of treatment.

**Preventing relapse in the central nervous system:** Most children with AML will also get intrathecal chemotherapy (given directly into the cerebrospinal fluid, or CSF) to help prevent leukemia from relapsing in the brain or spinal cord. [Radiation therapy](#)<sup>3</sup> to the brain is used less often.

### **Consolidation (intensification)**

About 85% to 90% of children with AML go into remission after induction therapy. This means no signs of leukemia are detected using standard lab tests, but it does not necessarily mean that the leukemia has been cured.

Consolidation (intensification) begins after the induction phase. The purpose is to kill any remaining leukemia cells by using more intensive treatment.

Some children have a brother or sister who would be a good stem cell donor. For these children, a [stem cell transplant](#)<sup>4</sup> might be recommended once the leukemia is in remission, especially if the AML has some poorer [prognostic factors](#)<sup>5</sup>. Most studies have found this improves the chance for long-term survival over chemo alone, but it is also more likely to cause serious complications. For children with good prognostic factors, some doctors may recommend just giving intensive [chemotherapy](#)<sup>6</sup>, and reserving the stem cell transplant in case the AML relapses.

For most children without a good stem cell donor, consolidation consists of the chemo drug cytarabine (ara-C) in high doses. Daunorubicin may also be added. It is usually given for at least several months.

Intrathecal chemo (into the CSF) is usually given every 1 to 2 months for as long as intensification continues.

Maintenance chemo is not needed for children with AML (other than [those with APL](#)<sup>7</sup>).

An important part of treatment for AML is supportive care (proper nursing care, nutritional support, antibiotics, and blood transfusions). The intense treatment needed for AML usually destroys much of the bone marrow (causing severe shortages of blood cells) and can cause other serious complications. Without antibiotic treatment of [infections](#)<sup>8</sup> or [transfusion support](#)<sup>9</sup>, the current high remission rates would not be possible.

### Refractory or recurrent AML

Less than 15% of children have **refractory AML** (leukemia that does not respond to initial treatment). These leukemias are often very hard to cure, and doctors may recommend a [stem cell transplant](#)<sup>10</sup> if it can be done.

Generally, the outlook for a child whose AML **relapses** (comes back) after treatment is slightly better than if the AML never went into remission, but this depends on how long the initial remission was. In more than half of cases of relapse, the leukemia can be put into a second remission with more chemo. The chance of getting a second remission is better if the first remission lasted for at least a year, but long-term second remissions are rare without a stem cell transplant. Many different combinations of standard chemo drugs have been used in these situations, but the results have been mixed.

Another option for some children with refractory or recurrent AML is treatment with the [targeted drug](#)<sup>11</sup> gemtuzumab ozogamicin (Mylotarg).

Most children whose leukemia has relapsed are good candidates for [clinical trials](#)<sup>12</sup> testing new treatment regimens. The hope is that some sort of a remission can be attained so that a stem cell transplant can be considered. Some doctors may advise a stem cell transplant even if there is no remission. This can sometimes be successful.

### Hyperlinks

1. [www.cancer.org/cancer/leukemia-in-children/treating/chemotherapy.html](http://www.cancer.org/cancer/leukemia-in-children/treating/chemotherapy.html)
2. [www.cancer.org/treatment/finding-and-paying-for-treatment/choosing-your-treatment-team/pediatric-cancer-centers.html](http://www.cancer.org/treatment/finding-and-paying-for-treatment/choosing-your-treatment-team/pediatric-cancer-centers.html)
3. [www.cancer.org/cancer/leukemia-in-children/treating/radiation.html](http://www.cancer.org/cancer/leukemia-in-children/treating/radiation.html)
4. [www.cancer.org/cancer/leukemia-in-children/treating/bone-marrow.html](http://www.cancer.org/cancer/leukemia-in-children/treating/bone-marrow.html)
5. [www.cancer.org/cancer/leukemia-in-children/detection-diagnosis-staging/prognostic-factors.html](http://www.cancer.org/cancer/leukemia-in-children/detection-diagnosis-staging/prognostic-factors.html)
6. [www.cancer.org/cancer/leukemia-in-children/treating/chemotherapy.html](http://www.cancer.org/cancer/leukemia-in-children/treating/chemotherapy.html)
7. [www.cancer.org/cancer/leukemia-in-children/treating/children-with-apl.html](http://www.cancer.org/cancer/leukemia-in-children/treating/children-with-apl.html)

8. [www.cancer.org/treatment/treatments-and-side-effects/physical-side-effects/infections/infections-in-people-with-cancer.html](http://www.cancer.org/treatment/treatments-and-side-effects/physical-side-effects/infections/infections-in-people-with-cancer.html)
9. [www.cancer.org/treatment/treatments-and-side-effects/treatment-types/blood-transfusion-and-donation.html](http://www.cancer.org/treatment/treatments-and-side-effects/treatment-types/blood-transfusion-and-donation.html)
10. [www.cancer.org/cancer/leukemia-in-children/treating/bone-marrow.html](http://www.cancer.org/cancer/leukemia-in-children/treating/bone-marrow.html)
11. [www.cancer.org/cancer/leukemia-in-children/treating/targeted-therapy.html](http://www.cancer.org/cancer/leukemia-in-children/treating/targeted-therapy.html)
12. [www.cancer.org/treatment/treatments-and-side-effects/clinical-trials.html](http://www.cancer.org/treatment/treatments-and-side-effects/clinical-trials.html)

## References

National Cancer Institute. Childhood Acute Myeloid Leukemia/Other Myeloid Malignancies Treatment (PDQ®)—Health Professional Version. Accessed at <https://www.cancer.gov/types/leukemia/hp/child-aml-treatment-pdq> on December 29, 2018.

Tarlock K, Cooper TM. Acute myeloid leukemia in children and adolescents. UpToDate. 2018. Accessed at [www.uptodate.com/contents/acute-myeloid-leukemia-in-children-and-adolescents](http://www.uptodate.com/contents/acute-myeloid-leukemia-in-children-and-adolescents) on December 29, 2018.

Last Medical Review: February 12, 2019 Last Revised: February 12, 2019

---

# Treatment of Children With Acute Promyelocytic Leukemia (APL)

Treatment of acute promyelocytic leukemia (APL), a subtype of acute myeloid leukemia (AML), differs from the usual AML treatment. This leukemia usually responds well to treatment, which is given in 3 phases:

- Induction
- Consolidation (also called intensification)
- Maintenance

## Induction

Many children with APL have bleeding and blood-clotting issues when APL is diagnosed, which can cause serious problems during early treatment. Because of this, children with APL must be treated carefully and are often given an anticoagulant (“blood thinner”) to help prevent or treat these problems.

Children with APL get a [non-chemotherapy drug](#)<sup>1</sup> similar to vitamin A called **all-trans retinoic acid (ATRA)**. ATRA alone can often put APL into remission, but combining it with [chemotherapy](#)<sup>2</sup> (usually daunorubicin and cytarabine) gives better long-term results. APL rarely spreads to the brain or spinal cord, so intrathecal chemotherapy is usually not needed.

In adults, ATRA is often combined with **arsenic trioxide (ATO)**, another [non-chemo drug](#)<sup>3</sup>, instead of chemo, as the initial treatment of APL. The results seem to be at least as good, and without some of the side effects of chemo. The combination of ATRA and ATO is now being studied in children as well.

## Consolidation (intensification)

This is usually similar to induction, using both ATRA and chemotherapy (daunorubicin, sometimes along with cytarabine). Because of the success of this treatment, a [stem cell transplant](#)<sup>4</sup> is not usually advised as long as the leukemia stays in remission.

ATRA plus ATO is also being studied as an option for consolidation therapy.

## Maintenance

Children with APL may get maintenance therapy with ATRA (often with the chemo drugs methotrexate and 6-mercaptopurine) for about a year.

## Relapsed APL

If the leukemia comes back after treatment, most often it can be put into a second remission. Arsenic trioxide is a drug that is very effective in this setting. ATRA plus chemo may be another option. A [stem cell transplant](#)<sup>5</sup> may be considered once a second remission is achieved.

## Hyperlinks

1. [www.cancer.org/cancer/leukemia-in-children/treating/targeted-therapy.html](http://www.cancer.org/cancer/leukemia-in-children/treating/targeted-therapy.html)

2. [www.cancer.org/cancer/leukemia-in-children/treating/chemotherapy.html](http://www.cancer.org/cancer/leukemia-in-children/treating/chemotherapy.html)
3. [www.cancer.org/cancer/leukemia-in-children/treating/targeted-therapy.html](http://www.cancer.org/cancer/leukemia-in-children/treating/targeted-therapy.html)
4. [www.cancer.org/cancer/leukemia-in-children/treating/bone-marrow.html](http://www.cancer.org/cancer/leukemia-in-children/treating/bone-marrow.html)
5. [www.cancer.org/cancer/leukemia-in-children/treating/bone-marrow.html](http://www.cancer.org/cancer/leukemia-in-children/treating/bone-marrow.html)

## References

National Cancer Institute. Childhood Acute Myeloid Leukemia/Other Myeloid Malignancies Treatment (PDQ®)—Health Professional Version. Accessed at <https://www.cancer.gov/types/leukemia/hp/child-aml-treatment-pdq> on December 29, 2018.

Tarlock K, Cooper TM. Acute myeloid leukemia in children and adolescents. UpToDate. 2018. Accessed at [www.uptodate.com/contents/acute-myeloid-leukemia-in-children-and-adolescents](http://www.uptodate.com/contents/acute-myeloid-leukemia-in-children-and-adolescents) on December 29, 2018.

Last Medical Review: February 12, 2019 Last Revised: February 12, 2019

---

# Treatment of Children With Juvenile Myelomonocytic Leukemia (JMML)

Juvenile myelomonocytic leukemia (JMML) is fairly rare, so it has been hard to study which treatment might be best. There is no clear single best [chemotherapy](#)<sup>1</sup> treatment for this leukemia. A [stem cell transplant](#)<sup>2</sup> is the treatment of choice when possible, as it offers the best chance to cure JMML. About half of the children with JMML who get a stem cell transplant are still free of leukemia after several years. Sometimes, even if the leukemia recurs, a second stem cell transplant can be helpful.

Because JMML is hard to treat with current chemo drugs, taking part in a [clinical trial](#)<sup>3</sup> looking at newer drugs may be a good option for children who can't get a stem cell transplant.

## Hyperlinks

1. [www.cancer.org/cancer/leukemia-in-children/treating/chemotherapy.html](http://www.cancer.org/cancer/leukemia-in-children/treating/chemotherapy.html)
2. [www.cancer.org/cancer/leukemia-in-children/treating/bone-marrow.html](http://www.cancer.org/cancer/leukemia-in-children/treating/bone-marrow.html)
3. [www.cancer.org/treatment/treatments-and-side-effects/clinical-trials.html](http://www.cancer.org/treatment/treatments-and-side-effects/clinical-trials.html)

## References

Caywood EH, Kolb EA. Juvenile myelomonocytic leukemia. UpToDate. 2018. Accessed at [www.uptodate.com/contents/juvenile-myelomonocytic-leukemia](http://www.uptodate.com/contents/juvenile-myelomonocytic-leukemia) on November 29, 2018.

National Cancer Institute. Childhood Acute Myeloid Leukemia/Other Myeloid Malignancies Treatment (PDQ®)—Health Professional Version. Accessed at <https://www.cancer.gov/types/leukemia/hp/child-aml-treatment-pdq> on December 29, 2018.

Last Medical Review: February 12, 2019 Last Revised: February 12, 2019

---

# Treatment of Children With Chronic Myeloid Leukemia (CML)

Chronic myeloid (myelogenous) leukemia (CML) is rare in children, but it does occur. Treatment in children is similar to what is used for adults.

[Targeted drugs](#)<sup>1</sup>, such as imatinib (Gleevec), dasatinib (Sprycel), and nilotinib (Tasigna), attack cells with the Philadelphia chromosome, which is the key gene abnormality in CML cells. These drugs are usually very good at controlling CML, often for long periods of time and with less severe side effects than chemotherapy drugs. However, it's not yet clear if these drugs can cure CML when used alone, and they must be taken every day.

Imatinib is usually the drug tried first. If it doesn't work or if it becomes less effective over time, another drug may be tried.

If targeted drugs are no longer helpful, high-dose chemotherapy with a [stem cell transplant](#)<sup>2</sup> offers the best chance for a cure. Doctors are now studying whether adding

targeted drugs to stem cell transplant regimens can help increase cure rates.

For more information on CML and its treatment, see [Chronic Myeloid Leukemia](#).<sup>3</sup>

## Hyperlinks

1. [www.cancer.org/cancer/leukemia-in-children/treating/targeted-therapy.html](http://www.cancer.org/cancer/leukemia-in-children/treating/targeted-therapy.html)
2. [www.cancer.org/cancer/leukemia-in-children/treating/bone-marrow.html](http://www.cancer.org/cancer/leukemia-in-children/treating/bone-marrow.html)
3. [www.cancer.org/cancer/chronic-myeloid-leukemia.html](http://www.cancer.org/cancer/chronic-myeloid-leukemia.html)

## References

National Cancer Institute. Childhood Acute Myeloid Leukemia/Other Myeloid Malignancies Treatment (PDQ®)—Health Professional Version. Accessed at <https://www.cancer.gov/types/leukemia/hp/child-aml-treatment-pdq> on December 29, 2018.

Last Medical Review: February 12, 2019 Last Revised: February 12, 2019

## Written by

The American Cancer Society medical and editorial content team  
([www.cancer.org/cancer/acs-medical-content-and-news-staff.html](http://www.cancer.org/cancer/acs-medical-content-and-news-staff.html))

Our team is made up of doctors and oncology certified nurses with deep knowledge of cancer care as well as journalists, editors, and translators with extensive experience in medical writing.

American Cancer Society medical information is copyrighted material. For reprint requests, please see our Content Usage Policy ([www.cancer.org/about-us/policies/content-usage.html](http://www.cancer.org/about-us/policies/content-usage.html)).

**cancer.org | 1.800.227.2345**