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Treating Chronic Myeloid Leukemia

If you've been diagnosed with chronic myeloid leukemia (CML), your treatment team will discuss your options with you. It's important to weigh the benefits of each treatment option against the possible risks and side effects.

How is CML treated?

Targeted therapy drugs are the main treatment for chronic myeloid leukemia (CML), but some patients might also need other treatments.

- [Targeted Therapies for Chronic Myeloid Leukemia](#)
- [Interferon Therapy for Chronic Myeloid Leukemia](#)
- [Chemotherapy for Chronic Myeloid Leukemia](#)
- [Radiation Therapy for Chronic Myeloid Leukemia](#)
- [Surgery for Chronic Myeloid Leukemia](#)
- [Stem Cell Transplant for Chronic Myeloid Leukemia](#)

Common treatment approaches

Treatment options for people with chronic myeloid leukemia (CML) depend on the [phase](#) of their disease (chronic, accelerated, or blast phase), their age, other prognostic factors, and the availability of a stem cell donor with matching tissue type.

- [Treating Chronic Myeloid Leukemia by Phase](#)
- [How Do You Know If Treatment for Chronic Myeloid Leukemia Is Working?](#)

Who treats CML?

Based on your treatment options, you might have different types of doctors on your

treatment team. These doctors could include:

- A **hematologist**: a doctor who treats blood disorders.
- A **radiation oncologist**: a doctor who treats cancer with radiation therapy
- A **medical oncologist**: a doctor who treats cancer with medicines such as chemotherapy
- A **surgical oncologist (oncologic surgeon)**: a doctor who uses surgery to treat cancer

You might have many other specialists on your treatment team as well, including physician assistants, nurse practitioners, nurses, nutrition specialists, social workers, and other health professionals.

- [Health Professionals Associated with Cancer Care](#)

Making treatment decisions

It's important to discuss all treatment options, including their goals and possible side effects, with your doctors to help make the decision that best fits your needs. You may feel that you need to make a decision quickly, but it's important to give yourself time to absorb the information you have learned. Ask your cancer care team questions.

If time permits, it is often a good idea to seek a second opinion. A second opinion can give you more information and help you feel more confident about the treatment plan you choose.

- [Questions To Ask About Chronic Myeloid Leukemia](#)
- [Seeking a Second Opinion](#)

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're 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.

- [Clinical Trials](#)

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 harmful.

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.

- [Complementary and Alternative Medicine](#)

Help getting through cancer treatment

People with cancer need support and information, no matter what stage of illness they may be in. Knowing all of your options and finding the resources you need will help you make informed decisions about your care.

Whether you are thinking about treatment, getting treatment, or not being treated at all, you can still get supportive care to help with pain or other symptoms. Communicating with your cancer care team is important so you understand your diagnosis, what treatment is recommended, and ways to maintain or improve your quality of life.

Different types of programs and support services may be helpful, and can be 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, 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.

- [Palliative Care](#)

- [Find Support Programs and Services in Your Area](#)

Choosing to stop treatment or choosing no treatment at all

For some people, when treatments have been tried and are no longer controlling the cancer, it could be time to weigh the benefits and risks of continuing to try new treatments. Whether or not you continue treatment, there are still things you can do to help maintain or improve your quality of life.

Some people, especially if the cancer is advanced, might not want to be treated at all. There are many reasons you might decide not to get cancer treatment, but it's important to talk to your doctors and you make that decision. Remember that even if you choose not to treat the cancer, you can still get supportive care to help with pain or other symptoms.

- [If Cancer Treatments Stop Working](#)

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.

Targeted Therapies for Chronic Myeloid Leukemia

Chronic myeloid leukemia (CML) cells contain an abnormal gene, *BCR-ABL*, that isn't found in normal cells. This gene makes a protein, BCR-ABL, which causes CML cells to grow and reproduce out of control. BCR-ABL is a type of protein known as a tyrosine kinase. Drugs known as **tyrosine kinase inhibitors** (TKIs) that target BCR-ABL are the standard treatment for CML. These include:

- Imatinib (Gleevec)
- Dasatinib (Sprycel)

- Nilotinib (Tasigna)
- Bosutinib (Bosulif)
- Ponatinib (Iclusig)

These drugs seem to work best when CML is in the [chronic phase](#)¹, but they also can help patients with more advanced disease (accelerated or blast phases). In most people, the TKIs don't seem to make the leukemia go away forever, so these drugs need to be taken indefinitely. But for some people who have very good, long-lasting responses to treatment, it might be possible to stop taking these drugs, or at least lower the dose. (See [Treating Chronic Myeloid Leukemia by Phase](#) to learn more.)

These drugs are pills you take at home. To get the best outcomes, it's important to take them exactly the way your doctor tells you to take them. Skipping pills or adjusting doses can effect the way TKI treatment works. See [Oral Chemotherapy: What You Need to Know](#)² for more on how to best manage cancer treatments you take at home.

All of these drugs can have serious interactions with some other drugs, over-the-counter supplements, and even certain foods (such as grapefruit and pomegranates). Be sure that your doctor always has an up-to-date list of everything you're taking, including over-the-counter drugs, vitamins, and herbal supplements. You also need to check with your doctor before starting any new medicine, to be sure it's safe.

It's also important to understand that all of the TKIs can harm a fetus if taken during pregnancy.

TKIs used to treat chronic phase CML

These TKIs are available as of 2018. Any of them might be used as the first (or frontline) treatment of chronic phase CML.

Imatinib

Imatinib (Gleevec) was the first drug to specifically target the BCR-ABL tyrosine kinase protein, because of this it's known as a **first-generation tyrosine kinase inhibitor**.

Almost all CML patients respond to treatment with imatinib, and most of these responses seem to last for many years.

Imatinib is taken by mouth as a pill with food, usually once a day.

Generic imatinib is also available. Studies have shown that it works as well as and causes the same kinds of side effects as the brand name, Gleevec.

Side effects of imatinib

Common side effects can include diarrhea, nausea, muscle pain, and fatigue. These are generally mild. Some people have itchy skin rashes. Most of these symptoms can be treated, if needed.

Another common side effect is fluid build-up around the eyes, feet, or abdomen (belly). In rare cases the fluid may collect in the lungs or around the heart, which can cause trouble breathing. Some studies have suggested that some of this fluid build-up may be caused by effects of the drug on the heart, though this is rare. It's not yet clear how serious this is or if it might go away if treatment is stopped. If you are taking this drug, tell your doctor right away if you notice sudden weight gain or fluid build-up anywhere in the body or have trouble breathing.

A person's white blood cell and platelet counts could possibly drop. When this happens at the beginning of treatment, it might be because the blood-forming cells that are making these are part of the leukemia process. If this is the case, normal blood-forming cells take over and the blood counts will begin to rise over time.

Your doctor might tell you to stop taking the drug for a short period if your blood counts get too low. This can also happen later on in treatment. Your doctor may lower the dose of imatinib to see if your blood counts improve.

In some patients, imatinib seems to stop working over time. This is known as **imatinib resistance**. Resistance to imatinib seems to be caused by changes in the genes of the CML cells. Sometimes this resistance can be overcome by increasing the dose of imatinib, but some patients need to change to a different drug, such as one of the other TKIs.

Dasatinib

Dasatinib (Sprycel) is another TKI that targets the BCR-ABL protein. Because it was developed after imatinib, it's called a **second-generation** TKI.

This drug is a pill taken once a day with or without food.

Dasatinib can be used as the first treatment for CML, but it can also be helpful for patients who can't take imatinib because of side effects or because imatinib isn't

working.

Side effects of dasatinib

The possible side effects of dasatinib seem to be similar to those of imatinib, including fluid build-up, lowered blood cell counts, nausea, diarrhea, and skin rashes.

A serious side effect that can occur with this drug is fluid build-up around the lung (called a **pleural effusion**). This side effect is more common in patients taking dasatinib twice a day. The fluid can be drained off with a needle, but it can build up again, and the dose of dasatinib may need to be decreased.

Nilotinib

Nilotinib (Tasigna) is another second-generation TKI that targets the BCR-ABL protein. This drug can be used as a first treatment for CML, and is also used for people who can't take imatinib or whose CML no longer responds to it.

It's taken as a pill. The patient cannot eat 2 hours before taking nilotinib and for 1 hour after taking it.

Side effects of nilotinib

Side effects of nilotinib seem to be mild, but can include fluid build-up, lowered blood cell counts, nausea, diarrhea, rash, and some blood chemical changes that may need to be treated (for instance, low potassium and magnesium levels). It can cause high blood sugar and pancreatitis, but this is rare.

This drug can also affect the rhythm of the heart, causing a condition called **prolonged QT syndrome**. This usually doesn't cause any symptoms, but can be serious or even fatal. Because of this, patients should have an electrocardiogram (EKG) before starting nilotinib and then again while being treated. This heart rhythm problem can sometimes be caused by nilotinib interacting with other drugs or supplements, so it's especially important to be sure that your cancer doctor knows about any medicines you take, including over-the-counter medicines, vitamins, herbs, and supplements. You also need to check with your doctor before starting any new medicine, to be sure it's safe.

TKIs used when firstline TKIs stop working

Bosutinib

Bosutinib (Bosulif) is another TKI that targets the BCR-ABL protein. It can be used as the first treatment for CML, but most often it's used if another TKI is no longer working.

This drug is taken as a pill with food once a day.

Side effects of bosutinib

Common side effects are usually mild and include diarrhea, nausea, vomiting, abdominal (belly) pain, rash, fever, fatigue, and low blood cell counts (including low platelet counts, low red blood cell counts, and low white blood cell counts). Less often, this drug can also cause problems with fluid retention, liver damage, and severe allergic reaction. Your doctor will check your blood test results regularly to watch for problems with your liver and low blood counts.

Ponatinib

Ponatinib (Iclusig) is a newer, third-generation TKI targeting the BCR-ABL protein. Because this drug can cause some serious side effects, it's only used to treat patients with CML if all of the other TKIs don't work or if their leukemia cells have a gene change called the *T315I mutation*. Ponatinib is the first TKI to work against CML cells that have this mutation.

This drug is a pill taken once a day with or without food.

Side effects of ponatinib

Most side effects are mild and can include abdominal (belly) pain, headache, rash or other skin problems, and fatigue. High blood pressure is also fairly common, and it may need to be treated with a blood pressure drug.

There's also a risk of serious **blood clots** that can lead to heart attacks and strokes, or block arteries and veins in the arms and legs. Rarely, blood clots in patients taking this drug have cut off circulation, and lead to an arm or leg needing to be amputated (cut off). Surgery or another procedure may be needed to treat these blood clots. The risk of serious blood clots is higher in older patients; those with certain risk factors, such as high blood pressure, high cholesterol, or diabetes; and those who have already had a heart attack, stroke, or poor circulation.

Less often, this drug can also weaken the heart muscle, leading to a condition known as congestive heart failure (CHF). It can also cause liver problems, including liver failure, as well as pancreatitis (inflammation of the pancreas, which can lead to severe belly

pain, nausea, and vomiting).

More information about targeted therapy

To learn more about how targeted drugs are used to treat cancer, see [Targeted Cancer Therapy](#)³.

To learn about some of the side effects listed here and how to manage them, see [Managing Cancer-related Side Effects](#)⁴.

Hyperlinks

1. www.cancer.org/cancer/chronic-myeloid-leukemia/detection-diagnosis-staging/staging.html
2. www.cancer.org/treatment/treatments-and-side-effects/treatment-types/chemotherapy/oral-chemotherapy.html
3. www.cancer.org/treatment/treatments-and-side-effects/treatment-types/targeted-therapy.html
4. www.cancer.org/treatment/treatments-and-side-effects/physical-side-effects.html

References

See all references for Chronic Myeloid Leukemia (www.cancer.org/cancer/chronic-myeloid-leukemia/references.html)

Bristol-Myers Squibb. Highlights of prescribing information: Sprycel (dasatinib). 11/2017. Accessed at https://packageinserts.bms.com/pi/pi_sprycel.pdf on May 16, 2018.

Jabbour E, Kantarjian H. Chronic myeloid leukemia: 2018 update on diagnosis, therapy and monitoring. *Am J Hematol*. 2018 Mar;93(3):442-459.

National Comprehensive Cancer Network, Clinical Practice Guidelines in Oncology (NCCN Guidelines®), Chronic Myeloid Leukemia, Version 4.2018 -- January 24, 2018. Accessed at https://www.nccn.org/professionals/physician_gls/pdf/cml.pdf on May 16, 2018.

Novartis Pharmaceuticals Corporation. Highlights of prescribing information: Gleevec (imatinib mesylate). 9/2017. Accessed at www.pharma.us.novartis.com/sites/www.pharma.us.novartis.com/files/gleevec_tabs.pdf

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Pfizer Laboratories. Highlights of prescribing information: Bosulif (bosutinib). 12/2018. Accessed at labeling.pfizer.com/ShowLabeling.aspx?id=884 on May 16, 2018.

Takeda Pharmaceuticals Company Limited. Highlights of prescribing information: Iclusig (ponatinib). 12/2017. Accessed at iclusig.com/pi on May 16, 2018.

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Interferon Therapy for Chronic Myeloid Leukemia

Interferons are a family of substances naturally made by our immune system (CML). Interferon-alpha is a man-made drug that acts like the substance our immune system naturally produces. It reduces the growth and division of leukemia cells. Interferon-alpha is the type most often used in treating chronic myeloid leukemia (CML).

Interferon was once the best treatment for CML, but now, the [tyrosine kinase inhibitors](#) are the mainstay of treatment and interferon is rarely used.

To treat CML, this drug is most often given as a daily injection (shot) under the skin. It may also be injected into a muscle or vein. It's given for many years.

Side effects of interferon

Interferon can cause significant side effects. These include "flu-like" symptoms like muscle aches, bone pain, fever, headaches, fatigue, nausea, and vomiting. Patients taking this drug may have problems thinking and concentrating. Interferon can also lower blood cell counts.

Side effects continue as long as the drug is used, but can become easier to tolerate over time. They get better after the drug is stopped. Still, some patients find it hard to deal with these side effects every day and may need to stop treatment because of them.

More information about immunotherapy

To learn more about how drugs that work on the immune system are used to treat cancer, see [Cancer Immunotherapy](#)¹.

To learn about some of the side effects listed here and how to manage them, see [Managing Cancer-related Side Effects](#)².

Hyperlinks

1. www.cancer.org/treatment/treatments-and-side-effects/treatment-types/immunotherapy.html
2. www.cancer.org/treatment/treatments-and-side-effects/physical-side-effects.html

References

See all references for Chronic Myeloid Leukemia (www.cancer.org/cancer/chronic-myeloid-leukemia/references.html)

American Society of Clinical Oncology. Leukemia - Chronic Myeloid - CML: Treatment Options. 11/2016. Accessed at www.cancer.net/cancer-types/leukemia-chronic-myeloid-cml/treatment-options on May 16, 2018.

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Chemotherapy for Chronic Myeloid Leukemia

Chemotherapy (chemo) is the use of anti-cancer drugs that are injected into a vein or taken by mouth. These drugs enter the bloodstream and reach all areas of the body, making this type of treatment useful for cancers that spread throughout the body, like chronic myeloid leukemia (CML). Any drug used to treat cancer (including [tyrosine kinase inhibitors](#) or TKIs) can be considered chemo, but here chemo is used to mean treatment with conventional cytotoxic (cell-killing) drugs that mainly kill cells that are

growing and dividing rapidly.

Chemo was once one of the main treatments for CML. It's seldom used now because TKIs like imatinib (Gleevec[®]) work much better. Today, chemo may be used to treat CML when the TKIs have stopped working. It's also used as part of a stem cell transplant.

Chemo drugs used to treat CML

The chemo drug hydroxyurea (Hydrea[®]) is taken as a pill, and can help quickly lower very high white blood cell counts and shrink an enlarged spleen. Other drugs sometimes used include cytarabine (Ara-C), busulfan, cyclophosphamide (Cytosan[®]), and vincristine (Oncovin[®]).

Omacetaxine (Synribo[®]) is a chemo drug that was approved to treat CML that's resistant to TKIs and progresses to the [accelerated phase](#)¹. It can also help some patients whose CML has developed the T315I mutation that keeps most TKIs from working (discussed in the section about [targeted therapy](#)).

Side effects of chemotherapy

Chemo drugs work by attacking cells that divide quickly, which is why they work against cancer cells. But other cells in the body, such as those in the bone marrow, 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.

Possible side effects depend on the type and dose of drugs given and how long they are taken. Some common side effects of chemo include:

- Hair loss
- Mouth sores
- Loss of appetite
- Nausea and vomiting
- Low white blood cell counts (leukopenia), which increases the risk of serious infection
- Low blood platelet counts (thrombocytopenia), which can lead to easy bruising or bleeding
- Low red blood cell counts (anemia), which can lead to feeling tired and weak

Still, different drugs can have different side effects. For example, vincristine can cause nerve damage (neuropathy) leading to numbness, tingling, or even pain or weakness in the hands or feet. Lung damage from busulfan is rare, but can be severe. Before starting treatment, speak with your health care team about the drugs you'll get and their possible side effects. Most side effects last a short time and go away once treatment is over, but some can be permanent.

While getting treatment, be sure to tell your cancer care team about any side effects you have. There may be ways to treat them or keep them from getting worse. For instance, there are drugs that work well to prevent or reduce nausea and vomiting.

For information on infections and how to avoid them, see [Infections in People With Cancer](#)².

If your platelet counts are very low, you may be given platelet transfusions to help protect against bleeding. Likewise, if low red blood cell counts are causing problems (like shortness of breath and/or weakness), you may be treated with [red blood cell transfusions](#)³.

More information about chemotherapy

For more general information about how chemotherapy is used to treat cancer, see [Chemotherapy](#)⁴.

To learn about some of the side effects listed here and how to manage them, see [Managing Cancer-related Side Effects](#)⁵.

Hyperlinks

1. www.cancer.org/cancer/chronic-myeloid-leukemia/detection-diagnosis-staging/staging.html
2. www.cancer.org/treatment/treatments-and-side-effects/physical-side-effects/low-blood-counts/infections.html
3. www.cancer.org/treatment/treatments-and-side-effects/treatment-types/blood-transfusion-and-donation.html
4. www.cancer.org/treatment/treatments-and-side-effects/treatment-types/chemotherapy.html
5. www.cancer.org/treatment/treatments-and-side-effects/physical-side-effects.html

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Teva Pharmaceuticals, Highlights of prescribing information: SYNRIBO (omacetaxine mepesuccinate). 06/2017. Accessed at www.accessdata.fda.gov/drugsatfda_docs/label/2017/203585s005lbl.pdf on May 16, 2018.

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Radiation Therapy for Chronic Myeloid Leukemia

Radiation therapy is treatment with high-energy rays or particles to destroy cancer cells.

Radiation is seldom part of the treatment for patients with chronic myeloid leukemia (CML), but it might be used in certain situations.

Some people have symptoms if swollen internal organs (such as an enlarged spleen) press on other organs. For instance, pressure against the stomach may affect appetite. If these symptoms are not helped by other treatments, radiation to shrink the spleen may be an option.

Radiation can also be useful in treating pain from bone damage caused by the growth of leukemia cells within the bone marrow.

Radiation therapy may be given in low doses to the whole body, as part of a [stem cell transplant](#).

Side effects of radiation therapy

The main short-term sideeffects of radiation therapy depend on what part of the body is treated.

- Fatigue (tiredness) is a common side effect (no matter what part of the body is treated).
- Skin changes can occur in the treated area which range from mild redness to blistering and peeling.
- If radiation is aimed at the head or neck, the inside lining of your mouth and throat may become red and irritated.
- Radiation to the belly or pelvis can cause nausea and vomiting and/or diarrhea.

More information about radiation therapy

To learn more about how radiation is used to treat cancer, see [Radiation Therapy](#)¹.

To learn about some of the side effects listed here and how to manage them, see [Managing Cancer-related Side Effects](#)².

Hyperlinks

1. www.cancer.org/treatment/treatments-and-side-effects/treatment-types/radiation.html
2. www.cancer.org/treatment/treatments-and-side-effects/physical-side-effects.html

References

See all references for Chronic Myeloid Leukemia (www.cancer.org/cancer/chronic-myeloid-leukemia/references.html)

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Surgery for Chronic Myeloid Leukemia

Leukemia cells spread widely throughout the bone marrow and other organs, so surgery cannot be used to cure this type of cancer. Surgery rarely has any role even in diagnosing chronic myeloid leukemia (CML), since a blood test or [bone marrow aspirate and biopsy](#)¹ are usually all that's needed.

Splenectomy

If leukemia spreads to the spleen, it can become large enough to compress nearby organs and cause symptoms. If [chemotherapy](#) or [radiation](#) doesn't help shrink the spleen, it may be removed with surgery. This operation, called a splenectomy, is meant to improve the symptoms of an enlarged spleen — it has no role in curing CML.

Splenectomy may also improve blood cell counts and lower the need for [blood product transfusions](#)². One of the spleen's normal functions is to remove worn-out blood cells from the bloodstream. If leukemia or other diseases cause the spleen to become too large, it may become too active in removing blood cells, leading to a shortage of red blood cells or platelets. Taking out the spleen may improve red blood cell and platelet counts in some patients.

Most people have no problem living without a spleen, but the risk for certain bacterial infections is increased. This is why doctors often recommend certain vaccines be given before the spleen is removed.

Hyperlinks

1. www.cancer.org/cancer/chronic-myeloid-leukemia/detection-diagnosis-staging/how-diagnosed.html
2. www.cancer.org/treatment/treatments-and-side-effects/treatment-types/blood-transfusion-and-donation.html

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Stem Cell Transplant for Chronic Myeloid Leukemia

Stem cell transplant is not a common treatment for chronic myeloid leukemia (CML) today. In the past, before tyrosine kinase inhibitors (TKIs) were available, SCT was often used to treat CML. Now, TKIs are the standard treatment, and transplants are being used far less often.

Because allogeneic SCT offers the only proven chance to cure CML, doctors may still recommend a transplant for younger patients, particularly children. Transplant is more likely to be considered for those with an available matched donor, like a well-matched brother or sister.

Transplant may also be recommended if CML is not responding well to TKIs. It's also an important option for people with CML that's advancing to or diagnosed in the accelerated or blast phases.

What is a stem cell transplant?

The usual doses of chemotherapy drugs can cause serious side effects by damaging quickly dividing cells such as the bone marrow. Even though higher doses of these drugs might be better at killing leukemia cells, they're not given because the severe damage to bone marrow cells would cause lethal shortages of blood cells.

For a stem cell transplant (SCT), high doses of chemo are given to kill the leukemia cells. Sometimes the whole body also is given a low dose of radiation. This treatment kills the leukemia cells, but also damages the normal bone marrow cells. Then after these treatments, the patient receives a transplant of blood-forming stem cells to restore

the bone marrow.

Blood-forming stem cells used for a transplant can come from either from blood (called a peripheral blood stem cell transplant, or PBSCT) or from the bone marrow (called a bone marrow transplant, or BMT). Bone marrow transplant was done more commonly in the past, but it has largely been replaced by PBSCT.

The 2 main types of stem cell transplants are allogeneic and autologous.

For an **autologous transplant**, the patient's own stem cells are collected from the blood or bone marrow and then given back after treatment. The problem is that leukemia cells may be collected with the stem cells.

In an **allogeneic transplant**, the stem cells come from someone else (a donor). To lower the chance of complications, the donor needs to "match" the patient's tissue type. Often, a close relative, like a brother or sister is a good match. Less often, a matched unrelated donor may be found.

Because collecting the patient's stem cells can also collect leukemia cells, allogeneic transplants are the main type of transplant used to treat CML. Allogeneic stem cell transplant is the only known cure for CML. Still, this type of transplant can cause severe or even life-threatening complications and side effects, and it's often not be a good option in people who are older or have other health problems.

For more details on transplants, see [Stem Cell Transplant \(Peripheral Blood, Bone Marrow, and Cord Blood Transplants\)](#)¹.

Hyperlinks

1. www.cancer.org/treatment/treatments-and-side-effects/treatment-types/stem-cell-transplant.html

References

See all references for Chronic Myeloid Leukemia (www.cancer.org/cancer/chronic-myeloid-leukemia/references.html)

Jabbour E, Kantarjian H. Chronic myeloid leukemia: 2018 update on diagnosis, therapy and monitoring. *Am J Hematol*. 2018;93(3):442-459.

National Comprehensive Cancer Network, Clinical Practice Guidelines in Oncology (NCCN Guidelines®), Chronic Myeloid Leukemia, Version 4.2018 -- January 24, 2018. Accessed at www.nccn.org/professionals/physician_gls/pdf/cml.pdf on May 16, 2018.

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How Do You Know If Treatment for Chronic Myeloid Leukemia Is Working?

If you have chronic myeloid leukemia (CML) and are being treated with targeted drugs, your doctor will check your blood counts, examine you, and do other tests like bone marrow biopsy and PCR (of blood and/or bone marrow). These check-ups will be at least every 3 months for at least the first year of treatment. They're done to see how well the CML is responding to the treatment you're getting. If you are taking your medicine correctly and the CML is not responding, you may be switched to another drug.

Studies have suggested that a fast response (within 3 to 6 months) is linked to better outcomes.

These are the ways doctors look for different kinds of responses to treatment:

Hematologic response

Hematologic response is based on the number of cells in your blood. The test used to measure this is a CBC or complete blood count. It's done on a sample of blood taken from your arm.

- **Complete hematologic response:** Also called CHR, is when all your blood cell counts have returned to normal, there are no immature cells seen in your blood, and your spleen is back to a normal size. You have no symptoms of CML.
- **Partial hematologic response:** A partial hematologic response means that your blood counts are better, but there are still signs or symptoms of CML. It means your

white blood cell count is less than half of what it was before treatment, your platelet count is still high, and/or your spleen has shrunk, but is still enlarged.

Cytogenetic response

This test is done on a sample of your bone marrow. It's done with either cytogenetics or FISH testing. These tests find altered (mutated) chromosomes. (They are discussed in [Tests for Chronic Myeloid Leukemia](#).¹)

- A **complete cytogenetic response** (CCyR) is when no cells with the Philadelphia chromosome can be found in your bone marrow.
- A **partial cytogenetic response** (PCyR) is when 1% to 34% of the cells still have the Philadelphia chromosome.
- A **major cytogenetic response** (MCyR) means less than 35% of your cells have the Philadelphia chromosome. It includes both a complete and partial response.
- A **minor cytogenetic response** occurs when more than 35% of your cells still have the Philadelphia chromosome.

Molecular response

Molecular response uses the PCR test. It can be done on either your blood or bone marrow. It's based on the number of leukemia cells in your blood.

- A **complete molecular response** (CMR) means that the PCR test does not find the *BCR-ABL* gene in your blood.
- A **major molecular response** (MMR) means that the amount of *BCR-ABL* gene in your blood is 1/1000th (or less) of what's expected in someone with untreated CML.
- An **early molecular response** (EMR) means that there is 10% or less *BCR-ABL* gene in your blood after 3 months and 6 months of treatment.

You may hear the terms **long-term deep molecular response** or a **durable complete molecular response**. This is a long-lasting complete molecular response. It's the goal of CML treatment.

Hyperlinks

1. www.cancer.org/cancer/chronic-myeloid-leukemia/detection-diagnosis-staging/how-diagnosed.html

References

See all references for Chronic Myeloid Leukemia (www.cancer.org/cancer/chronic-myeloid-leukemia/references.html)

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Treating Chronic Myeloid Leukemia by Phase

Treatment options for people with chronic myeloid leukemia (CML) depend on the [phase](#)¹ of their disease (chronic, accelerated, or blast phase), their age, other prognostic factors, and the availability of a stem cell donor with matching tissue type.

Chronic phase

The standard treatment for chronic phase CML is a [tyrosine kinase inhibitor](#) (TKI) like imatinib (Gleevec), nilotinib (Tasigna), dasatinib (Sprycel), or bosutinib (Bosulif). If the first drug stops working or it never really worked well at all, the dose may be increased or another TKI might be tried. Ponatinib (Iclusig) is an option after all of the other TKIs have been tried or if the leukemia cells later develop the T315I mutation.

Switching to another TKI is also an option if a person can't take the first drug because of side effects.

Rarely, people in chronic phase may be treated with an allogeneic stem cell transplant (SCT). This treatment is discussed in detail in [Stem Cell Transplant for Chronic Myeloid Leukemia](#).

Monitoring treatment results

Monitoring the patient to see how they respond to treatment is very important. Blood counts are checked often. The blood is also checked with a polymerase chain reaction (PCR) test to measure the amount of the *BCR-ABL* gene. The bone marrow is checked, too, to see if the Philadelphia chromosome is there. Testing for the *BCR-ABL* gene or the Philadelphia chromosome is usually done about 3 months after a TKI is started, and then every 3 to 6 months after that. If the results show that treatment is working well, the patient stays on their current drug. If the results show that treatment isn't working well, and the patient is taking the drug the way they should, a new drug or treatment may be needed.

If the CML is responding well to treatment, 3 months after starting treatment, the patient should have:

- A complete hematologic response (CHR), and
- Some type of cytogenetic response, and/or
- A reduction of the number of copies of *BCR-ABL* on the PCR test by 90% or more

If treatment is working well, 18 months after starting treatment, the patient should have:

- A complete hematologic response (CHR), and
- A complete cytogenetic response (CCyR), and/or
- A major molecular response (MMR)

For more on these different types of response, see [How Do You Know If Treatment for Chronic Myeloid Leukemia Is Working?](#)

How often is treatment successful?

Up to about 70% of people have a complete cytogenetic response (CCyR) within 1 year of starting imatinib, and the rate of CCyR is even higher with other TKIs. After a year, even more patients will have had a CCyR. Many of these patients also have a complete molecular response (CMR).

But even in patients in whom the *BCR-ABL* gene can no longer be found while on treatment, it's often not clear if they are cured, so most people need to stay on a TKI indefinitely. In patients who have a deep, long-lasting response to treatment (usually for at least 2 or 3 years), some doctors might suggest stopping the drug for a time and closely monitoring with blood tests to see if the CML returns. In clinical trials so far, typically about half of these patients can stop treatment without the CML returning. Another option might be lowering the dose of the TKI, which can reduce side effects.

If the CML does return after stopping or lowering the dose of the TKI, it's been found to respond well when the original treatment is restarted.

If the first treatment doesn't work

If the leukemia doesn't respond well to the first treatment, there are several options.

- Increasing the dose of the drug. This helps some people, although the higher dose often has worse side effects.
- Switching to another TKI, for example from imatinib to dasatinib, nilotinib, or bosutinib. The doctor may check the CML cells for genetic changes (mutations) to help decide which drug would be best.
- [Interferon](#) or [chemotherapy](#) (chemo) may be tried for those who can't take the TKIs or those for whom they are not working,
- [Stem cell transplant](#) may be an option, especially for younger people who have a donor with a matching tissue type.

Treating CML after a stem cell transplant

Some people who have a stem cell transplant may not get a complete response. If they *do not* have graft-versus-host disease (GVHD), doctors may try to get their new immune system to fight the leukemia. One way to do this is by slowly lowering the doses or stopping the immune suppressing drugs they are taking. This is done very carefully in order to have an anti-leukemia effect without getting too much GVHD. Patients are watched closely during this time. Another approach that helps some patients is an infusion of lymphocytes taken from the person who donated the stem cells for the transplant (called **donor lymphocyte infusion**). This can induce an immune reaction against the leukemia. Other drugs may also be helpful. Most experts agree that these patients should take part in a clinical trial.

In patients who *do* have GVHD after a stem cell transplant, boosting the immune system further is not likely to help. These patients are often treated with a TKI like imatinib.

Accelerated phase

When CML is in accelerated phase, leukemia cells begin to build up in the body quickly, causing symptoms. The leukemia cells often acquire new gene mutations, which help

them grow and might make treatments less effective.

The treatment options for accelerated phase CML depend on what treatments the patient has already had. In general, the options are a lot like those for patients with chronic phase CML. But patients with accelerated phase CML are less likely to have a long-term response to any treatment.

If the patient hasn't had any treatment, a [TKI](#) will be used. Imatinib (often at higher doses than used for chronic phase CML) is an option for most people. Most patients in this phase respond to treatment with imatinib, but the responses do not seem to last as long as they do in patients in the chronic phase. The newer drugs like dasatinib and nilotinib are often used in this phase, and other drugs are under study.

If the patient is already getting imatinib, the dose may be increased. Another option is to switch to one of the other TKIs. Sometimes the CML cells are tested to see if they have genetic changes (mutations) that may mean that a certain TKI is more or less likely to work (see the section below called CML with the T315I mutation). In CML without that mutation, ponatinib is an option after all of the other TKIs have been tried.

[Interferon](#) is another option, but it's also much less effective in this phase than in the chronic phase. Some patients have some response when chemo is added to the TKI, but these responses are usually shorter than 6 months.

An [allogeneic stem cell transplant](#) may be the best option for most patients who are young and healthy enough to have this treatment. Most doctors prefer that the leukemia be controlled, preferably in remission, before starting the transplant procedure. To achieve this, [chemo](#) will often be used.

In some cases, an autologous SCT may be an option to try to get the CML back into the chronic phase, but it's very unlikely to result in a cure.

Blast phase

In the blast phase of CML, the leukemia cells become more abnormal. The disease acts like an acute leukemia, with blood counts getting higher and symptoms appearing or getting worse.

For people with blast phase CML who haven't been treated before, high-dose imatinib may be helpful. But it works in a smaller number of people and for shorter lengths of time than when used earlier in the course of the disease. Newer [TKIs](#), such as dasatinib, nilotinib, and bosutinib, seem to be better in this phase, particularly if they

hadn't been used earlier. Ponatinib may also be used, but only after all of the other TKIs have been tried. Patients who respond to these drugs may want to consider a [stem cell transplant](#), if possible.

Most often, the leukemia cells in this phase act like cells of [acute myeloid leukemia \(AML\)](#)², but they're often resistant to the chemo drugs normally used to treat AML. Standard chemo for AML will bring about a remission in about 1 out of 5 patients, but this is usually short-lived. If remission does occur, it may be a chance to consider some type of stem cell transplant.

A smaller number of patients have blast cells that act like cells of acute lymphoblastic leukemia (ALL). These cells are more sensitive to chemo drugs. Remissions can be induced in about half of these patients with drugs like vincristine, prednisone, and doxorubicin, along with imatinib, if that hasn't been given yet. Like patients with ALL, these patients are at risk for having leukemia cells in the fluid that surrounds the brain and spinal cord, so they often get chemo (cytarabine or methotrexate) put directly into that fluid (like during a spinal tap). Radiation therapy to the brain is another option, but is used less often. For more information, see [Acute Lymphocytic Leukemia](#)³.

Allogeneic SCT is less successful for blast phase CML than for earlier phases, and the long-term survival rate is less than 20%. Still, it's the only known option that may cure the disease. It's more likely to work if the CML can be brought back to the chronic phase before the transplant.

Because most patients with blast phase CML can't be cured, [palliativetreatment](#)⁴ (intended to relieve symptoms rather than cure the disease) is important. For instance, radiation therapy can help shrink an enlarged spleen or reduce pain from areas of bone damaged by leukemia. [Chemo](#) (usually with drugs such as hydroxyurea) may relieve some symptoms for a time.

[Clinical trials](#)⁵ of new combinations of chemo, targeted agents, and biologic therapies are important options.

CML with the T315I mutation

As was mentioned in the section about [targeted therapy](#), in some patients on TKI treatment, the cancer cells develop a gene change called the *T315I mutation* that keeps most of the TKIs from working. If your CML stops responding to treatment with a TKI, another one may be tried. Your doctor may also check to see if the cancer cells have developed the T315I mutation. If they have, you may be switched to ponatinib, which is the only TKI that works for CML with this mutation. If this doesn't work or you can't take

it because of side effects, you may be started on chemotherapy (chemo). Omacetaxine (Synribo[®]) is a newer chemo drug that has been shown to help sometimes in this situation, but other chemo drugs may help, too.

Hyperlinks

1. www.cancer.org/cancer/chronic-myeloid-leukemia/detection-diagnosis-staging/staging.html
2. www.cancer.org/cancer/acute-myeloid-leukemia.html
3. www.cancer.org/cancer/acute-lymphocytic-leukemia.html
4. www.cancer.org/treatment/treatments-and-side-effects/palliative-care.html
5. www.cancer.org/treatment/treatments-and-side-effects/clinical-trials.html

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See all references for Chronic Myeloid Leukemia (www.cancer.org/cancer/chronic-myeloid-leukemia/references.html)

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