



National Comprehensive  
Cancer Network®

2026

## NCCN Guidelines for Patients®

Cancer care recommendations from leading experts at the  
National Comprehensive Cancer Network® (NCCN®)

# Chronic Myeloid Leukemia



Presented with support from



Available online at  
[NCCN.org/patientguidelines](https://www.nccn.org/patientguidelines)



## NCCN Guidelines for Patients®

The essential guide for people facing cancer.

Based on care recommendations from leading cancer experts.

Explains high-quality cancer care provided at  
state-of-the-art cancer centers.

Reviewed and revised every year.

Did you know that top cancer centers across the United States work together to improve cancer care? This alliance of leading cancer centers is called the National Comprehensive Cancer Network® (NCCN®).

Because cancer care is always evolving, NCCN develops and frequently updates evidence-based cancer care recommendations used by health care providers worldwide. These recommendations are known as the NCCN Clinical Practice Guidelines in Oncology (NCCN Guidelines®).

The NCCN Guidelines for Patients plainly explain these expert recommendations, so you can talk with your care team about the best care for you.

**These NCCN Guidelines for Patients are based on the NCCN Guidelines®  
for Chronic Myeloid Leukemia Version 1.2026 — July 16, 2025.**

View the NCCN  
Guidelines for  
Patients free online:  
[NCCN.org/  
patientguidelines](https://www.nccn.org/patientguidelines)

Find an NCCN  
Cancer Center  
near you:  
[NCCN.org/  
cancercenters](https://www.nccn.org/cancercenters)

Learn how the  
NCCN Guidelines  
for Patients are  
developed:  
[NCCN.org/patient-  
guidelines-process](https://www.nccn.org/patient-guidelines-process)

Connect with us



@NCCNorg



@NCCNVideo



NCCN



@NCCNorg

## Supporters



NCCN Guidelines for Patients are supported by funding from the  
NCCN Foundation®

**NCCN Foundation gratefully acknowledges the following corporate supporters for helping to make available these NCCN Guidelines for Patients: Azurity Pharmaceuticals and Novartis Pharmaceuticals Corporation.**

NCCN independently adapts, updates, and hosts the NCCN Guidelines for Patients. Our corporate supporters do not participate in the development of the NCCN Guidelines for Patients and are not responsible for the content and recommendations contained therein.

To make a gift, visit  
[NCCNFoundation.org/Donate](https://www.nccn.org/Donate)

To learn more, visit online  
[NCCN.org/patientguidelines](https://www.nccn.org/patientguidelines)  
or email  
[PatientGuidelines@NCCN.org](mailto:PatientGuidelines@NCCN.org)

## Contents

- 4 Chapter 1  
About CML
- 9 Chapter 2  
Testing for CML
- 19 Chapter 3  
Types of treatment
- 31 Chapter 4  
Supportive care
- 37 Chapter 5  
Chronic phase CML
- 46 Chapter 6  
Advanced phase CML
- 53 Chapter 7  
Other resources
- 57 Words to know
- 60 NCCN Contributors
- 61 NCCN Cancer Centers
- 62 Index

©2026 National Comprehensive Cancer Network, Inc. All rights reserved.

NCCN Guidelines for Patients and illustrations herein may not be reproduced in any form for any purpose without the express written permission of NCCN. No one, including doctors or patients, may use the NCCN Guidelines for Patients for any commercial purpose and may not claim, represent, or imply that the NCCN Guidelines for Patients that have been modified in any manner are derived from, based on, related to, or arise out of the NCCN Guidelines for Patients. The NCCN Guidelines are a work in progress that may be redefined as often as new significant data become available. NCCN makes no warranties of any kind whatsoever regarding its content, use, or application and disclaims any responsibility for its application or use in any way.

NCCN Foundation seeks to support the millions of patients and their families affected by a cancer diagnosis by funding and distributing NCCN Guidelines for Patients. NCCN Foundation is also committed to advancing cancer treatment by funding the nation's promising doctors at the center of innovation in cancer research. For more details and the full library of patient and caregiver resources, visit [NCCN.org/patients](https://www.nccn.org/patients).

National Comprehensive Cancer Network (NCCN) and NCCN Foundation  
3025 Chemical Road, Suite 100, Plymouth Meeting, PA 19462 USA

# 1

## About CML

- 5 What is chronic myeloid leukemia?
- 6 What is blood?
- 8 How is CML treated?
- 8 How can I get the best care?

Chronic myeloid leukemia (CML) is caused by a single, specific abnormal gene that's created when a piece of chromosome 9 and a piece of chromosome 22 break off and trade places. This results in a shortened chromosome 22 called the Philadelphia (Ph) chromosome and a fusion gene called *BCR::ABL1*. Treatment aims to reduce the number of CML cells with the *BCR::ABL1* gene.

### What is chronic myeloid leukemia?

Chronic myeloid leukemia (CML) is a type of blood cancer. CML is caused by a single, specific abnormal gene called *BCR::ABL1*. It's created when a piece of chromosome 9 and a piece of chromosome 22 break off and switch places. This results in an abnormal chromosome 22 that contains a small part of chromosome 9. This new chromosome 22 is referred to as the Philadelphia (Ph) chromosome. The Ph chromosome contains the *BCR::ABL1* gene.

The *BCR::ABL1* gene makes a protein that leads to uncontrolled blood cell production and growth. This cell growth ultimately leads to CML. When the body makes abnormal blood cells, it doesn't make enough of the normal blood cells a person's body needs and this can cause symptoms.

### How this guide can help you

Making decisions about cancer care is stressful. There's a lot to learn, and you don't know what the future holds.

Use this guide to get the information and support you need.

Patients, doctors, and other health care professionals trust the NCCN Guidelines for Patients. This guide uses clear, everyday language to explain current cancer care recommendations made by respected experts in the field. Their recommendations are based on the latest research and practices at leading cancer centers.

Your health is unique to you, so your cancer care should be, too. As you read this guide, you'll learn which treatments are likely to provide the best results for you. And you'll be better prepared to talk with your care team.

*BCR::ABL1* isn't found in normal blood cells. It isn't passed down from birth parents to children. If you have the *BCR::ABL1* gene, then you have CML.

Read more to learn about the types of blood cells, how blood is made, and where CML starts.

## What is blood?

There are 4 main components of blood—plasma, red blood cells, white blood cells, and platelets. Blood’s function is to move oxygen and nutrients throughout your body and carry away waste. Blood also plays an important role for the immune system and in preventing bleeding.

### Types of blood cells

Your blood contains different types of cells that float in plasma. Plasma is a clear, yellowish fluid made up of mostly water.

There are 3 types of blood cells:

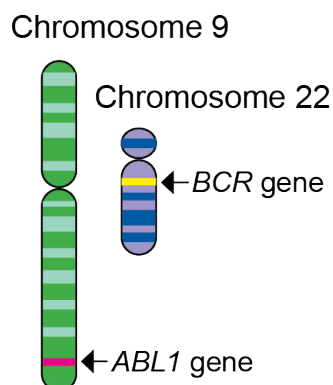
- Red blood cells (erythrocytes) carry oxygen throughout the body.
- White blood cells (leukocytes) help fight and prevent infection. White blood cells include granulocytes (or neutrophils), monocytes, and lymphocytes.
- Platelets (thrombocytes) help control bleeding.

In CML, there are usually too many granulocytes. Granulocytes include neutrophils, eosinophils, and basophils. Sometimes, there are too few or too many platelets as well. The “chronic” in chronic myeloid leukemia means this cancer worsens slowly without treatment.

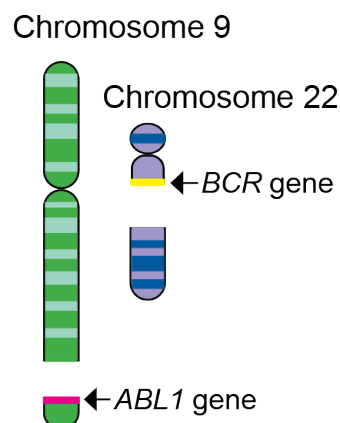
### Philadelphia chromosome

**Chronic myeloid leukemia (CML) is caused by a single, specific, abnormal gene that’s created when a piece of chromosome 9 and a piece of chromosome 22 break off and trade places. This results in a shortened chromosome 22 called the Philadelphia (Ph) chromosome and a fusion gene called *BCR::ABL1*.**

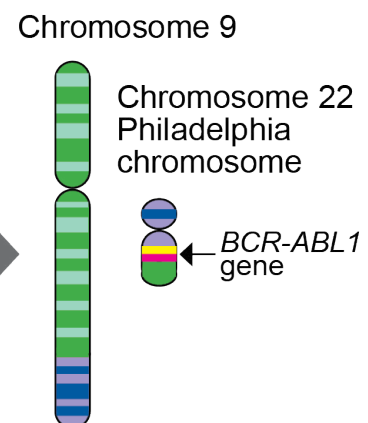
#### Normal chromosomes



#### Chromosomes break



#### Changed chromosomes



## How are blood cells formed?

Bone marrow is the organ that creates blood in our body. It's the sponge-like tissue in the center of most bones. Inside your bone marrow are early blood-forming cells called blood (hematopoietic) stem cells. At any given time, the bone marrow contains cells in various stages of development, from very immature to nearly mature. Once a blood stem cell fully

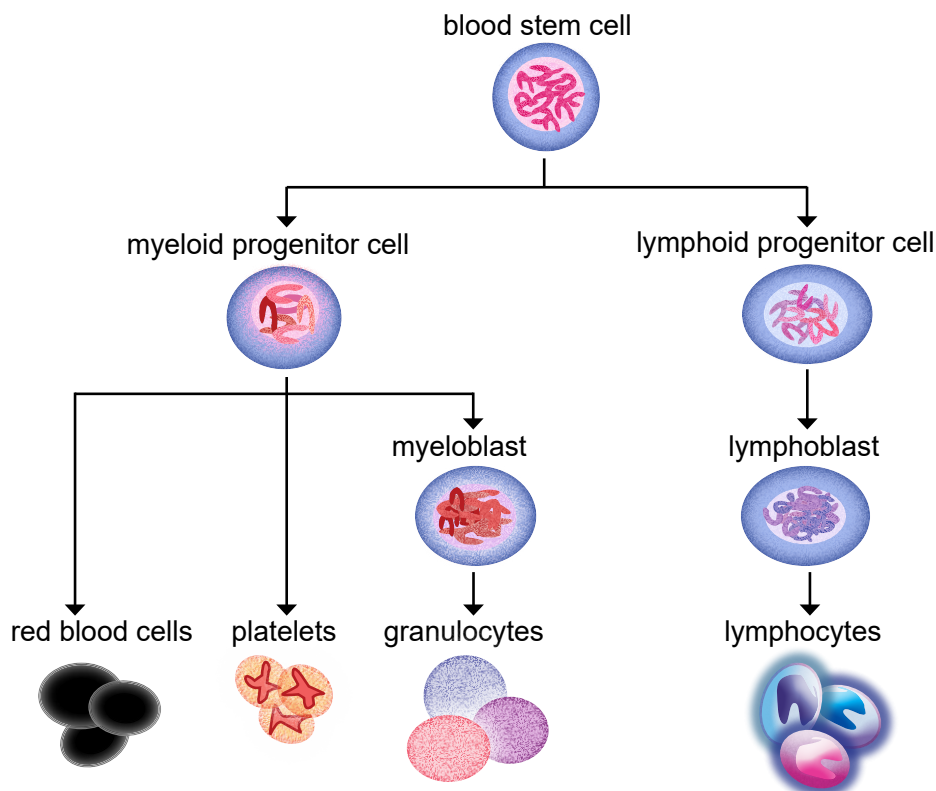
develops into a red blood cell, white blood cell, or platelet, it's released into the bloodstream as needed.

The role of blood stem cells is to make cells called intermediary cells that will become red blood cells, white blood cells, and platelets. These intermediaries are called progenitor cells or precursor cells.

### Blood cell formation

**All blood cells start as blood stem cells. A blood stem cell has to go through many stages to become a red blood cell, white blood cell, or platelet. CML affects the myeloid progenitor cells and causes too many granulocytes (a type of white blood cell) to be made. However, advanced phase CML can also affect the lymphoid progenitor cells.**

Copyright © 2022 National Comprehensive Cancer Network® (NCCN®). [www.nccn.org](http://www.nccn.org)



There are different types of progenitor cells:

- **Lymphoid progenitor cells** form into lymphoblasts that mature into lymphocytes.
- **Myeloid progenitor cells** form into myeloblasts and other non-lymphoid blood cells.

CML is thought to arise from blood stem cells that make an increased amount of myeloid progenitor cells. However, an advanced form of CML can cause an increased amount of lymphoid or myeloid progenitor cells to be made. Most people are diagnosed with an early form of CML called chronic phase CML. The phases of CML are discussed later in this guide.

In CML, the very immature or most immature cells (myeloblasts or lymphoblasts) are often referred to simply as blasts.

### How is CML treated?

CML is usually treated with a type of drug therapy called targeted therapy. Treatment aims to stop the activity of the BCR::ABL1 protein and reduce the number of CML cells with the *BCR::ABL1* gene to at least 1 percent (1%) or less, ideally as close as possible to zero.

This guide will discuss in greater detail how CML is diagnosed and treated. It will provide an overview of the types of tests and treatments and what to expect during testing and treatment.

### How can I get the best care?

Advocate for yourself. You have an important role to play in your care. Many people feel more satisfied when they actively take part in planning their cancer care.

The NCCN Guidelines for Patients will help you play a larger role in your care. Discuss the recommendations in this guide with your care team. Ask questions about your options and share your goals and concerns.

Don't know what to ask? You're not alone. That's why we include suggested questions to ask at the end of chapters.

Keep reading to find the best care for you.



#### **We want your feedback!**

**Our goal is to provide helpful and easy-to-understand information on cancer.**

**Take our survey to let us know what we got right and what we could do better.**

[NCCN.org/patients/feedback](https://www.nccn.org/patients/feedback)

# 2

## Testing for CML

- 10 General health tests
- 11 Blood tests
- 13 Fertility (all genders)
- 13 Bone marrow tests
- 14 CML biomarker and genetic testing
- 17 Heart tests
- 18 Key points
- 18 Questions to ask

Treatment planning starts with testing. Accurate testing is needed to diagnose and treat chronic myeloid leukemia (CML). This chapter presents an overview of possible tests you might receive and what to expect.

Results from blood tests and possibly a biopsy will be used to determine your treatment plan. Treatment will be based on these findings. This chapter provides a general overview of some of the tests you might have. Tests to plan treatment can be found in **Guide 1**.

### General health tests

Some general health tests are described next.

#### Medical history

A medical history is a record of all health issues and treatments you've had in your life. It asks about past surgeries and current health conditions. Bring a list of old and new medicines and any over-the-counter medicines, herbals, or supplements you take. Some supplements interact with and affect medicines that your care team may prescribe. Also, tell your care team about any symptoms you have.

#### Family history

To date, there's no compelling evidence that having a family history of chronic myeloid leukemia (CML) increases the chances of developing CML. However, some other types of cancers can run in families. Your doctor will ask about the health history of family members who are blood relatives. This information is called family history.

Ask family members on both sides of your family about their health concerns, like heart disease, cancer, and diabetes, and at what age they were diagnosed. It's important to know the specific type of cancer, where the cancer started, if it's in multiple locations, and if they had genetic testing.

#### Guide 1 Testing for CML

Medical history and physical exam that includes spleen size

Complete blood count with differential

Chemistry profile, including uric acid

Bone marrow aspirate and biopsy

qPCR using IS for *BCR::ABL1* found in blood

Hepatitis virus screening

Distress screening

### Physical exam

During a physical exam, your health care team may:

- › Check your height and weight
- › Listen to your lungs and heart
- › Look in your eyes, ears, nose, and throat
- › Feel and apply pressure to parts of your body to see if organs are of normal size, are soft or hard, or cause pain when touched
- › Feel for enlarged lymph nodes in your neck, underarm, and groin
- › Feel your abdomen and below your left ribcage to see if your spleen is enlarged. An enlarged spleen is one sign of CML.

### Distress screening

Dealing with a cancer diagnosis can be stressful and may cause further distress. Distress is an unpleasant experience of a mental, physical, social, or spiritual nature. It can affect how you feel, think, and act. Distress might include feelings of sadness, fear, helplessness, worry, anger, and guilt. You may also experience depression, anxiety, and sleep issues. Your treatment team will screen your level of distress. This is part of your cancer care.

### Performance status

Performance status is a rating of a person's general level of fitness and ability to perform daily tasks. It's one factor taken into consideration when choosing a treatment plan.

### Blood tests

Blood tests check for signs of disease and how well organs are working. They are also used to look for hormones and other chemicals produced by your tumor. Blood tests require a sample of blood, which is removed through a needle placed into a vein in your arm. Blood samples are sent to a lab for testing.

Some possible blood tests are described next.

#### Complete blood count and differential

A complete blood count (CBC) measures the levels of red blood cells, white blood cells, and platelets in your blood. A CBC is a key test that gives a picture of your overall bone marrow health.

A differential counts the number of each type of white blood cell (neutrophils, lymphocytes, monocytes, eosinophils, and basophils). It also checks if the counts are in balance with each other. CML often causes high white blood cell counts and/or high platelet counts but can sometimes cause low red blood cell counts.

#### Chemistry profile

A chemistry profile or panel measures the levels of different substances released into your blood by the liver, bone, and other organs. When CML is present, the chemistry panel can be abnormal.

### **Creatinine**

Creatinine is a waste produced in the muscles. Every person generates a fixed amount of creatinine every day based on how much muscle they have. It's filtered out of the blood by the kidneys. The level of creatinine in the blood tells how well the kidneys are working. Higher levels of creatinine generally mean the kidneys aren't working well.

### **Hepatitis virus screening**

Hepatitis B and C virus infections can affect the liver. A hepatitis blood test will show if you had hepatitis in the past or if you have it today. Some cancer treatments can wake up (or reactivate) the virus. If this happens, it can harm the liver.

### **HLA typing**

Human leukocyte antigen (HLA) is a protein found on the surface of most cells. It plays an important role in your body's immune response. HLAs mark your body's cells. Your body detects these markers to tell which cells are yours. In other words, all of your cells have the same set of HLAs. Each person's set of HLAs is called the HLA type or tissue type.

HLA typing is a test that detects a person's HLA type. This test is done before a donor (allogeneic) hematopoietic cell transplant (HCT). To find a donor match, your proteins will be compared to the donor's proteins to see how many proteins are the same. A very good match is needed for a transplant to be a treatment option. Otherwise, your body will reject the donor cells or the donor cells will react against your body. Blood samples from you and your blood relatives will be tested first.

### **Liver function tests**

Liver function tests (LFTs) look at the health of your liver by measuring chemicals that are made or processed by the liver. Levels that are too high or low signal that the liver isn't working well or the bile ducts might be blocked.

### **Pregnancy test**

People who can become pregnant will be given a pregnancy test before treatment begins.

### **Uric acid**

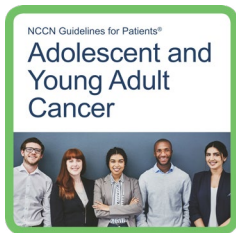
Uric acid is released by cells when DNA breaks down. It's a normal waste product that dissolves in the blood and is filtered by the kidneys where it leaves the body in the urine. Too much uric acid in the body is called hyperuricemia. With CML, it can be caused by fast turnover (cell death) of white blood cells. High uric acid might be a side effect of treatment. Very high levels of uric acid in the blood can cause kidney stones, kidney damage, and gout (inflammation of joints). You may be prescribed allopurinol for a brief period of time after starting primary treatment.

### Fertility (all genders)

Treatment with targeted therapy and other forms of systemic therapy can affect your fertility, or the ability to have children. If you think you want children in the future, ask your care team how cancer and cancer treatment might affect your fertility.

Fertility preservation is all about keeping your options open, whether you know you want to have children later in life or aren't sure at the moment. Fertility and reproductive specialists can help you sort through what may be best for your situation.

More information on fertility preservation can be found at *NCCN Guidelines for Patients: Adolescent and Young Adult Cancer* [NCCN.org/patientguidelines](https://www.nccn.org/patientguidelines) and on the [NCCN Patient Guides for Cancer](#) app.



### Pregnancy during treatment

If you are pregnant or breastfeeding during your cancer diagnosis, treatment may need to be adjusted. Speak to your care team if you have any concerns.

### Bone marrow tests

Leukemias start in the bone marrow. To diagnose CML and determine the CML phase, samples of bone marrow are removed and tested before starting any treatment. Your bone marrow sample should be reviewed by a pathologist who is an expert in the diagnosis of CML. This review is often referred to as histology, histopathology, or hematopathology review. The pathologist will note the overall appearance and the size, shape, and type of your cells. Tests will be done on the biopsied cells.

There are 2 types of bone marrow tests that are often done at the same time:

- Bone marrow aspirate
- Bone marrow biopsy

Your bone marrow is like a sponge holding liquid and cells. An aspirate withdraws some of the liquid and cells out of the sponge, and a biopsy removes a piece of the sponge.

A bone marrow aspirate and biopsy are a bedside procedure. It's not surgery and doesn't require going to an operating room. In some people, imaging may be needed to locate the correct part of the hip to place the needle. Your care team will try to make you as comfortable as possible during the procedure.

The samples are usually taken from the back of the hip bone (pelvis). You will likely lie on your belly or side. For an aspirate, a hollow needle will be pushed through your skin and into the bone marrow. Liquid bone marrow will then be drawn into a syringe. For the biopsy, a wider needle will be used to remove a small piece of bone marrow. You may feel bone

## 2 Testing for CML

pain at your hip for a few days. Your skin may bruise.

Although the diagnosis of CML can be made by genetic testing of peripheral (circulating) blood, a bone marrow biopsy is required to fully stage the disease.

### Flow cytometry

Flow cytometry is a laboratory method used to detect, identify, and count specific cells. Flow cytometry involves adding a light-sensitive dye to cells. The dyed cells are passed through a beam of light in a machine. The machine measures the number of cells.

Flow cytometry may be used on cells from circulating (peripheral) blood or from a bone marrow aspirate. A blood test can count the number of white blood cells, but it can't detect

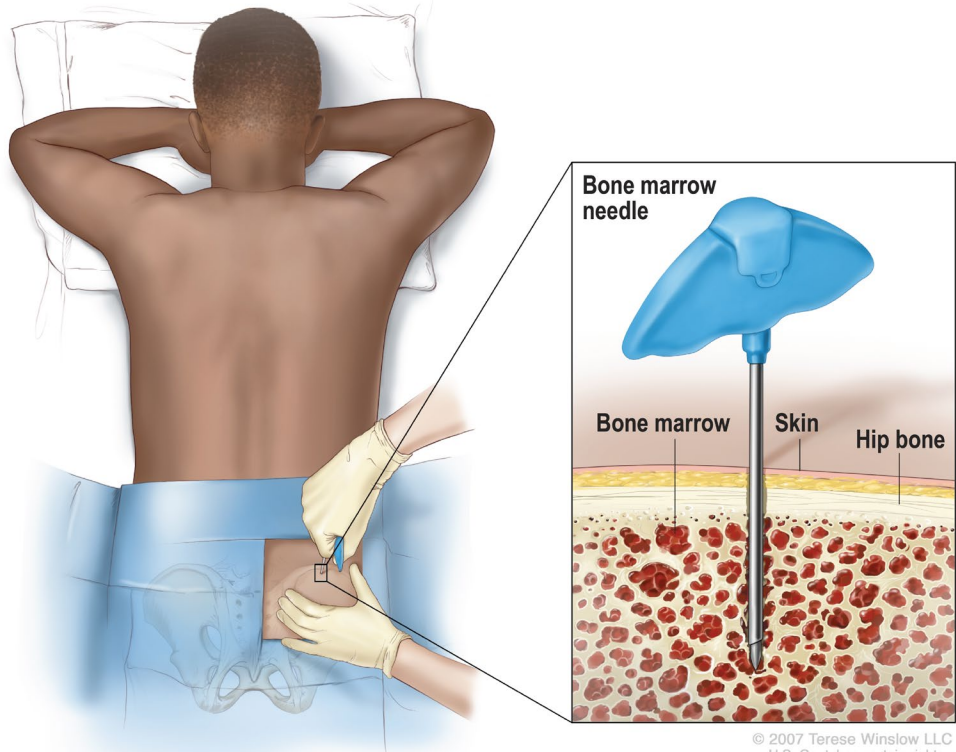
the subtle differences between different types of blood cancers. Flow cytometry can help detect these subtle differences.

### CML biomarker and genetic testing

Biomarker and genetic tests are used to learn more about your type of CML, to target treatment, and to determine the likely path your cancer will take, called a prognosis. This genetic testing is different from family history genetic testing or genetic cancer risk testing. This testing looks for changes only in the blood cells that have developed over time, and not changes in the rest of your body's cells. Testing will look for the Philadelphia (Ph) chromosome, which is used to diagnose and to help determine the CML phase. You may be

### Bone marrow aspirate and biopsy

Samples of bone and marrow are removed in a bone marrow biopsy. A bone marrow aspirate and biopsy are needed to diagnose CML.



© 2007 Terese Winslow LLC  
U.S. Govt. has certain rights

## 2 Testing for CML

placed into a risk group based on the types of genetic abnormalities found.

Inside of our cells are DNA (deoxyribonucleic acid) molecules. These molecules are tightly packaged into what is called a chromosome. Chromosomes contain most of the cell's genetic information. Normal human cells contain 23 pairs of chromosomes for a total of 46 chromosomes. Each chromosome contains thousands of genes. Genes are coded instructions for the proteins your cells make. A mutation is when something goes wrong in the genetic code.

### CML mutation testing

Mutation testing uses methods such as karyotype, fluorescence in situ hybridization (FISH), polymerase chain reaction (PCR), and next-generation sequencing (NGS) to look for changes or abnormalities that are

unique to CML cells (genes and chromosomes). A sample of your blood or bone marrow will be used to see if the CML cancer cells have any specific mutations.

Some mutations may determine the type of treatment given. Subtle, new, drug-resistant mutations in the *BCR::ABL1* gene may emerge over time. They can happen as CML progresses to advanced phases, such as the accelerated or blast phase. Some mutations lead to resistance to certain targeted therapies. There are many possible mutations.

### FISH

Fluorescence in situ hybridization (FISH) is a method that involves special dyes called probes that attach to pieces of DNA. For example, in CML the probes attach to the *BCR* gene and the *ABL1* gene. The *BCR::ABL1* gene is detected when the colors of the probes

**“During the early stages, it’s often difficult to listen and absorb everything because it’s easy to get overwhelmed, feel numb, or have racing thoughts. Bring someone with you to every appointment: they can help take notes and keep a list of your questions to ensure they are answered. More importantly, they will be there to provide comfort for you during this difficult time.”**



## 2 Testing for CML

overlap due to the Philadelphia chromosome translocation. A translocation is the switching of parts between 2 chromosomes. The *BCR::ABL1* translocation can also be written as t(9;22).

FISH can identify translocations that are too small to be detected with some methods. It can only be used for known changes. It can't detect all of the possible changes found within genes or chromosomes. A bone marrow sample is usually needed to get all of the information your care team needs to plan your treatment.

### Karyotype

A karyotype is a picture of chromosomes. Normal human cells contain 23 pairs of chromosomes for a total of 46 chromosomes. A karyotype will show extra, missing, rearranged, or abnormal pieces of chromosomes. Since a

karyotype requires growing cells, a sample of bone marrow or blood must be used.

### Chromosome translocation and gene rearrangement

Chromosome translocation and gene rearrangement is the switching of parts between 2 chromosomes. When described at the chromosome level, it's called a translocation. When described at the gene level, it's called rearrangement. For example, a translocation between chromosome 9 and 22 is written as t(9;22) and is known as the Philadelphia (Ph) chromosome. Its gene rearrangement is written as *BCR::ABL1*.

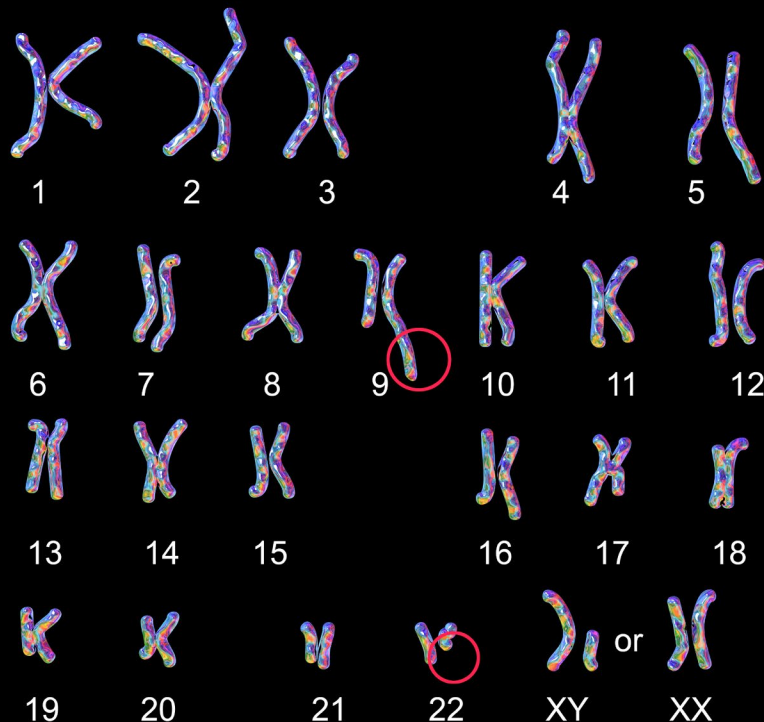
### Next-generation sequencing

Next-generation sequencing (NGS) is a method used to determine a portion of a person's DNA sequence. It shows if a gene

### CML karyotype

A karyotype is a picture of your chromosomes. The study of chromosomes is called cytogenetics.

In CML, a piece of chromosome 9 and a piece of chromosome 22 break off and trade places. This results in a shortened chromosome 22 called the Philadelphia (Ph) chromosome (shown in image).



has any mutations that might affect how the gene works. NGS looks at the gene in a more detailed way than other methods and can find mutations that other methods might miss.

### PCR

A polymerase chain reaction (PCR) is a technique that can make millions or billions of copies of your DNA or RNA (genetic information). PCR is very sensitive. It can find 1 abnormal cell among more than 100,000 normal cells. These copies, called PCR products, might be used for NGS.

### qPCR (IS)

A special PCR called quantitative reverse transcriptase polymerase chain reaction (qPCR) is used in CML. It provides a rough estimate of the proportion of cells with the *BCR::ABL1* gene. This is done by comparing the *BCR::ABL1* gene level with a gene needed for maintaining basic cell functions (called a “housekeeping” gene), such as *ABL1*. The number found in your blood is compared to an international standard, or baseline, called the International Scale (IS). This is the most important test for monitoring responses to treatment. Ask your care team if they are using qPCR (IS). It’s the gold standard for detecting and measuring *BCR::ABL1*.

A qPCR (IS) should be done at initial diagnosis to look for the presence of the *BCR::ABL1* gene on the Philadelphia chromosome. This test might be referred to as real-time or reverse transcriptase (RT)-PCR. The qPCR level of *BCR::ABL1* shows how your disease is responding to CML therapy and is an important measure of treatment progress. You will have this test often after starting treatment.

**A qPCR (IS) is the only test sensitive enough to detect very low levels of the *BCR::ABL1* gene.**

## Heart tests

Heart, or cardiac, tests are used to see how well the heart works. These tests might be used to monitor treatment side effects or to measure your heart function before you start treatment. You might be referred to a heart specialist called a cardiologist.

- **An electrocardiogram (ECG or EKG)** shows electrical activity in the heart.
- **An echocardiogram (or echo)** uses sound waves to make pictures of the heart.

### Key points

- A diagnosis of chronic myeloid leukemia (CML) is confirmed using a bone marrow aspirate and bone marrow biopsy.
- Genetic and biomarker tests are used to learn more about your CML, to target treatment, and to determine the likely course your cancer will take.
- A special polymerase chain reaction (PCR) test called qPCR (IS) measures the proportion of cells with the *BCR::ABL1* gene mutation. It's the gold standard for detecting and measuring *BCR::ABL1*.
- In some cases, CML cells can develop additional mutations, which can affect treatment options.
- Talk to your care team if you are or plan to become pregnant. Certain treatments for CML will need to be avoided if you are pregnant or breastfeeding.

### Questions to ask

- What can you tell me about my cancer?
- Is there a cancer center or hospital nearby that specializes in CML?
- What tests will I have? How often will they be repeated?
- Will my insurance pay for this test?
- Who will talk with me about the next steps and when?

### What's next?

The next chapter provides information on the 3 phases of CML, the types of treatment, what to expect from treatment, and some possible side effects of treatment.

# 3

## Types of treatment

- 20 Your care team
- 21 Three phases of CML
- 22 Systemic therapy
- 23 Targeted therapy
- 26 Hematopoietic cell transplant
- 28 Clinical trials
- 30 Key points
- 30 Questions to ask

### 3 Types of treatment

This chapter presents an overview of the possible types of treatment and what to expect. CML is usually treated with targeted therapy. A targeted therapy focuses on specific or unique features of cancer cells, such as the protein made by the *BCR::ABL1* gene.

Chronic myeloid leukemia (CML) is highly treatable and may be curable in certain cases. Most people will need lifelong treatment. It's important to have regular talks with your care team about your goals for treatment and your treatment plan.

### Your care team

Treating cancer takes a team approach. Treatment decisions should involve a multidisciplinary team of health care and

psychosocial care professionals from different backgrounds who have knowledge and experience in your type of cancer. This team is united in planning and implementing your treatment. Ask who will coordinate your care.

Your team might include the following specialists:

- ▶ **A hematologist or hematologic oncologist** is a medical expert in blood diseases and blood cancers and treats these conditions. There are some hematologists who specialize in CML.
- ▶ **A medical oncologist** treats cancer using systemic (drug) therapy.
- ▶ **A pathologist or hematopathologist** analyzes the cells and tissues removed during a biopsy and provides cancer diagnosis, staging, and information about biomarker testing.

**“I was so fortunate to find an expert oncologist and health care team that I could talk to openly and honestly during and after my treatment. This shared decision-making made me feel like I had a say about my care.”**



### Three phases of CML

This section provides an in-depth explanation of the CML phases.

CML can have 3 phases:

- Chronic
- Accelerated
- Blast

Most people with CML are diagnosed in the chronic phase, but a small number are diagnosed in the accelerated or blast phase. During treatment, CML can sometime progress from chronic phase to accelerated or blast phase.

Phases are based on the percentage of immature white blood cells (blasts) found in the blood and bone marrow. Normal bone marrow contains up to 5 percent (5%) blasts. This means that it's normal to have fewer than 5 blasts for every 100 blood cells. In chronic phase CML, the number of blasts may be higher than 5% but is usually less than 15%. Fifteen percent or more blasts is a sign of advanced phase CML. The accelerated and blast phases are considered advanced phases.

#### Phase 1 – Chronic phase

The first phase of CML is called chronic phase. In this phase, there's an increased number of white blood cells in the blood, bone marrow, or both. Fewer than 15 out of every 100 blood cells are myeloblasts (<15%).

Without treatment, CML typically progresses very slowly in the chronic phase. It may take several months or years to reach the next phase. Compared to other phases, chronic phase CML typically responds better to treatment.

#### Phase 2 – Accelerated phase

The second phase of CML is called accelerated phase. In this phase, the number of myeloblasts is higher than normal or there are chromosome changes that suggest the number of myeloblasts is going to increase soon. The number of white blood cells may also be high. Other cells called promyelocytes and eosinophils may be increased. There may be a very low number of platelets in the blood caused by CML and not by treatment. In the accelerated phase, CML cells may grow faster.

In all phases, CML cells contain the Philadelphia chromosome (Ph+). However, in the accelerated phase, there may be additional abnormal DNA changes (mutations) within Ph+ cells.

#### Phase 3 – Blast phase

The third and final phase of CML is called blast phase, or blast crisis. Once CML is in blast phase, it can be life-threatening and very difficult to treat with medicine alone. As a result, the major focus of treatment of CML is to prevent blast phase. Blast phase happens after a series of events, including additional gene mutations and resistance to targeted drug therapy. However, some people are diagnosed as having blast phase CML before starting any treatment.

### 3 Types of treatment

A blast is an immature white blood cell. In blast phase CML, blast cells may be found in tissues and organs outside of the bone marrow or blood. Treatment for blast phase CML is based on whether the blasts are myeloid or lymphoid.

- In **myeloid** blast phase CML, the number of myeloblasts is very high, at least 30 out of every 100 cells (30 percent). Myeloblasts are responsible for non-lymphoid blood cells in bone marrow, such as granulocytes, a type of white blood cell, as well as platelets.
- In **lymphoid** blast phase CML, any number of abnormal lymphoblasts is concerning. Lymphoblasts normally mature into lymphocytes, a type of white blood cell. However, in blast phase CML, the normal maturation process is impaired.

The following pages provide a general overview of the types of treatment used to treat CML.

## Systemic therapy

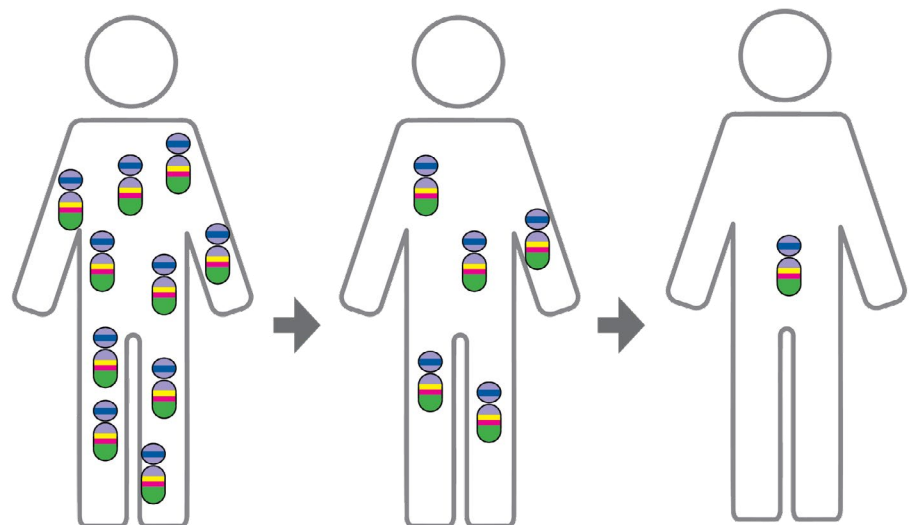
CML is treated with systemic therapy. Systemic therapy is drug therapy that works throughout the body. It includes targeted therapy, chemotherapy, and steroids. CML is usually treated with targeted therapy.

All systemic treatments listed in this guide are recommended and appropriate. When helpful, NCCN experts also assign a level of preference to their recommendations for systemic therapies:

- **Preferred therapies** have the most evidence they may work better and may be safer than other therapies.
- **Other recommended therapies** can provide effective results but may have less evidence, more side effects, or may not work quite as well as preferred therapies.
- **Therapies used in certain cases** work best for individuals with specific cancer features or health circumstances.

### CML treatment

Treatment aims to stop the activity of the **BCR::ABL1** protein and reduce the number of CML cells with the **BCR::ABL1** gene to as close to zero as possible.



## 3 Types of treatment

Goals of systemic therapy should be discussed before treatment is started. The choice of therapy takes into consideration many factors, including your age, other serious health issues, and future treatment possibilities like a hematopoietic cell transplant. Your preferences about treatment are important. If you have any religious or personal beliefs about certain kinds of treatment, now is the time to share them with your care team.

### Chemotherapy

Chemotherapy kills fast-dividing cells throughout the body, including cancer cells and normal cells. Chemotherapy combined with targeted therapies are the preferred treatment for blast phase CML.

### Steroids

Steroids are human-made versions of hormones made by the adrenal glands. The adrenal glands are small structures found near the kidneys, which help regulate blood pressure and reduce inflammation. Steroids also are toxic to lymphoid cells and may be part of a treatment for lymphoid blast phase CML. Steroids can cause short-term and long-term side effects.

## Targeted therapy

Targeted therapy is a form of systemic therapy that focuses on specific or unique features of cancer cells. Targeted therapies seek out how cancer cells grow, divide, and move in the body. These drugs stop the action of molecules that help cancer cells grow and/or survive.

**People with CML should seek treatment at cancer centers experienced in this type of cancer.**

### Tyrosine kinase inhibitor

A tyrosine kinase inhibitor (TKI) is a type of targeted therapy that blocks the signals that cause cancer to grow and spread.

Tyrosine kinases are proteins in cells that are important for many cell functions. The protein made by the *BCR::ABL1* gene is a tyrosine kinase. It moves or transfers chemicals, called phosphates, from one molecule to another. TKIs are targeted drugs that block this transfer, which stops the uncontrolled cell growth in CML.

There are several TKIs that are used for treatment of CML. These TKIs are slightly different from one another, but they generally work in a similar way. They may cause different side effects. You might not be given a certain TKI if you have a health condition, such as lung or heart issues, or certain mutations. Sometimes, a TKI will stop working if a new drug-resistant mutation develops in CML cells. Switching to a different TKI can often help.

## 3 Types of treatment

### TKIs used to treat CML

The following TKIs might be used to treat your CML (listed in alphabetical order):

- Asciminib (Scemblix)
- Bosutinib (Bosulif)
- Dasatinib (Sprycel)
- Imatinib (Gleevec)
- Nilotinib (Tasigna)
- Ponatinib (Iclusig)

TKIs are divided into first, second, and third generations. In general, each generation of a drug gets more specific and better at targeting certain mutations. This means that second- and third-generation TKIs are usually more potent and better at targeting certain mutations. However, they might have different side effects. TKI options will be based on your specific situation.

If CML doesn't seem to be responding to one TKI, then another TKI will be given. Certain TKIs may work better and be less toxic. The TKI dose might be increased or decreased depending on how CML is responding to treatment and if you are experiencing side effects. If you're having many side effects, you and your care team may discuss changing to another TKI. You'll be closely monitored during treatment.

The following TKIs are listed in alphabetical order and not in order of importance.

#### Asciminib

Asciminib targets a different area of *BCR::ABL1* gene from other TKIs. Asciminib is approved for people with newly diagnosed chronic phase CML, as well as those who received prior therapy. At a higher dose, it's a treatment for those with chronic phase CML with a *BCR::ABL1* gene mutation called T315I. It should be avoided in people who have had pancreatitis and can increase blood pressure.

#### Bosutinib

Bosutinib is a second-generation TKI. It may not be preferred for people who have liver or stomach and digestion (gastrointestinal) issues. There's a lower-cost generic bosutinib option.

#### Dasatinib

Dasatinib is a second-generation TKI. It may not be prescribed if you have lung (pulmonary) disease or breathing issues. There's a lower-cost generic dasatinib option.

#### Imatinib

Imatinib was the first TKI approved by the U.S. FDA to treat CML. Imatinib has been studied for a long time and is still a very good treatment option. It's a good option for those who are older, who have other more serious health issues, or for those who have low-risk chronic phase CML where an aggressive treatment might not be needed. There's a lower-cost generic imatinib option.



### Warnings about supplements and drug interactions

Some supplements and foods can affect the ability of a drug used to treat CML to do its job. This is called a drug interaction.

You might be asked to **stop taking certain herbal supplements**, such as:

- Curcumin (turmeric)
- Ginkgo biloba
- Green tea extract
- St. John's Wort
- Antioxidants

You might be asked to **avoid certain foods**, such as:

- Grapefruit
- Star fruit
- Black mulberry or raspberry
- Wild grape
- Pomegranate

Certain medicines can also affect the ability of a drug to do its job. Antacids, heart or blood pressure medicine, and antidepressants are just some of the medicines that might interact with systemic therapy or supportive care medicines given during systemic drug therapy. Therefore, it's very important to tell your care team about any medicines, vitamins, over-the-counter drugs, herbals, or supplements you're taking.

**Bring a list with you to every visit.**

## 3 Types of treatment

### Nilotinib

Nilotinib is a second-generation TKI. Nilotinib may not be best for those who have heart issues, are at risk for heart issues, or have electrolyte abnormalities. Sudden deaths have occurred in those taking nilotinib. Nilotinib may cause increased blood sugar or worsen peripheral vascular disease. Nilotinib prolongs the QT interval, which is detectable on an electrocardiogram (ECG or EKG). You will likely have heart tests to monitor your heart. There's a lower-cost generic nilotinib option and a non-generic form that has no mealtime restrictions and can be taken with food.

### Ponatinib

Ponatinib is a third-generation TKI. It's the preferred treatment for people with a *BCR::ABL1* gene mutation called T315I, but it may also be used as a treatment option in those without T315I. Ponatinib can have some serious side effects and isn't used as a first-line therapy. You might be referred to a cardiologist to monitor your heart if you receive this treatment.



**When you are going through treatment that's difficult, remember it's about the cancer. Don't let the side effects of treatment become your focus!"**

### TKI side effects

TKIs can cause side effects. If you feel unwell or a side effect is interfering with your ability to do daily tasks, tell your care team. There may be ways to help you feel better. It's very important to continue to take your medicine even if you don't feel well. Speak to your care team before making any changes!

Side effects are common among TKIs. These include low blood counts, fatigue, and musculoskeletal pain. You may feel nauseated, have diarrhea, and vomit. Changes in your skin may occur, such as a rash. You may feel tired and get headaches and fevers. Fluid buildup in limbs (edema) or around certain organs may occur. Severe side effects include heart and liver issues and kidney failure.

### If pregnant or breastfeeding

TKIs can pass into human breast milk or harm an unborn baby. There are treatment options.

## Hematopoietic cell transplant

A hematopoietic cell transplant (HCT) is a cancer treatment that replaces a person's bone marrow and immune system with donor cells to fight the leukemia. An HCT replaces hematopoietic stem cells that have been destroyed by high doses of chemotherapy and/or radiation therapy as part of the transplant process. A hematopoietic stem cell is an immature cell that can develop into any type of blood cell. HCTs are performed in specialized centers.

- Most people with CML don't need an HCT.

## 3 Types of treatment

There are 2 types of HCTs:

- **Autologous**—stem cells come from you.
- **Allogeneic**—stem cells come from a donor who may or may not be related to you. Compared to an autologous HCT, an allogeneic HCT introduces new immune cells from the donor, which may be able to detect and eliminate cancer cells better than your immune system was able to (known as graft-versus-leukemia effect).  
**Only an allogeneic HCT is used as a possible treatment option in CML.**

### Conditioning

Before an HCT, treatment is needed to destroy bone marrow cells. This is called conditioning, and it creates room for transplanted healthy stem cells. It also weakens the immune system, so your body won't kill the transplanted cells. Chemotherapy is used for conditioning. Radiation therapy may also be given as part of conditioning treatment.

After conditioning, you'll receive a transfusion of healthy stem cells from a donor who is matched to you. A transfusion is a slow injection of blood products into a vein. This can take several hours. The transplanted stem cells will travel to your bone marrow and grow. New, healthy blood cells will form. This is called engraftment. It usually takes about 2 to 4 weeks. Until then, you will have little or no immune defense. You may need to stay in a very clean room at the hospital or be given antibiotics to prevent or treat infection.

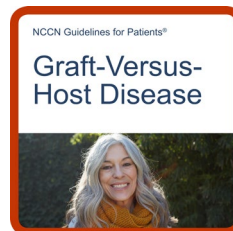
You may need blood transfusions. A red blood cell transfusion is used to treat anemia (below normal red blood cell count). A platelet transfusion is used to treat a low platelet count or bleeding. While waiting for the cells to engraft, you will likely feel tired and weak. HCT has very serious and life-threatening side effects.

The goal of the transplant is for the new immune system to recognize the leukemia as foreign and destroy it and to provide you with new, healthy bone marrow.

### Possible side effects

Every treatment has side effects. You'll be monitored for infections, disease relapse, and graft-versus-host disease (GVHD). In GVHD, the donor cells attack your normal, healthy tissue, such as your organs and skin. There are treatments for GVHD. Ask your care team about the possible side effects or complications of HCT and how this might affect your quality of life.

For more information, see *NCCN Guidelines for Patients: Graft-Versus-Host Disease*, available at [NCCN.org/patientguidelines](https://www.nccn.org/patientguidelines) and on the [NCCN Patient Guides for Cancer](#) app.



### Clinical trials

You may also be able to receive treatment through a clinical trial. A clinical trial is a type of medical research study. After being developed and tested in a lab, potential new ways of treating cancer need to be studied in people.

If found to be safe and effective in a clinical trial, a drug, device, or treatment approach may be approved by the U.S. FDA.

Everyone with cancer should carefully consider all of the treatment options available for their cancer type, including standard treatments and clinical trials. Talk to your doctor about whether a clinical trial may make sense for you.

#### Phases

Most cancer clinical trials focus on treatment and are done in phases.

- **Phase 1** trials study the safety and side effects of an investigational drug or treatment approach.
- **Phase 2** trials study how well the drug or approach works against a specific type of cancer.
- **Phase 3** trials test the drug or approach against a standard treatment. If the results are good, it may be approved by the FDA.
- **Phase 4** trials study the safety and benefit of an FDA-approved treatment.



### Finding a clinical trial

#### In the United States

NCCN Cancer Centers  
[NCCN.org/cancercenters](https://www.nccn.org/cancercenters)

The National Cancer Institute (NCI)  
[cancer.gov/about-cancer/treatment/clinical-trials/search](https://www.cancer.gov/about-cancer/treatment/clinical-trials/search)

#### Worldwide

The U.S. National Library of Medicine (NLM)  
[clinicaltrials.gov](https://clinicaltrials.gov)

#### Need help finding a clinical trial?

NCI's Cancer Information Service (CIS)  
1.800.4.CANCER (1.800.422.6237)  
[cancer.gov/contact](https://www.cancer.gov/contact)

## 3 Types of treatment

### Who can enroll?

It depends on the clinical trial's rules, called eligibility criteria. The rules may be about age, cancer type and stage, treatment history, or general health. They ensure that participants are alike in specific ways and that the trial is as safe as possible for the participants.

### Informed consent

Clinical trials are managed by a research team. This group of experts will review the study with you in detail, including its purpose and the risks and benefits of joining. All of this information is also provided in an informed consent form. Read the form carefully and ask questions before signing it. Take time to discuss it with people you trust. Keep in mind that you can leave and seek treatment outside of the clinical trial at any time.

### Will I get a placebo?

Placebos (inactive versions of real medicines) are almost never used alone in cancer clinical trials. It's common to receive either a placebo with a standard treatment, or a new drug with a standard treatment. You will be informed, verbally and in writing, if a placebo is part of a clinical trial before you enroll.

### Are clinical trials free?

There's no fee to enroll in a clinical trial. The study sponsor pays for research-related costs, including the study drug. But you may need to pay for other services, like transportation or childcare, due to extra appointments. During the trial, you'll continue to receive standard cancer care. This care is often covered by insurance.



**share with us.**

**Take our survey and help make the  
NCCN Guidelines for Patients  
better for everyone!**

[NCCN.org/patients/comments](https://www.nccn.org/patients/comments)

### Key points

- ▶ Systemic therapy is drug therapy that works throughout the body. It includes targeted therapy, chemotherapy, and steroids.
- ▶ Chronic myeloid leukemia (CML) is usually treated with targeted therapy. Targeted therapy focuses on specific or unique features of cancer cells.
- ▶ A tyrosine kinase inhibitor (TKI) is a type of targeted therapy that blocks the signals that cause certain cancers, such as CML, to grow and spread.
- ▶ A hematopoietic cell transplant (HCT) is a cancer treatment that replaces a person's bone marrow and immune system with donor cells to fight the leukemia.
- ▶ A clinical trial is a type of medical research study.

### Questions to ask

- ▶ What phase is my CML and how does the phase affect my treatment options?
- ▶ What can I expect from treatment?
- ▶ Are there resources to help me pay for treatment or other care I may need?
- ▶ Am I candidate for a clinical trial?
- ▶ Am I candidate for a hematopoietic cell transplant?

### What's next?

Now that you've read about the different types of treatment and what to expect, the next chapter talks about some of the common side effects of treatment and what might be done to manage those effects.

# 4

## Supportive care

- 32 What is supportive care?
- 32 Side effects
- 35 Late effects
- 35 Survivorship
- 36 Key points
- 36 Questions to ask

Supportive care helps manage the symptoms of cancer and the side effects of treatment. This chapter discusses possible side effects.

### What is supportive care?

Supportive care is an important part of cancer care. The goal is to improve your quality of life during and after cancer treatment. Supportive care is for everyone with cancer and their families, not just for those at the end of life. It's also known as palliative care.

Supportive care includes a wide range of services. Supportive care prevents or manages the symptoms of cancer and the side effects of cancer treatment, like pain and cancer-related fatigue. It also addresses the mental, social, emotional, and spiritual concerns faced by people with cancer.

Supportive care provides help with additional needs, such as:

- Making treatment decisions
- Coordinating your care
- Paying for care
- Planning for advanced care and end of life

Read more about the types of support you may receive in *NCCN Guidelines for Patients: Palliative Care*, available at [NCCN.org/patientguidelines](https://www.nccn.org/patientguidelines) and on the [NCCN Patient Guides for Cancer](#) app.



### Side effects

Cancer treatment can cause unwanted health issues called side effects. Some side effects may be unpleasant. Others may be harmful to one's health. Side effects depend on many factors. These factors include the drug type and dose, length of treatment, and the person.

Some potential side effects are described next. They are listed in alphabetical order and not in order of importance.

#### **Diarrhea or constipation**

Diarrhea is frequent and watery bowel movements. Your care team will tell you how to manage diarrhea. It's important to drink lots of fluids and make sure there's no infection causing the diarrhea.

Constipation is also common, especially if taking certain pain medicines, including narcotic pain medication and some anti-nausea medications. Constipation means having less frequent and more difficult bowel movements. Drinking fluids, staying active, and taking medicines for constipation are often recommended.

### Emotional distress

Depression, anxiety, and sleeping issues are common during a cancer diagnosis. Talk to your care team and those with whom you feel most comfortable about how you're feeling. There are services, people, and medicine that may be recommended to help relieve your distress.

### Fatigue

Fatigue is a state of physical or mental tiredness that can feel like lack of energy, motivation, or stamina. Fatigue may be caused by cancer, or it may be a side effect of treatment. Let your care team know how you are feeling and if fatigue is getting in the way of doing the things you enjoy. Eating a balanced diet, exercise, yoga, acupuncture, and massage therapy can help. It's important to try to stay active.

### Infection

Infections occur more frequently and are more severe in people with a weakened immune system. Drug treatment for CML can weaken the body's natural defense against infections. If not treated early, infections can be fatal.

Neutropenia, a low number of white blood cells, can lead to frequent or severe infections. When someone with neutropenia also develops a fever, it's called febrile neutropenia (FN). With FN, your risk of severe infection may be higher than normal. This is because a low number of white blood cells leads to a reduced ability to fight infections. FN is a side effect of some types of systemic therapy.

FN is considered a medical emergency requiring IV antibiotics to be started quickly. It's important to talk with your care team about

**Tell your care team about all side effects, including new or worsening symptoms, so they can be managed.**

how to prevent FN, what to look for, and who to contact in an emergency.

### Loss of appetite

The side effects from cancer or its treatment and the stress of having cancer might cause you to feel not hungry or sick to your stomach (nauseated). You might have a sore mouth or difficulty swallowing.

Healthy eating is important during treatment. It includes eating a balanced diet, eating the right amount of food, and drinking enough fluids. A registered dietitian who's an expert in nutrition and food can help.

### Low blood cell counts

Some cancer treatments can cause low blood cell counts.

- **Anemia** is a condition where your body doesn't have enough healthy red blood cells, resulting in less oxygen being carried to your cells. You might tire easily if you're anemic.
- **Neutropenia** is a decrease in neutrophils, a type of white blood cell. This puts you at risk for infection.
- **Thrombocytopenia** is a condition where there aren't enough platelets in the blood. This puts you at risk for bleeding.

### Nausea and vomiting

Nausea and vomiting are common side effects of treatment. You may be given medicine to prevent nausea and vomiting. Sometimes adjusting the amount of food you eat in one sitting can help.

### Neuropathy and neurotoxicity

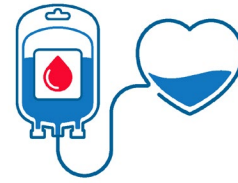
Some treatments can damage the nervous system (neurotoxicity), causing neuropathy and problems with concentration, memory, and thinking. Neuropathy is a nerve problem that causes pain, numbness, tingling, or muscle weakness in different parts of the body. It usually begins in the hands or feet and gets worse with additional cycles of treatment. Most of the time, neuropathy improves gradually and may eventually go away after treatment.

### Pain

Tell your care team about any pain or discomfort. You might meet with a palliative care specialist or with a pain specialist to manage pain.

### Transfusions

Blood transfusions may be needed during CML treatment. A transfusion is a slow infusion of blood products, such as red blood cells or platelets, into a vein. Over time, the body may begin to reject blood transfusions if your immune system develops antibodies against the blood cells from other donors.



### Transfusions

A transfusion is a common procedure to replace blood or blood components (red blood cells or platelets). It's given through an IV (intravenous) line, a tiny tube that's inserted into a vein with a small needle.

- The whole process can take about 1 to 4 hours, depending on how much blood is needed.
- Most transfusions use blood from a donor. Some people choose a family member or friend to donate blood.
- Blood transfusions are usually very safe. Donated blood is carefully tested, handled, and stored.
- Most people's bodies handle blood transfusions very well. But, like any medical procedure, there are some risks. Speak with your care team for specific information about your risks.
- Systemic therapy can affect how bone marrow makes new blood cells. Some people getting treatment for cancer might need a transfusion of red blood cells or platelets.

### Health insurance coverage

It's important to let your care team know about any changes to your insurance. Gaps in coverage can impact your CML treatment if the TKI can't be taken continuously. There are resources available to help you pay for treatment. Don't be afraid to ask your care team for help.

### Late effects

Late effects are side effects that occur months or years after a disease is diagnosed or treatment has ended. Late effects may be caused by cancer or its treatment. They may include physical, mental, and social health concerns and second cancers. The sooner late effects are treated the better. Ask your care team about what late effects could occur. This will help you know what to look for.

### Survivorship

A person is a cancer survivor from the time of diagnosis until the end of life. After treatment, your health will be monitored for side effects of treatment and the return of cancer. This is part of your survivorship care plan. It's important to keep any follow-up doctor visits and imaging test appointments. Seek good routine medical care, including regular doctor visits for preventive care and cancer screening.

A personalized survivorship care plan will contain a summary of possible long-term effects of treatment, called late effects, and list follow-up tests. Find out how your primary care provider will coordinate with specialists for your follow-up care.

As you age and the longer you are on a TKI, consider incorporating other team members, such as a cardio-oncologist who specializes in managing the side effects that CML and TKIs can have on the heart.

### Supportive care resources

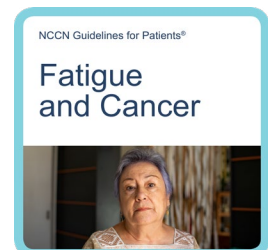
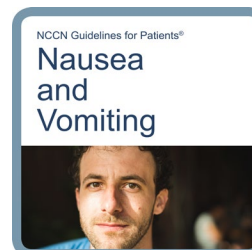
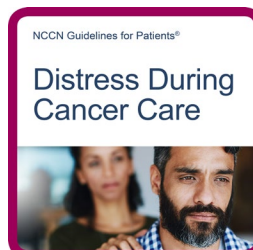
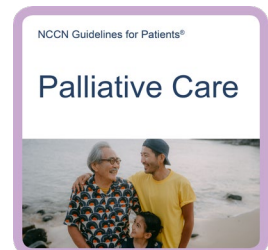
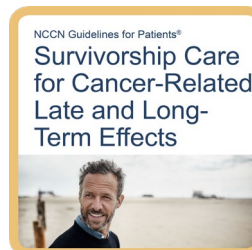
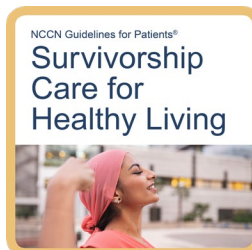
More information on supportive care is available at

[NCCN.org/patientguidelines](https://www.nccn.org/patientguidelines)

and on the

[NCCN Patient Guides for Cancer](#)

app.



### Key points

- Supportive care is health care that relieves symptoms caused by cancer or its treatment and improves quality of life. Supportive care is always given.
- All cancer treatments can cause unwanted health issues called side effects. Side effects depend on many factors. These factors include the drug type and dose, length of treatment, and the person.
- Some side effects are very rare. Ask your care team what to expect.
- Tell your care team about any new or worsening symptoms.
- A person is a cancer survivor from the time of diagnosis until the end of life. After treatment, your health will be monitored for side effects of treatment and the return of cancer.

### Questions to ask

- What side effects can I expect from treatment?
- How are these side effects treated?
- What should I do if I notice changes in my condition?
- What should I do if I notice side effects on weekends and other non-office hours?
- Will my care team be able to communicate with the emergency department or urgent care team?

### What's next?

The next chapter provides in-depth information on chronic phase CML, how it's treated, and treatment response milestones.



**Increasing evidence highlights that a good patient-doctor relationship and positive communication substantially influence patients' quality of life and treatment outcomes. Talk with your care team. Get to know them and help them get to know you."**

# 5

## Chronic phase CML

- 38 Overview
- 38 Risk groups
- 39 First treatment
- 40 Monitoring
- 40 Response milestones
- 43 Second treatment
- 44 Treatment-free remission
- 45 Key points
- 45 Questions to ask

## 5 Chronic phase CML

In chronic phase CML, there's an increased number of granulocytes in the blood, bone marrow, or both. The goal of treatment is to stop the activity of the *BCR::ABL1* protein and reduce the number of CML cells with the *BCR::ABL1* gene to less than 1 percent (1%) within 12 months, and ideally as close to zero as possible.

### Overview

CML is often diagnosed during the chronic phase of the disease. In this phase, there's an increased number of white blood cells called granulocytes in the blood, bone marrow, or both. Fewer than 15 out of every 100 blood cells are blasts (written as <15%) in chronic phase CML.

Chronic phase CML responds well to treatment. However, if left untreated, chronic phase CML can progress to accelerated phase CML or blast phase CML, which is more difficult to treat effectively.

Chronic phase CML is highly treatable. Treatment is a type of targeted therapy called a tyrosine kinase inhibitor (TKI). You can expect a near-normal to normal life expectancy if CML goes into remission and you continue to take medicine as prescribed.

Not everyone's disease responds to treatment in the same way.

When planning your treatment, your care team will consider these factors:

- Specific variations (called transcript type) within the *BCR::ABL1* gene
- The presence of mutations in other genes known to play a role in blood cancer development, such as *ASXL1*
- Your age and if you have any other serious health concerns, called comorbidities
- Side effects and toxicity of a TKI
- Possible drug interactions between a chosen TKI and any medicines, herbals, supplements, and over-the-counter drugs you're taking or foods you're eating
- Whether your insurance plan will cover a particular TKI (there are lower-cost generic TKIs available)
- Your goals for treatment and your preferences about treatment options

### Risk groups

In general, people in the same risk group are expected to respond to treatment in the same way. As a result, doctors often use risk groups to help plan treatment. Ask how your risk group will affect your treatment. See **Guide 2**.

## 5 Chronic phase CML

In CML, your risk score is calculated using your:

- Age
- Spleen size on physical exam
- Blood counts

Based on this information, you will receive one of the following:

- Sokal score
- Hasford (EURO) score
- EUTOS long-term survival (ELTS) score

This score places you into a risk group:

- Low risk
- Intermediate risk
- High risk

## First treatment

The first or main treatment given is called primary treatment. It's based on your risk score.

### Low risk

For low risk, the preferred treatment options are:

- Imatinib or generic imatinib (generic imatinib is the same in dosage, safety, strength, quality, and performance as imatinib)
- Second-generation TKI (bosutinib, dasatinib, or nilotinib) or generic second-generation TKI
- Asciminib
- Clinical trial

### Guide 2 Risk groups

<b>Low risk</b>	<ul style="list-style-type: none"><li>• Sokal score is less than 0.8</li><li>• Hasford (EURO) score is 780 or less</li><li>• EUTOS long-term survival (ELTS) score is 1.5680 or less</li></ul>
<b>Intermediate risk</b>	<ul style="list-style-type: none"><li>• Sokal score is between 0.8 and 1.2</li><li>• Hasford (EURO) score is between 781 and 1480</li><li>• ELTS score is between 1.5680 and 2.2185</li></ul>
<b>High risk</b>	<ul style="list-style-type: none"><li>• Sokal score is more than 1.2</li><li>• Hasford (EURO) score is more than 1480</li><li>• ELTS score is more than 2.2185</li></ul>

### Intermediate or high risk

For intermediate or high risk, the preferred treatment options are:

- Second-generation TKI (bosutinib, dasatinib, or nilotinib) or generic second-generation TKI
- Asciminib
- Clinical trial

Other recommended options include:

- Imatinib or generic imatinib
- Clinical trial

## Monitoring

A special test is used to see how your CML is responding to targeted therapy. You'll be monitored at certain points during treatment with a test called qPCR using the International Scale (IS). **A qPCR (IS) is the only test sensitive enough to detect very low levels of *BCR::ABL1*.**

### qPCR (IS) scores

The qPCR (IS) score uses a standard baseline of 100%. This is the starting point or value that your results are measured against. It's the average of what's observed in untreated individuals. Note that it's possible to have a value of greater than 100%.

Changes in qPCR (IS) scores are often described in terms of log changes. Log changes can be down (decrease) or up (increase). A 1-log increase means that the value has gone up at least 10 times from the lowest it's been. For example, an increase

of *BCR::ABL1* from 0.12% to 1.2% would be a 1-log increase. Any log increase during treatment is cause for concern and would prompt your oncologist to re-evaluate your CML and consider changing treatment.

## Response milestones

For CML, treatment results are discussed in terms of response milestones. The goal is to reach certain response milestones within a specific timeframe and maintain those milestones.

Some important milestones are as follows:

- **Early molecular response (EMR)** is defined as *BCR::ABL1* less than 10% at 3 months and 6 months. It's a sign of how well treatment will work in the long term. The next milestone is complete cytogenetic response by 12 months.
- **Complete cytogenetic response (CCyR)** is the absence of the Philadelphia chromosome (written as Ph-) and the *BCR::ABL1* level in the blood is 1% or less. This *BCR::ABL1* blood level is often used to establish that a CCyR has been achieved. A CCyR should be achieved within 12 months.
- **Major molecular response (MMR)** is defined as *BCR::ABL1* less than 0.1%. MMR can predict a deep molecular response.
- **Deep molecular response (DMR)** is when *BCR::ABL1* can only be detected by the most sensitive tests or can't be detected at all. In a DMR, *BCR::ABL1* (IS) is at 0.01% or less.

## 5 Chronic phase CML

For more detailed response definitions, see **Guide 3**.

Your care team will monitor how your CML is responding to TKI treatment. They will use the qPCR (IS) test to measure levels of *BCR::ABL1* in your blood at 3, 6, and 12 months. They are looking for specific treatment response milestones. These are described in **Guide 4**.

### Not meeting milestones

If treatment doesn't meet certain milestones, then it's possible your CML is resistant to the TKI you're taking.

If this is the case, you'll be asked if you:

- Missed or forgot to take any doses
- Are taking certain medicines, over-the-counter drugs, herbals, or supplements, eating certain foods, or if there were any changes to other medicines you might take for your heart, allergies, or digestion.

### Guide 3

#### Response types and definitions

<b>Complete hematologic (blood) response (CHR)</b>	<ul style="list-style-type: none"><li>• Blood counts are normal</li><li>• No immature cells, such as myelocytes, promyelocytes, or blasts, in blood</li><li>• No signs and symptoms of disease (spleen is normal size)</li></ul>
<b>Cytogenetic (Philadelphia chromosome) response</b>	<ul style="list-style-type: none"><li>• Complete cytogenetic response (CCyR): No Philadelphia chromosomes are found (Ph-) and <i>BCR::ABL1</i> (IS) is 1% or less</li><li>• Major cytogenetic response (MCyR): Ph+ are between 0% and 35%</li><li>• Partial cytogenetic response (PCyR): Ph+ are between 1% and 35%</li><li>• Minor cytogenetic response: Ph+ are between 36% and 65%</li></ul>
<b>Molecular (<i>BCR::ABL1</i>) response</b>	<ul style="list-style-type: none"><li>• Early molecular response (EMR): <i>BCR::ABL1</i> (IS) is 10% or less at 3 and 6 months</li><li>• Major molecular response (MMR): <i>BCR::ABL1</i> (IS) is 0.1% or less</li><li>• Deep molecular response (DMR): <i>BCR::ABL1</i> (IS) is 0.01% or less (MR4.0) or <i>BCR::ABL1</i> (IS) is 0.0032% or less (MR4.5)</li></ul>
<b>Relapse</b>	<ul style="list-style-type: none"><li>• Any sign of loss of response</li></ul>

**Guide 4**

**Early treatment milestones using *BCR::ABL1* (IS)**

<b>At 3 months</b>	Possible TKI resistance if <i>BCR::ABL1</i> (IS) is more than 10%.
	Milestone met if <i>BCR::ABL1</i> (IS) is between 10% and 1% (EMR).
	Milestone met if <i>BCR::ABL1</i> (IS) is between 1% and 0.1% (CCyR).
	Milestone met if <i>BCR::ABL1</i> (IS) is 0.1% or less (MMR).
<b>At 6 months</b>	TKI resistance if <i>BCR::ABL1</i> (IS) is more than 10%. Milestone not met.
	Milestone met if <i>BCR::ABL1</i> (IS) is between 10% and 1% (EMR).
	Milestone met if <i>BCR::ABL1</i> (IS) is between 1% and 0.1% (CCyR).
	Milestone met if <i>BCR::ABL1</i> (IS) is 0.1% or less (MMR).
<b>At 12 months</b>	TKI resistance if <i>BCR::ABL1</i> (IS) is more than 10%. Milestone not met.
	Possible TKI resistance if <i>BCR::ABL1</i> (IS) is between 10% and 1% (EMR).
	<i>BCR::ABL1</i> (IS) is between 1% and 0.1% (CCyR), then milestone met if goal is long-term survival. Milestone not met if goal is treatment-free remission.
	Milestone met if <i>BCR::ABL1</i> (IS) is 0.1% or less (MMR).

- Red shows milestone not met. TKI resistance.
- Yellow shows area of concern and possible TKI resistance.
- Light green milestone is based on the treatment goal.
- Green shows milestone goal met.
- Orange shows area of concern and possible TKI resistance.

## 5 Chronic phase CML

It's very important to tell your care team about any teas you drink, like green tea, and any supplements you take, such as turmeric. It might be one reason your treatment isn't meeting certain milestones. Another reason might be that your CML has a new drug-resistant mutation. Your care team will consider this and order any mutation or biomarker testing as needed.

### Second treatment

Second treatment options are based on qPCR (IS) results and if primary treatment milestones were met. Response milestones are measured as the percentage of cells with *BCR::ABL1* using qPCR (IS). The goal is to reduce the

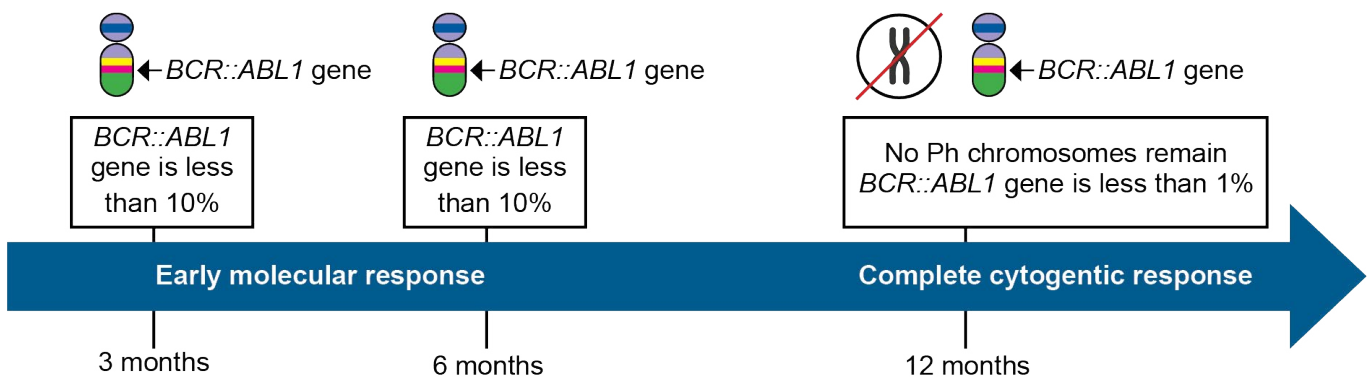
number of CML cells with *BCR::ABL1* to less than 1% within 12 months.

#### Milestone not met

If the *BCR::ABL1* (IS) level is more than 10% after 6 months or more than 1% after 12 months, it means that treatment milestones were not met or maintained. If you've been taking your medicine regularly, the next option is to switch to another TKI (not including imatinib) and consider mutation testing. If a hematopoietic cell transplant (HCT) is an option in the future, you might talk with a transplant expert.

### Chronic phase CML treatment response goals

Tests at 3, 6, and 12 months will measure levels of the *BCR::ABL1* gene in your blood. The goal is for *BCR::ABL1* to be less than 10% at 3 months and 6 months, called an early molecular response. It's a sign of how well treatment will work in the long term. The next milestone is complete cytogenetic response by 12 months. In a complete cytogenetic response, no Philadelphia (Ph) chromosomes remain and *BCR::ABL1* is 1% or less.



### Possible TKI resistance

You might have TKI resistance if the number of *BCR::ABL1* (IS) cells is:

- More than 10% after 3 to 6 months
- More than 1% after 12 months

You might have additional biomarker and mutation testing before continuing treatment. Since a treatment response can still occur with more time, you might remain on the same TKI or switch to a different TKI.

### Milestone might have been reached

For those with *BCR::ABL1* (IS) between 0.1% and 1% at 12 months, if the treatment goal is:

- Long-term survival, then the milestone is met and you will continue with the same TKI.

- Treatment-free remission (TFR), then the milestone isn't met. You might switch to a different TKI, be referred to a center that specializes in CML, or be recommended for a clinical trial.

## Treatment-free remission

For some, it may be possible to discontinue or stop TKI therapy if certain guidelines (criteria) have been met. This is called treatment-free remission. Your care team should consult with a CML specialist and review with you in detail the potential risks and benefits. You will need to agree (consent) to stop therapy and be aware of the TKI withdrawal side effects and the very low risk of developing treatment-resistant disease in the future.

**“Leukemia impacts all bodies—physical, emotional, mental and spiritual. It takes support in all areas to get through the journey. Attitude and perspective are areas you can work on to impact your own healing.”**



**It's very important to take all medicine exactly as prescribed and not miss or skip doses.**

Frequent monitoring is needed for those in remission who have stopped taking TKI therapy. You will need to have blood tests more often. This is to make sure that your *BCR::ABL1* level is closely monitored. If the *BCR::ABL1* level increases above 0.1%, you will need to re-start treatment. There's a chance that your cancer might return (relapse) if you stop taking the targeted therapy. Ask your care team about the risks.

### Milestone met

If milestones have been reached, you'll stay on your TKI unless you met criteria to consider stopping treatment. It's very important not to stop your medication without your doctor's advice or skip doses of your medicine. Missing doses allows the leukemia cells to grow and potentially develop TKI resistance. Monitoring will continue indefinitely.

### Key points

- In chronic phase chronic myeloid leukemia (CML), there's an increased number of white blood cells called granulocytes found in blood, bone marrow, or both. Also, there are fewer than 15 blasts out of every 100 blood cells.
- Chronic phase CML is highly treatable.

- Treatment for chronic phase CML is based partly on risk group. In general, people in the same risk group are expected to respond to treatment in the same way.
- Treatment results are discussed in terms of milestones. The goal is to reach and maintain certain treatment milestones within a specific timeframe.
- Two very important milestones are early molecular response at 3 months and 6 months and complete cytogenetic response by 12 months.
- The minimal goal of treatment is to reduce the number of CML cells with *BCR::ABL1* to less than 1% within 12 months.

### Questions to ask

- What is my risk group and how does it affect my treatment options?
- What are my options if treatment doesn't work as expected or doesn't reach certain milestones?
- What should I do if I miss a dose?
- How will treatment impact my day-to-day activities? Will I be able to work?
- How long will treatment last? Will I have to spend time in the hospital?

### What's next?

The next chapter provides in-depth information on advanced phase CML and how it's treated.

# 6

## Advanced phase CML

- 47 Overview
- 48 Testing
- 49 Treatment planning
- 49 Accelerated phase
- 50 Blast phase
- 51 After an HCT
- 52 Key points
- 52 Questions to ask

Accelerated phase and blast phase are known as advanced phase CML. These phases are defined by an increase in blasts, additional gene mutations, and leukemia that's growing and evolving. The goal of treatment is to prevent CML from progressing. A hematopoietic cell transplant (HCT) offers people with blast phase CML the best chance of long-term disease control.

### Overview

Treatment for advanced phase CML aims to prevent CML from progressing. Advanced phase CML includes accelerated and blast phases. In all phases, CML cells contain the Philadelphia (Ph) chromosome. However, in the accelerated and blast phases, there may be new abnormal changes within chromosomes.

- **In accelerated phase CML**, the blasts are myeloid.
- **In blast phase CML**, the blasts can be myeloid or lymphoid.

For definitions of advanced phase CML, see **Guide 5**.

#### Guide 5 Definitions of advanced phase CML

##### Accelerated phase

Any of the following:

- Blood myeloblasts are between 15% and 29%
- Blood myeloblasts and promyelocytes total 30% or more
- Blood basophils are 20% or more
- Platelet count is  $100 \times 10^9/L$  or less and not due to therapy
- Additional mutations are found in Ph+ cells

##### Blast phase

###### For myeloid blast phase

- 30% or more blasts are found in blood, bone marrow, or both
- Blast cells are found in tissues and organs outside of the blood or bone marrow

###### For lymphoid blast phase

- Any increase in lymphoblasts in blood or bone marrow

### Testing

Before treatment, you will have tests to confirm the advanced phase of CML—accelerated or blast phase. The phase is based on the number and type of blasts, if there are any new mutations, and if CML has spread to tissues and organs outside of the bone marrow or blood.

### Mutation testing

New mutations in the *BCR::ABL1* gene may occur over time. This can happen as CML progresses to advanced phases or it can happen during treatment for CML.

Mutation testing is used to look for these new mutations. Testing can be performed on blood or bone marrow. It should be done prior to starting treatment for advanced phase CML and for any convincing evidence of loss of response to treatment.

**Standard of care is the best-known way to treat a particular disease based on past clinical trials. There may be more than one treatment that's considered standard of care. Ask your care team what treatment options are available and if a clinical trial might be right for you.**



### Treatment planning

Factors such as your age, medical history, test results, any prior tyrosine kinase inhibitor (TKI) therapy, and treatment goals will be used for treatment planning. The goal of treatment is to stop CML from progressing any further.

Your care team will consider the following when planning treatment for advanced phase CML:

- Did your CML progress while being treated using TKI therapy?
- Did your CML progress while not being treated?
- Are you a candidate for a hematopoietic cell transplant (HCT)?
- Is there any leukemia in your central nervous system (CNS)?

- What mutations does your CML have?
- What TKIs did you take before? Did your CML not respond or was it resistant to certain TKIs?

Human leukocyte antigen (HLA) testing might be done if an HCT is planned.

### Accelerated phase

In accelerated phase CML, the percentage of myeloblasts is higher than normal. Platelet count might be low.

#### Treatment options

The treatment goal is to stop CML from progressing to blast phase. For long-term control, an allogeneic (donor) HCT is likely needed. For treatment options, see **Guide 6**.

#### Guide 6

#### Treatment options: Accelerated phase

##### Clinical trial

---

##### Preferred (listed in alphabetical order)

- Bosutinib
  - Dasatinib
  - Nilotinib
  - Ponatinib
- 

##### Used in certain cases

- Imatinib or generic Imatinib
  - Asciminib
-

### Blast phase

The blasts in blast phase CML can be myeloid (myeloblasts) or lymphoid (lymphoblasts). In blast phase CML, the percentage of blasts is higher than normal.

- **In myeloid blast phase**, at least 30 out of every 100 cells (30%) are blasts.
- **In lymphoid blast phase**, any number of abnormal lymphoblasts is concerning.

Blasts may be found in tissues and organs outside of the bone marrow or blood. A lumbar puncture might be done if CML is suspected in the fluid that surrounds the spine or brain.

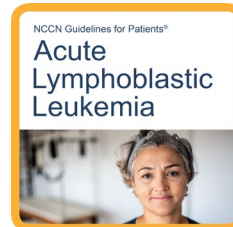
An allogeneic (donor) HCT or intensive chemotherapy would follow initial TKI treatment for blast phase CML.

### Lymphoid blast phase treatment options

Options for lymphoid blast phase include:

- Clinical trial
- Acute lymphoblastic leukemia (ALL)-type induction chemotherapy with a TKI (preferred)
- TKI with steroids (used in certain cases)

For more information on ALL-type induction therapies, see *NCCN Guidelines for Patients: Acute Lymphoblastic Leukemia* at [NCCN.org/patientguidelines](https://www.nccn.org/patientguidelines) and on the [NCCN Patient Guides for Cancer](https://www.nccn.org/patientguides) app.

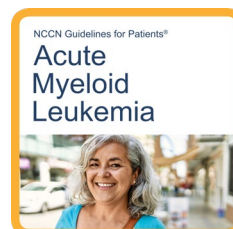


### Myeloid blast phase treatment options

Options for myeloid blast phase include:

- Clinical trial
- Acute myeloid leukemia (AML)-type induction chemotherapy with a TKI (preferred)
- TKI alone (used in certain cases)

For more information on AML-type induction therapies, see *NCCN Guidelines for Patients: Acute Myeloid Leukemia* at [NCCN.org/patientguidelines](https://www.nccn.org/patientguidelines) and on the [NCCN Patient Guides for Cancer](https://www.nccn.org/patientguides) app.



**In a complete molecular response, *BCR::ABL1* is as close to zero as possible.**

### After an HCT

A hematopoietic cell transplant (HCT) is used to prevent CML from progressing. It's a treatment given to cure CML. However, this doesn't always happen. An allogeneic HCT uses healthy blood (hematopoietic) stem cells from a donor who may or may not be related to you. HCTs are performed at specialized treatment centers.

How your body responds to an HCT is based on your age, if you have other serious health issues (comorbidities), donor type, and transplant center. You will have qPCR (IS) after an HCT to see if any cells with the Philadelphia (Ph) chromosome or *BCR::ABL1* gene remain.

HCT is sometimes done for long-term disease control for people whose CML progressed to blast phase and then achieved remission with chemotherapy.

### Complete molecular response

Following an HCT, you'll be monitored with qPCR. This is done using a sample of your blood. qPCR will be done every 3 months for 2 years, then every 3 to 6 months. If qPCR is negative (no signs of CML), then you will continue to be monitored. You might have to take TKI therapy for at least 1 year after transplant if you had accelerated phase CML or blast phase CML before, or your PCR test remains positive.

### Not in complete molecular response or in relapse

If Ph chromosomes or *BCR::ABL1* genes remain after the HCT, or CML has returned, then treatment options include:

- TKI alone
- TKI with donor lymphocyte infusion
- Clinical trial

In a donor lymphocyte infusion, you will receive white blood cells from the same person who donated blood-forming cells for the HCT. Treatment options are based on the type(s) of TKI you had before, your current health, *BCR::ABL1* mutations, and other factors. Your wishes are also important.

### Key points

- Accelerated phase and blast phase are known as advanced phase chronic myeloid leukemia (CML). These phases are defined by an increase in blasts, additional gene mutations, and leukemia that's growing and evolving.
- In all phases, CML cells contain the Philadelphia (Ph) chromosome. However, in the accelerated and blast phase, there may be new abnormal changes within chromosomes (gene mutations).
- TKIs are often used to treat advanced phase CML. Chemotherapy or steroids may be added if in blast phase. For long-term control, an allogeneic (donor) hematopoietic cell transplant (HCT) is needed.
- You'll be monitored after an HCT. If CML returns, then you'll begin treatment.

### Questions to ask

- Which treatment do you recommend and why?
- How long will I be on this treatment?
- Does the treatment order matter?
- What side effects can I expect from this treatment?
- Am I candidate for an allogeneic HCT or a clinical trial?

### What's next?

The next chapter provides information about resources and where you can get support.

# 7

## Other resources

54 What else to know

54 What else to do

54 Where to get help

55 Questions to ask

Want to learn more? Here's how you can get additional help.

### What else to know

This guide helps you know your options so you can make informed decisions and improve your cancer care. But it's not the only resource that you have.

Ask for as much information and help as you need. Many people are interested in learning more about:

- Finding an oncologist who's an expert in CML
- Making treatment decisions
- The details of treatment and possible side effects
- Getting financial help
- Coping with cancer treatment and other health challenges

### What else to do

Your health care center can help you with next steps. It often has on-site resources to help meet your needs and find answers to your questions. Health care centers can also inform you of resources in your community.

In addition to help from your providers, the resources listed in the next section provide support for many people like yourself.

### Where to get help

Look through the list below and visit the provided websites to learn more about these organizations.

#### **AnCan Foundation**

[ancan.org](http://ancan.org)

#### **Blood Cancer United**

[bloodcancerunited.org/patientsupport](http://bloodcancerunited.org/patientsupport)

#### **BMT InfoNet (Blood & Marrow Transplant Information Network)**

[bmtinfolnet.org](http://bmtinfolnet.org)

#### **CancerCare**

[cancercares.org](http://cancercares.org)

#### **Cancer Hope Network**

[cancerhopenetwork.org](http://cancerhopenetwork.org)

#### **CML Buster Foundation**

[Cmlbf.org](http://Cmlbf.org)

#### **GRACE**

[cancergrace.org](http://cancergrace.org)

#### **Imerman Angels**

[imermanangels.org](http://imermanangels.org)

#### **Leukemia Research Foundation**

[leukemiarf.org](http://leukemiarf.org)

#### **National Bone Marrow Transplant Link (nbmtLINK)**

[nbmtlink.org](http://nbmtlink.org)

### NMDP

[nmdp.org](http://nmdp.org)

### Stupid Cancer

[stupidcancer.org](http://stupidcancer.org)

### TargetCancer Foundation

[targetcancer.org](http://targetcancer.org)

### Triage Cancer

[triagecancer.org](http://triagecancer.org)

## Questions to ask

- Who can I talk to about help with housing, food, and other basic needs?
- What help is available for transportation, childcare, and home care?
- What other services are available to me and my caregivers?
- How can I connect with others and build a support system?
- Who can I talk to if I don't feel safe at home, at work, or in my neighborhood?

“

You'll get through this! There are many resources and survivors that are available, just reach out when you're ready. I wasn't a group person, but I found the conversations, either as a group or one-on-one extremely beneficial.”



**Let us know what  
you think!**

**Please take a moment to  
complete an online survey about  
the NCCN Guidelines for Patients.**

**[NCCN.org/patients/response](http://NCCN.org/patients/response)**



## Words to know

### **accelerated phase CML (AP-CML)**

The second phase of chronic myeloid leukemia progression, when the number of blast cells is increased.

### **acute lymphoblastic leukemia (ALL)**

A fast-growing cancer that causes too many immature white blood cells called lymphoblasts to be made.

### **acute myeloid leukemia (AML)**

A fast-growing cancer that causes too many immature white blood cells called myeloblasts to be made.

### **advanced phase CML**

Chronic myeloid leukemia that has progressed from the chronic phase. The number of immature blood cells (blast cells) is high, and it is causing symptoms. Advanced phase includes accelerated and blast phases.

### **allogeneic hematopoietic cell transplant (HCT)**

A treatment in which the patient receives healthy, immature blood-forming cells from another person to replace damaged or diseased cells in the bone marrow. Also called allogeneic stem cell transplant (SCT).

### ***BCR::ABL1* gene**

An abnormal gene that's formed when the *BCR* gene and *ABL1* gene join on the Philadelphia chromosome. Also called *BCR::ABL1* fusion gene.

### ***BCR::ABL1* protein**

An abnormal protein that's made by the *BCR::ABL1* fusion gene and causes too many abnormal white blood cells to be made.

### **blast cell**

An immature white blood cell. Can be myeloid or lymphoid.

### **blast phase CML (BP-CML)**

The final phase of chronic myeloid leukemia, which has the highest number of blast cells in the blood and bone marrow and can be life-threatening. Blasts can be myeloid or lymphoid. Also called blast crisis.

### **blood stem cell**

An immature blood-forming cell from which all other types of blood cells are made. Also called hematopoietic stem cell.

### **bone marrow**

The soft, sponge-like tissue in the center of most bones where blood cells are made.

### **bone marrow aspirate**

The removal of a small amount of liquid bone marrow to test for disease.

### **bone marrow biopsy**

The removal of a small amount of solid bone and bone marrow to test for disease.

### **chemotherapy**

Drugs that kill fast-dividing cells, including cancer cells and normal cells.

### **chromosomes**

Long strands that contain bundles of coded instructions (genes) in cells for making and controlling cells.

### **chronic myeloid leukemia (CML)**

A slow-growing cancer that starts in the bone marrow and causes too many granulocytes to form.

### **chronic phase CML (CP-CML)**

The first phase of chronic myeloid leukemia, when the number of white blood cells is higher than normal but may not cause symptoms.

### **complete cytogenetic response (CCyR)**

When tests don't find any copies of the Philadelphia chromosome.

### **cytogenetics**

The study of chromosomes.

### **deep molecular response (DMR)**

No copies of the abnormal *BCR::ABL1* gene or copies are detected at a very low level using a very sensitive test.

### **donor lymphocyte infusion (DLI)**

Procedure in which a person receives white blood cells from the same person who donated blood-forming cells for the hematopoietic cell transplant.

### **early molecular response (EMR)**

When *BCR::ABL1* is less than 10 percent (10%) at 3 months and 6 months.

### **flow cytometry**

A test that looks at certain substances on the surface of cells to identify the type of cells present.

### **fluorescence in situ hybridization (FISH)**

A lab test that uses special dyes to look for abnormal changes in a cell's genes.

### **fusion gene**

A gene that's made when parts of 2 separate genes join.

### **gene**

A set of coded instructions in cells for making new cells and controlling how cells behave.

### **graft-versus-host disease (GVHD)**

A disease that occurs when transplanted blood stem cells attack a patient's normal cells.

### **granulocyte**

A type of white blood cell that has small particles (granules).

### **hematologist**

A doctor who's an expert in blood diseases.

### **hematopathologist**

A doctor who specializes in blood diseases by looking at cells under a microscope.

### **hematopoietic cell**

An immature blood-forming cell from which all other types of blood cells are made. Also called blood stem cell.

### **hematopoietic cell transplant (HCT)**

A treatment that replaces damaged or diseased cells in the bone marrow with healthy blood-forming cells. Also called stem cell transplant (SCT) or bone marrow transplant (BMT).

### **human leukocyte antigen (HLA)**

Special proteins on the surface of cells that help the body to tell its own cells apart from foreign cells.

### **immune system**

The body's natural defense against infection and disease.

### **International Scale (IS)**

A standardized scale for measuring and reporting results of a very sensitive test that measures the number of cells that have the *BCR::ABL1* gene. Lower numbers mean less evidence of leukemia.

### **log increase or decrease**

An increase or decrease in the number of cells that have the *BCR::ABL1* gene.

### **lymphoid**

Referring to a type of white blood cell called a lymphocyte.

**major molecular response (MMR)**

An improvement related to treatment, when tests detect a 3-log reduction in *BCR::ABL1* levels. It means that there are 1,000 times fewer cells with the *BCR::ABL1* gene than the standardized baseline level.

**molecular response**

An improvement related to treatment, when tests detect a decrease in the number of cells that have the *BCR::ABL1* gene.

**mutation testing**

A test that looks for abnormal changes in genes (the coded instructions in cells for making and controlling cells).

**myeloid**

Referring to a type of white blood cell called a granulocyte.

**pathologist**

A doctor who's an expert in testing cells and tissue to find disease.

**Philadelphia (Ph) chromosome**

An abnormal, short chromosome 22 that's formed when parts of chromosomes 9 and 22 switch with each other. It contains the *BCR::ABL1* gene.

**primary treatment**

The first, or main, treatment given to treat a disease.

**prognosis**

The likely or expected course and outcome of a disease.

**quantitative reverse transcriptase polymerase chain reaction (qPCR)**

A very sensitive test that measures the number of cells in the blood or bone marrow that have the *BCR::ABL1* gene.

**relapse**

The return or worsening of cancer after a period of improvement.

**remission**

When there are minor or no signs of a disease.

**resistance**

When cancer does not respond to a drug treatment.

**supportive care**

Treatment for the symptoms or health conditions caused by cancer or cancer treatment.

**targeted therapy**

Treatment with drugs that target a specific or unique feature of cancer cells.

**transfusion**

Replacing lost blood with new blood.

**translocation**

When pieces of 2 chromosomes break off and switch places with each other.

**treatment-free remission (TFR)**

The ability to maintain a deep molecular response after stopping therapy.

**treatment response**

An outcome or improvement in disease that's caused by treatment.

**tyrosine kinase inhibitor (TKI)**

A type of targeted drug that attaches to the *BCR::ABL1* protein so that it can't send growth signals.

# NCCN Contributors

This patient guide is based on the NCCN Clinical Practice Guidelines in Oncology (NCCN Guidelines®) for Chronic Myeloid Leukemia, Version 1.2026. It was adapted, reviewed, and published with help from the following people:

Dorothy A. Shead, MS  
Senior Director  
Patient Information Operations

Tanya Fischer, MEd, MSLIS  
Senior Medical Writer

Lisa Diehl  
Production Layout Artist

The NCCN Clinical Practice Guidelines in Oncology (NCCN Guidelines®) for Chronic Myeloid Leukemia, Version 1.2026 were developed by the following NCCN Panel Members:

\*Neil P. Shah, MD, PhD/Chair  
UCSF Helen Diller Family  
Comprehensive Cancer Center

\*Ximena Jordan Bruno, MD  
Abramson Cancer Center  
at the University of Pennsylvania

Moshe Talpaz, MD  
University of Michigan Rogel Cancer Center

Ravi Bhatia, MD/Vice-Chair  
O'Neal Comprehensive  
Cancer Center at UAB

Lori Maness, MD  
Fred and Pamela Buffett Cancer Center

Tiffany N. Tanaka, MD  
UC San Diego Moores Cancer Center

Jessica K. Altman, MD  
Robert H. Lurie Comprehensive Cancer  
Center of Northwestern University

Leland Metheny, MD  
Case Comprehensive Cancer Center/  
University Hospitals Seidman Cancer Center  
and Cleveland Clinic Taussig Cancer Institute

Srinivas Tantravahi, MBBS  
Huntsman Cancer Institute  
at the University of Utah

Maria Amaya, MD, PhD  
University of Colorado Cancer Center

Sanjay Mohan, MD, MSCI  
Vanderbilt-Ingram Cancer Center

James Thompson, MD, MS  
Roswell Park Comprehensive Cancer Center

Kebede H. Begna, MD  
Mayo Clinic Comprehensive Cancer Center

Javid J. Moslehi, MD  
UCSF Helen Diller Family  
Comprehensive Cancer Center

\*Steven Tsai, MD, PhD  
UCLA Jonsson  
Comprehensive Cancer Center

\*Ellin Berman, MD  
Memorial Sloan Kettering Cancer Center

\*Vivian Oehler, MD  
Fred Hutchinson Cancer Center

Jennifer Vaughn, MD, MSPH  
The Ohio State University Comprehensive  
Cancer Center - James Cancer Hospital  
and Solove Research Institute

Onyee Chan, MD  
Moffitt Cancer Center

\*Iskra Pusic, MD, MSCI  
Siteman Cancer Center at Barnes-  
Jewish Hospital and Washington  
University School of Medicine

Jeanna Welborn, MD  
UC Davis Comprehensive Cancer Center

\*Joan Clements  
CML Buster Foundation

Lindsay Rein, MD  
Duke Cancer Institute

\*David T. Yang, MD  
University of Wisconsin  
Carbone Cancer Center

Robert H. Collins, Jr., MD  
UT Southwestern Simmons  
Comprehensive Cancer Center

Michal G. Rose, MD  
Yale Cancer Center/Smilow Cancer Hospital

## NCCN

Peter T. Curtin, MD  
City of Hope National Medical Center

Koji Sasaki, MD, PhD  
The University of Texas  
MD Anderson Cancer Center

Kristina Gregory, RN, MSN  
Senior Vice President,  
Clinical Information Programs

Magdalena B. Czader, MD, PhD  
Indiana University Melvin and Bren Simon  
Comprehensive Cancer Center

William Shomali, MD  
Stanford Cancer Institute

Hema Sundar, PhD  
Senior Manager, Global Clinical Content

\*Daniel J. DeAngelo, MD, PhD  
Dana-Farber/Brigham and  
Women's Cancer Center

B. Douglas Smith, MD  
Johns Hopkins Kimmel Cancer Center

Michael Drazer, MD, PhD  
The UChicago Medicine  
Comprehensive Cancer Center

\*Michael Styler, MD  
Fox Chase Cancer Center

\* Reviewed this patient guide. For disclosures, visit [NCCN.org/disclosures](https://www.nccn.org/disclosures).

# NCCN Cancer Centers

For contact information visit [NCCN.org/cancercenters](https://www.nccn.org/cancercenters).

Abramson Cancer Center  
at the University of Pennsylvania  
*Philadelphia, Pennsylvania*

Case Comprehensive Cancer Center/  
University Hospitals Seidman Cancer Center and  
Cleveland Clinic Taussig Cancer Institute  
*Cleveland, Ohio*

City of Hope National Medical Center  
*Duarte, California*

Dana-Farber/Brigham and Women's Cancer Center |  
Mass General Cancer Center  
*Boston, Massachusetts*

Duke Cancer Institute  
*Durham, North Carolina*

Fox Chase Cancer Center  
*Philadelphia, Pennsylvania*

Fred & Pamela Buffett Cancer Center  
*Omaha, Nebraska*

Fred Hutchinson Cancer Center  
*Seattle, Washington*

Huntsman Cancer Institute at the University of Utah  
*Salt Lake City, Utah*

Indiana University Melvin and Bren Simon  
Comprehensive Cancer Center  
*Indianapolis, Indiana*

Johns Hopkins Kimmel Cancer Center  
*Baltimore, Maryland*

Mayo Clinic Comprehensive Cancer Center  
*Phoenix/Scottsdale, Arizona*  
*Jacksonville, Florida*  
*Rochester, Minnesota*

Memorial Sloan Kettering Cancer Center  
*New York, New York*

Moffitt Cancer Center  
*Tampa, Florida*

O'Neal Comprehensive Cancer Center at UAB  
*Birmingham, Alabama*

Robert H. Lurie Comprehensive Cancer Center  
of Northwestern University  
*Chicago, Illinois*

Roswell Park Comprehensive Cancer Center  
*Buffalo, New York*

Siteman Cancer Center at Barnes-Jewish Hospital  
and Washington University School of Medicine  
*St. Louis, Missouri*

St. Jude Children's Research Hospital/  
The University of Tennessee Health Science Center  
*Memphis, Tennessee*

Stanford Cancer Institute  
*Stanford, California*

The Ohio State University Comprehensive Cancer Center -  
James Cancer Hospital and Solove Research Institute  
*Columbus, Ohio*

The UChicago Medicine Comprehensive Cancer Center  
*Chicago, Illinois*

The University of Texas MD Anderson Cancer Center  
*Houston, Texas*

UC Davis Comprehensive Cancer Center  
*Sacramento, California*

UC San Diego Moores Cancer Center  
*La Jolla, California*

UCLA Jonsson Comprehensive Cancer Center  
*Los Angeles, California*

UCSF Helen Diller Family Comprehensive Cancer Center  
*San Francisco, California*

University of Colorado Cancer Center  
*Aurora, Colorado*

University of Michigan Rogel Cancer Center  
*Ann Arbor, Michigan*

University of Wisconsin Carbone Cancer Center  
*Madison, Wisconsin*

UT Southwestern Simmons  
Comprehensive Cancer Center  
*Dallas, Texas*

Vanderbilt-Ingram Cancer Center  
*Nashville, Tennessee*

Yale Cancer Center/Smilow Cancer Hospital  
*New Haven, Connecticut*

# Index

- accelerated phase** 21, 47, 49
- BCR::ABL1* gene** 5, 8, 16–17, 23–24, 26, 51
- BCR::ABL1* protein** 5, 8, 22, 38
- biomarker tests** 14–17
- blast** 8, 50
- blast phase** 21–23, 47, 50
- bone marrow aspirate and biopsy** 14
- breastfeeding** 13, 26
- chemotherapy** 23, 50
- chronic phase** 21, 38–45
- clinical trials** 28–29
- complete cytogenetic response (CCyR)** 40–42
- deep molecular response (DMR)** 40–42
- early molecular response (EMR)** 40–42
- fertility** 13
- flow cytometry** 14–15
- fluorescence in situ hybridization (FISH)** 16
- genetic tests** 14–17
- heart tests** 17
- hematopoietic cell transplant (HCT)** 26–27, 51
- human leukocyte antigen (HLA) typing** 12
- International Scale (IS)** 17, 40
- karyotype** 16–17
- log increase or decrease** 40
- lymphoblast** 8, 22, 50
- major molecular response (MMR)** 40–42
- monitoring** 17, 40, 45
- mutations and mutation testing** 15, 48
- myeloblast** 8, 22, 50
- performance status** 11
- Philadelphia (Ph) chromosome** 5–6
- pregnancy** 13, 26
- quantitative reverse transcriptase polymerase chain reaction (qPCR)** 17, 40
- response types** 41
- response milestones** 40–45
- risk groups** 38–40
- side effects** 32–35
- steroids** 23
- targeted therapy** 23–26
- translocation** 16
- treatment-free remission** 44–45
- treatment milestones** 40–45
- types of response** 41
- tyrosine kinase inhibitor (TKI)** 23–26



# NCCN Guidelines for Patients<sup>®</sup>

Cancer care recommendations from leading experts at the  
National Comprehensive Cancer Network<sup>®</sup> (NCCN<sup>®</sup>)

# Chronic Myeloid Leukemia

To support the NCCN Guidelines for Patients, visit

[NCCNFoundation.org/Donate](https://www.nccn.org/donate)

**NCCN**

National Comprehensive  
Cancer Network<sup>®</sup>

3025 Chemical Road, Suite 100  
Plymouth Meeting, PA 19462  
215.690.0300

[NCCN.org/patients – For Patients](https://www.nccn.org/patients) | [NCCN.org – For Clinicians](https://www.nccn.org)

PAT-N-1934-0526