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# Myeloproliferative Neoplasms

CLASSIC TYPES

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**It's easy to  
get lost in the  
cancer world**



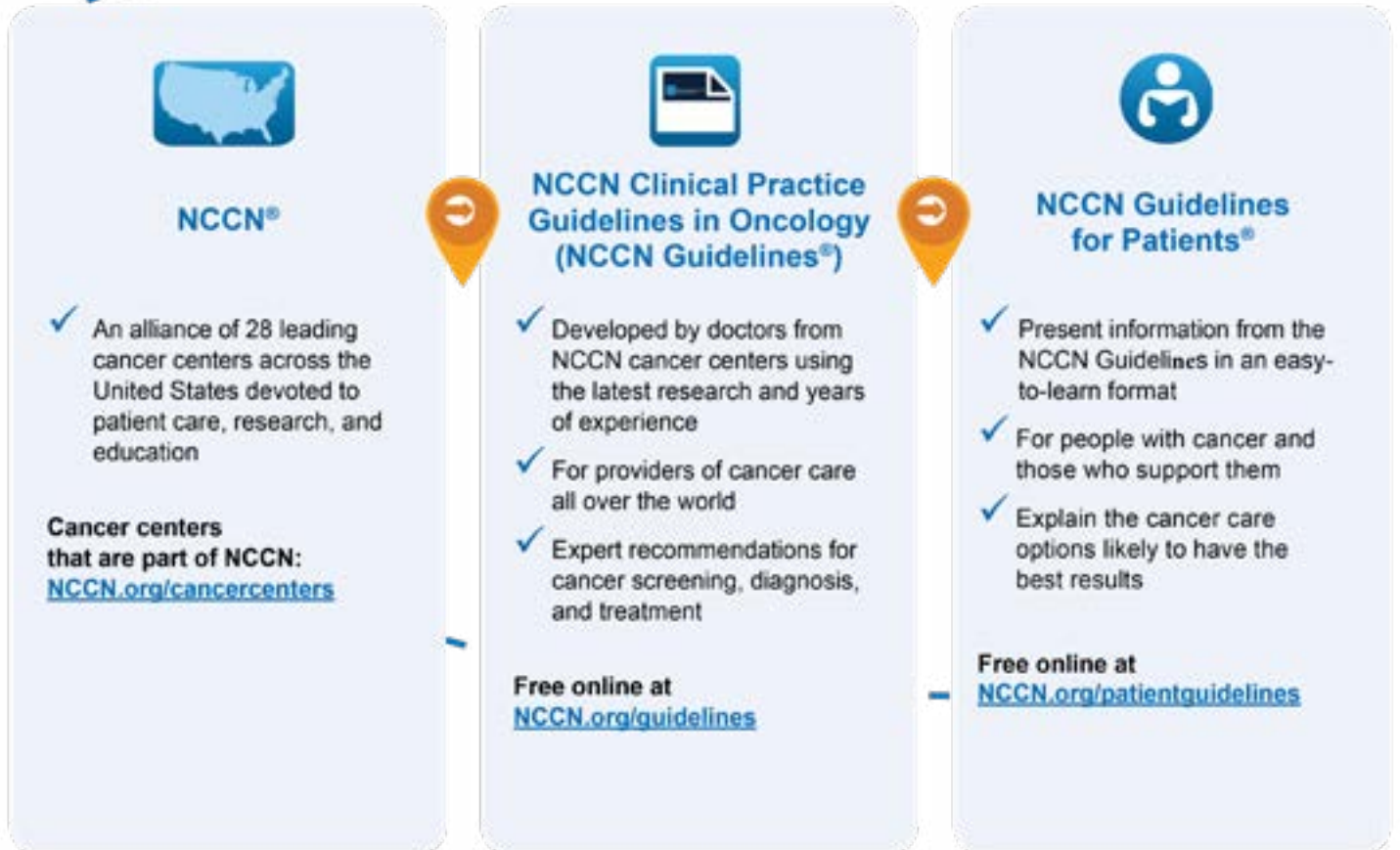
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- ✓ Step-by-step guides to the cancer care options likely to have the best results
- ✓ Based on treatment guidelines used by health care providers worldwide
- ✓ Designed to help you discuss cancer treatment with your doctors

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3025 Chemical Road, Suite 100  
Plymouth Meeting, PA 19462  
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### **MPN Research Foundation**

The NCCN Guidelines for myelofibrosis provide clarity for physicians and patients about their options and what to expect upon diagnosis with PV, ET or MF. These are long awaited by the patient community and we are so glad to see it come to fruition. [mpnresearchfoundation.org](http://mpnresearchfoundation.org)

## Endorsed by

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LLS is dedicated to developing better outcomes for blood cancer patients through research, education and patient services and is happy to have this comprehensive resource available to patients. [LLS.org/PatientSupport](http://LLS.org/PatientSupport)

### **MPN Cancer Connection**

MPN Cancer Connection (MPN-CC) recognizes MPN patients are in fact “cancer patients” and should have full access to programs, benefits and resources available in your area. MPN-CC is pleased to support the comprehensive resource provided by the NCCN Patient Guidelines for Myeloproliferative Neoplasms (MPNs). [MPNCancerConnection.org](http://MPNCancerConnection.org)

### **MPN Education Foundation**

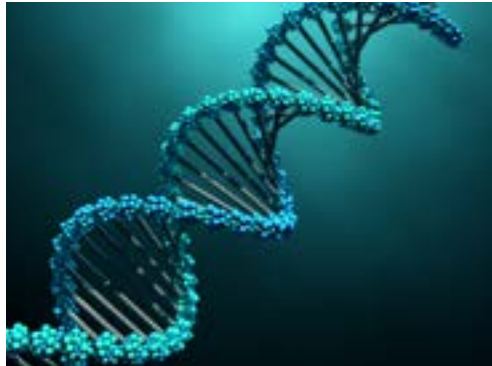
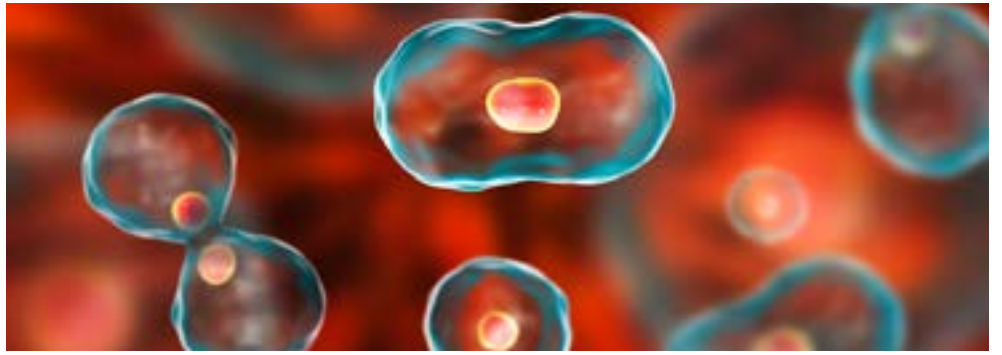
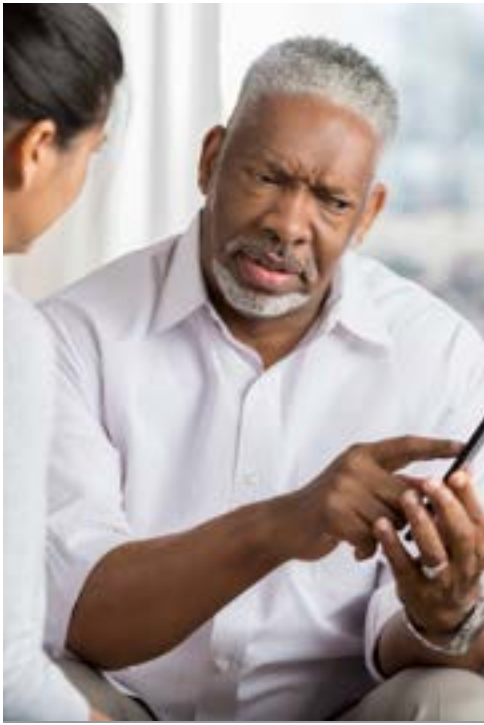
Educating physicians and patients about evidence-based diagnostic algorithms and treatment for the myeloproliferative neoplasms will benefit both, and will improve patient access to those treatments. The MPN Education Foundation is pleased to endorse this landmark effect on behalf of those we serve. [mpninfo.org](http://mpninfo.org)

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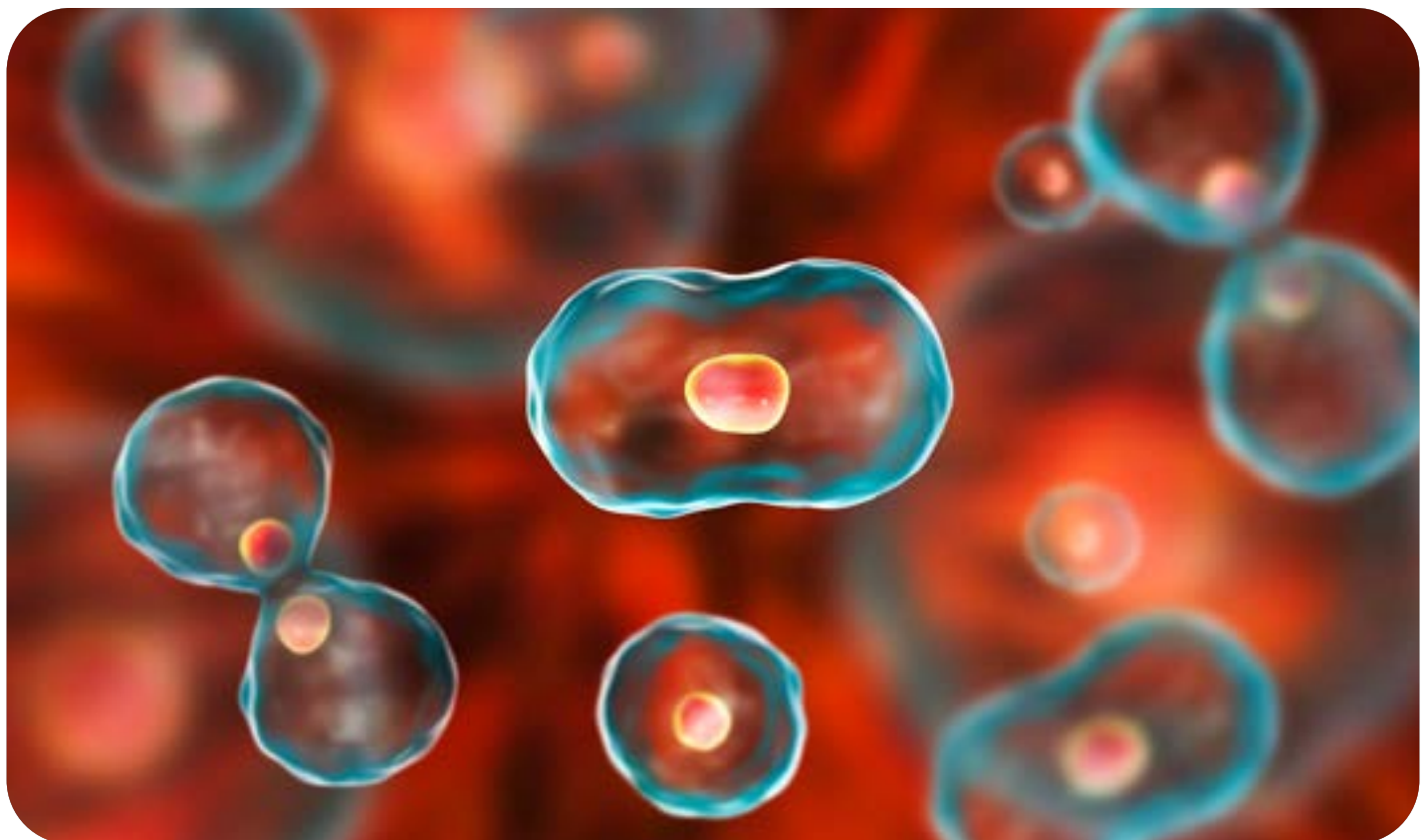
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# 1

## MPN basics

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Myeloproliferative neoplasms (MPNs) are a type of blood cancer. This chapter reviews some basics about MPNs, such as the 3 classic types.

## Blood

To learn about blood cancers, you first must know about blood. Blood is one of the fluids in the body. It consists of blood cells that move within plasma. Plasma is mostly water.

### Blood cells

As shown in **Figure 1**, there are 3 main types of blood cells:

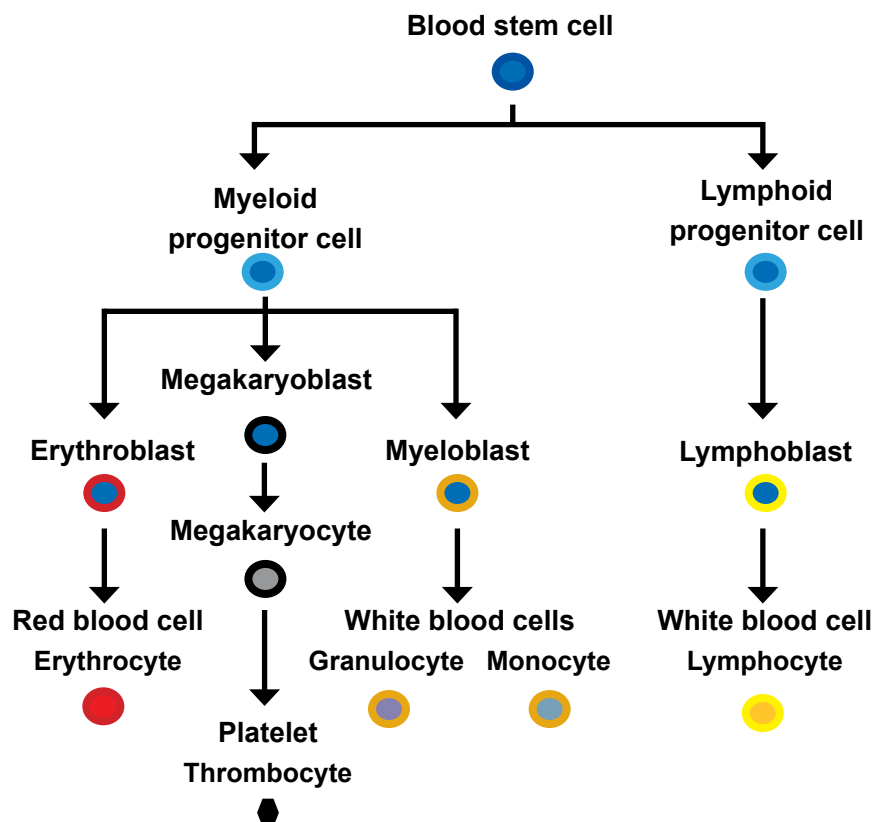
- Red blood cells (also called erythrocytes)
- White blood cells (leukocytes), which include granulocytes, monocytes, and lymphocytes
- Platelets (thrombocytes)

Blood cells have important jobs. Red blood cells carry oxygen throughout the body. White blood cells help fight germs. Platelets help control bleeding.

**Figure 1**  
**Blood cells**

There are three main types of blood cells. These types are red blood cells, white blood cells, and platelets. Blood stem cells are the cells from which all blood cells are formed. They go through a series of changes to become mature blood cells.

Illustration Copyright © 2020 National Comprehensive Cancer Network® (NCCN®).



Your blood cells don't live forever. Many have a short lifespan. Blood cells are being replaced in your body all the time.

### How blood cells form

Most blood cells are formed in bone marrow. Bone marrow is the sponge-like tissue in the center of most bones.

Your bone marrow contains blood-forming cells. These cells are called blood stem cells or hematopoietic stem cells. They are the cells from which all blood cells are formed.

Blood stem cells can make exact copies of themselves. They can also make new cells that are a step closer to being mature blood cells. These cells are called progenitor cells.

Unlike stem cells, progenitor cells are set to become a certain type of blood cell. There are 2 types of blood progenitor cells:

- Myeloid progenitor cells
- Lymphoid progenitor cells

Myeloid progenitor cells change and become red blood cells, platelets, and white blood cells. These white blood cells are called granulocytes and monocytes. Granulocytes include neutrophils, eosinophils, and basophils.

Lymphoid progenitor cells change and become a type of white blood cell called lymphocytes. There are 3 types of lymphocytes. They are B cells, T cells, and natural killer cells.

## Blood cancers

Blood cancers are a group of cancers. They form from blood cells in bone marrow. Blood cancers include leukemia, myelodysplastic syndromes (MDS), and myeloproliferative neoplasms (MPNs).

### MPN

MPNs form from blood stem cells within the myeloid cell line. "Myelo" means marrow. "Proliferative" means growing and refers to making too many cells. A neoplasm is any abnormal growth.

MDS are also cancers of blood stem cells. Unlike MPNs, people with MDS don't have enough blood cells.

## Classic MPN types

There are many types of MPNs. This book is about treatment for the 3 classic types. The classic types are:

- Polycythemia vera (PV),
- Essential thrombocythemia (ET), and
- Primary myelofibrosis (PMF).

Chronic myeloid leukemia (CML) is a type of MPN. Some people call it a classic MPN, but it is often discussed by itself. Its treatment is based on a cancer marker that the other classic MPN types do not have.

### **Polycythemia vera**

PV causes too many red blood cells. High levels of white blood cells and platelets may also be present. The red blood cells build up in bone marrow and blood. As a result, blood becomes thicker than normal.

### **Essential thrombocythemia**

ET causes too many megakaryocytes and platelets. Megakaryocytes are a type of blood cell within bone marrow. Platelets are tiny pieces of megakaryocytes. Having too many platelets is called thrombocythemia. “Essential” means the thrombocythemia is caused by a problem in the blood cell-making process within bone marrow.

### **Primary myelofibrosis**

Myelofibrosis (MF) is scarring of the bone marrow. If you haven’t had another type of MPN, MF is called “primary myelofibrosis” or PMF. In early phases of PMF, scarring may have not yet formed.

When scarring forms, the scar tissue may replace bone marrow. With less bone marrow, the number of blood cells may drop. As a result, the spleen and liver may begin to make blood cells and grow larger in size. These organs may also enlarge because they trap abnormal blood cells.

MF can also occur if you have PV or ET. If you have PV that became MF, it is called “post-PV myelofibrosis.” If you have ET that became myelofibrosis, it is called “post-ET myelofibrosis.”



A rare disease isn’t so rare when you have one.

– Jean

Survivor, Polycythemia vera

## Health risks

MPNs are chronic blood cancers. This means they do not go away on their own. Without treatment, MPNs get worse over time. It can take many years for MPNs to cause symptoms or serious health conditions.

### Symptoms

People may have symptoms for a long time before they learn they have an MPN. The most common symptom is feeling tired despite sleep (fatigue). Other symptoms include itchy skin, night sweats, bone pain, fever, and weight loss. These symptoms differ across MPNs.

### Blood clots and bleeding

A person with an MPN is more likely to have bleeding and blood clots. Bleeding is often minor but can be severe. Blood clots (or thrombosis) can block blood vessels and

sometimes be deadly. Blood clots can cause a pulmonary embolism or stroke. **See Figure 2.**

### Other cancers

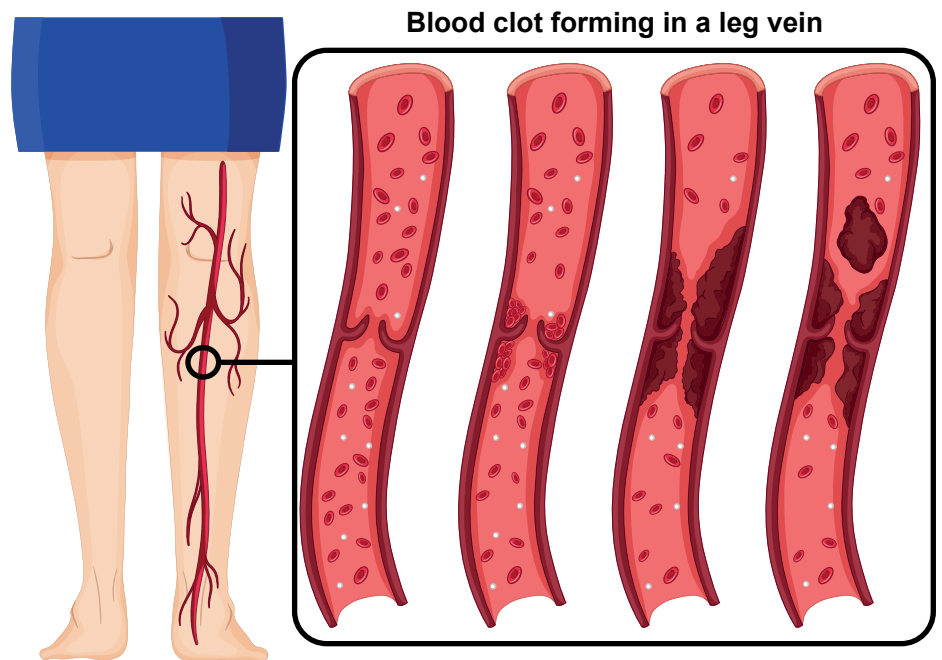
MPNs can transform. ET and PV can evolve into MF. Although rare, MPNs can change into acute myeloid leukemia (AML).

### Potentially shorter lifespan

MPNs are slow-growing cancers. Many people can live for years with the proper treatment. But, people with an MPN may have a shorter lifespan compared to their peers. Among the subtypes, people with MF typically have shorter lifespans than people with ET or PV.

**Figure 2**  
**Blood clot**

This image shows a blood clot forming in a leg vein. On the far right, a piece of the blood clot has detached. It could travel through the heart to the lungs and get stuck. This is called a pulmonary embolism. Pulmonary embolisms can be deadly.



## Review

- Most blood cells are formed in bone marrow. Blood stem cells are the cells from which all blood cells are formed.
- MPNs are a type of blood cancer. They are cancers that form from blood-forming stem cells within the myeloid cell line.
- The 3 classic MPNs are polycythemia vera (PV), essential thrombocythemia (ET), and primary myelofibrosis (PMF).
- People may have symptoms for a long time before they learn they have an MPN.
- MPNs can lead to serious health conditions, such as blood clots and AML.



Being diagnosed with PV at age 39 was a shock. I had never heard of MPN.

– Susan

Survivor, Polycythemia vera



# 2

## Tests for MPN

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14 Symptom survey

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14 Blood tests

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16 Bone marrow tests

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17 Genetic tests

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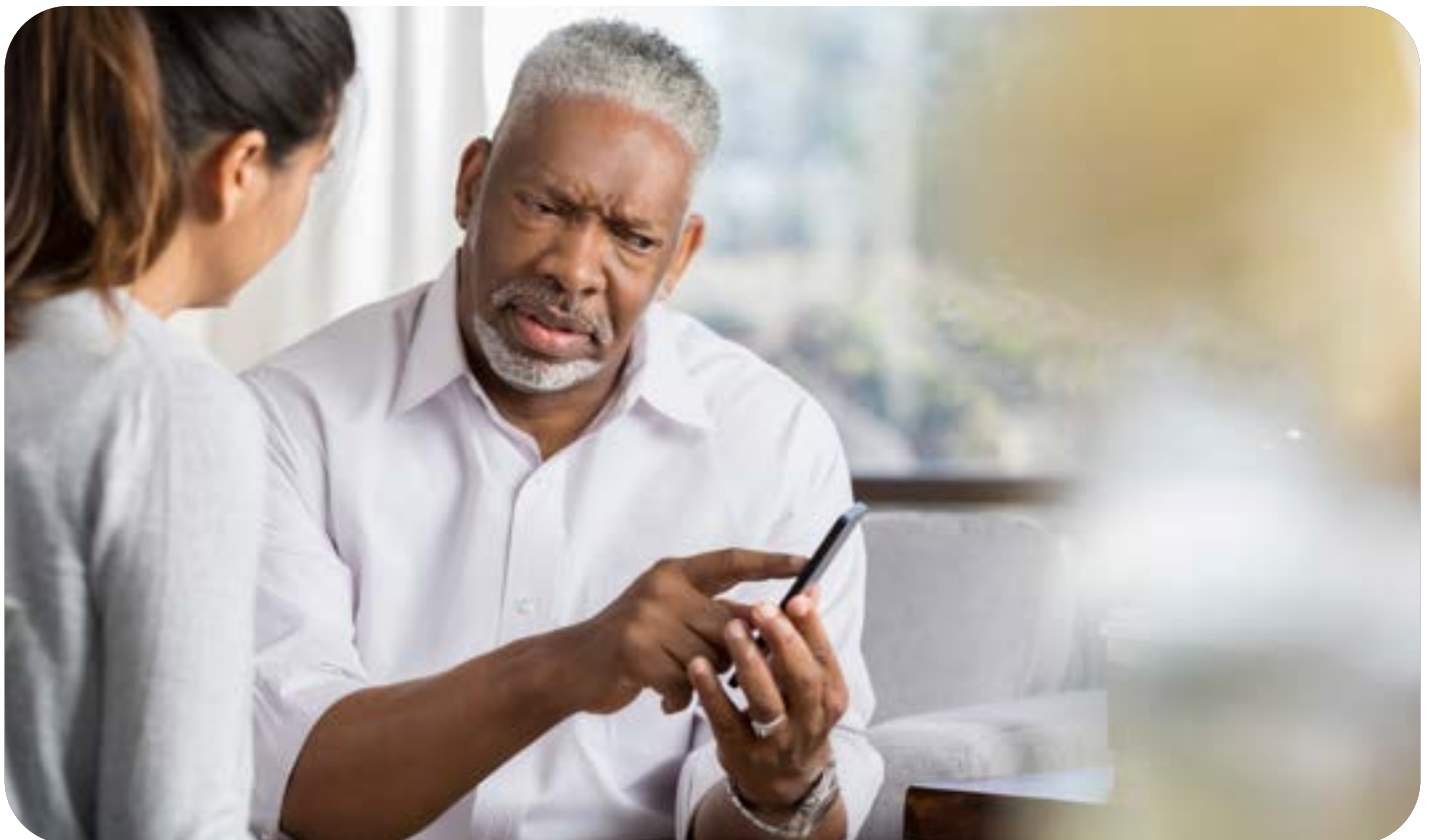
19 HLA tests

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19 Blood clotting tests

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20 Review



Tests that are used to identify (diagnose) MPNs are explained in this chapter. Other tests that are needed to prepare for treatment are also described.

Doctors plan treatment using many sources of information. These sources include the health care listed in [Guide 1](#). Another source is you. Tell your doctor your concerns and goals for treatment. Together, you can share in the decision-making process. Read **Part 6** to learn more about making treatment decisions.

## General health tests

### Medical history

Your doctor will want to know about any health problems and their treatment during your lifetime. Be prepared to talk about:

- Illnesses
- Injuries
- Health conditions
- Symptoms
- Medications
- Blood transfusions

A very important issue is the health of your heart and blood vessels (cardiovascular system). Be sure to report if you have had:

- High blood pressure (hypertension)
- Diabetes
- Blood clots
- Abnormal bleeding

### Guide 1 Tests for MPN

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Medical history

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Physical exam

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MPN-SAF TSS

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CBC with differential

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Blood smear

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Comprehensive metabolic panel

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Uric acid

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LDH

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Liver function tests

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EPO level

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Iron studies

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Bone marrow aspiration and biopsy

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*BCR-ABL1* testing

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Bone marrow cytogenetics

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Molecular testing

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HLA testing if needed

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Blood clotting tests if needed

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## Medications

It may help to bring a list of old and new medicines to your doctor's office.

Your doctor may also want to know about your diet, exercise level, and smoking history.

Some cancers and other health conditions can run in families. Be prepared to tell the health history of your close blood relatives. Such family includes your siblings, parents, and grandparents.

### Physical exam

A physical exam is a study of your body. It is done to look for signs of disease. It is also used to help assess what treatments may be options.

During this exam, expect the following to be checked:

- Your body temperature
- Your blood pressure
- Your pulse and breathing rate
- Your weight
- How your lungs, heart, and gut sound
- How your eyes, skin, nose, ears, and mouth look
- The size or hardness of your organs
- Level of pain when you are touched

MPN may cause your spleen and liver to be larger than normal.

## Symptom survey

Sometimes MPNs cause symptoms. These symptoms can be assessed with a survey called the MPN Symptom Assessment Form Total Symptom Score. It's also called the MPN-SAF TSS, for short. This survey lists 10 symptoms. Each symptom is rated on a scale from 0 to 10. Higher scores point to worse symptoms.

## Blood tests

Blood tests are useful for diagnosing MPNs. They can help to find other diseases, too. They require a sample of your blood. Samples of blood can be removed with a blood draw.

### Blood draw

Some blood draws require no eating and drinking for hours. A blood draw is performed with a needle inserted into a vein. Your blood samples will be sent to a lab.

### CBC with differential

A complete blood count (CBC) measures parts of the blood. Test results include:

- White blood cell count
- Red blood cell count
- Platelet count
- Hematocrit
- Hemoglobin level

A count is the number of blood cells within a blood sample. Hematocrit is the percentage of red blood cells in blood. Hemoglobin level is the amount of hemoglobin, which is a protein found within red blood cells.

Cancer and other health problems can cause low or high counts. With MPNs, one or more cell counts are high. Hematocrit and hemoglobin levels are high in polycythemia vera (PV).

### Differential

There are several types of white blood cells. A differential counts the number of each type. It also checks if the counts are in balance with each other. Your doctor can determine the cause of an abnormal white blood count from this test.

### Blood smear

A blood smear is a study of a drop of blood. It reveals important information about your blood cells.

- ▶ A blood smear can show which types of cells are present. With MPNs, sometimes blood-forming cells from the bone marrow are found in blood.
- ▶ A blood smear can also reveal if the blood cells look normal or not. Finding cells with an abnormal shape or size can be a clue as to what disease you have.

### Comprehensive metabolic panel

Chemicals in your blood come from your liver, bone, kidneys, and other organs. A comprehensive metabolic panel often includes tests for up to 14 chemicals. The tests show if the level of chemicals is too low or high. Abnormal levels can be caused by cancer or other health problems.

### Uric acid

Certain phases of MPNs produce many bone marrow cells. In turn, uric acid levels increase. High uric acid levels can cause gout and kidney stones. Testing should be done to assess if you

have excess uric acid in your blood. Excess uric acid is called hyperuricemia.

### LDH

Lactate dehydrogenase (LDH) is a protein that is in most cells. Dying cells release LDH into blood. High levels of LDH can be a sign of myelofibrosis (MF).

### Liver function tests

Your liver is an organ in the upper right side of your abdomen. It does many important jobs, such as remove toxins from blood. Liver function tests assess for chemicals in blood that are made or processed by the liver. With MPN, there may be abnormal results if the cancer is in the bone or liver.

### EPO level

Erythropoietin (EPO) is a hormone made by your kidneys. Low levels of EPO can cause low levels of red blood cells—anemia. But, anemia has other causes. EPO testing can help show if EPO is the cause.

EPO results can also help to diagnose PV. In PV, high red blood cell counts suppress EPO levels.

### Iron studies

Iron is a mineral that your body uses to make hemoglobin. Iron testing may help find the cause of high platelet counts. The cause may be iron-deficiency anemia instead of cancer. Iron studies can also help to diagnose PV. Despite having high hemoglobin levels, almost all people with PV have low iron levels.

## Bone marrow tests

Tests on bone marrow can be very helpful. They can be used to diagnose MPN and get a sense of its outlook (prognosis). It can also show how much scarring (fibrosis) is present.

### Aspiration and biopsy

A bone marrow aspiration removes a small amount of liquid bone marrow. A bone marrow biopsy removes a small piece of bone with marrow. These procedures may be done at the same time. You will likely lie on your side as shown in **Figure 3**.

### Lab tests

The samples will be sent to a lab. A pathologist will then study the samples with a microscope. A pathologist is a doctor who's an expert in testing cells to find disease.



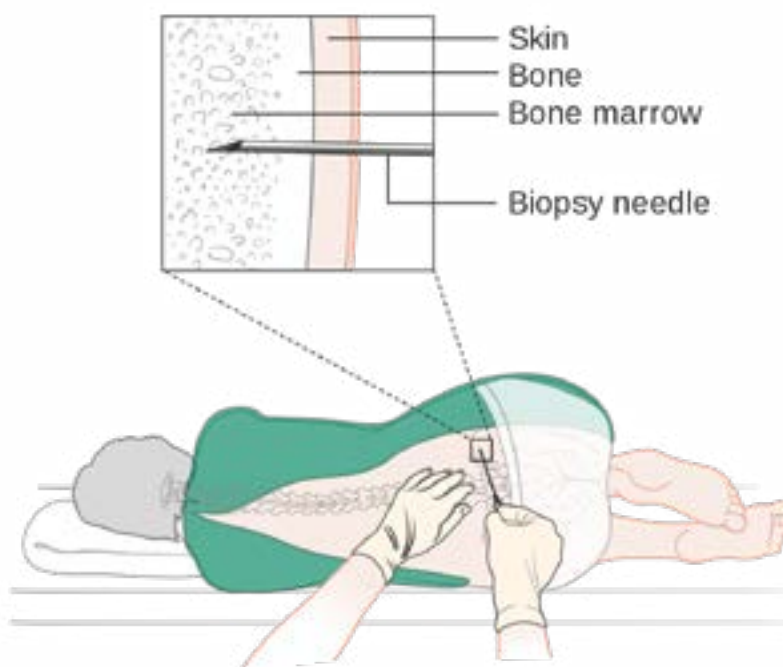
A diagnosis was made because of migraines due to a high platelet count and following blood work and a bone marrow biopsy confirmed ET.

– Antje  
Survivor, Essential  
thrombocythemia

### Figure 3 Bone marrow aspiration and biopsy

**A bone marrow aspiration removes a small amount of liquid bone marrow. A bone marrow biopsy removes a small piece of bone with marrow. These procedures are often done on the back of the hip.**

By Cancer Research UK - Original email from CRUK, CC BY-SA 4.0 <https://commons.wikimedia.org/w/index.php?curid=34332967>





## Genetic tests

Genetic information tells cells what to do. It is found in almost every cell in the body. It is found within a part of a cell called the nucleus.

**See Figure 4.**

Genetic information is stored in 46 long strands of DNA (deoxyribonucleic acid). A gene is a small segment of DNA with complex instructions. Each strand of DNA is carried and protected in a chromosome.

Genetic information is passed down from parents to a child. For most people, genetic changes related to cancer occur after birth. These changes are found only in the cancer cells. Much less often, people are born with genetic errors that increase their chance for getting cancer. These errors are present in all cells.

Genetic tests assess for abnormal changes in genes and chromosomes within cancer cells. These tests are used for diagnosis and treatment planning. Genetic tests are performed by pathologists.

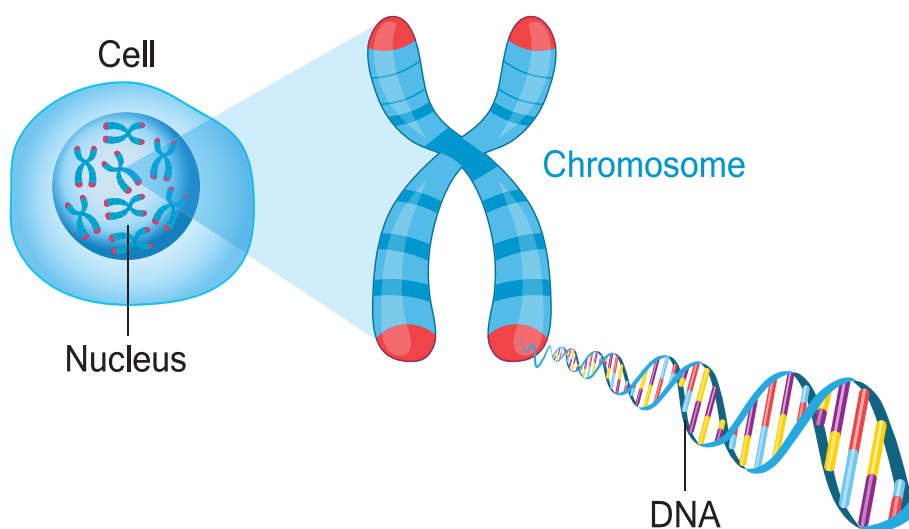
### ***BCR-ABL1* testing**

Chronic myeloid leukemia (CML) is a type of MPN. Its hallmark is the *BCR-ABL1* fusion gene. Normal blood cells do not have this gene. This gene is formed when parts of chromosomes 9 and 22 switch places with each other.

Testing for *BCR-ABL1* is advised to rule out CML. Testing requires either a bone marrow or blood sample. The lab test used to detect the *BCR-ABL1* gene is either fluorescence in situ hybridization (FISH) or multiplex RT-PCR.

**Figure 4**  
**Genetic information**

**Most human cells contain genetic information. The information tells cells how to build your body and make it work. It is stored in DNA. A gene is a small segment of DNA that contains complex instructions. DNA is not one long strand but a set of 46 strands. Each strand is carried and protected in a chromosome.**



### Bone marrow cytogenetics

Cytogenetics is the study of chromosomes. Chromosomes can be studied with a lab test called a karyotype. FISH may be used, too. Tests should be performed on bone marrow if a sample can be obtained. A blood sample can be used instead, but abnormal changes are less likely to be found.

Cytogenetics are done for a few reasons, such as to assess:

- **Clonality.** MPNs are a clonal disease. This means all the cancer cells came from the same parent cell. Molecular testing is most often used to test for clonality, but cytogenetics can be used instead.
- **Complex karyotype.** A complex karyotype is when there are 3 or more unrelated defects in chromosomes that occur in more than one cell.
- **Late-phase changes.** In the late phases of the cancer, it is common for MPN cells to have abnormal chromosomes. A part of or the entire chromosome may be missing. There may be an extra part in a chromosome. Parts of chromosomes may have switched places with each other.
- **Treatment results.** Treatment is working well if tests show that cells no longer have abnormal chromosomes. Abnormal chromosomes may reappear when treatment stops working.

### Molecular testing

Molecular testing includes tests of genes or their products (proteins). There are 3 common gene mutations among MPNs as well as less common ones. A blood or bone marrow sample may be used for testing. Test results are used for diagnosis and prognosis.

#### *JAK2* mutations

The most common mutation among MPNs is the *JAK2 V617F* mutation. It is present in cancer cells among almost every person with polycythemia vera (PV). It is also present in cancer cells in over half of the people with essential thrombocythemia (ET) or primary myelofibrosis (PMF). Testing for *JAK2 V617F* is needed for anyone who may have an MPN.

A small subset of people with PV have *JAK2* exon 12 mutations. Testing for these mutations should be done if the *JAK2 V617F* mutation is absent.

#### *CALR* and *MPL* mutations

People with ET or MF should be tested for *CALR* and *MPL* mutations if the *JAK2 V617F* mutation is absent. A small subset of these people do not have *JAK2*, *CALR*, or *MPL* mutations. When all 3 mutations are absent, the cancer is called triple-negative MPN.

#### Multi-gene NGS

Multi-gene next-generation sequencing (NGS) can detect many mutations. This lab test may be more helpful than testing for a few mutations. If done, it should include testing for *JAK2*, *CALR*, and *MPL*. Mutations in *ASXL1*, *EZH2*, *TET2*, *IDH1*, *IDH2*, *SRSF2*, and *SF3B1* genes may also be included.

## HLA tests

Human leukocyte antigens (HLA) are proteins found on the surface of most cells. They are markers of your cells. They allow your body to tell which cells are yours and which cells are foreign.

HLA testing detects a person's HLA type. You will receive this test if a transplant of healthy stem cells from a donor may be a treatment option. This treatment is described more in **Part 5**. HLA testing is performed on a blood sample.

HLA testing is needed to find the right donor for you. A donor's HLA type must be a near-perfect match to you for treatment to work. Otherwise, your body will reject the donor stem cells or the donor cells will react against your body.



Family and online education and support sites (MPN-NET) have been vital to my emotional stability and critical to my understanding of PV.

– Susan

Survivor, Polycythemia vera

## Blood clotting tests

Your body stops bleeding by turning blood into a gel-like form. The gel-like blood forms into a solid mass called a blood clot. Proteins, called coagulation factors, are needed for clotting.

An impaired clotting process sometimes occurs among people with an MPN. One cause of impaired clotting is von Willebrand disease (VWD). This disease usually occurs when platelet counts are very high. The high platelet counts limit how well the von Willebrand proteins clot.

Your doctor may order clotting tests if you have:

- Abnormal bleeding
- Increased platelets, especially over 1 million
- An enlarged spleen
- A surgery scheduled that may cause major bleeding.

There are 3 common clotting (coagulation) tests:

- **Prothrombin time** is how long it takes for your blood to clot. It measures how well all coagulation factors work together.
- **Partial thromboplastin time** is how long it takes for your blood to clot. It measures coagulation factors from two of three pathways.
- **Fibrinogen activity** is a measure of how well a blood protein called fibrinogen is working.

## Review

- A medical history is a report of all health events and treatment in your lifetime. Expect to be asked questions about your health and the health of some family members.
- Your doctor will study your body to assess your health. He or she will touch parts of your body to see if anything feels abnormal.
- Your doctor will decide if and what type of disease you have based on blood tests, bone marrow tests, and genetic tests.
- HLA testing is needed if you will receive a transplant of blood stem cells from a donor.
- Tests of how well your blood clots may be done if your doctor suspects that it is not clotting normally.



It is so good to talk to someone that understands what we are going through.

– Jean

Survivor, Polycythemia vera

# 3

## Polycythemia vera

- 22 Diagnosis
- 23 Initial treatment
- 26 Monitoring
- 28 Changing treatment
- 29 Review





This chapter is a treatment guide for the MPN called polycythemia vera (PV, for short). It starts with describing how PV is found. It also explains the treatment process and options. Discuss with your doctor which options are right for you.

## Diagnosis

Signs of PV are commonly found with a blood test that was given for another reason. The blood test may reveal a high hemoglobin level. Or, a lot of your blood may consist of red blood cells (high hematocrit). These results may lead to more testing, which confirm PV. **See Figure 5.**

PV is also found due to its symptoms or related health problems. Such health problems include blood clots and bleeding. A common

symptom is unpleasant skin sensations. These sensations often occur shortly after a bath or shower. Your skin may itch, tickle, sting, or burn.

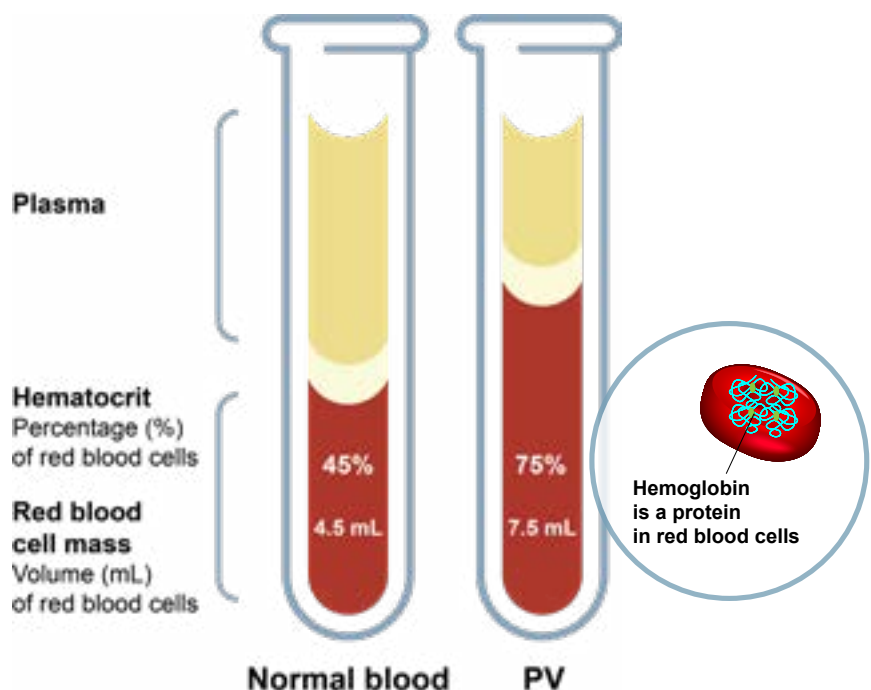
There are many other common symptoms. You may have headaches, dizziness, vision changes, and fatigue. Your abdomen may hurt. You may quickly feel full when eating.

You may have PV if testing finds that:

- You have a high red cell mass, hematocrit, or hemoglobin level
- There are too many cells in your bone marrow
- The abnormal blood cells have a *JAK2* mutation that is common to PV or your EPO levels are low

### Figure 5 Signs of PV

A diagnosis of PV requires a high red cell mass, hematocrit, or hemoglobin level. Red cell mass is the volume of red blood cells. Hematocrit is the percentage of red blood cells in blood. The hemoglobin level is the amount of hemoglobin in the blood.



## Initial treatment

At this time, PV does not have a cure, but with treatment, most people live many years. One aim of treatment is to stop the health problems caused by PV. This is mostly achieved by reducing red blood cell counts. A second goal is to relieve symptoms.

Treatment is based on your chance for having a blood clot. Your doctor will assess whether you are at low or high risk for blood clots. [Guide 2](#) lists treatment based on risk.

- **Low risk** includes people who are younger than 60 years of age and have never had a blood clot.
- **High risk** includes people who are 60 years of age and older or have had a blood clot.

### Blood clots

You might need to get imaging to look for a blood clot. Imaging makes pictures of the insides of your body. It allows your doctor to see blood flow and blood clots.

Imaging includes ultrasound, computed tomography (CT), and magnetic resonance imaging (MRI). Venography involves an injection of a dye followed by x-rays.

You might need an anticoagulant if you have a blood clot. Anticoagulants are also called blood thinners. Low-molecular-weight heparin (LMWH) is a blood thinner that you can inject into your skin at home. Warfarin, abigatran, rivaroxaban, apixaban, and edoxaban are oral blood thinners.

If your platelets are very high, you may receive plateletpheresis. This procedure withdraws your blood and removes platelets. Your platelet-reduced blood will then be returned to your body.

## Guide 2

### Initial treatment by risk group

<b>Low risk</b>	<ul style="list-style-type: none"> <li>• Assess for new blood clots and major bleeding</li> <li>• Manage cardiovascular risk factors</li> <li>• Aspirin</li> </ul>
<b>High risk</b>	<ul style="list-style-type: none"> <li>• Assess for new blood clots and major bleeding</li> <li>• Manage cardiovascular risk factors</li> <li>• Aspirin</li> <li>• Hydroxyurea or interferons</li> </ul>

### Bleeding

Treatment for PV includes assessing for and treating bleeding. Bleeding is common in PV but often isn't severe.

Bleeding related to PV includes easy bruising, nosebleeds, or heavy menstrual periods. PV may also cause bleeding in your digestive tract or blood in your urine. You may see a liver or bowel expert if sudden bleeding occurs.

### Cardiovascular risk factors

Cardiovascular is a word that refers to the heart and blood vessels. There are cardiovascular risk factors that increase your chance for a blood clot. These risk factors include:

- High blood pressure (hypertension)
- Diabetes
- Smoking

If needed, your treatment team can help you to reduce cardiovascular risk factors. Reducing risk factors is advised for low- and high-risk groups.

Medications can help treat high blood pressure and diabetes. There are also medications that reduce cravings to smoke. If you smoke, it's important that you quit.

Healthy living can reduce cardiovascular risk factors. Health experts can help you make and follow plans for healthy living. Such plans can focus on eating healthy foods, exercising, and taking medications as prescribed. You can also get help with a plan to quit smoking.

### Aspirin

Most people with PV receive aspirin. It is advised for low- and high-risk groups. It has many health benefits, such as:

- Prevents blood clots
- Reduces your chance for heart attacks and strokes
- Prevents death from cardiovascular diseases

Smoking blocks the action of aspirin. You'll have to quit smoking for aspirin to work.

Aspirin for PV is prescribed at a low dose. A low dose consists of 80 to 100 milligrams a day. Higher doses should be avoided. High doses increase the chance of bleeding in your bowels.

Your doctor may tell you not to take aspirin if you have had major bleeding. Aspirin increases the likelihood of bleeding. Aspirin should be withheld 1 week prior to any surgery. You may restart aspirin 24 hours after surgery unless you have a bleeding risk.



I find it very important to be in the care of a MPN specialist and a local hematologist, who work together on my behalf.

– Eric  
Survivor, Polycythemia vera

### Phlebotomy

A goal of treatment is to lower hematocrit. Hematocrit is the percentage of red blood cells in blood. For many people, hematocrit should be below 45 percent (%). For women, a target of below 42 percent is often used.

Lowering hematocrit will likely reduce blood thickness. As a result, your chance for getting blood clots will decrease. You may also have fewer headaches, less itchiness, and fewer vision problems.

Phlebotomy is the procedure that is used to lower hematocrit. It consists of having your blood withdrawn with a needle like when donating blood. Phlebotomy works by causing iron deficiency, so don't take iron supplements. **See Figure 6.**

How often phlebotomy is needed differs between people. Some people need it every

other week. If your hematocrit is high, you may need it once or twice a week.

### Hydroxyurea or interferons

Hydroxyurea and interferons are cytoreductive medications for PV. Cytoreductive treatment reduces the number of blood cells. It is not needed for low-risk PV. NCCN experts recommend cytoreductive treatment for high-risk PV.

Hydroxyurea is commonly received. Interferon may be an option if you're pregnant or young. Some people put off taking hydroxyurea by first taking interferon.

Hydroxyurea and interferons can cause unwanted health issues. Such health issues are called side effects. Ask your treatment team for a complete list of side effects of your treatments. Learn how side effects might be prevented or treated.

### Figure 6 Phlebotomy

**Phlebotomy is a procedure that is commonly used to control PV. It involves withdrawing your blood much like when donating blood. The goal is to reduce your hematocrit. This will reduce your chance for getting blood clots. Phlebotomy may also reduce symptoms.**



## Monitoring

After starting treatment, you will need to meet with your treatment team often. NCCN experts advise having visits every 3 to 6 months. You may need more frequent visits if problems arise. During visits, the status of the cancer and the results of treatment will be checked.

### Status of PV

PV may get better or worse after starting treatment. To assess the status of PV, you may get blood tests. The MPN-SAF Total Symptom Score is used to assess symptoms.

### Starting cytoreductive treatment

If you're not taking cytoreductive treatment, you might need it in the future. Signs that it may be needed include:

- A new blood clot
- Major bleeding
- Frequent phlebotomy
- The need for phlebotomies doesn't decrease over time
- Enlarged spleen
- Too many platelets
- Too many white blood cells
- Worsening MPN symptoms

Cytoreductive treatment should only be started if the cancer has not progressed to myelofibrosis (MF). A bone marrow aspiration and biopsy are needed to make sure MF is not present. If you now have MF, read **Part 5** to learn about treatment options.



## Pregnancy and PV

You may want to have a baby after learning you have PV. Before getting pregnant, it may help to meet with an obstetrician who's an expert in high-risk pregnancies. This doctor can tell you what health care you will need.

NCCN experts advise taking aspirin during and shortly after pregnancy. For a low-risk pregnancy, low-molecular-weight heparin (LMWH) may be received instead of aspirin for 2 weeks before labor. For a high-risk pregnancy, you may take LMWH with aspirin throughout the pregnancy.

Aspirin may be stopped 1 to 2 weeks before labor. LMWH should be stopped 12 to 24 hours before labor. After giving birth, restart treatment for 6 weeks.

Hydroxyurea should not be taken during pregnancy or while breastfeeding. It may harm your baby. You may switch from hydroxyurea to interferon before and during pregnancy.



### Results of cytoreductive treatment

After starting cytoreductive treatment, your blood will be checked for changes. Your doctor will check your counts to be sure that they are not too high or low.

For research, there are standards for assessing treatment results. Know that your treatment may be working but may not match these standards. In research, treatment results are described by these 4 groups:

#### Complete remission

Complete remission is defined by:

- No cancer symptoms and signs for at least 12 weeks
- Hematocrit less than 45 percent without phlebotomies, and normal or near-normal blood counts for at least 12 weeks
- No blood clots or bleeding events, and PV isn't changing into another cancer
- Bone marrow consists of a normal number of cells, cells that look normal, and minor, if any, bone marrow scarring (fibrosis)

#### Partial remission

Partial remission is like a complete remission except bone marrow cells are still abnormal.

#### No response

No response is less than a partial remission.

#### Progressive disease

Progressive disease is a worsening of the cancer.



## Surgery during treatment for PV

You might need surgery while being treated for PV. Your surgery team will assess your risk for bleeding and blood clots. Be prepared to report your health history. If surgery is planned, you can take steps to prevent bleeding and blood clots.

Prior to surgery, your blood counts should be close to normal. You may need more phlebotomies to stay below 45 percent for 3 months prior to surgery.

If on aspirin, stop taking it 1 week before surgery. Likewise, stop taking any blood thinners before surgery. You may restart 24 hours after surgery unless you have a bleeding risk.

You may stay on cytoreductive treatment until the surgery unless otherwise told by your surgery team.

## Changing treatment

Sometimes cytoreductive treatment works at first then stops. Sometimes, it doesn't work enough or at all. When one treatment fails, another treatment may be received.

Signs to change your cytoreductive treatment include:

- Severe side effects
- Treatment stops working
- A new blood clot
- Major bleeding
- Frequent phlebotomy
- The need for phlebotomies doesn't decrease
- Enlarged spleen
- Too many platelets
- Too many white blood cells
- Worsening MPN symptoms

Your next treatment depends on if PV has transformed and your prior treatments. If it has transformed into MF or acute myeloid leukemia (AML), read **Part 5** for treatment options. If it hasn't transformed, changing the type of cytoreductive treatment may be needed. Treatment options are listed in [Guide 3](#).

You may have been on hydroxyurea but it stopped working or caused severe side effects. If this happened, ruxolitinib may be an option. Ruxolitinib is a medicine that is called a kinase inhibitor. It blocks a kinase called JAK and in turn stops growth signals within MPN cells. Ask your treatment team for a list of its side effects.

If you haven't taken hydroxyurea, it may be an option now. Likewise, interferon may be an option if not received before.

A clinical trial may be an option. A clinical trial is a type of research that studies a promising test or treatment in people. It gives people access to health care that otherwise couldn't usually be received. Ask your treatment team if there is a clinical trial that is right for you.

Be aware that busulfan is not advised by NCCN experts. It may increase the likelihood of AML and other cancers.

### Guide 3 Second-line treatment options

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Ruxolitinib if hydroxyurea is not an option

---

Hydroxyurea if not received before

---

Interferon if not received before

---

Clinical trial

---

## Review

- PV is commonly found after a blood test that was given for another reason. A key feature of PV is a high number of red blood cells.
- Treatment options are based on low or high risk for blood clots. Aspirin to control symptoms is an option if you're at low or high risk. Phlebotomy may be received to reduce hematocrit. For high-risk PV, cytoreductive treatment may be an option to reduce blood counts.
- You will need to meet with your doctor often. During visits, the status of the cancer and the results of treatment will be checked.
- If the cancer is getting worse, you may receive another type of cytoreductive treatment. Ruxolitinib and joining a clinical trial are other options.



I was diagnosed with PV when I was 57 years old. Now I am 72. Most of the time my disease has been under control.

– Gail

Survivor, Polycythemia vera

# 4

## Essential thrombocythemia

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This chapter is a treatment guide for the MPN called essential thrombocythemia (ET, for short). It starts with describing how ET is found. It also explains the treatment process and options. Discuss with your doctor which options are right for you.

## Diagnosis

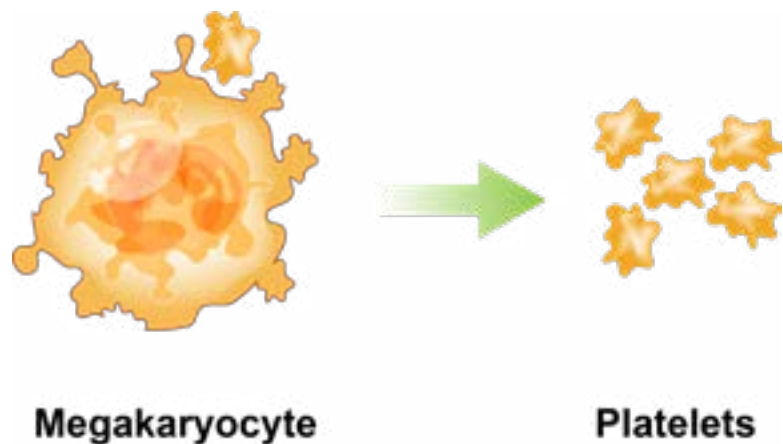
ET is commonly found after a blood test that was given for another reason. It is also found due to related symptoms or health events. Common symptoms of ET are fatigue, headaches, dizziness, or vision changes. ET can also cause blood clots (thrombosis), abnormal bleeding (hemorrhage), and miscarriage during pregnancy.

You may have ET if testing finds that:

- Your platelet count is very high over time. There is no secondary cause for the high platelet counts.
- There are too many abnormal platelet-forming cells (megakaryocytes) in your bone marrow. **See Figure 7.**
- Other types of MPN, myelodysplastic syndromes (MDS), and other myeloid neoplasms have been ruled out.
- The abnormal blood cells have a mutation that is common to ET like *JAK2*, *CALR*, or *MPL* mutations.

### Figure 7 Key ET features

A diagnosis of ET requires high numbers of megakaryocytes and platelets. A megakaryocyte is a rare type of bone marrow cell. When mature, it extends its arms through the wall of a blood vessel and releases platelets into the bloodstream.



## Initial treatment

At this time, ET is not cured, but with treatment, most people have a normal or near-normal lifespan. One aim of treatment is to prevent health problems caused by ET. The second goal is to relieve symptoms.

Treatment is based on your chance for having a blood clot. Your doctor will assess your risk using a tool called the International Prognostic Score of Thrombosis (IPSET-thrombosis).

**Guide 4** lists treatment based on risk.

- **Very low risk** describes people who are 60 years of age or younger, don't have a *JAK2* mutation, and never had a blood clot.
- **Low risk** describes people who are 60 years of age or younger, have a *JAK2* mutation, and never had a blood clot.
- **Intermediate risk** describes people who are older than 60 years of age, don't have a *JAK2* mutation, and never had a blood clot.
- **High risk** describes people who 1) have had a blood clot or 2) are older than 60 years and have a *JAK2* mutation.

### Blood clots

For all risk groups, treatment includes assessing for and treating blood clots. You might need to get imaging to assess for a blood clot. Imaging makes pictures of the insides of your body. It allows your doctor to see blood flow and blood clots.

Imaging includes ultrasound, computed tomography (CT), and magnetic resonance imaging (MRI). Venography involves an injection of a dye followed by x-rays.

You might need an anticoagulant if you have a blood clot. Anticoagulants are also called blood thinners. Low-molecular-weight heparin (LMWH) is a blood thinner that you can inject into your skin at home. Warfarin, abigatran, rivaroxaban, apixaban, and edoxaban are oral blood thinners.

If your platelets are very high, you may receive plateletpheresis. This procedure withdraws your blood and removes platelets. Your platelet-reduced blood will then be returned to your body.

### VWD

Treatment for ET includes assessing for the onset of von Willebrand disease (VWD). VWD can impair normal blood clotting and cause major bleeding. Testing may be done if your spleen enlarges, platelet counts increase, if bleeding starts, or before having surgery. Blood clotting (coagulation) tests are used to diagnose acquired VWD.

### Major bleeding

Treatment for ET includes assessing for and treating major bleeding. Bleeding isn't very common for ET. It is more likely if platelet counts are very high.

Bleeding related to ET includes easy bruising, nosebleeds, or heavy menstrual periods. ET may also cause bleeding in your digestive tract or blood in your urine. You may see a liver or bowel expert if sudden bleeding occurs. You may receive plateletpheresis if bleeding is severe.



### Cardiovascular risk factors

Cardiovascular is a word that refers to the heart and blood vessels. There are cardiovascular risk factors that increase your chance for a blood clot. These risk factors include:

- High blood pressure (hypertension)
- Diabetes
- Smoking

If needed, your treatment team will help you to reduce cardiovascular risk factors. Reducing risk factors is advised for all risk groups.

Medications can help treat high blood pressure and diabetes. There are also medications that reduce cravings to smoke. If you smoke, it's important that you quit.

Healthy living can reduce cardiovascular risk factors. Health experts can help you make and follow plans for healthy living. Such plans can focus on eating healthy foods, exercising, and taking medications as prescribed. You can also get help with a plan to quit smoking.

### Aspirin

Most people with ET receive aspirin. It is advised for all risk groups. It has many health benefits, such as:

- Prevents blood clots
- Reduces your chance for heart attacks and strokes
- Prevents death from cardiovascular diseases

Smoking blocks the action of aspirin. You'll have to quit smoking for aspirin to work.

Aspirin for ET is prescribed at a low dose. A low dose consists of 80 to 100 milligrams a day. Higher doses should be avoided. High doses increase the chance of bleeding in your bowels.

Your doctor may tell you not to take aspirin if you have had major bleeding. Aspirin increases the likelihood of bleeding. Aspirin may also not be received if you have VWD.

## Guide 4

### Initial treatment by risk group

#### Very low risk

#### Low risk

#### Intermediate risk

- Assess for new blood clots, onset of VWD, and major bleeding
- Manage cardiovascular risk factors
- Aspirin

#### High risk

- Assess for new blood clots, onset of VWD, and major bleeding
- Manage cardiovascular risk factors
- Aspirin
- Hydroxyurea, interferons, or anagrelide

### Hydroxyurea, interferons, or anagrelide

Hydroxyurea, interferons, and anagrelide are cytoreductive medications for ET. Cytoreductive treatment reduces the number of blood cells. NCCN experts recommend cytoreductive treatment for high-risk ET.

Hydroxyurea is commonly received. Interferon may be an option if you're pregnant or young. Some people put off taking hydroxyurea by first taking interferon. Anagrelide works well, but its side effects may be worse than hydroxyurea.

Cytoreductive treatment can cause unwanted health issues. Such health issues are called side effects. Ask your treatment team for a complete list of side effects of your treatments. Learn how side effects might be prevented or treated.



Routine blood work showed high platelets and a following bone marrow biopsy confirmed the ET diagnosis.

– Lyn  
Survivor, Essential  
thrombocythemia



## Cytoreductive treatment

### Hydroxyurea

Hydroxyurea is a chemotherapy drug. It is a type of chemotherapy called an antimetabolite. This type prevents the “building blocks” of DNA from being used. As a result, new cells can't be made.

### Interferons

Interferons naturally exist in your body as part of your disease-fighting (immune) system. They can also be made in the lab and be used to treat MPN. When used as treatment, interferon is given in much higher amounts than what the body makes.

Interferons for MPN are interferon alfa-2b, peginterferon alfa-2a, and peginterferon alfa-2b.

### Anagrelide

Anagrelide is a medicine that reduces the number of platelets. It works by blocking an enzyme called phospholipase A2. This stops megakaryocytes from maturing and making platelets.

## Monitoring

After starting treatment, you will need to meet with your treatment team often. A visit every 3 to 6 months is advised. You may need more frequent visits if problems arise. During visits, the status of the cancer and the results of treatment will be checked.

### Status of ET

ET may get better or worse after starting treatment. To assess the status of ET, you may get blood tests. The MPN-SAF Total Symptom Score is used to assess symptoms.

### Starting cytoreductive treatment

If you're not taking cytoreductive treatment, you might need it in the future. Signs that it may be needed include:

- A new blood clot
- Acquired VWD
- Major bleeding
- Enlarged spleen
- Too many platelets
- Too many white blood cells
- MPN symptoms
- Aspirin isn't relieving symptoms

Cytoreductive treatment should only be started if the cancer has not progressed to myelofibrosis (MF). A bone marrow aspiration and biopsy are needed to make sure MF is not present. If you now have MF, read **Part 5** to learn about treatment options.



## Pregnancy and ET

You may want to have a baby after learning you have ET. Before getting pregnant, it may help to meet with an obstetrician who's an expert in high-risk pregnancies. This doctor can tell you what health care you will need.

NCCN experts advise taking aspirin during and shortly after pregnancy. For a low-risk pregnancy, low-molecular-weight heparin (LMWH) may be received instead of aspirin for 2 weeks before labor. For a high-risk pregnancy, you may take LMWH with aspirin throughout the pregnancy.

Aspirin may be stopped 1 to 2 weeks before labor. LMWH should be stopped 12 to 24 hours before labor. After giving birth, restart treatment for 6 weeks.

Hydroxyurea should not be taken during pregnancy or while breastfeeding. It may harm your baby. You may switch from hydroxyurea to interferon before and during pregnancy.

### Results of cytoreductive treatment

After starting cytoreductive treatment, your blood will be checked for changes. Your doctor will check your counts to be sure that they are not too high or low.

For research, there are standards for assessing treatment results. Know that your treatment may be working but may not match these standards. In research, treatment results are described by these 4 groups:

#### Complete remission

Complete remission is defined by:

- No cancer symptoms and signs for at least 12 weeks
- Normal or near-normal blood counts for at least 12 weeks
- No blood clots or bleeding events and ET isn't changing into another cancer
- Megakaryocytes look normal, and there is minor, if any, bone marrow scarring (fibrosis)

#### Partial remission

Partial remission is like a complete remission except megakaryocytes look abnormal.

#### No response

No response is less than a partial remission.

#### Progressive disease

Progressive disease is a worsening of the cancer.



## Surgery during treatment for ET

You might need surgery while being treated for ET. Your surgery team will assess your risk for bleeding and blood clots. Be prepared to report your health history. If surgery is planned, you can take steps to prevent bleeding and blood clots.

If on aspirin, stop taking it 1 week before surgery. Likewise, stop taking any blood thinners before surgery. You may restart 24 hours after surgery unless you have a bleeding risk.

You may stay on cytoreductive treatment until the surgery, unless otherwise told by your surgery team.

## Changing treatment

Sometimes cytoreductive treatment works at first then stops. Sometimes, it doesn't work enough or at all. When one treatment fails, another treatment may be received. Signs to change your cytoreductive treatment include:

- Severe side effects
- Treatment stops working
- A new blood clot
- Acquired VWD
- Major bleeding
- Enlarged spleen
- Too many platelets
- Too many white blood cells
- MPN symptoms
- Aspirin isn't relieving symptoms

Your next treatment depends on if ET has transformed and on your prior treatments. If it has transformed into MF or acute myeloid leukemia (AML), read **Part 5** for treatment options. If it hasn't transformed, changing the type of cytoreductive treatment may be needed. Treatment options are listed in [Guide 5](#).

A clinical trial may be an option. A clinical trial is a type of research that studies a promising test or treatment in people. It gives people access to health care that otherwise couldn't usually be received. Ask your treatment team if there is a clinical trial that is right for you.

Be aware that busulfan is not advised by NCCN experts. It may increase the likelihood of AML and other cancers.

### Guide 5

#### Second-line treatment options

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Hydroxyurea if not received before

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Interferon if not received before

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Anagrelide if not received before

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Clinical trial

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Available treatments have successfully controlled my counts for over 25 years. When one treatment loses efficacy, I cycle to another.

– Antje  
Survivor, [Essential thrombocythemia](#)

## Review

- ET is commonly found after a blood test that was given for another reason. ET causes a high number of platelets.
- Treatment options are based on risk for blood clots. Your risk may be very low, low, intermediate, or high.
- Aspirin is an treatment option for all risk groups. For high-risk ET, cytoreductive treatment may be an option to reduce blood counts.
- You will need to meet with your doctor often. During visits, the status of the cancer and the results of treatment will be checked.
- If the cancer is getting worse, you may receive another type of cytoreductive treatment. Joining a clinical trial is another option.



Family, friends, and medical personnel have provided emotional and spiritual support to me.

– RuthAnne  
Survivor, Essential  
thrombocythemia



# 5

## Myelofibrosis

40	Diagnosis
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This chapter is a treatment guide for myelofibrosis (MF, for short). It starts with describing how MF is found. It also explains the treatment process and options. Discuss with your doctor which options are right for you.

## Diagnosis

MF is scarring of the bone marrow. It can occur in people with or without a history of MPNs.

- If you haven't had another type of MPN, myelofibrosis is called "primary myelofibrosis" or PMF.
- If you've had polycythemia vera (PV) that became myelofibrosis, it is called "post-PV myelofibrosis."
- If you've had essential thrombocythemia (ET) that became myelofibrosis, it is called "post-ET myelofibrosis."

### PMF

PMF is often found because of symptoms. The most common symptom is severe fatigue. Another common symptom is quickly feeling full when eating. You may have discomfort under your ribs on the left side. Other symptoms include losing weight without trying, low fever, bone pain, and night sweats.

PMF is also found because of tests given for other reasons. Your doctor may detect that your liver or spleen is big. Your blood results may be abnormal. These results may lead to more tests, which confirm PMF.

There are 2 stages of PMF:

- Prefibrotic PMF (prePMF); also called early PMF
- Overt PMF

The stages were created to help tell prefibrotic PMF apart from ET.

### Prefibrotic PMF

You may have prefibrotic PMF if testing finds that:

- Your bone marrow has many abnormal platelet-forming cells (megakaryocytes). Counts of other marrow and blood cells are normal or slightly low.
- There is minor or no bone marrow scarring (fibrosis).
- Other types of MPN, myelodysplastic syndromes (MDS), and other myeloid neoplasms have been ruled out.
- The abnormal blood cells have a *JAK2*, *CALR*, *MPL*, or other mutation that is linked to PMF. Otherwise, there is no other molecular cause for MF.

Also, you must have at least 1 of the following 4 conditions:

- Anemia that isn't caused by another health condition
- High white blood cell count
- High LDH level
- Enlarged spleen

**Overt PMF**

You may have overt PMF if testing finds that:

- Your bone marrow has many abnormal megakaryocytes. Major scarring (fibrosis) of your bone marrow is present.
- Other types of MPN, MDS, and other myeloid neoplasms have been ruled out.
- The abnormal blood cells have a *JAK2*, *CALR*, *MPL*, or other mutation that is linked to PMF. Otherwise, there is no other molecular cause for MF.

Also, you must have at least 1 of the following 4 conditions:

- Anemia that isn't caused by another health condition
- High white blood cell count
- High LDH level
- Enlarged spleen
- Young blood cells in your blood that should only be in your bone marrow



*My PMF has been indolent and progressing very slowly. Exercise and a good diet have helped my quality of life.*

– Vivian

Survivor, Primary myelofibrosis

**Post-PV myelofibrosis**

You may have post-PV myelofibrosis if you have:

- PV with higher-grade bone marrow fibrosis.

Also, you must have 2 of the following 4 conditions:

- Anemia that isn't caused by another condition
- Young blood cells in blood that should only be in bone marrow
- An enlarged spleen
- Weight loss or night sweats caused by MF

**Post-ET myelofibrosis**

You may have post-ET myelofibrosis if you have:

- ET with higher-grade bone marrow fibrosis

Also, you must have 2 of the following 5 conditions:

- Anemia that isn't caused by another condition
- Young blood cells in blood that should only be in bone marrow
- A high LDH level
- An enlarged spleen
- Weight loss or night sweats caused by MF

## Initial treatment

There are 3 treatment goals for MF. The first is to reduce symptoms. Symptoms are often related to an enlarged spleen and anemia. The second treatment goal is to improve blood counts. The third goal is to reduce the chance of MPN progressing to AML.

Treatment is based on the outlook of the cancer and symptoms. Doctors use scoring systems to assess the outlook of the cancer (prognosis). Some scoring systems have been in use and others are being studied. Some surveys being studied use information on abnormal chromosomes and genes that may affect the prognosis and treatment planning.

NCCN experts prefer the Dynamic International Prognostic Scoring System-PLUS (DIPSS-PLUS) during the course of treatment. The MPN-SAF Total Symptom Score is used to assess current symptoms. [Guide 6](#) lists treatment based on risk groups and symptoms.

- **Low risk** is a score of zero on the DIPSS-PLUS.
- **Intermediate-1** is a score of 1 on the DIPSS-PLUS.
- **Intermediate-2** is a score of 2 or 3 on the DIPSS-PLUS.
- **High risk** is a score between 4 and 6 on the DIPSS-PLUS.

### Guide 6

#### Initial treatment by risk group

<b>Low risk without symptoms</b>	<ul style="list-style-type: none"> <li>• Watch and wait (observation)</li> <li>• Clinical trial</li> </ul>
<b>Low risk with symptoms</b>	<ul style="list-style-type: none"> <li>• Ruxolitinib</li> <li>• Interferons</li> <li>• Hydroxyurea</li> <li>• Clinical trial</li> </ul>
<b>Intermediate-1</b>	<ul style="list-style-type: none"> <li>• Watch and wait (observation)</li> <li>• Ruxolitinib</li> <li>• Clinical trial</li> <li>• Allogeneic HCT</li> </ul>
<b>Intermediate-2 and high risk</b>	<ul style="list-style-type: none"> <li>• Allogeneic HCT</li> <li>• Clinical trial</li> <li>• Ruxolitinib</li> <li>• Fedratinib</li> <li>• Anemia treatment</li> </ul>

### Watch and wait

Watch and wait is also sometimes called observation. It is an option for low- and intermediate-1-risk MF without symptoms. It is a period of testing to watch for changes in cancer status. Treatment may be started if symptoms appear. Starting treatment before symptoms appear doesn't help improve outcomes.

### Clinical trial

A clinical trial is an option for all risk groups. Ask your treatment team if there is a clinical trial that is right for you. Clinical trials can help answer these questions:

- Does treatment reduce bone marrow fibrosis?
- Does treatment improve cell counts and reduce symptoms?
- Does treatment make stem cell transfusions unnecessary?
- Does treatment prevent or delay leukemia?



Our MPNs are so individual and respond differently to different treatments.

– Lyn

Survivor, Post-ET myelofibrosis



## Clinical trials

Clinical trials study how safe and helpful tests and treatments are for people. When found to be safe and helpful, they may become tomorrow's standard of care.

Because of clinical trials, the tests and treatments in this book are now widely used to help people with MPN. All new tests and treatments that may improve your care and treatment need to be tested in clinical trials.

To join a clinical trial, you must meet the conditions of the study. Patients in a clinical trial are often alike in terms of their cancer and general health. This helps ensure that any change is from the treatment and not because of differences between patients.

If you decide to join a clinical trial, you will need to review and sign a paper called an informed consent form. This form describes the study in detail, including the risks and benefits. Even after you sign a consent form, you can stop taking part in a clinical trial at any time.

Ask your treatment team if there is an open clinical trial that you can join. There may be clinical trials where you're getting treatment or at other treatment centers nearby. You can also find clinical trials through the websites listed in the last chapter.

### Ruxolitinib

Ruxolitinib is a medicine that is called a kinase inhibitor. It blocks a kinase called JAK and in turn stops growth signals within MPN cells.

It is an option for low- and intermediate-1-risk MF when symptoms are present. It is also an option for intermediate-2 and high-risk MF when a transplant is not an option and the platelet count is greater than 50,000.

### Hydroxyurea or interferons

Interferon and hydroxyurea are options for low-risk MF with symptoms. Both control MF by reducing the number of blood cells. Cell counts may return to normal levels.

### Allogeneic HCT

Allogeneic hematopoietic cell transplant (HCT) is a transplant of healthy stem cells from a donor. It is an option for intermediate-2- and high-risk MF.

It may also be an option for intermediate-1 if platelets are low or MPN cells have complex cytogenetics. A complex karyotype is when there are 3 or more unrelated defects in chromosomes that occur in 2 or more cells.

Allogeneic HCT provides the only chance for a cure. However, it is not safe for everyone. Most people are not able to undergo allogeneic HCT.



## Kinase inhibitors

Kinases are molecules that move chemicals, called phosphates, from one molecule to another. Kinase inhibitors stop the phosphates from being moved. In turn, growth signals within cells are often blocked. This reduces the number of new cells being made.

Ruxolitinib and fedratinib are kinase inhibitors used to treat myelofibrosis. Ruxolitinib is also used to treat polycythemia vera (PV).

Ruxolitinib and fedratinib stop a kinase called JAK. JAK is part of a cell receptor that helps cells grow. It is a key to blood stem cells becoming blood cells.



### Fedratinib

Fedratinib is a medicine that is called a kinase inhibitor. It blocks a kinase called JAK and in turn stops growth signals within MPN cells.

It is an option for intermediate-2 and high-risk MF. It is an option when a transplant can't be done and the platelet count is greater than 50,000.

Although fedratinib works like ruxolitinib, more research is needed to learn how well fedratinib treats MF.

### Anemia treatment

Many people with MF have anemia. Anemia is a condition of low red blood cells or hemoglobin. For intermediate-2 and high-risk MF, treatment for anemia may be the main type of care you will receive. It is an option if you can't have an allogeneic HCT and you don't have other symptoms.



## Allogeneic HCT

A stem cell transplant replaces unhealthy stem cells with healthy ones. An allogeneic hematopoietic cell transplant (HCT) uses healthy stem cells from a donor. Testing is needed to find a donor who's a good match for you. An allogeneic transplant is an intense treatment, so not everyone can get it.

You'll first receive treatment to kill your bone marrow and most MPN cells. Next, you'll receive the donor cells. These cells will form new, healthy marrow. They will also attack cancer cells that weren't killed by prior treatment. Visit the websites listed in the last chapter for more information on transplants.

## Supportive care

Supportive care aims to improve your quality of life. It is sometimes called palliative care. It's important for everyone, not just people at the end of life.

Supportive care can prevent or relieve emotional or physical problems caused by cancer or its treatment. Health problems caused by treatment are called side effects. Ask your treatment team for a full list of side effects of your treatment. In [Guide 7](#), some of the supportive care needs of people with MF are listed.

### Anemia

Your doctor will first assess for causes of anemia other than MF. Common causes include bleeding, breakdown of red blood cells, and low iron, B12, or folate levels. These causes should be treated.

Standard treatment of anemia with symptoms is a red blood cell transfusion. Additional treatment options are based on erythropoietin (EPO) levels.

Most white blood cells should be removed from donated blood for a transfusion. This will help prevent the donated blood from attacking your body. It also prevents you from getting cytomegalovirus (CMV).

### Iron overload

Iron overload is a term for too much iron in your body. It can occur if you've had many red blood cell transfusions. Iron chelation is a type of drug that removes extra iron from your body. It is an option at times for low- and intermediate-1-risk MF. Your doctor may prescribe iron chelation

if you've had more than 20 transfusions, blood ferritin is greater than 2500 ng/mL, or both.

### Bleeding

Platelet transfusion is used when:

- There is bleeding caused by low platelets
- Your platelet count is lower than 10,000 m<sup>3</sup>
- Your platelet count is higher than 10,000 m<sup>3</sup> and there is bleeding

Most white blood cells should be removed from the donated blood. This will help prevent the blood from attacking your body. It also prevents you from getting CMV.

Transfusions may not stop bleeding. In this case, antifibrinolytic agents may be used. These drugs help your blood to clot.

### Infections

You may be prone to infections because of the cancer, its treatment, or both. Ask your doctor which vaccinations are safe for you. If you get infections often, your doctor may prescribe antibiotics.

Instead of antibiotics, you may receive granulocyte colony-stimulating factor (G-CSF) or granulocyte-macrophage colony-stimulating factor (GM-CSF) if you have low neutrophil counts. These medicines can cause your spleen to rupture. Thus, they should be used with caution if you have an enlarged spleen.

### Tumor lysis syndrome

Tumor lysis syndrome (TLS) occurs when the waste released by dead cells is not quickly cleared out of the body. This results in kidney damage and severe blood electrolyte disturbances. It can be life-threatening.

Induction chemotherapy may cause TLS. Induction chemotherapy is a treatment for advanced-stage MF or acute myeloid leukemia (AML). This treatment kills many cancer cells and results in too much waste too quickly.

TLS may be prevented by high amounts of fluids during chemotherapy. Decreasing uric

acid levels with allopurinol or rasburicase is another option. Rasburicase may be given as the first treatment if your blast cells are quickly increasing. It may also be first received if your uric acid level is high or your kidney(s) are damaged.

**Guide 7**  
**Supportive care**

<p><b>Anemia</b></p>	<p>Treat other causes of anemia</p> <ul style="list-style-type: none"> <li>• Replace iron, folate, and B12 if needed</li> <li>• Treat hemolysis if needed</li> <li>• Red blood cell transfusions</li> </ul>	<p>→</p> <p>If EPO is lower than 500 mU/mL:</p> <ul style="list-style-type: none"> <li>• Erythropoiesis-stimulating agents (darbepoetin alfa and epoetin alfa)</li> <li>• Clinical trial</li> </ul> <p>If EPO is 500 mU/mL or higher:</p> <ul style="list-style-type: none"> <li>• Clinical trial</li> <li>• Danazol</li> <li>• Lenalidomide with or without prednisone</li> <li>• Thalidomide with or without prednisone</li> </ul>
<p><b>Iron overload</b></p>	<ul style="list-style-type: none"> <li>• Iron chelation</li> </ul>	
<p><b>Bleeding or low platelet counts</b></p>	<ul style="list-style-type: none"> <li>• Platelet transfusions</li> <li>• Antifibrinolytic agents if transfusions fail</li> </ul>	
<p><b>Infections</b></p>	<ul style="list-style-type: none"> <li>• Vaccinations</li> <li>• Antibiotics for recurrent infections</li> <li>• G-CSF or GM-CSF for recurrent infections when neutrophils are low</li> </ul>	
<p><b>Tumor lysis syndrome</b></p>	<ul style="list-style-type: none"> <li>• Prevent with hydration</li> <li>• Manage hyperuricemia with allopurinol or rasburicase</li> </ul>	
<p><b>Heart disease</b></p>	<ul style="list-style-type: none"> <li>• Counseling and treatment</li> </ul>	
<p><b>High blood counts in PMF</b></p>	<ul style="list-style-type: none"> <li>• Cytoreductive therapy</li> </ul>	

### Heart (cardiovascular) disease

Cardiovascular is a word that refers to the heart and blood vessels. Cardiovascular risk factors increase your chance for heart disease. These risk factors include:

- High blood pressure (hypertension)
- Diabetes
- Smoking

If needed, your doctor will help you to reduce cardiovascular risk factors. Medications can help treat high blood pressure and diabetes. There are also medications that reduce cravings to smoke.

Healthy living can reduce cardiovascular risk factors. Health experts can help you make and follow plans for healthy living. Such plans can focus on eating healthy foods, exercise, and taking medications as prescribed. You can also get help with a plan to quit smoking.

### High blood counts

You may have high blood counts if you have PMF. In this case, you may receive cytoreductive treatment, such as hydroxyurea. Cytoreductive treatment is sometimes received in addition to other treatments to relieve certain symptoms.

## Monitoring

After starting treatment, you will need to meet with your treatment team often. A visit every 3 to 6 months is advised. You may need more frequent visits if problems arise. During visits, the status of the cancer and the results of treatment will be checked.

### Status of MF

Over time, the cancer may get worse. To assess the cancer, you may get blood tests. The MPN-SAF Total Symptom Score is used to assess symptoms. If the cancer appears worse, a bone marrow aspiration and biopsy may be needed. You may start active treatment if you were on a watch-and-wait treatment approach.

### Treatment results

There is more than one type of treatment result.

- **Anemia response** is an improvement in hemoglobin level or not needing transfusions anymore.
- **Spleen response** is a major decrease in spleen size.
- **Symptom response** is a major decrease in symptoms.
- **Clinical improvement** is one or more of these responses and no signs of the cancer worsening.

For research, there are standards for assessing treatment results. Know that your treatment may be working but may not match these standards. In research, treatment results are described by these 4 groups:

- **Complete remission** is the best outcome. There are no signs of the cancer. Your bone marrow and blood results are normal or near normal. Your symptoms have cleared. Your liver and spleen are of normal size.
- **Partial remission** is a good response. Your blood work may be normal. Otherwise, your bone marrow results are normal and your blood results are returning to normal. Your symptoms have

cleared. Your liver and spleen are of normal size.

- **No response** is less than a partial remission.
- **Progressive disease** is a worsening of the cancer.

MF may also be assessed for changes in cell structures.

- **Cytogenetic remission** is defined by fewer or the absence of abnormal chromosomes.
- **Molecular remission** is defined by fewer or the absence of molecular markers of MF.

### Changing treatment

Sometimes treatment works at first then stops. This is called a relapse. Sometimes, treatment doesn't work enough or at all. MF can transform into AML.

When one treatment fails, another treatment may be received. Read *Initial treatment* in this chapter to learn what your next treatment might be based on your risk. If the cancer is now advanced-stage MF or AML, read the next section for treatment options.

## Advanced-stage MF or AML

Before treatment, you may receive more tests. These tests may include bone marrow aspiration and biopsy. Lab tests on bone marrow or blood may include cytogenetics, flow cytometry, and molecular testing for AML-related mutations.

### Clinical trial

A clinical trial is an option. Ask your treatment team if there is a clinical trial that is right for you.

### Hypomethylating agents or chemotherapy

Besides a clinical trial, hypomethylating agents or chemotherapy is an option. Hypomethylating agents include azacitidine and decitabine. Chemotherapy for AML is used.

If you will get chemotherapy, the regimen that you will receive will partly depend on whether allogeneic HCT is an option. If it's not an option, low-intensity induction chemotherapy is used. If allogeneic HCT is an option, intensive induction chemotherapy is used to achieve a remission.

Visit this webpage to read about induction chemotherapy for AML: [NCCN.org/patients/guidelines/cancers.aspx#aml](https://www.nccn.org/patients/guidelines/cancers.aspx#aml).

### Allogeneic HCT

An allogeneic HCT provides the only chance for a cure. However, it is not safe for everyone. Most people are not able to undergo allogeneic HCT.

## Review

- MF is scarring of the bone marrow.
- Primary MF means you haven't had another type of MPN. On the other hand, PV or ET can worsen and become MF. If this occurs, PV is called post-PV myelofibrosis and ET is called post-ET myelofibrosis.
- Scar tissue in bone marrow may cause blood cell counts to drop. Scarring may not be present in early MF.
- Initial treatment is based on risk groups. A clinical trial is an option for all risk groups. Observation is an option for low- and intermediate-1-risk cancers without symptoms. Otherwise, active treatment with cytoreductive treatment or *JAK2* inhibitors is used. Allogeneic HCT is an option for intermediate- and high-risk MF.
- Besides cancer treatment, you may receive supportive care to prevent or reduce symptoms related to the cancer.
- You will need to meet with your doctor often. He or she will assess the status of the cancer and how you feel.
- For advanced-stage MF or AML, a clinical trial is an option. Other options are hypomethylating agents or chemotherapy. These treatments may be followed by an allogeneic transplant for some people.



# 6

## Making treatment decisions

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Having cancer is very stressful. There is a lot to learn in what feels like a short amount of time. This chapter can help you make decisions that reflect your beliefs, wishes, and values.

## It's your choice

The role patients want in choosing their treatment differs. You may feel uneasy about making treatment decisions. This may be due to a high level of stress. It may be hard to hear or know what others are saying. Stress, pain, and drugs can limit your ability to make good decisions. You may feel uneasy because you don't know much about cancer. You've never heard the words used to describe cancer, tests, or treatments. Likewise, you may think that your judgment isn't any better than your doctors'.

Letting others decide which option is best may make you feel more at ease. But, whom do you want to make the decisions? You may rely on your doctors alone to make the right decisions. However, your doctors may not tell you which to choose if you have more than one good option. You can also have loved ones help. They can gather information, speak on your behalf, and share in decision-making with your doctors. Even if others decide which treatment you will receive, you still have to agree by signing a consent form.

On the other hand, you may want to take the lead or share in decision-making. Most patients do. In shared decision-making, you and your doctors share information, weigh the options, and agree on a treatment plan. Your doctors know the science behind your plan but you know your concerns and goals. By working

together, you are likely to get a higher quality of care and be more satisfied. You'll likely get the treatment you want, at the place you want, and by the doctors you want.

## Questions to ask your doctors

You may meet with experts from different fields of medicine. Strive to have helpful talks with each person. Prepare questions before your visit and ask questions if the person isn't clear. You can also take notes and get copies of your medical records.

It may be helpful to have your spouse, partner, family member, or a friend with you at these visits. A patient advocate or navigator might also be able to come. They can help to ask questions and remember what was said. Suggested questions to ask are listed on the following pages.



I discuss my treatment plan and changes with my local hematologist and then involve my specialist in the final decision. This way we are all on the same page and find the best treatment for me.

– Antje  
Survivor, Essential  
thrombocythemia

## What's my diagnosis and prognosis?

It's important to know that there are different types of cancer. Cancers with the same name can even greatly differ. Based on your test results, your doctor can tell you which type of cancer you have. He or she can also give a prognosis. A prognosis is a prediction of the pattern and outcome of a disease. Knowing the prognosis may affect what you decide about treatment.

1. Where did the cancer start? In what type of cell? Is this cancer common?
2. What is the cancer risk group? Does this group mean the cancer is advanced?
3. Is this a fast- or slow-growing MPN?
4. What are my chances that MPN will become AML?
5. What tests do you recommend for me?
6. Where will the tests take place? How long will the tests take and will any test hurt?
7. What if I am pregnant?
8. How do I prepare for testing?
9. Should I bring a list of my medications?
10. Should I bring someone with me?
11. How often are these tests wrong?
12. Would you give me a copy of the pathology report and other test results?
13. Who will talk with me about the next steps? When?

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## What are my options?

There is no single treatment practice that is best for all people. There is often more than one treatment option along with clinical trial options. Your doctor will review your test results and recommend treatment options.

1. What will happen if I do nothing?
  2. Can I just carefully monitor the cancer?
  3. Do you consult NCCN recommendations when considering options?
  4. Are you suggesting options other than what NCCN recommends? If yes, why?
  5. Do your suggested options include clinical trials? Please explain why.
  6. Is an allogenic stem cell transplant an option for me?
  7. How do my age, health, and other factors affect my options? What if I am pregnant?
  8. Which option is proven to work best?
  9. Which options lack scientific proof?
  10. What are the benefits of each option? Does any option offer a cure or long-term cancer control? Are my chances any better for one option than another? Are any options less time-consuming? Less expensive?
  11. What are the risks of each option? What are possible complications? What are the rare and common side effects? Short-lived and long-lasting side effects? Serious or mild side effects? Other risks?
  12. How do you know if treatment is working?
  13. What are my options if treatment doesn't work?
  14. What can be done to prevent or relieve the side effects of treatment?
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## What does each option require of me?

Many patients consider how each option will practically affect their lives. This information may be important because you have family, jobs, and other duties to take care of. You also may be concerned about getting the help you need. If you have more than one option, choosing the option that is the least taxing may be important to you:

1. Will I have to go to the hospital or elsewhere? How often? How long is each visit?
2. What do I need to think about if I will travel for treatment?
3. Do I have a choice of when to begin treatment? Can I choose the days and times of treatment?
4. How do I prepare for treatment? Do I have to stop taking any of my medicines? Are there foods I will have to avoid?
5. Should I bring someone with me when I get treated?
6. Will the treatment hurt?
7. How much will the treatment cost me? What does my insurance cover?
8. Will I miss work or school? Will I be able to drive?
9. Is home care after treatment needed? If yes, what type?
10. How soon will I be able to manage my own health?
11. When will I be able to return to my normal activities?

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## What is your experience?

Research suggests that patients treated by experienced doctors have better results. It is important to learn if a doctor is an expert in the cancer treatment he or she is offering.

1. Are you board-certified? If yes, in what area?
2. How many patients like me have you treated?
3. How many procedures like the one you're suggesting have you done?
4. Is this treatment a major part of your practice?
5. How many of your patients have had complications?

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## Deciding between options

Deciding which option is best can be hard. Doctors from different fields of medicine may have different opinions on which option is best for you. This can be very confusing. Your spouse or partner may disagree with which option you want. This can be stressful. In some cases, one option hasn't been shown to work better than another. Some ways to decide on treatment are discussed next.

### Second opinion

After finding out you have cancer, it is normal to want to start treatment as soon as possible. While cancer can't be ignored, there is time to have another doctor review your test results and suggest a treatment plan. This is called getting a second opinion, and it's a normal part of cancer care.

Getting a second opinion doesn't mean you don't trust the first doctor. In fact, most doctors who are diagnosed with cancer will see more than one doctor before beginning treatment. What's more, some health plans require a second opinion. If your health plan doesn't cover the cost of a second opinion, you have the choice of paying for it yourself.

If the two opinions are the same, you may feel better about the treatment you accept to have. If the two opinions differ, think about getting a third opinion. Choosing your cancer treatment is a very important decision. It can affect your length and quality of life.

### Support groups

Besides speaking with health experts, it may help to talk to people who have walked in your shoes. Support groups often consist of people at different stages of treatment. Some may be

in the process of deciding while others may be finished with treatment. At support groups, you can ask questions and hear about the experiences of other people with Hodgkin lymphoma. Keep in mind that your cancer may have different characteristics (eg, stage, number of unfavorable risk factors) than other people. Therefore, you may have different treatment options than others.

### Compare benefits and downsides

Every option has benefits and downsides. Consider these when deciding which option is best for you. Talking to others can help identify benefits and downsides you haven't thought of. For example, you can decide how aggressive you want to be with treatment at the cost of increasing negative long-term side effects. Scoring each factor from 0 to 10 can also help since some factors may be more important to you than others. You should feel comfortable discussing your goals of care with your health care team.



It is important to seek a second opinion from a specialist to confirm the diagnosis and lay out future plans.

– Kye

Survivor, Polycythemia vera

## Websites

### Leukemia & Lymphoma Society

[LLS.org/information-specialists](https://lls.org/information-specialists)

### MPN Cancer Connection

[MPNCancerConnection.org](https://mpncancerconnection.org)

### MPN Education Foundation

[mpninfo.org](https://mpninfo.org)

### MPN Research Foundation

[mpnresearchfoundation.org](https://mpnresearchfoundation.org)

### National Cancer Institute (NCI)

[cancer.gov/types/myeloproliferative](https://cancer.gov/types/myeloproliferative)

### National Coalition for Cancer Survivorship

[canceradvocacy.org/toolbox](https://canceradvocacy.org/toolbox)

### NCCN for Patients®

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

### Voices of MPN (Supported by Incyte)

[voicesofmpn.com](https://voicesofmpn.com)

## Review

- ▶ Shared decision-making is a process in which you and your doctors plan treatment together.
- ▶ Asking your doctors questions is vital to getting the information you need to make informed decisions.
- ▶ Getting a second opinion, attending support groups, and comparing benefits and risks may help you decide which treatment is best for you.

## myMPN

myMPN is the first-ever natural history databank for classic MPNs. Your information will be used to find better treatments. You will also be informed about new clinical trials. All information is protected. You decide how your information is used. Visit [mpnresearchfoundation.org/myMPN](https://mpnresearchfoundation.org/myMPN) for more information and to register.



## Words to know

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

A fast-growing cancer that starts in the bone marrow and causes too many young white blood cells to be made.

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

A cancer treatment that replaces blood stem cells with donor stem cells which in turn make a new immune system and attack the cancer cells.

### **anemia**

A condition in which the number of red blood cells is low.

### **anticoagulant**

A medicine that reduces blood clotting. Also called blood thinner.

### ***BCR-ABL1* gene**

An abnormal gene that is formed when parts of chromosomes 9 and 22 break off and switch with each other. This gene is found on the Philadelphia chromosome and is the key feature of chronic myeloid leukemia.

### **biopsy**

Removal of small amounts of tissue from the body to be tested for disease.

### **blood clot**

A thickened mass of blood. Also called a thrombosis.

### **blood smear**

A test that involves viewing a drop of blood with a microscope to assess features of blood cells.

### **blood stem cell**

A blood-forming cell from which all other types of blood cells are formed. 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 aspiration**

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 stop the life cycle of cells so they don't increase in number.

### **chromosome**

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

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

A cancer of blood-forming cells that causes too many white blood cells called granulocytes to form.

### **clinical trial**

Research on a test or treatment to assess its safety or how well it works.

### **coagulation test**

A test of the proteins that cause blood to clot.

### **complete blood count (CBC)**

A test of the number of blood cells in a sample.

### **complete remission**

No signs of cancer are present after treatment.

### **complex karyotype**

The presence of 3 or more unrelated defects in chromosomes that occur in 2 or more cells.

### **comprehensive metabolic panel**

Tests of up to 14 chemicals in your blood.

### **computed tomography (CT)**

A test that uses x-rays from many angles to make a picture of the inside of the body.

**conditioning treatment**

Treatment that is used to destroy cells in the bone marrow to prepare your body for a stem cell transplant.

**cytogenetic testing**

A test that uses a microscope to examine a cell's chromosomes.

**CMV**

cytomegalovirus

**cytoreductive treatment**

Treatment that reduces the number of blood cells.

**deoxyribonucleic acid (DNA)**

A chain of chemicals in cells that contains coded instructions for making and controlling cells.

**diagnosis**

To identify a disease.

**differential**

Measurement of the different types of white blood cells present in a blood sample.

**DIPSS-PLUS**

Dynamic International Prognostic Scoring System-PLUS

**erythromelalgia**

A health condition that turns skin red and may cause painful, burning sensations.

**erythropoiesis-stimulating agent**

A drug that helps bone marrow to make more red blood cells.

**erythropoietin (EPO)**

A substance that helps bone marrow to make more red blood cells.

**essential thrombocythemia (ET)**

A cancer of blood-forming cells that causes a high number of platelets.

**fatigue**

Severe tiredness despite getting enough sleep that limits one's ability to function.

**fluorescence in situ hybridization (FISH)**

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

**G-CSF**

granulocyte colony-stimulating factor

**gene**

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

**GM-CSF**

granulocyte-macrophage colony-stimulating factor

**HCT**

hematopoietic cell transplant

**hematocrit**

The percentage of red blood cells in blood.

**hematopoietic stem cell**

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

**hemoglobin**

A protein in red blood cells that carries oxygen.

**human leukocyte antigen (HLA)**

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

**imaging**

A test that makes pictures of the insides of your body.

**immune system**

Your body's natural defense against infection and disease.

**immunomodulator**

A medicine that changes some parts of your body's disease-fighting system.

**immunotherapy**

A medicine that increases the activity of your body's disease-fighting system.

**intensive chemotherapy**

Treatment with high doses of strong cancer drugs that are more likely to cause severe side effects.

**IPSET-thrombosis**

International Prognostic Score of Thrombosis

**iron**

A mineral needed to make new red blood cells.

**iron chelation**

Treatment that is used to remove excess iron from your body.

**iron overload**

The buildup of excess iron in your body.

**karyotype**

A test that uses a microscope to examine a cell's chromosomes.

**lactate dehydrogenase (LDH)**

A protein that helps to make energy in cells.

**liver function tests**

Tests that measure chemicals made or processed by the liver.

**LMWH**

Low-molecular-weight heparin.

**magnetic resonance imaging (MRI)**

A test that uses a magnetic field and radio waves to make pictures of the insides of the body.

**medical history**

All health events and medications taken to date.

**megakaryocyte**

A bone marrow cell that makes platelets.

**MPN-SAF TSS**

MPN Symptom Assessment Form Total Symptom Score

**mutation**

An abnormal change in the instructions within cells for making and controlling cells.

**myelofibrosis (MF)**

Scarring of the bone marrow.

**myeloproliferative neoplasm (MPN)**

A cancer of blood-forming cells that causes too many blood cells to form.

**myeloproliferative neoplasm (MPN)**

A cancer of blood-forming cells that causes too many blood cells to form.

**NGS**

Next-generation sequencing

**no response**

Test results show no meaningful change in cancer status after treatment.

**observation**

A period of testing for changes in cancer status.

**partial response**

Test results still show signs of cancer but also improvement after treatment.

**pathologist**

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

**phlebotomy**

Withdrawal of blood.

**physical exam**

A review of the body by a health expert for signs of disease.



**platelet**

A type of blood cell that helps control bleeding. Also called thrombocyte.

**plateletpheresis**

A procedure that withdraws blood, removes platelets, and then returns your altered blood to your body.

**platelet transfusion**

A slow injection of platelets into a vein.

**polycythemia vera (PV)**

Cancer of blood-forming cells that causes too many red blood cells.

**prePMF**

prefibrotic primary myelofibrosis.

**prognosis**

The pattern and outcome of a disease.

**post-ET myelofibrosis**

Advanced essential thrombocythemia with scarring in the bone marrow.

**post-PV myelofibrosis**

Advanced polycythemia vera with scarring in the bone marrow.

**primary myelofibrosis (PMF)**

Scarring of the bone marrow not due to other bone marrow problems.

**progression**

A worsening of cancer.

**red blood cell**

A type of blood cell that carries oxygen from the lungs to the rest of the body.

**red blood cell transfusion**

A slow injection of red blood cells into a vein.

**relapse**

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

**side effect**

An unhealthy or unpleasant physical or emotional condition caused by treatment.

**spleen**

A small organ to the left of your stomach that is part of the immune system.

**supportive care**

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

**TLS**

Tumor lysis syndrome

**white blood cell**

A type of blood cell that helps fight infections in the body.

**VWD**

von Willebrand disease

# NCCN Contributors

This patient guide is based on the NCCN Clinical Practice Guidelines in Oncology (NCCN Guidelines<sup>®</sup>) for Myeloproliferative Neoplasms. It was adapted, reviewed, and published with help from the following people:

Dorothy A. Shead, MS  
Director, Patient Information  
Operations

Erin Vidic, MA  
Medical Writer

Tanya Fischer, MEd, MSLIS  
Medical Writer

Susan Kidney  
Design Specialist

Laura J. Hanisch, PsyD  
Medical Writer/Patient  
Information Specialist

Rachael Clarke  
Senior Medical Copyeditor

Kim Williams  
Creative Services Manager

The NCCN Clinical Practice Guidelines in Oncology (NCCN Guidelines<sup>®</sup>) for Myeloproliferative Neoplasms Version 3.2019 were developed by the following NCCN Panel Members:

Aaron T. Gerds, MD, MS/Chair  
Case Comprehensive Cancer Center/  
University Hospitals Seidman Cancer  
Center and Cleveland Clinic Taussig  
Cancer Institute

Sanjay R. Mohan, MD  
Vanderbilt-Ingram Cancer Center

Pankit Vachhani, MD  
O'Neal Comprehensive  
Cancer Center at UAB

Jason Gotlib, MD, MS/Vice-Chair  
Stanford Cancer Institute

Vivian Oehler, MD  
Fred Hutchinson Cancer Research Center/  
Seattle Cancer Care Alliance

\*Martha Wadleigh, MD  
Dana-Farber/Brigham and Women's  
Cancer Center

Prithviraj Bose, MD  
The University of Texas  
MD Anderson Cancer Center

Stephen Oh, MD, PhD  
Siteman Cancer Center at Barnes-  
Jewish Hospital and Washington  
University School of Medicine

Katherine Walsh, MD  
The Ohio State University Comprehensive  
Cancer Center - James Cancer Hospital  
and Solove Research Institute

Michael W. Deininger, MD, PhD  
Huntsman Cancer Institute  
at the University of Utah

Animesh Pardhanani, MBBS, PhD  
Mayo Clinic Cancer Center

## NCCN Staff

Mary Anne Bergman  
Guidelines Coordinator

Ivana Gojo, MD  
The Sidney Kimmel Comprehensive  
Cancer Center at Johns Hopkins

Nikolai Podoltsev, MD, PhD  
Yale Cancer Center/Smilow Cancer Hospital

Hema Sundar, PhD  
Oncology Scientist/Senior Medical Writer

\* Krishna Gundabolu, MBBS  
Fred & Pamela Buffett Cancer Center

Raajit Rampal, MD, PhD  
Memorial Sloan Kettering Cancer Center

Elizabeth Hexner, MD  
Abramson Cancer Center  
at the University of Pennsylvania

Erik Ranheim, MD, PhD  
University of Wisconsin  
Carbone Cancer Center

Lindsay Rein, MD  
Duke Cancer Institute

Gabriela Hobbs, MD  
Massachusetts General Hospital  
Cancer Center

\*David S. Snyder, MD  
City of Hope National Medical Center

Catriona Jamieson, MD, PhD  
UC San Diego Moores Cancer Center

Brady L. Stein, MD, MHS  
Robert H. Lurie Comprehensive Cancer  
Center of Northwestern University

Andrew T. Kuykendall, MD  
Moffitt Cancer Center

Moshe Talpaz, MD  
University of Michigan Rogel Cancer Center

Brandon McMahon, MD  
University of Colorado Cancer Center

Swapna Thota, MD  
Roswell Park Comprehensive Cancer Center

\* Reviewed this patient guide.

For disclosures, visit [www.nccn.org/about/disclosure.aspx](http://www.nccn.org/about/disclosure.aspx).

# NCCN Cancer Centers

Abramson Cancer Center  
at the University of Pennsylvania  
Philadelphia, Pennsylvania  
800.789.7366  
[penmedicine.org/cancer](http://penmedicine.org/cancer)

Fred & Pamela Buffett Cancer Center  
Omaha, Nebraska  
800.999.5465  
[nebraskamed.com/cancer](http://nebraskamed.com/cancer)

Case Comprehensive Cancer Center/  
University Hospitals Seidman Cancer  
Center and Cleveland Clinic Taussig  
Cancer Institute  
Cleveland, Ohio  
800.641.2422 • UH Seidman Cancer Center  
[uhhospitals.org/services/cancer-services](http://uhhospitals.org/services/cancer-services)  
866.223.8100 • CC Taussig Cancer Institute  
[my.clevelandclinic.org/departments/cancer](http://my.clevelandclinic.org/departments/cancer)  
216.844.8797 • Case CCC  
[case.edu/cancer](http://case.edu/cancer)

City of Hope National Medical Center  
Los Angeles, California  
800.826.4673  
[cityofhope.org](http://cityofhope.org)

Dana-Farber/Brigham and  
Women's Cancer Center  
Massachusetts General Hospital  
Cancer Center  
Boston, Massachusetts  
877.332.4294  
[dfbwcc.org](http://dfbwcc.org)  
[massgeneral.org/cancer](http://massgeneral.org/cancer)

Duke Cancer Institute  
Durham, North Carolina  
888.275.3853  
[dukecancerinstitute.org](http://dukecancerinstitute.org)

Fox Chase Cancer Center  
Philadelphia, Pennsylvania  
888.369.2427  
[foxchase.org](http://foxchase.org)

Huntsman Cancer Institute  
at the University of Utah  
Salt Lake City, Utah  
877.585.0303  
[huntsmancancer.org](http://huntsmancancer.org)

Fred Hutchinson Cancer  
Research Center/Seattle  
Cancer Care Alliance  
Seattle, Washington  
206.288.7222 • [seattlecca.org](http://seattlecca.org)  
206.667.5000 • [fredhutch.org](http://fredhutch.org)

The Sidney Kimmel Comprehensive  
Cancer Center at Johns Hopkins  
Baltimore, Maryland  
410.955.8964  
[hopkinskimmelfcancercenter.org](http://hopkinskimmelfcancercenter.org)

Robert H. Lurie Comprehensive  
Cancer Center of Northwestern  
University  
Chicago, Illinois  
866.587.4322  
[cancer.northwestern.edu](http://cancer.northwestern.edu)

Mayo Clinic Cancer Center  
Phoenix/Scottsdale, Arizona  
Jacksonville, Florida  
Rochester, Minnesota  
800.446.2279 • Arizona  
904.953.0853 • Florida  
507.538.3270 • Minnesota  
[mayoclinic.org/departments-centers/mayo-clinic-cancer-center](http://mayoclinic.org/departments-centers/mayo-clinic-cancer-center)

Memorial Sloan Kettering  
Cancer Center  
New York, New York  
800.525.2225  
[mskcc.org](http://mskcc.org)

Moffitt Cancer Center  
Tampa, Florida  
800.456.3434  
[moffitt.org](http://moffitt.org)

The Ohio State University  
Comprehensive Cancer Center -  
James Cancer Hospital and  
Solove Research Institute  
Columbus, Ohio  
800.293.5066  
[cancer.osu.edu](http://cancer.osu.edu)

O'Neal Comprehensive  
Cancer Center at UAB  
Birmingham, Alabama  
800.822.0933  
[uab.edu/onealcancercenter](http://uab.edu/onealcancercenter)

Roswell Park Comprehensive  
Cancer Center  
Buffalo, New York  
877.275.7724  
[roswellpark.org](http://roswellpark.org)

Siteman Cancer Center at Barnes-  
Jewish Hospital and Washington  
University School of Medicine  
St. Louis, Missouri  
800.600.3606  
[siteman.wustl.edu](http://siteman.wustl.edu)

St. Jude Children's Research Hospital  
The University of Tennessee  
Health Science Center  
Memphis, Tennessee  
888.226.4343 • [stjude.org](http://stjude.org)  
901.683.0055 • [westclinic.com](http://westclinic.com)

Stanford Cancer Institute  
Stanford, California  
877.668.7535  
[cancer.stanford.edu](http://cancer.stanford.edu)

UC San Diego Moores Cancer Center  
La Jolla, California  
858.657.7000  
[cancer.ucsd.edu](http://cancer.ucsd.edu)

UCSF Helen Diller Family  
Comprehensive Cancer Center  
San Francisco, California  
800.689.8273  
[cancer.ucsf.edu](http://cancer.ucsf.edu)

University of Colorado Cancer Center  
Aurora, Colorado  
720.848.0300  
[coloradocancercenter.org](http://coloradocancercenter.org)

University of Michigan  
Rogel Cancer Center  
Ann Arbor, Michigan  
800.865.1125  
[rogelcancercenter.org](http://rogelcancercenter.org)

The University of Texas  
MD Anderson Cancer Center  
Houston, Texas  
800.392.1611  
[mdanderson.org](http://mdanderson.org)

University of Wisconsin  
Carbone Cancer Center  
Madison, Wisconsin  
608.265.1700  
[uwhealth.org/cancer](http://uwhealth.org/cancer)

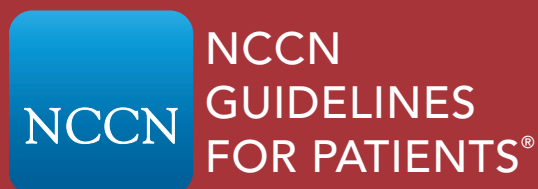
Vanderbilt-Ingram Cancer Center  
Nashville, Tennessee  
800.811.8480  
[vicc.org](http://vicc.org)

Yale Cancer Center/  
Smilow Cancer Hospital  
New Haven, Connecticut  
855.4.SMILOW  
[yalecancercenter.org](http://yalecancercenter.org)

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# Myeloproliferative Neoplasms

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