MPN Cancer CONNECTION

A Guide to Myelofibrosis (MF)

This resource is made possible by grant funding through CTI BioPharma

Things You Should Know

Whether you were just diagnosed, have been living with MF for some time, or you're looking for more information for a family member or friend, learning about MF can be overwhelming. Having a simple place to find information is important, which is why we designed this guide to help you understand the basics. We hope this is a helpful place to start.

Learn more about MF

- Defining MF
- Symptoms you may have
- How to understand your blood counts
- How your MF may change over time
- Treatment options
- How to talk to your doctor about MF

Defining MF

- MF is a type of blood cancer in a group of blood cancers called myeloproliferative neoplasms, or MPNs.
- Myelo means bone marrow and fibrosis means a buildup of scar tissue.
- Together, myelofibrosis means the buildup of scar tissue in bone marrow. When scar tissue takes up too much space in the bone marrow, the bone marrow can no longer make blood cells like it should.
- There are three main types of cells in your blood—red blood cells, white blood cells, and platelets.
- Having scar tissue may mean the bone marrow makes too much of one of these blood cells, or it may not make enough.

Causes of MF

Nobody knows exactly what causes MF. However, about **90% of patients with MF have a mutation in their genes**. A mutation in genes means a change in genes that is not passed down from your family.

Genes that may have mutations

- JAK2
- CALR
- MPL family

The two main phenotypes of MF

There are two phenotypes of MF. A phenotype is a characteristic of a disease—or how it behaves.

1 **Myelodepletive (cytopenic) MF**
When the body does not make **enough** of one or more types of blood cells

2 **Myeloproliferative MF**
when the body makes **too much** of one or more types of blood cells

Some people may have both phenotypes of MF at the same time. This means they may make too many of one type of blood cell and not enough of another. An example of this would be someone whose body makes too many red blood cells, but not enough platelets.

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Symptoms of MF

Symptoms of MF may be different from person to person. Your symptoms will depend on which type of MF you have and your risk level. We will talk more about risk level later in this guide. Some symptoms people with MF may have include:

- Pale skin
- Frequent infections
- Bleeding or bruising easily due to a low platelet count
- Pain in the stomach, feeling full, decreased appetite, and weight loss from having an enlarged spleen or enlarged liver
- Night sweats
- Itching skin
- Fever
- Bone or joint pain
- Weight loss

What your blood cell counts say about your MF

Blood cell counts tell you how many of each type of cell would be found within a certain amount of blood.

Important: Having counts above or below those listed here does not mean that you have MF. Any diagnoses should be made by a doctor.

People with **Myelodepletive (cytopenic) MF** have one or more blood cell counts that are **LOWER** than these normal ranges.

Blood cell type	Normal ranges per liter (L)
Red blood cells (RBCs)	**For males:** **4.3 to 5.9** trillion
	For females: **3.5 to 5.5** trillion
White blood cells (WBCs)	**4.5 to 11.0** billion
Platelets	**150 to 400** billion

People with **Myeloproliferative MF** have one or more blood cell counts that are **HIGHER** than these normal ranges.

Normal cell counts may be different amongst individuals. Please contact your doctor to determine if your levels are normal.

Review MF treatment options here.

See the discussion guide for ideas on how to talk to your doctor about your blood counts.

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MF Over Time

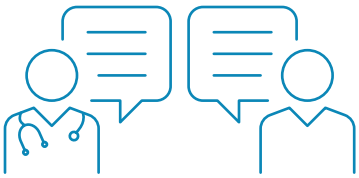
Either type of MF phenotype (cytopenic [myelodepletive]) or myeloproliferative can be either primary or secondary. Primary MF develops on its own, and secondary MF develops after having another type of MPN, like polycythemia vera or essential thrombocythemia. MF can be a progressive disease, which means that it may get worse over time. In some cases, it may develop into acute myeloid leukemia (AML).

- About **70%** of people with MF have **Primary MF**
- About **30%** of people with MF have **Secondary MF**

Low risk and intermediate or high risk

Some people with MF have a low risk of developing AML. People with low risk may not have symptoms.

Others with MF may be intermediate or high risk, which means they may need a treatment for their MF. They may also need help with their symptoms.



There are tools to help you learn your risk level. If you don't know your risk level, ask your doctor. **Your risk level can change over time.** It is also important to talk to your doctor about any changes in your health.

Treatment Depends on How Your MF Changes Over Time

The type of treatment a person needs depends on how their disease is now and how it is or is not progressing. For example, people over the age of 60 are at higher risk of their disease progressing. Learning about your risk level may help you and your doctor choose the right treatment for you.

Treatment depends (in part) on risk level

Low risk	Intermediate risk	High risk
Regular doctor visits to check for signs and symptoms	Regular doctor visits to check for signs and symptoms	Regular doctor visits to check for signs and symptoms
May or may not need treatment	Medical treatment should be a strong consideration	Should be receiving medical treatment
	Stem cell transplant may be right for some	Stem cell transplant may be right for some
		Supportive care to manage anemia or other complications

LEARN MORE **Learn more about MF treatment options here.**

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What Treatments are Available for MF?

Most medicines for MF aim to relieve the symptoms of the disease, but do not cure it.

The following are some common medicines used to treat symptoms of MF:

Medicine	Who uses this medicine?	What impact does it have on blood cell counts?	What are the possible benefits?	What are the possible side effects?	Specific benefits for people with anemia?
Ruxolitinib (Jakafi®)	• Used in people with low-risk MF • FDA approved for people with intermediate-risk and high-risk MF	• Can reduce platelet count • Can reduce red blood cell count	May reduce spleen size and improve spleen-related symptoms and fatigue	May cause low platelet count, low red blood cell counts, bruising, dizziness, and headache, risk of infection	No
Fedratinib (Inrebic®)	FDA approved for people with intermediate-risk-2 (INT-2) and high-risk MF	• Can reduce platelet count • Can reduce red blood cell count	Can reduce spleen size and symptom burden	Common side effects include low blood count, nausea, vomiting, diarrhea, loss of appetite and raised liver enzymes	No
Pacritinib (Vonjo™)	FDA approved for people with intermediate-risk and high-risk MF	Does not cause low white blood cell or low platelet counts	• Can stop the biological pathways that cause MF symptoms • Can reduce spleen volume without reducing platelet count	May cause diarrhea nausea, anemia, and swelling in legs	Yes
Hydroxyurea (Hydrea®)	Used in people with low-risk MF*	Can reduce blood cell counts (platelets, white blood cells, red blood cells)	May reduce spleen size	May cause below normal blood cell counts, fatigue, skin changes, diarrhea, constipation, skin cancer	No
Peginterferon alfa-2a (Pegasys®, Intron®A, Roferon®A)	Used in people with low-risk MF*	Can reduce blood cell counts (platelets, white blood cells, red blood cells)	Can treat an enlarged spleen, bone pain, itching, night sweats	May cause fatigue, joint or muscle pain, flu-like illness, itching, throat swelling, or depression	No

*Though Interferon alfa and Hydroxyurea are not FDA-approved for MF treatment, they are often used off-label by doctors in clinical practice to treat MF.

Other possible procedures

- **Stem cell transplant:** This is the only procedure that may cure MF, but it comes with with extreme risks, including risk of fatality. This procedure is not appropriate for most older people or those with other health concerns.
- Splenectomy (removal of the spleen) is performed only in extreme cases.
- Palliative care focuses on pain relief.

Having a low red blood cell count (also called anemia) can be a serious complication of MF. If you have anemia, you may need medicine that specifically treats anemia or a blood transfusion. **Be sure to ask your doctor about how to manage anemia.**

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Living with MF

Managing your MF will depend on your risk level, whether you have symptoms, and how your MF progresses over time. In many cases, treating symptoms is the most important thing. Always talk to your doctor about what the right next steps are for you. All people with MF should be monitored closely and visit their doctor regularly.

We understand the word cancer may feel overwhelming or scary. We have a list of resources to help you find the information you need during this time.

Find more treatment and support resources here.

You will find additional information about treatments, including copay assistance and other support resources.

Notes

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