LEUKEMIA & LYMPHOMA SOCIETY®
fighting blood cancers

MPN Options

Use this Guide to understand your treatment options for polycythemia vera, essential thrombocythemia and myelofibrosis.

POLYCYTHEMIA VERA (PV) TREATMENT OPTIONS

Low-Dose Aspirin	Low-dose aspirin may reduce the risk of blood clots and helps prevent platelets from sticking together, making it less likely for blood clots to form. The most common side effects are upset stomach and heartburn.
Therapeutic Phlebotomy Blood removed from the vein, similar to donating blood.	Phlebotomy reduces the number of blood cells and decreases blood volume. After phlebotomy, the blood is thinner and flows better. The immediate effect of phlebotomy is the decrease of certain symptoms such as headaches, ringing in the ears and dizziness. Eventually, however, phlebotomy results in iron deficiency.
Treatments to Reduce Itching A troublesome symptom that occurs in many PV patients is itchy skin (pruritus).	To help prevent pruritus, it is suggested that patients bathe less frequently. To reduce itching, patients should bathe or shower in cool water and use a gentle soap. Patients should also avoid hot tubs, heated whirlpools and hot showers or baths. It is also important to keep skin well moisturized with lotion and try not to scratch as it can damage the skin. Aspirin and antihistamines such as diphenhydramine (Benadryl®) may help itching that does not go away. Side effects of antihistamines include dry mouth, drowsiness, dizziness and restlessness. Other treatment options for itching include light therapy (phototherapy) using psoralen and ultraviolet A light. Interferon alpha or pegylated interferon may be effective.
Medications to Reduce the Number of Blood Cells	High-risk PV patients may be prescribed cytoreductive drugs to reduce the number of blood cells, which eliminates a need for phlebotomy and decreases the risk of clotting.
Busulfan (Myleran®) and Chlorambucil (Leukeran®) These drugs may be prescribed for patients who cannot tolerate other medications.	These drugs are associated with an increased risk of leukemia in PV patients and are only prescribed to treat PV when other therapies have not been effective in patients at least 70 to 80 years old. Due to the risk of leukemia, they are not prescribed to younger patients.

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Hydroxyurea (Hydrea®)

This chemotherapy drug is available as a pill. It is prescribed to help decrease the number of blood cells made in the bone marrow and to reduce the size of the spleen. It is used for high-risk patients as well as patients who cannot tolerate frequent phlebotomy and for patients with high blood counts and enlarged spleens.

Rare side effects are mouth ulcers, change in the sense of taste, skin ulcers or rash. There is some controversial data that long-term therapy with hydroxyurea is associated with an increased risk of acute leukemia, so it is frequently avoided as therapy for younger patients who have many years ahead of them.

Interferon alfa (Intron® A [alfa-2b] and Roferon®-A [alfa-2a] and sustained-release preparations PEG-Intron® [peginterferon alfa-2b] and Pegasys® [peginterferon alfa-2a])

Interferon is a biological agent used to stimulate the immune system to fight the overproduction of red blood cells. It may be used for patients who are either intolerant or resistant to hydroxyurea or for younger patients for whom hydroxyurea is not recommended. Interferon is not used for most patients because, compared to other treatments for PV, it is less convenient to administer (it is given by intramuscular or subcutaneous injection) and may cause troublesome side effects.

Some patients experience moderately severe flu-like symptoms, confusion, depression or other complications.

Ruxolitinib (Jakafi®)

Ruxolitinib is a JAK1/JAK2 inhibitor approved by the Food and Drug Administration (FDA) as a second-line therapy to treat people with PV who do not respond to or cannot take hydroxyurea. JAK proteins send signals that affect the production of blood cells in the bone marrow. When JAKs send too many signals, they cause the body to make too many blood cells. Ruxolitinib works by inhibiting the JAK proteins and reducing the overactive signaling. This oral drug is not associated with major toxicity and over time may slightly increase the risk of infectious complications such as pneumonia and urinary tract infections as well as shingles.

Clinical Trial

A carefully planned and monitored research study that tests how well new medical approaches work in patients. The goal is to develop new treatments, improve quality of life and increase survival. LLS Information Specialists can help conduct clinical-trial searches. When appropriate, personalized clinical-trial navigation by trained nurses is also available.

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ESSENTIAL THROMBOCYTHEMIA (ET) TREATMENT OPTIONS

Patients Being Observed Without Therapy	For patients with low-risk disease and no symptoms, the risk of complications may be low. The doctor may prescribed low-dose aspirin or no therapy at all. The doctor will monitor the patient closely through regular exams, watching for any signs of disease progression.
Low-Dose Aspirin	Low-dose aspirin may reduce the risk of clotting complications. Low dose aspirin helps prevent platelets from sticking together, making it less likely for patients to form blood clots which can cause heart attacks or strokes. The most common side effects include upset stomach and heartburn. Low-dose aspirin may also increase bleeding risk in patients with extremely high platelet counts. For these reasons, the use of aspirin in treating ET needs to be individualized.
Anagrelide (Agrylin®) This is a non-cytotoxic drug (an agent that does not kill cells) that decreases the body's production of platelets.	Anagrelide is not associated with an increased risk of leukemia, but may be considered as effective as hydroxyurea in reducing certain types of clots. Side effects of anagrelide include fluid retention, heart and blood pressure problems, headaches, dizziness, nausea and diarrhea.
Hydroxyurea (Hydrea®) This chemotherapy drug is available as a pill. It is prescribed to help decrease the number of blood cells made in the bone marrow.	Hydroxyurea is often successful in decreasing the platelet count within several weeks, with few short-term side effects. In some patients it may lower red blood cell counts, causing anemia; other rare side effects are mouth ulcers, change in the sense of taste, skin ulcers or rash. There is some controversial evidence that hydroxyurea is associated with an increased risk of developing acute leukemia after long-term therapy and is frequently avoided as therapy for younger patients who have many years of treatment ahead of them.
Interferon alfa (Intron® A [alfa-2b] and Roferon®-A [alfa-2a] and sustained-release preparations PEG-Intron® [peginterferon alfa-2b] and Pegasys® [peginterferon alfa-2a]) Interferon is a biological agent used to stimulate the immune system to fight the overproduction of red blood cells. It may be used for patients who are either intolerant or resistant to hydroxyurea or for younger patients for whom hydroxyurea is not recommended. However, it is not used in most patients because, in comparison with other treatments for ET, it is less convenient to administer—it is given by injection.	Interferon is a treatment for lowering platelet counts in patients with ET. It may cause troublesome side effects. Some patients experience moderately severe flu like symptoms, confusion, depression or other complications.
Busulfan (Busulfex®, Myleran®) and Pipobroman (Vercyte®) These drugs are used as second-line treatments in older patients who are unresponsive or intolerant to hydroxyurea.	These drugs are associated with an increased risk of developing acute leukemia after long-term use and are generally used in older patients.

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Plateletpheresis This is a process that uses a special machine to skim platelets from a patient's blood and then returns the remaining blood components to the patient.	This process is used only in emergency situations, such as acute clotting complications, when the platelet count is very high and needs to be reduced quickly. The platelet-reducing effect of this therapy is temporary.
Clinical Trial	A carefully planned and monitored research study that tests how well new medical approaches work in patients. The goal is to develop new treatments, improve quality of life and increase survival. LLS Information Specialists can help conduct clinical-trial searches. When appropriate, personalized clinical-trial navigation by trained nurses is also available.

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MYELOFIBROSIS (MF) TREATMENT OPTIONS

Asymptomatic Patients	Some people remain stable and symptom-free for many years. However, these patients need to be monitored closely through regular medical checkups and examinations to detect any signs and symptoms of disease progression.
Ruxolitinib (Jakafi®)	Ruxolitinib is a prescription JAK1/JAK2 inhibitor that is available as a pill. JAK proteins send signals that affect the production of blood cells in the bone marrow. When JAKs send too many signals, they cause the body to make too many blood cells. Ruxolitinib works by inhibiting the JAK proteins and reducing the overactive signaling. It is indicated for treatment of patients with intermediate- or high-risk myelofibrosis. The most common side effects are low platelet count, low red blood cell count, bruising, dizziness and headache. This oral drug is not associated with major toxicity and over time may slightly increase the risk of infectious complications such as pneumonia and urinary tract infections as well as shingles.
Interferon alfa (Intron A®, Roferon-A®, Pegasys®) Interferon is a biological agent used to stimulate the immune system to fight the overproduction of red blood cells. It is given by subcutaneous injection.	It has been used to treat an enlarged spleen, bone pain and high platelet count in selected MF patients. Due to its effects on the immune system, interferon alfa may worsen thyroid abnormalities, diabetes mellitus, or autoimmune disorders. Interferon alfa may also cause or worsen depression.
Splenectomy	Removal of the spleen may be considered if other forms of therapy have not reduced the pain or complications associated with an enlarged spleen. However, the benefits and risks of this procedure need to be weighed before a decision is made.
Stem Cell Transplantation In this procedure, the patient receives high doses of chemotherapy or radiation therapy to destroy the diseased bone marrow. Then, healthy hematopoietic (blood-forming) stem cells from a compatible donor (a related or unrelated person whose stem cells "match" the patient's) are infused into the patient. The transplanted healthy cells travel to the patient's bone marrow, replacing the defective stem cells. The new cells grow and provide a supply of red blood cells, white blood cells and platelets.	Allogeneic stem cell transplantation is the only current treatment with the potential to cure MF, but it also carries a high risk of life-threatening side effects. Whether or not a patient is a candidate for transplantation is determined by medical indications and the availability of a donor. Reduced-intensity or "nonmyeloablative" allogeneic stem cell transplantation is a type of transplant that is being used to treat some patients with MF. Compared to a standard allogeneic stem cell transplantation, a reduced-intensity transplant delivers lower doses of chemotherapy drugs and/or radiation to the patient in preparation for the transplant. This approach may benefit older and sicker patients who are unable to tolerate high doses of chemotherapy drugs used in a standard allogeneic stem cell transplantation.

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Radiation Therapy Radiation therapy uses high-powered beams, such as x-rays, to kill cancer cells.	Radiation may be useful for a small number of patients to treat an enlarged spleen, bone pain and tumors outside the marrow.
Clinical Trial	A carefully planned and monitored research study that tests how well new medical approaches work in patients. The goal is to develop new treatments, improve quality of life and increase survival. LLS Information Specialists can help conduct clinical-trial searches. When appropriate, personalized clinical-trial navigation by trained nurses is also available.

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