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2nd Edition

Myelofibrosis

A treatment guide for patients and their families

Support a healthy lifestyle through treatment

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For guide to ACUTE MYELOID LEUKEMIA

CONTENT REVIEWED BY A DISTINGUISHED MEDICAL ADVISORY BOARD

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Transform 1
M16-191

Myelofibrosis Research Study

Do you or someone you know have myelofibrosis?

The TRANSFORM-1 study is a research study evaluating how safe and well an investigational medication (navitoclax) works in combination with ruxolitinib (medication that is the current standard treatment) compared to ruxolitinib.

Participants must meet the following criteria:

- 18 years of age or older
- Diagnosed with primary myelofibrosis, post-polycythemia vera myelofibrosis, or post-essential thrombocythemia myelofibrosis
- Currently have Intermediate-2 or High-Risk myelofibrosis
- Have not received prior treatment with a JAK-2 inhibitor therapy
- Have splenomegaly (enlarged spleen)

For more information, ask your doctor about the TRANSFORM-1 Study or visit **www.clinicaltrials.gov** (NCT04472598) to see if you qualify.

You'll find further information about myelofibrosis at **www.myelofibrosisresearch.com**.

Navitoclax is an investigational medication that is not approved by the FDA or other global health authorities. Safety and efficacy have not been established.

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2nd Edition

Myelofibrosis

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Manage your diagnosis with knowledge and support

Discovering you have myelofibrosis (pronounced MY-eh-loh-fy-BROH-sis), a disease that many people have never heard of, can be overwhelming. Learning as much as you can about this rare disease and its treatment will empower you to make the decisions that are ahead. And know that you do not have to go through this alone. You will be surrounded by a multidisciplinary team of doctors and specialists who will provide support and guidance.

Myelofibrosis is a hematologic (blood) cancer that falls into the category of myeloproliferative neoplasms (MPNs), a group of cancers that affect the bone marrow. Following are the three main types of MPNs and their defining characteristics.

- Essential thrombocythemia (ET) — too many platelets
- Polycythemia vera (PV) — too many red blood cells
- Primary myelofibrosis — too many fibroblasts, which are connective tissue cells that make and secrete collagen proteins

This guide focuses on primary myelofibrosis and its treatment, side effects and ongoing supportive care.

UNDERSTANDING MYELOFIBROSIS

This rare cancer begins in the bone marrow when abnormal blood stem cells produce immature cells that grow quickly and take over. The immature cells become fibrous and fill the bone marrow with scar tissue. As a result, not enough normal blood cells can be made, and other organs, such as the spleen or liver, may try to compensate and become enlarged in the process.

Bone marrow is the soft, spongy center of some bones. It is where blood is created and is made up of blood stem cells, more mature blood-forming cells, fat cells and supporting tissues. Blood stem cells can become:

- Red blood cells that carry oxygen from the lungs to other parts of the body
- White blood cells that fight off infection and other foreign intruders in the body
- Platelets that help blood to clot and to stop bleeding

Both ET and PV can progress to myelofibrosis. When myelofibrosis develops from ET or PV, it is known as secondary myelofibrosis, which may also be referred to as either post-ET myelofibrosis or post-PV myelofibrosis. When myelofibrosis develops spontaneously, it is known as primary myelofibrosis. Although myelofibrosis is considered a chronic (slow-growing) blood cancer, in some cases it can transform to acute myeloid leukemia.

Myelofibrosis affects the development of red blood cells, white blood cells and platelets. They are often misshapen and immature so they cannot perform their normal function. As a result, there are low levels of red blood cells and too many white blood cells.

People may or may not have symptoms at diagnosis, and the progression of myelofibrosis can vary from person to person. The most common symptoms are fatigue, feeling full quickly, weight loss, fever, bone pain, night sweats, itching (especially after bathing), abdominal discomfort or bloating. In the absence of symptoms, blood tests may indicate abnormal amounts of cells that can prompt the need for further testing.

CLASSIFYING AND DETERMINING RISK

Multiple tests are used to diagnose myelofibrosis and to rule out other types of blood and bone marrow cancer that have similar features. Testing typically includes a physical exam, a symptom survey, blood tests, a bone marrow biopsy, and chromosome and specific gene testing, among others. Imaging studies to evaluate the size of the liver and spleen are also commonly included in the initial evaluation.

Along with examining your test results, your doctor will use a prognostic scoring system that determines your prognosis (outcome) based on risk factors. You will be placed into a risk category to better treat your individual symptoms.

The Dynamic International Prognostic Scoring System (DIPSS) Plus, which scores risk during treatment, is commonly used. It divides risk into four tiers: low risk, intermediate-1 risk, intermediate-2 risk and high risk. You will be treated based on your risk score and other factors (see *Treatment Planning*, page 4).

Multiple prognostic scoring systems exist. If you are unsure which one your doctor used, ask. Some differentiate between whether the patients are asymptomatic or symptomatic. ■

Molecular testing may assist with diagnosis and treatment planning

Researchers have discovered that many cancers are caused by genetic mutations. The results of genetic and molecular testing help your doctor to better personalize your treatment. By understanding which mutations (changes) in certain genes are present, your doctor can better treat your myelofibrosis and may revise the prognosis and treatment plan as any changes occur.

Identifying any genetic mutations associated with myelofibrosis will be a part of the diagnostic process. Blood tests will indicate whether levels of uric acid, bilirubin and lactate dehydrogenase (LDH) are elevated. Additional tests may be needed to confirm the diagnosis and may include cytogenetic analysis, which examines a sample of tissue, blood or bone marrow to look for changes in chromosomes, and molecular testing, which checks these samples for certain genes, proteins and other molecules.

Doctors do not know what causes myelofibrosis, but research has discovered that approximately half of patients with the disease have a mutation in the *JAK2* gene, which is the most common mutation found in people with myelofibrosis. Having this mutation may qualify you for targeted therapies designed to target that gene.

Other common mutations found in people with myelofibrosis include the calreticulin (*CALR*) gene and the myeloproliferative leukemia (*MPL*) gene. Less common gene mutations that are associated with myelofibrosis include *ASXL1, EZH2, TET2, IDH1, IDH2, SF3B1, SRSF2, TET2* and *U2AF1*. The *BCR-ABL1* gene is often tested to rule out chronic myelogenous leukemia (CML).

Some mutations may help predict how quickly myelofibrosis will progress. New risk scoring systems have been developed that include information from DIPSS Plus while also incorporating prognostic information from key mutations. Your doctor may discuss these additional scoring systems with you.

R E S E A R C H S T U D Y

Do you or someone you know have a Myeloproliferative Neoplasm (MPN) or Chronic Myelomonocytic Leukemia (CMML)?

We are conducting a research study to evaluate the safety and effectiveness of an investigational medication called navitoclax in adults with the following types of blood and bone marrow conditions: Polycythemia Vera (PV), Essential thrombocythemia (ET), Myelofibrosis (MF), and Chronic Myelomonocytic Leukemia (CMML).

Patients must meet the following criteria:

- 18 years of age or older
- Documented diagnosis of primary or secondary Myelofibrosis (MF), Essential Thrombocythemia (ET) or Polycythemia Vera (PV) or Chronic Myelomonocytic Leukemia (CMML)
- Have failed, are intolerant to or unwilling to use at least one standard treatment
- Additional study entrance criteria will apply

Scan here to be matched to a clinical trial

Navitoclax is an investigational drug that is being studied in myelofibrosis patients. It is not approved by the FDA or other global regulatory health authorities. Its safety and efficacy are being evaluated.

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Shared decision-making leads to personalized treatment

The introduction of new treatments over the past few years has helped more people manage their myelofibrosis and is providing them with a better quality of life. Furthermore, ongoing research in clinical trials is showing promise that more new treatments will be available for this rare cancer in the future.

Because myelofibrosis is such a rare cancer, both finding a local hematologist/oncologist and getting a referral to a blood cancer specialist with experience in myelofibrosis are recommended. You will likely also work with a multidisciplinary team that will co-manage your treatment, making partnership and shared decision-making important. The team may include a nurse or advanced practice provider, a stem cell transplant specialist, a pharmacist and others.

Myelofibrosis is often treated like a chronic condition that will progress. For the majority of patients, the main goal is to minimize their symptoms, which includes improving blood counts and reducing the chance myelofibrosis will progress to acute myeloid leukemia. The only potential cure for myelofibrosis is a stem cell transplant, but patients must meet certain criteria.

Your doctor will base your treatment on several factors – your symptoms, age and general health – that are combined into a risk score, which is assigned at diagnosis. The more aggressive the disease, the more aggressive the treatment is likely to be.

The Dynamic International Prognostic Scoring System (DIPSS) Plus includes four risk groups, ranging from low to high. In general, patients in the low-risk group, who may or may not have symptoms, may be treated with watchful waiting, a clinical trial or targeted therapy. Patients in the high-risk group may receive a stem cell transplant, targeted therapy or a clinical trial.

As you and your doctor discuss your treatment plan, ask about how the suggested therapies and potential side effects may best fit in with your expectations and your desired quality of life. Monitoring at follow-up appointments will be essential because as your disease changes, so, too, will your treatment.

The following treatment options may be used alone or in combination.

WATCHFUL WAITING

Instead of actively treating, this approach involves close monitoring. It may be considered if you have slow-growing disease, or if you have very mild or no symptoms. This option, typically for people who are in the lower risk groups, offers the possibility of avoiding the side effects of treatment as long as possible and, hopefully, without affecting the outcome. It is important to make and keep regular checkups to look for signs and symptoms because treatment should begin as soon as the disease progresses or symptoms appear.

STEM CELL TRANSPLANTATION

A stem cell transplant is the only potential cure for myelofibrosis. This procedure replaces a patient's own stem cells with healthy stem cells. It may be considered risky for some older patients who have other health problems, so if you are considering one, you are encouraged to find a doctor and a transplant center with extensive experience. This may require traveling if you are not located near a transplant center. Some treatment centers offer financial assistance with travel and temporary lodging during treatment. Talk with your health care team at the transplant center and advocacy organizations to learn about the resources available to assist you.

The type commonly used for myelofibrosis is an allogeneic stem cell transplant. It uses stem cells donated by a family member or an unrelated donor identified through a national or international registry. Other less-matched donor options are under investigation and in clinical trials now to provide access for patients who have no available matched donor. The potential for cure with allogeneic transplantation comes from donor stem cells working directly against the cancer through the graft-versus-tumor effect (also called graft-versus-leukemia or graft-versus-cancer-cell). For the donor stem cells to be effective, transplant recipients must first receive a conditioning treatment, consisting of chemotherapy and possibly radiation therapy, to weaken their own stem cells and allow the donor stem cells to replace them and start fighting the cancer. High-dose or myeloablative conditioning treatments are often reserved for transplant candidates in excellent health and with very active and aggressive cancer.

For older, sicker patients who may not be eligible for a higher intensity transplant, a non-myeloablative stem cell transplant or mini-transplant may be an option. A reduced-intensity conditioning treatment is often used to spare some patients from the very high doses of chemotherapy. This procedure uses milder doses of chemotherapy and radiation therapy for conditioning prior to the transplant. The potential success of this approach depends entirely on the anti-cancer effect of the new immune system transplanted into the patient.

The allogeneic stem cell transplantation process has four distinct phases:

1. **Donor identification and evaluation**, which includes tissue typing of related and unrelated donors, selecting the best match and requesting a thorough health evaluation of the donor as well as availability for the transplant time frame.
2. **Conditioning**, which includes chemotherapy and radiation therapy.
3. **Stem cell infusion**, during which the harvested donor stem cells are infused into the recipient's body intravenously (through a vein).
4. **Recovery and engraftment**, in which healthy cells begin to grow. There are typically signs of this in the first 30 days, but an enlarged spleen and scar tissue in the bone marrow, common in patients with myelofibrosis, can delay the engraftment process compared to that for other diseases. The patient will be at risk for bleeding and infection while the weakened immune system recovers. This process may take multiple years and will require ongoing use of prophylactic anti-viral and anti-bacterial medications as well as repeat inoculations with childhood vaccines. The number of red cells, white cells and platelets will continue to be monitored until they are back to safe levels. Long-term monitoring and management of Graft-versus-Host

Disease will also occur, if applicable. Transplant recipients also remain at risk for chronic GvHD and may require lifelong treatment for this condition.

Talk with your doctor about the benefits and risks so you know what to expect with this treatment option. This includes potential short-term or long-term side effects, as well as the amount of help you will need from a caregiver. Once you are home, you may need help. If a family member or friend cannot help, you may need to consider hiring a temporary caregiver.

CLINICAL TRIALS

Medical research studies explore new treatment strategies and combinations for myelofibrosis. Trials may offer access to new therapies not yet approved and may be an option to consider for first-line treatment (before any other treatment is given), or if the current treatment is not effective.

A variety of trials are underway to identify new mutations, create new types of therapy or combinations, improve treatments for side effects and more. Ask your doctor if you should consider a trial immediately after diagnosis or at any other time.

DRUG THERAPY

Targeted therapy uses drugs or other substances to slow or stop the progression of disease. These are drugs that travel throughout the body via the bloodstream. Targeted therapy is intended to affect primarily cancer cells, leaving healthy cells alone. These therapies for myelofibrosis are given orally as a pill.

The type of targeted therapy approved blocks a type of enzyme called a kinase. In myelofibrosis, the *JAK1* and/or *JAK2* enzymes are involved with blood cell production. The approved therapies are kinase inhibitors that target *JAK1* and *JAK2*. Blocking these enzymes helps prevent cancer cells from growing.

This treatment is intended to decrease spleen size and improve symptoms and does not alter the natural course of this illness. Because the currently approved targeted therapies can worsen low blood counts, they are typically restricted to patients with only mild anemia or thrombocytopenia, which can be side effects of treatment or the disease.

Chemotherapy uses drugs to kill rapidly growing cells throughout the body. It may be given intravenously (IV) into a vein or taken orally as a pill. It is often used in high doses to prepare for a stem cell transplant. In some cases, it is used to treat symptoms of anemia or to reduce high platelet and white blood cell counts.

Immunotherapy in the form of immunomodulators can be used to slow the growth of cancer by reducing the number of blood cells.

COMMON DRUG THERAPIES

- fedratinib (Inrebic)
- hydroxyurea
- interferon
- ruxolitinib (Jakafi)

As of 10/12/21

Corticosteroids are drugs used to treat some blood cancers and can help nausea, vomiting, weight loss, fatigue, night sweats and fevers. They can be used alone or in combination with other types of antiemetic (anti-nausea) therapy. They are given orally or intravenously (IV).

RADIATION THERAPY

High-energy radiation to destroy cancer cells and shrink tumors may be given to prepare for an allogeneic stem cell transplant, reduce the size of the spleen, decrease bone pain or shrink tumors that have developed outside of the marrow.

SURGERY

This treatment is primarily used to remove the spleen (a procedure called a splenectomy) if it becomes very large, is causing anemia or is lowering platelet counts. ■

ONGOING CARE

Follow-up appointments and symptom management are key

Managing a chronic disease requires regular monitoring. It is especially important with myelofibrosis because other symptoms and side effects may develop throughout the course of the disease that will need to be addressed. Some of the most common symptoms include anemia, enlarged spleen, fatigue and abnormal blood cell counts. Talk with your doctor about setting up a schedule for follow-up appointments. Be sure to let your medical team know about new symptoms that occur between appointments.

The following therapies may be included to manage symptoms from myelofibrosis or its treatment.

Androgens are a type of hormone therapy that promotes the development of male hormones. They may be given to improve anemia.

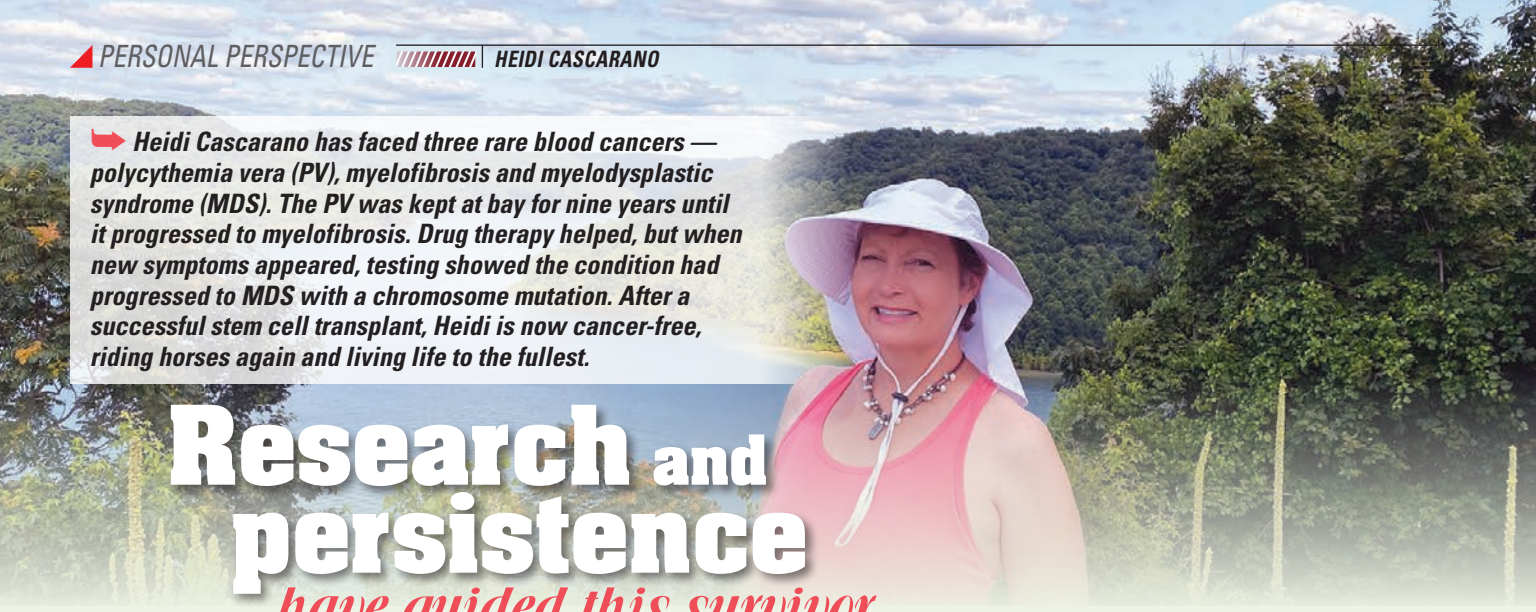
Blood transfusions are given for severe anemia. Blood donated by another person may be given intravenously (IV) to a patient through a vein in the arm.

Erythropoietin is a growth factor designed to stimulate bone marrow stem cells to make red blood cells, which may improve anemia. Your doctor will check your erythropoietin level to determine if you may benefit from this therapy.

Granulocyte colony stimulating factors and **granulocyte macrophage stimulating factors** are drugs that may be administered to help the body make white blood cells other than lymphocytes. White blood cells are often damaged by treatment, which can increase the risk of infection.

Phlebotomy is the removal of blood from the body through a vein in the arm. This is sometimes used to remove extra red blood cells.

Platelet transfusions may be given to a patient with a very low platelet count. Platelets donated by another person may be given intravenously to a patient through a vein in their arm. ■



Heidi Cascarano has faced three rare blood cancers — polycythemia vera (PV), myelofibrosis and myelodysplastic syndrome (MDS). The PV was kept at bay for nine years until it progressed to myelofibrosis. Drug therapy helped, but when new symptoms appeared, testing showed the condition had progressed to MDS with a chromosome mutation. After a successful stem cell transplant, Heidi is now cancer-free, riding horses again and living life to the fullest.

Research and persistence *have guided this survivor*

At my annual physical in 2008, my bloodwork showed abnormal numbers, and I was referred to a hematologist. He ran more tests and found the *JAK2* mutation was present in my blood. I was diagnosed with polycythemia vera (PV).

The diagnosis was shocking. I was scared and feeling alone living in Chicago and missing my family in Tennessee. Then I lost my job and my health insurance ran out, so I paid for my treatments with cash. I was originally pre-med and had some medical knowledge, but I didn't know anything about PV. So, I turned to the internet, but statistics online were scary. Life expectancy was not good, and there was an increased risk the PV could progress to myelofibrosis.

The hematologist recommended I start oral chemotherapy, but he admitted it would not reverse the disease and it would come with a lot of side effects. I only took one pill before deciding I wanted a second opinion.

The next doctor I met with recommended a treatment plan consisting of watching and waiting for symptoms while taking phlebotomies to reduce my red blood cell count and baby aspirin to prevent blood clots. PV can cause the blood to thicken and clot easily, so phlebotomies are used to reduce the number of red blood cells.

I followed that plan and, while I adjusted to living life with PV, I found several online support groups. The MPN Research Foundation and other sources offered great information. I also became more involved with my church and developed a stronger relationship with God.

In one of my groups, others were discussing a type of immunotherapy that could reverse fibrosis in bone marrow. My goal became to reduce or eliminate as much of the fibrosis as possible to prevent progression to myelofibrosis. I asked about trying immunotherapy, but the doctor felt the side effects would be worse than the disease. I trusted his decision and went on with my life.

Then over time, I started feeling more fatigued than I should and developed mild bone pain, itching and night sweats. I suspected something was wrong, so I asked for a bone marrow biopsy to confirm, but he didn't think I needed one. I realized I needed a specialist who had experience with treating MPNs.

The specialist I found was wonderful. He ordered a bone marrow biopsy that revealed the PV had progressed to myelofibrosis. We decided to try immunotherapy, but it didn't control it completely. We eventually switched to a targeted therapy. I felt much better on it, but it didn't improve my white blood cell count, which remained low.

Looking for more options, I read that some doctors were combining immunotherapy with targeted therapy. I asked the doctor if I could take both. He feared the two would cancel each other out because one is an immune stimulant and the other is an immune suppressant. I was persistent, and he eventually agreed. It was successful, and I showed a great response.

About four years later, I started getting constant mouth sores. My gut told me something was wrong. Another bone marrow biopsy revealed I had a chromosome 7 deletion. I now had myelodysplastic syndrome (MDS) with a prognosis of only 18 months. That's when my doctor and I discussed a stem cell transplant to try to save my life.

Six of my siblings were tested. None of them were a complete match, but my brother Carl was a 7/10 match, so we went with him. I am so grateful Carl made this sacrifice for me. Having the stem cell transplant brought us closer than we had ever been.

After the procedure, I spent a month in the hospital. The transplant reached 100 percent engraftment in three weeks, which was fantastic news. However, I developed ocular Graft-vs-Host Disease. My doctor sent me to the best GvHD specialists, and they treated it aggressively with steroid eye drops and autologous serum tears. I still take maintenance medications but am being weaned off them.

The last *JAK2* test revealed I had no detectable mutations, and I'm considered cancer-free. I moved back to Tennessee and bought a horse. I had ridden when I was younger, but started up again with riding lessons to get comfortable. A year later, I was back to jumping. ■

Heidi's Advice...

Listen to your gut. You know your body best. Educate yourself about the disease so you know what questions to ask.

Accept what has happened so you can make a plan to move forward. Sticking your head in the sand will not make the cancer go away.

Be proactive. Find the best doctors who have experience treating your type of cancer. An MPN specialist is incredibly valuable.

Speak up for yourself, especially if you want to try something different.

Find support, whether that's through friends or online groups. Talking with others going through a similar experience is helpful and educational.

TRANSFORM 2
M20-178

Myelofibrosis Research Study

Do you or someone you know have Relapsed/Refractory Myelofibrosis?

The TRANSFORM-2 study is a research study evaluating the efficacy and safety of an investigational medication (navitoclax) in combination with ruxolitinib compared to best available therapy.

Patients must meet the following criteria:

- 18 years of age or older
- Diagnosed with primary myelofibrosis, post-polycythemia vera myelofibrosis, or post-essential thrombocythemia myelofibrosis
- Currently have Intermediate-2 or High-Risk myelofibrosis
- Have received prior treatment with a single JAK2 inhibitor therapy
- Have splenomegaly (enlarged spleen)

For more information, ask your doctor about the TRANSFORM-2 Study or visit **www.myelofibrosisresearch.com** and **www.clinicaltrials.gov** (NCT04468984) to see if you qualify.

Navitoclax is an investigational drug that is not approved by the FDA or any other global health authority. Safety and efficacy have not been established.

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Abbvie, Inc.

Research holds the promise of more new therapies

Today, thousands of clinical trials across the country and around the world are evaluating new and better ways to prevent, diagnose and treat cancer, its symptoms and side effects. And they are bringing new hope to people diagnosed with myelofibrosis. The most recent treatment advances were made possible by people who participated in clinical trials.

Clinical trials are structured research studies that test the safety and effectiveness of new medical approaches or interventions. They evaluate methods for disease prevention and patient screening; tools and procedures to diagnose disease; new or improved treatments such as drugs or drug combinations, medical procedures or devices; and lifestyle or behavioral changes that may improve health and/or quality of life.

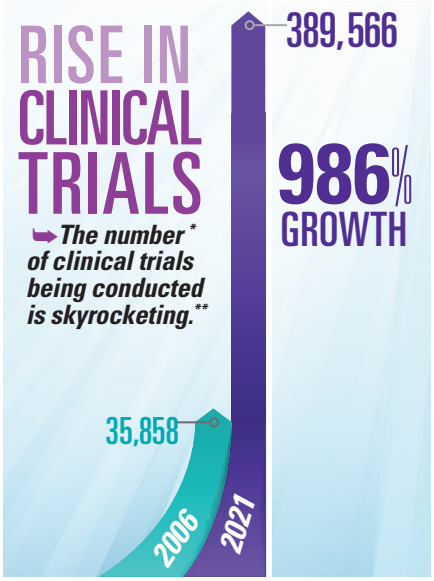
These carefully planned and structured studies are evolving and expanding to accommodate precision medicine, a personalized approach to treating and managing disease that considers a person's genetic variants and other unique factors. For more information about clinical trials and how to search for them, flip over this guide and read *Clinical Trials*, page 6.

CLINICAL TRIALS FOR MYELOFIBROSIS

Because myelofibrosis is a rare cancer, many trials are underway to research new and improved ways to treat it. Currently, the only treatment capable of curing myelofibrosis is a stem cell transplant. For people who are not matched with a compatible donor or who are not eligible for a transplant, the next best option may be a clinical trial that is investigating other less-matched donors and other non-transplant therapies. The most recent advancement is the approval of *JAK* inhibitors, which were introduced within the last 10 years.

Additional research being conducted for myelofibrosis and the management of its side effects include the following:

- Finding additional targeted therapies that target new mutations.
- Examining drugs that have been approved for myelodysplastic syndrome for use in people with myelofibrosis.
- Focusing on therapies that could be used in patients with very early disease or very advanced disease.
- Evaluating whether early intervention can reduce the risk of progression.
- Understanding how the interactions of molecular mutations can impact the disease's progression and whether these mechanisms can be targeted in a more effective way.
- Developing better ways to treat anemia.
- Determining whether the bone marrow fibrosis can be reversed.
- Exploring the use of immune checkpoint inhibitors, a type of immunotherapy.



▲ **This graph shows the total number of studies posted on ClinicalTrials.gov since the first posted date, February 29, 2000.**

**ClinicalTrials.gov, as of September 15, 2021.*
***All clinical trials, not exclusive to cancer.*

CHOOSING TO PARTICIPATE IN A CLINICAL TRIAL

Deciding whether to pursue a clinical trial as a treatment choice takes much consideration, and the element of uncertainty can cause some anxiety. Understanding more about the clinical trial process may make you more comfortable. Consult with your doctor, ask questions and consider getting a second opinion. You can also reach out to current or former clinical trial participants through local or online cancer support groups, as well as conduct your own online research.

Some cancer centers have a clinical trial coordinator who can help find trials. The trial coordinator will know all aspects of the study, explain eligibility requirements and help address any concerns. Ask the coordinator to provide additional information or printed materials, such as results from previous phases of the trial and the trial sponsor's track record on cancer drug development. An abundance of active clinical trials for myelofibrosis can be found at www.clinicaltrials.gov and below. It might be best to review these trials with your treating physician to determine whether there is one most suitable for you.

WHAT IS INFORMED CONSENT?

Once you express interest in a clinical trial, you will receive comprehensive information in a document known as an Informed Consent form. It details the purpose of the research, including your role in the trial, the treatment to be studied, how the trial will work, risks and benefits. The form also explains how you will be monitored, potential side effects of the treatment, the current standard of care for your type and stage of cancer, the safeguards in place to protect you, how to withdraw from the trial, alternative options and a detailed list of the costs the trial sponsor will cover.

The trial's medical team or administrator will go through the information with you. It's very important to get clear answers to all your questions. Signing the Informed Consent form does not lock you in to the trial or require that you participate. If you decide you no longer want to be involved, you may withdraw at any time and return to the standard-of-care treatment.

Signing the form also does not guarantee you will have a spot in the clinical trial. Each research study has its own unique eligibility criteria, such as cancer type, subtype, stage, biomarker or treatment history. Your age, gender and any additional health conditions may also be factors. ■

CLINICAL TRIALS

- **Be the Match | Jason Carter Clinical Trials Program** www.ctsearchsupport.org, 888-814-8610
- **Cancer Support Community** www.cancersupportcommunity.org/find-clinical-trial, 888-793-9355
- **Center for Information & Study on Clinical Research Participation** www.searchclinicaltrials.org
- **CenterWatch** www.centerwatch.com, 866-219-3440 ▸ **ClinicalTrials.gov** www.clinicaltrials.gov
- **Lazarex Cancer Foundation** www.lazarex.org, 877-866-9523, 925-820-4517
- **The Leukemia & Lymphoma Society** www.lls.org/treatment/types-of-treatment/clinical-trials/finding-a-clinical-trial ▸ **National Cancer Institute** www.cancer.gov/clinicaltrials, 800-422-6237
- **NCI Cancer Information Service** 800-422-6237

Effective monitoring requires frequent communication

Managing the side effects of myelofibrosis and its treatment is a primary focus of your multidisciplinary health care team members. The goal is to help you maintain a good quality of life beginning at diagnosis. They do this by drawing on a broad range of services known as supportive care.

Supportive care addresses the physical, emotional, practical, spiritual, financial and family-related challenges of people diagnosed with cancer. This includes assisting your children, family members, caregivers and others close to you. To access supportive care, start with your doctor's office. They will recommend resources that may include side effect and pain management; counseling about nutrition, fitness, mental health or spirituality; physical/occupational therapy; speech therapy; complementary medicine and others.

Feeling nervous or concerned about potential side effects is normal. It may reassure you to know that there are many ways to prevent and manage them. Knowing what to expect can help you prepare, so you are encouraged to discuss all possible side effects of each treatment with your doctor. And, keep in mind that you likely will not experience all of them because people respond differently, even if they have the same diagnosis and type of treatment.

To get the most out of supportive care, you can take an active role by keeping the lines of communication open with your health care team. Let them know when a side effect or symptom begins so it can be treated or managed before it gets worse.

POTENTIALLY SEVERE SIDE EFFECTS

Though serious side effects are rare, they can occur with certain treatments. Ask your doctor whether you are at risk, how to identify the symptoms that signal a severe side effect and when to seek emergency care. Report symptoms immediately so they can be treated right away.

- **Bleeding problems** (hemorrhages) and **bruising** may occur. Inform your health care team about any history of bleeding problems, and contact them immediately if you experience any of these symptoms: blood in your stools or black, tar-like stools; pink or brown urine; unexpected bleeding or severe bleeding you cannot control; vomit that looks like coffee grounds; coughing up blood or blood clots; increased bruising, dizziness, weakness or confusion; changes in speech; or a long-lasting headache.
- **Infection** can occur as a result of a low white blood cell count (neutropenia) or other factors. Contact your doctor immediately – do not wait until the next day – if you have any of these symptoms: oral temperature over 100.4° F, chills or sweating; body aches, chills and fatigue with or without fever; coughing, shortness of breath or painful breathing; abdominal pain; sore throat; mouth sores; painful, swollen or reddened skin; pus or drainage from an open cut or sore; pain or burning during urination; pain or sores around the anus; or vaginal discharge or itching.
- **Tumor lysis syndrome** (TLS) may occur after treatment of a fast-growing cancer, especially certain blood cancers. As tumor cells die, they break apart and release their contents into the blood. This causes a change in certain chemicals in the blood, which may cause damage to organs. There may also be worsening of your kidney function or an increase in the level of potassium in the blood. TLS can potentially cause damage to the kidneys, heart, liver or other organs. Symptoms may include vomiting, diarrhea, muscle cramps or twitches, neuropathy and decreased urination.

COMMON SIDE EFFECTS

Some of the common physical side effects of myelofibrosis and its treatment include abdominal pain, anemia, bone pain, diarrhea, fatigue, gout, Graft-versus-Host Disease (GvHD), headaches, leukocytosis (increased level of white blood cells), mouth sores, nausea and vomiting, pneumonitis (inflammation of the lung) and thrombocytopenia (low platelets).

Treatment may also affect you emotionally, bringing up anger, fear, anxiety, depression or loneliness. These feelings are normal and should be addressed. Taking care of your emotional well-being will help you manage physical side effects better. Get immediate medical attention for thoughts of suicide or death.

FINDING SUPPORT

The following types of support are also available to ensure your whole person is being cared for. If you have challenges in an area not listed here, talk with your health care team.

Social support is available in many forms. You may choose to speak with a therapist or attend an online, telephone or in-person group. Many advocacy programs offer one-on-one buddy programs that pair you with another peron who has the same type of cancer as you.

Spiritual guidance may be provided by a chaplain or spiritual care advisor at the hospital or from your religious community. Spiritual support is available to you even if you do not consider yourself a religious person.

Financial counseling may be accessible from a social worker or financial counselor. The stress and anxiety of paying for treatment and other related expenses can negatively affect your well-being. Understanding the costs ahead and making a plan can help you feel more in control. ■

MANAGING FATIGUE CAUSED BY ANEMIA

One of the biggest challenges with myelofibrosis is controlling the fatigue that results from it. Because myelofibrosis interferes with the development of healthy blood cells, not enough red blood cells are made, causing anemia. The main symptoms of anemia include fatigue and shortness of breath.

Part of your supportive care will likely include treatment for anemia. Therapies that may be used include androgens, blood transfusions, corticosteroids, erythropoiesis stimulating agents and immunomodulators.

Actions you can take to ease fatigue include the following:

- **Get enough sleep**
- **Take a walk, even for a few minutes**
- **Eat a well-balanced diet**
- **Engage in activities that you enjoy**

Because more therapies are needed, several other therapies for anemia are also being researched in clinical trials. These include novel *JAK* inhibitors, antifibrotic agents to reverse the development of bone marrow scarring, and growth factors.

Consider asking your doctor if you may be a candidate for a clinical trial that is exploring treatment options for anemia.

Prepare yourself to embrace the valuable role of caregiver

Stepping into the role of a caregiver may have been unexpected, but your support and dedicated efforts can make your loved one's myelofibrosis diagnosis more bearable. You may take on a variety of tasks and handle different responsibilities. Following are some suggestions for how you can contribute to your loved one's quality of life.

Empower your loved one to ask questions. He or she may feel afraid or unsure of speaking up and asking questions and may rely on your encouragement to learn about the disease and its treatment options.

Discuss concerns you both have before an appointment. Write a list of questions to share with the medical team.

Attend and participate in appointments. Telehealth appointments might be offered due to some centers having COVID-19 restrictions. You may be able to offer valuable information and insights about your loved one at appointments.

Track medications. Cancer medications are most effective when taken exactly as prescribed. Help your loved one stay on schedule with medications taken at home and those given at medical appointments. Use a calendar or reminder tool.

Encourage your loved one to share feelings and concerns. This may help you stay alert to physical and emotional changes that you can share with the medical team.

Learn what symptoms require a call to the doctor. Pay attention to changes such as fever, night sweats, bruising, weight loss or not eating.

Reduce the risk of infection. Myelofibrosis affects a person's blood counts and may make them susceptible to infections. Do your part by encouraging social distancing and wearing masks. Consider getting the COVID-19 vaccine to further protect your loved one.

Shop, cook and provide personal assistance and transportation. Side effects, such as fatigue, may prevent your loved one from taking care of these and other daily tasks.

Manage expectations. Don't feel pressure to get every question answered at a visit. Consider using email and portal systems between appointments. Also try to attend follow-ups with medical staff to close loops.

Take care of yourself. Rest, eat right, exercise and keep your own medical appointments to help you better care for your loved one. ■

Put these financial resources to work for you

PRESCRIPTION EXPENSES

America's Pharmacy ..... www.americaspharmacy.com, 888-495-3181
The Bone Marrow & Cancer Foundation ..... www.bonemarrow.org, 800-365-1336
Cancer*Care* Co-Payment Assistance Foundation ..... www.cancercarecopay.org, 866-552-6729
Cancer Financial Assistance Coalition ..... www.cancerfac.org
Children's Leukemia Research Association ..... www.childrensleukemia.org, 516-222-1944
Good Days ..... www.mygooddays.org, 972-608-7141
HealthWell Foundation ..... www.healthwellfoundation.org, 800-675-8416
The Leukemia & Lymphoma Society ..... www.lls.org, 800-955-4572
Medicine Assistance Tool ..... www.medicineassistancetool.org
National Organization for Rare Disorders ..... www.rarediseases.org, 203-744-0100
NeedyMeds ..... www.needymeds.org, 800-503-6897
Patient Access Network Foundation ..... www.panfoundation.org, 866-316-7263
Patient Advocate Foundation Co-Pay Relief ..... www.copays.org, 866-512-3861
RxAssist ..... www.rxassist.org
RxHope ..... www.rxhope.org
SingleCare ..... www.singlecare.com, 844-234-3057
Stupid Cancer ..... www.stupidcancer.org, 212-619-1040
Together Rx Access ..... www.togetherrxaccess.com, 800-444-4106

REIMBURSEMENT & PATIENT ASSISTANCE PROGRAMS

myAbbVie Assist ..... www.abbvie.com/patients/patient-assistance, 800-222-6885
Astellas Pharma Support Solutions ..... astellaspharmasupportsolutions.com/patient, 800-477-6472
Bristol-Myers Squibb ..... bms.com/patient-and-caregivers/get-help-paying-for-your-medicines.html, 800-721-8909
Bristol-Myers Squibb Access Support ..... bmsaccesssupport.bmscustomerconnect.com/patient, 800-721-8909
Bristol-Myers Squibb Patient Assistance Foundation ..... bmspaf.org, 800-736-0003
Daurismo Financial Assistance ..... pfizeroncologytogether.com/patient/financial-assistance, 877-744-5675
Genentech Access Solutions ..... genentech-access.com/patient, 877-436-3683
Genentech BioOncology Co-pay Assistance Program ..... copayassistancenow.com, 855-692-6729
Genentech Patient Foundation ..... gene.com/patients/patient-foundation, 888-941-3331
Idhifa Patient Support ..... www.bmsaccesssupport.bmscustomerconnect.com/patient, 800-721-8909
IncyteCARES for Jakafi ..... www.incytecares.com/jakafi/home.aspx, 855-452-5234
Inrebic Patient Support ..... www.bmsaccesssupport.bmscustomerconnect.com/patient, 800-721-8909
JazzCares ..... jazzcares.com, 833-533-5299
Mylotarg Financial Assistance ..... www.pfizeroncologytogether.com/patient/financial-assistance, 877-744-5675
Novartis Oncology Universal Co-pay Program ..... copay.novartisoncology.com, 877-577-7756
Novartis Patient Assistance Foundation ..... www.novartis.us/our-products/patient-assistance/patient-assistance-foundation-enrollment, 800-277-2254
Novartis Patient Assistance Now Oncology (PANO) ..... www.patient.novartisoncology.com/financial-assistance/pano, 800-282-7630
Onureg Patient Assistance ..... www.bmsaccesssupport.bmscustomerconnect.com/patient, 800-721-8909
Pfizer Oncology Together ..... pfizeroncologytogether.com/patient, 877-744-5675
Pfizer RxPathways ..... pfizerrxpathways.com, 844-989-7284
Rydapt NOW Access ..... us.rydapt.com/acute-myeloid-leukemia/patient-support/financial-resources, 800-282-7630
Tibsovo Financial Assistance ..... myagios.com/patient/paying-for-tibsovo.html, 844-409-1141
Venclexta Access Solutions ..... genentech-access.com/patient/brands/venclexta, 877-436-3638
Vyxeos Patient Support ..... jazzcares.com/patients/vyxeos/, 833-533-5299
Xospata Support Solutions ..... astellaspharmasupportsolutions.com/products/xospata/patient_assistance, 844-632-9272

TRAVEL RESOURCES

Air Charity Network ..... www.aircharitynetwork.org, 877-621-7177
American Cancer Society (Hope Lodge) ..... www.cancer.org/hopelodge, 800-227-2345
Angel Flight Central ..... www.angelflightcentral.org, 866-569-9464
Joe's House ..... www.joeshouse.org, 877-563-7468

➥ ***For Financial Resources,*** see opposite page. For more resources, go to PatientResource.com