- ESSENTIAL THROMBOCYTHEMIA
- POLYCYTHEMIA VERA
- MYELOFIBROSIS

Pediatric & Young Adult MPN™

MYELOPROLIFERATIVE NEOPLASMS

MPN ADVOCACY & EDUCATION INTERNATIONAL

mpnadvocacy.com

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Ann Brazeau, CEO

Kathleen Michael
Vice President

Dr. Nicole Kucine, MD,MS
Weill Cornell Medicine
MPN ZPediatric Advisor

MPN Advocacy & Education International

MPN Advocacy and Education International provides educational programs, materials, and resources for patients, caregivers, physicians, and entire healthcare teams to improve their understanding of myelofibrosis, polycythemia vera, and essential thrombocythemia. They are dedicated to making a difference in the lives of those affected by MPNs and strive to grow awareness and advocate on behalf of the MPN community.

Advocacy

Our advocacy efforts extend beyond responding to the unmet needs of the MPN Community. We identify concerns in a meaningful and productive way and create initiatives that impact quality care, treatment access, new drug development and represent MPN patients and organizations who are unable to address the issues surrounding a blood cancer diagnosis. Women and MPN and Pediatric and Young Adult initiatives have expanded the interest and exploration into the unmet needs of these patient groups.

Education

Dr. Nicole Kucine, MD, MS is our pediatric advisor and frequent speaker at our educational programs. Her website is at: https://pediatric-mpn.weill.cornell.edu/. MPN Education programs are held across the country and internationally each year. Our speakers are MPN specialists who share updated information on research, clinical trials, treatment options, and comprehensive quality of life direction.

Please visit our website at www.mpnadvocacy.com for more information on events, advocacy initiatives, patient support groups in your area and numerous resources.

MPN | GENERAL INFORMATION

Myelo – prefix referring to bone marrow

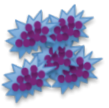
Proliferative – increasing the numbers of cells

Neoplasm – any new and abnormal growth, where cell multiplication is uncontrolled and progressive.

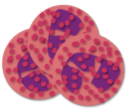
MYELOPROLIFERATIVE NEOPLASMS

Myeloproliferative neoplasms (MPNs) are chronic blood and bone marrow disorders. They involve overproduction of one or more bone marrow components. The bone marrow is the spongy material inside your bones where blood cells are made. Things that can overgrow in the bone marrow include:

Red blood cells



Platelets



White blood cells

(like scar tissue)

Fibrous tissue

MPNs are genetic diseases. Genes are made up of DNA and carry the necessary information to make all the different parts of our bodies. The body knows how to develop and work properly because the DNA has a "code" that exists in a particular order. When parts of the DNA's code get out of order, or there is too much, or missing pieces, of that code, a gene develops what is called a mutation. Certain gene mutations can cause an MPN. The mutations that cause MPN are usually thought to be "sporadic", or mutations that randomly appear and are not passed from parent to child, but there are some reports of children born with an MPN mutation and some families can be predisposed to developing MPN mutations and have multiple family members with MPNs.

Most Frequent

The most frequently mutated gene in MPNs is the JAK2 gene. This is found in the majority of patients with PV, as well as a large number of patients with ET and MF. CALR is another gene that can be mutated in ET and MF, as is MPL (although less frequently than CALR.) If a patient does not have a mutation in one of these 3 genes, they are referred to as "triple negative". Children seem to be triple negative more frequently than adults. There are also a number of important secondary mutations that can develop over time in MPNs that can have importance for treatment and outcomes in adults (but we don't know much about these mutations in children.)

Symptoms

MPNs can lead to a variety of different symptoms, and there is a broad spectrum as far as how well or sick a person with an MPN feels. Some of the major side effects of MPNs can include severe bleeding (most commonly in people with very high platelets) or blood clots. Unfortunately, MPNs can progress into acute leukemia. This happens most frequently with MF, then with PV, and least often with ET. Thankfully, this has only been reported in adults with MPNs.

More Common in Adults

MPNs are much more common in adults than children. While the exact incidence in children is not clear, we can estimate that MPNs are about 50-100 times more common in adults than kids. This means that many pediatricians, and even pediatric hematology/oncology specialists may never see a child with an MPN during their time in practice, or may only see 1 or 2 children.

MPN TYPES

There is a group of MPNs called the " classical MPNs" that is made up of 3 types of MPN. They are: essential thrombocytosis (ET), polycythemia vera (PV), and myelofibrosis (MF). Of these three, MF is the most rare in children. Let's take a closer look at each type.

1. Essential Thrombocytosis

ET is the mildest form of the classical MPNs. It is an overproduction of platelets, but sometimes there is also a small amount extra bone marrow fibrous tissue. Children with ET can be symptomatic at the time of presentation, or they can be picked up incidentally on routine screening blood work. Children frequently develop something called "reactive thrombocytosis", or high platelets in the setting of an infection or inflammation. A physician may think a child has reactive thrombocytosis when they are first found to have a high platelet count, and it may take time to identify if ET is the real cause of high platelets.

2. Polycythemia Vera

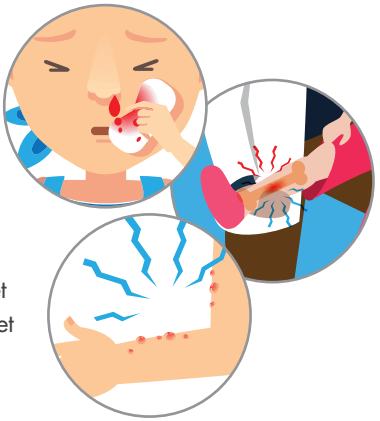
PV is usually more severe than ET. It is an overproduction of red blood cells, but can also include an overproduction of platelets and bone marrow fibrous tissue. PV is often detected when blood tests are done because a child is feeling sick. Sometimes, children present with an enlarged spleen or a blood clot in their liver, and that leads to the diagnosis of PV.

3. Myelofibrosis

MF is the least common, and traditionally most severe form, of the classical MPNs. Children often develop different forms of MF than adults. Some of these forms can self-resolve, while some can be extremely severe and potentially life threatening. MF tends to occur in younger patients than the other classical MPN. Among the classical MPN, there is some overlap in clinical findings. ET can transform to become PV, rarely PV can transform to ET, and both ET and PV can transform to MF. This is why these diseases are often referred to as a "spectrum" since they are so closely related.

SYMPTOMS OF MPNS

While children may be asymptomatic of their MPN, there are a variety of symptoms they might report. Some examples of symptoms that children with MPNs may have include:



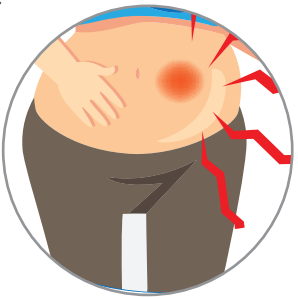
- Headache
- Abdominal pain
- Abdominal swelling
- Chest pain
- Fatigue
- Leg pains
- Itching
- Abnormal sensations in hands or feet
- Redness and swelling of hands or feet
- Bleeding

Presenting Feature

One presenting feature of an MPN in children can be something called Budd-Chiari syndrome. This is a condition where blood clots form in veins in the liver, leading to decreased or blocked blood flow from the liver. This can be very serious and can lead to backpressure and swelling and damage of other veins, an enlarged spleen, fluid in the belly, and possibly failure of the liver to work properly. Thankfully this is not common, but has happened to a number of children and teens with an MPN.

Most Look Normal on Examination

Most children with an MPN look normal on physical exam, but things their doctors may note on an exam, depending on how well or sick the child is may include:



- Enlarged spleen
- Enlarged liver
- Distended abdomen
- Bruising on the skin

WORKING UP A CHILD FOR AN MPN

If a doctor is evaluating your child for an MPN, there are number of tests that will need to be done. Blood tests that will be sent may include:

- Complete blood count (CBC) with differential
- Reticulocyte count
- Erythropoietin (EPO) level
- Lactate dehydrogenase (LDH) level
- Liver function tests
- Kidney function tests
- vonWillebrand testing (acquired bleeding disorder associated with MPNs)
- Genetic tests for mutations in one of the 3 common MPN genes
- Genetic tests for mutations in other MPN-associated genes

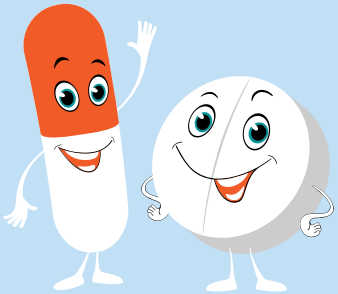
Depending on the symptoms and/or physical exam findings your child has, your doctor may also request:

- Abdominal ultrasound (to look at the liver and spleen)
- MRI/MRV of the head (to look at the brain and its veins)
- Echocardiogram (to look at the heart)

A Bone Marrow Examination is Needed

All children being evaluated for an MPN will need a bone marrow examination. This should be a standard part of the evaluation if an MPN is truly high on the differential for what is going on with your child. This will allow your doctor to get information on things like how much fibrosis is in the bone marrow, what do the early blood cells look like, and do your child's chromosomes look normal or are there missing/extra parts of them. Bone marrow procedures in children are generally done with sedation and local pain medicine to minimize fear and discomfort.

TREATMENTS FOR MPNS IN CHILDREN



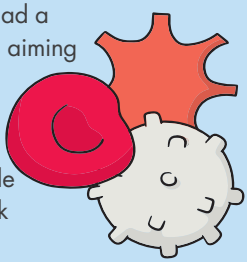
Many children with an MPN may not need to be treated. If they have no symptoms and their blood counts are relatively stable, there may be no benefit to treating other than observation. If a child with an MPN has mild symptoms like headaches, and they show no increased bleeding risk, aspirin is usually the front-line therapy used.

For children with PV, phlebotomy (removing blood through an IV) is also an appropriate therapy that can help improve blood counts and symptoms. For children with severe disease who are having blood clots or serious bleeding, or those with other symptoms that can't be well controlled, "cytoreductive" therapy is used. This includes stronger medicines, like hydroxyurea or interferon, which will reduce blood counts and attempt to alleviate symptoms. If your child's doctor is recommending treatment, you should discuss the risks and benefits of the therapy and make a decision together on what is best for your child and family.

Goals May be Different

The goal of cytoreductive treatment may be different in each child. In some children, the goal of therapy will be to improve counts just enough so that symptoms resolve. This may not require having a normal blood count. For example, if a child with ET has severe headaches that are not responding to aspirin, if he or she starts on a cytoreductive medication and the headaches get better at a platelet count around 750,000 then that should be the goal of therapy. Increasing medication doses to get the platelet count into the normal range may not be needed and may increase potential toxicity or side effects of the medication.

There is no evidence yet in children that aiming for a normal blood count is necessary. In a different situation, where a child had a blood clot, then aiming for a normal platelet count would be more reasonable to minimize risk of recurrence.



SOME KEY POINTS ABOUT MPNS IN KIDS

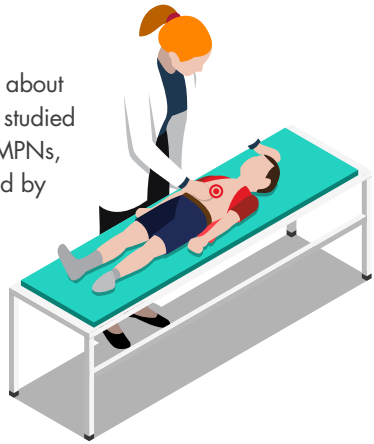
Most of the information we have about MPNs in children are based on evidence from studies and clinical trials in adults. There are people in the US and abroad who are working to learn more about these diseases specifically in children so we can have better evidence to provide for families. Some important points to remember when reading about MPNs that may be different for children include:

- Diagnostic criteria put out by major groups like the World Health Organization are based on adult findings and normal lab values, so they may not be appropriate for children and diagnosing the exact type of MPN your child has may not be possible

- Because there is not yet a lot of good data on MPNs in kids, some doctors will have different opinions about how to manage children with MPNs – this doesn't mean one doctor is right and one is wrong, it just means that it is important to talk to your doctor about his or her recommendations to understand why they are making those recommendations

- All children diagnosed with an MPN, or being seriously evaluated for an MPN, should have a bone marrow evaluation

- Many of the medications you read about being studied in MPNs are not yet studied in, or approved for, children with MPNs, so may not be offered for your child by your doctor.



SIGNS & SYMPTOMS

Signs of disease are characteristics that can be seen and objectively measured such as genetic mutations, blood counts, and visible rash. Symptoms are things experienced by the patient such as itching, headaches, and bone pain. Each MPN patient experiences signs and symptoms in different combinations over time.

Common to PV: headaches; dizziness; weakness; shortness of breath; double or blurred vision; itching all over after a warm bath; reddened skin, burning feeling on hands or feet; bleeding from gums; excessive sweating; excessive fatigue; gouty arthritis in a joint; enlarged spleen; decreased libido.

Common to ET: high platelet count; weakness; bleeding takes time to clot; headache; dizziness; chest pain; tingling/numbness in the hands and feet due to tiny vessel clots; blood clots in tiny blood vessels of the brain causing headaches, changes in speech or awareness; foggy brain; decreased libido; shortness of breath.

Common to MF: enlarged spleen, bone pain, extreme fatigue, unexplained weight loss, decreased libido.

Splenomegaly: enlarged spleen. Spleens get enlarged when they are overworked. Typically, they trap elderly-nonfunctioning red cells, break down the hemoglobin and ship it to

the liver for detoxification. When you consistently stress the system, the spleen gets larger. The spleen is also a major reservoir of platelets so it will also work overtime with increased platelet counts.

TESTS & PROCEDURES

Complete Blood Count (CBC) is a simple blood test that measures all the components of the whole blood including the number of each type of blood cell, the size and shape of red cells, the amount of hemoglobin, and hematocrit (percentage of red cells in the blood).

Blood Smear takes a small sample of blood and examines it under a microscope. It can reveal abnormal number, size and shape of blood cells, and is helpful to diagnose Myelofibrosis or Polycythemia Vera.

Erythropoietin (EPO) is a blood test that measures the level of EPO in your blood. EPO is a hormone that instructs the marrow to make new blood cells. With Polycythemia Vera, the EPO level is very low. People with Secondary Polycythemia usually have normal or high levels of EPO.

Bone Marrow Tests are two tests to see whether the bone marrow is healthy. The bone marrow is collected from needles inserted through the pelvic bone on the patient's backside. It can be done in an office or hospital. If done without twilight anesthesia, insist on adequate sedation and pain medication prior to the procedure. There are no stitches and there is some tenderness for a few days following.

Bone Marrow Aspirate is a technique for obtaining bone marrow fluid through a needle for microscopic examination, cytogenetics and flow cytometry.

Bone Marrow Biopsy is a technique where a small amount of bone containing marrow is obtained through slightly larger needle to identify the presence of myelofibrosis, and to assess marrow cellularity and architecture.

Flow Cytometry is a technique by which individual blood or marrow cells can be analyzed for clonality.

Cytogenetics is a technique used to analyze the number and integrity of a cell's chromosomes.

Phlebotomy or Venesection is the removal of whole blood from a vein. This is often used with Polycythemia Vera patients to reduce the number of red cells and induce iron deficiency to slow their accumulation. It controls the signs and symptoms but not the progression of the disease.

Coping with Myeloproliferative Neoplasms

If you've been diagnosed with myelofibrosis, polycythemia vera, or essential thrombocythemia, it's important to have a primary care physician, hematologist, and healthcare team that you feel comfortable with and at ease asking any question important to you. An empathetic, knowledgeable group can make your journey less trying.
It's okay to get a second opinion if you choose.
The more knowledgeable you are about your MPN the more empowered you will be in making decisions with your family, caregivers and physician(s).

Educate yourself and others

Educate yourself, caregiver, and family members. Learn everything you need to know about your diagnosis. Attend educational symposia and hear from the experts in the field of MPNs. View webcasts after events if you are unable to attend. Visit the numerous sites that focus on MPNs and read as much as possible. Ask questions.

Hematologist /Oncologist

Most hematologists have few, if any, MPN patients. That's okay. A great hematologist will support you getting a consultation with a MPN expert, and then follow the suggested treatment plan. Since these are long-term, chronic cancers, they can be monitored regularly and change treatments only when signs and symptoms change.

Prepare for appointments

Keep a calendar or journal where you note symptoms from day to day (some days are better than others) and any changes in medication.

Write questions that come up before your appointment

Take notes during your appointments or have someone else take notes. If you don't understand what your doctor is saying, ask for explanations.

Know whom to call if you need medical attention outside of a scheduled doctor appointment.

Create a Health History document that you can add to over time. This will help you and your doctors see what has changed over time.

Carry an ID Card & avoid possible complications

Place an ID card in a school bag or wallet that requests a call to your hematologist immediately if you are in an emergency situation that may require medical care or surgeries. List your MPN on that card with your physician's name and phone number.

If you are having elective surgery, make sure you meet with your entire healthcare team to discuss your MPN and whether you may require special needs.

Join a local support group

Parents and young adults can join a support group. There are many support groups across the country and abroad. Support group meetings are a safe place to hear from other patients and caregivers who share similar experiences, issues, and concerns. You will hear updates on research, clinical trials and treatment options.

FAQs FREQUENTLY ASKED QUESTIONS & ANSWERS

What are the main symptoms, treatments and tests for MF, PV, and ET in children?

Please read about symptoms, treatments and tests for children on the earlier pages of this booklet (immediately preceding these FAQ pages)

Are males and females affected differently?

Males and females with MPNs may simply have different issues. For example: females may require different approaches because of reproductive related considerations. More research needs to be spent on defining these differences and it is a subject matter MPN Advocacy and Education International discusses at all symposia.

What causes MF, PV, ET?

The most common cause of MPNs is an abnormal proliferation of white blood cells, red blood cells and platelets which can be caused by genetic mutations (called acquired defects or mutations). Exposure to certain chemicals and high levels of radiation have been possible culprits however more research needs to be done.

Is there a cure for MPNs?

The only known cure for some patients is a bone marrow transplant. This procedure is not recommended for everyone and many factors must be considered. Other treatments are being tested and research is ongoing to find a cure.

How long does one live with an MPN?

Many MPN patients live long and productive lives. Since 2005 when the JAK2 gene mutation was discovered, an avalanche of interest, research, and new drug development has emerged in the world of MPNs. This event has created such a great awareness and focus in the field that more accurate information is available, more treatment options are available and more are on the horizon, and a better understanding of prevalence and survival data is being collected.

Can children have bone marrow or stem cell transplants?

BMT or SCT is not a procedure for everyone and there are many factors to considered with your health care team. Some patients (of any age) do very well after a BMT or SCT. Typically, their donors are a perfect match and they were excellent candidates for this procedure.

Are MPNs inherited?

Some studies indicate that MPNs can be inherited. Research suggests that inherited gene mutations such as JAK 2, may predispose individuals to developing an MPN. People are not born with this mutation, it is acquired.

What is the JAK2 gene mutation?

A genetic mutation found in approximately 50% of myelofibrosis patients, 95% of polycythemia vera patients, and approximately 50% of essential thrombocythemia patients.

What is the CALR mutation?

Calreticulin mutation in early blood forming cells are associated with essential thrombocythemia and primary myelofibrosis.

Are there many other mutations seen in MPN patients?

Yes. For example, BCR-ABL1-negative, MPL, CBL, LNK, TET2, IDH1/2.

Who gets an MPN?

Infants are sometimes diagnosed with an MPN. Most often we hear about young adults or older patients but MPNs can strike anyone at any age.

Are there diets or healthy life choices that will make my diagnosis easier?

It is always wise to maintain a healthy diet and exercise plan no matter what. It is doubly important when living with a chronic disease or illness. Good nutrition maintains energy and strengthens the immune system. Physical activity every day promotes restful sleep and elevates your mood. Sleep or rest when able. Yoga or meditation can be beneficial to reduce stress and increase energy. Participate in a support group.

Will my child have to take medicine for his or her MPN?

Not all children with an MPN require treatment. How the disease presents, the type of MPN, the level of blood counts, and your child's symptoms, all contribute to the decision whether or not treatment is needed. Treatments can vary depending on the type of MPN, and may require medication or phlebotomy.

Are there pediatric MPN centers?

There are pediatric cancer centers and academic institutions who have experienced clinicians who see a number of pediatric MPN patients. Visit our website for a list of centers, mpnadvocacy.com/resources/mpn-centers.

Is there an environmental component to an MPN diagnosis?

There are many patients who believe their exposure to toxins, chemicals, and other hazardous waste may have played a role in their diagnosis. More studies are needed to confirm and explain these possibilities.

Are there other children with the same disease as my child?

Yes! While MPN are very rare in children, you are not alone. There are resources such as parent-run support groups on the internet, and connections are often made through the MPN Advocacy & Education International organization. Check out our list of support groups at mpnadvocacy/mpn-support-groups

Are there educational programs I can attend?

Yes. MPN Advocacy and Education International hosts educational programs each year in many different cities. Visit our events page to see where we will be this year. Mpnadvocacy.com/events/

Is there research being done specifically about MPNs?

Yes. The MPN Research Foundation funds cutting edge research projects. Most academic institutions with MPN researchers and clinicians are conducting specific MPN research and clinical trials.

Resources

Patient support; prescription and co-pay assistance and more

MPN Patient/Caregiver Support

If you are seeking a safe place to meet other patients and caregivers in your area, our website offers an updated list of MPN support groups across the country and internationally. Online groups are very active including Facebook and can provide useful information and support if you prefer using that format, for more information go to mpnadvocacy.com.

MPN Cancer Centers

If you are looking for a medical institution or MPN Cancer Center in your area, visit our website for a comprehensive list of alternatives, mpnadvocacy.com/resources/mpn-centers/ or call us 517.899.6889.

ADDITIONAL RESOURCES:

MPN Research Foundation

Research/Patient Support
mpnresearchfoundation.org

MPN Education Foundation

Patient Support
mpninfo.org

MPN Forum

MPN Quarterly Journal Publication
mpnforum.com

Patient Access Network (PAN) Foundation

Prescription Assistance
panfoundation.org
866-316-7263

Incyte Corporation

Prescription Assistance Program for Jakafi
incytecares.com
855-452-5234

Leukemia and Lymphoma Society

Co-pay Assistance
lls.org
877-557-2672

National Organization for Rare Disorders (NORD)

Advocacy/Education
rarediseases.org

BMTinfonet

Bone Marrow Transplant Support
bmtinfonet.org

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Glossary of Terms

Allogenic: Cells that are genetically different and immunologically incompatible; cell types that are antigenically distinct.

Anemia: When the number of red blood cells is below normal, it can result in fatigue, weakness, and shortness of breath.

Antibody: A molecule created to adhere to and interact with the antigen that triggered its synthesis. The antigen-antibody reaction is important to an immune response.

Antigen: A substance on the surface of a cell that triggers an immune response; it also reacts with the product of the response (the antibody). Part of the body's immune response.

Asymptomatic: Exhibits no symptoms

Blood Cancer: A malignant neoplasm of blood-forming tissue, characterized by abnormal proliferation of leukocytes.

Blood Clot: Blood forms a clot when it comes in contact with a "thrombogenic" substance causing the blood to convert from a liquid to a solid state (also known as coagulation). Thrombogenic substances include collagen, tissue factor and von Willebrand factor.

Bone Marrow: The soft, fatty, vascular tissue inside bones that produce blood cells.

Bone Marrow Aspiration: A technique for obtaining bone marrow tissue through a needle (usually in the pelvic bone) for examination.

Bone Marrow Biopsy: A procedure used to remove soft tissue, called marrow, from inside the bone. Blood and tissue is tested for disease or disease progression. Can be done under local anesthetic, or with "conscious sedation" to ensure a minimum discomfort from the procedure.

Bone Marrow Transplant (BMT): A procedure to replace damaged or destroyed bone marrow with healthy bone marrow stem cells. Also called a Stem Cell Transplant. Autologous transplants use the patient's own cells. Allogeneic transplants use cells from a donor for a patient.

CAL-Reticulan: A mutation of the CALR gene is known to cause MPN.

Chemotherapy: A cancer treatment that can be given orally and/or intravenously using chemical agents or drugs that are selectively destructive to specific cancer cells.

Chronic Myeloid/Myelogenous Leukemia: The bone marrow produces excessive white blood cells, caused by the Philadelphia chromosome or the BCR-ABL fusion gene, Since granulocytes are involved in the type of leukemia, it is also referred to as CGL o chronic granulocytic leukemia.

Coagulation: The process by which the blood converts from liquid to a semisolid mass (blood clot), caused by a thrombogenic substance.

Complete Blood Count/CBC: A blood test that measures the concentration of white blood cells, red blood cells, and platelets in the blood.

Constitutional Symptom: Something that affects the health status of a patient and indicates a disease (e.g. unexpected weight loss, fever, vomiting, fainting).

Cytokine: A small nonantibody protein chemical released by cells that instruct other cells. Regulate the intensity and duration of immune response and mediate cell-to-cell communication. Cytokines included interferons, interleukins, lymphonkine and chemokine.

Deep Vein Thrombosis: A blood clot that forms in a deep vein of the body, usually the thigh or leg. If and when the clot breaks off and moves through the bloodstream, it becomes an embolism, which can get lodged in the brain, heart or lungs, causing severe damage.

Donor: Some MPN patients choose to have Bone Marrow Transplants. Donors who are considered a Perfect Match are the optimal candidates and are often a family member. Many donors are unknown to the recipient although some do connect after successful transplants.

Embolism: A blood clot that breaks off and moves through the bloodstream, lodges in a blood vessel and blocks it. An embolism can become lodged in the brain, heart, lungs or other area, causing severe damage.

Erythrocytes: Red blood cells; carry oxygen from lungs to the tissues.

Erythromelalgia: Sudden dilation of peripheral blood vessels, often triggered by heat or exertion; causes throbbing, burning, or severe itchiness of the skin. Usually affects hands and feet.

Essential Thrombocythemia (ET): Blood disorder characterized by the overproduction of platelets and megakaryocytes in bone marrow. Also known as primary thrombocytosis.

Fatigue: A condition marked by extreme tiredness and inability to function normally due to a lack of energy.

Fibrosis: Thickening and scarring of connective tissue.

Foggy Brain: Common symptoms of MPN patients include loss of thought clarity, difficulty in thinking, short-term memory loss, routine tasks are forgotten.

Gene Mutation: A change in the DNA sequence. Gene mutations that are often associated with MPNs include JAK2V617F mutation, MPL mutation and calreticulin (CALR) mutation.

Genes: The basic building blocks of heredity that are present in all cells.

Gout: Acute arthritis (swelling) of joints, typically the big toe, due to excess uric acid that isn't processed through the kidneys. Uric acid crystalizes and accumulates in the joints.

Graft vs Host Disease: Complication of allogeneic SCT in which the new immune cells in the transplanted marrow treat the recipient's tissues (the patient's) as foreign and cause an immunologic attack.

Hematologist/Oncologist: A physician who specializes in blood diseases and cancers. Many hematologists treat tumor cancers as well as blood cancers.

Hematocrit: Percentage of red blood cells in a volume of whole blood. The percentage by volume of whole blood that consists of blood cells (the remainder is plasma).

Hematopoiesis: Formation and development of blood cells.

Hematopoietic Stem Cell: A cell that develops into any type of specialized blood cell.

Hemoglobin: The part of the red blood cell that carries oxygen.

Hypertension: High blood pressure

Idiopathic: The cause for a disease process is unknown; also called Agnogenic.

JAK Inhibitor: A medication that blocks the activity of one or more of the JAK enzymes (JAK1, JAK2, JAK3, TYK2). JAK inhibitors are used to treat certain types of cancer and inflammatory conditions.

JAK positive: The somatic genetic mutation found in approximately 50 percent of myelofibrosis patients, 95 percent of polycythemia vera patients, and approximately 50 percent of essential thrombocythemia patients.

JAK 2 (JAK2v617F): The genetic mutation found in approximately 50 percent of myelofibrosis patients, 95 percent of polycythemia vera patients, and approximately 50 percent of essential thrombocythemia patients.

Leukocytes: White blood cells; kill micro-organisms (infection) that invades the body.

Leukocytosis: Overproduction of white cells

Lymphocyte: A type of white blood cell (leukocyte) that is responsible for the immune response and aids in defending the body against disease. There are two primary types of lymphocytes: B cells and T cells.

Matched Unrelated Donor: Someone, not related to the patient, who donates his/her marrow stem cells for transplantation to someone with a blood cancer or disorder.

Mean Platelet Volume: Measures the average amount (volume) of platelets. Used with platelet count to diagnose some diseases.

MPL gene: A mutation of the MPL gene is known to cause MPN.

MRI Scan: A scan that uses magnets and radio frequency waves to produce images inside the body.

Mutation: The change or alteration of something. Gene mutation changes the way a gene functions.

Myelofibrosis (MF): Two definitions (descriptive and disease): 1) increased fibrosis in the bone marrow, and 2) a rare bone marrow cancer that disrupts normal blood cell production. Causes excessive fibrous scar tissue formation. Symptoms include anemia and enlarged spleen.

Myeloproliferative Neoplasms (MPN): Diseases of the blood and bone marrow, in which the body makes too many blood cells. The three main types MPNs are: polycythaemia vera (PV), essential thrombocythaemia (ET), and myelofibrosis.

Neoplasm: An abnormal mass of tissue that results when cells divide more than they should or do not die when they should.

Neutropenia: A significant decrease in the number of white blood cells.

Night Sweats: Episodes of excessive sweating while sleeping.

Pegylated: Time-released medication

Petechiae: Flat, red, pinpoint spots under the skin caused by bleeding.

Philadelphia Chromosome: An abnormality of chromosome 22, which is associated with chronic myeloid leukemia (CML).

Phlebotomy, Venesection: Withdrawing blood from the body, usually in large amounts, for treatment purposes. Phlebotomy is a mainstay of treatment for the polycythemia vera (PV) to lower hemoglobin and hematocrit levels.

Platelet Count: The number of platelets in a given volume of blood. Either quoted as per liter (e.g., reference range of 150-400 x 109 per liter) or per microliter (reference range of 150,000-400,000).

Platelets: Small cell fragments that help blood clot.

Polycythemia Vera (PV): Blood disorder of the bone marrow where the stem cells produce excessive clonal red cells that rapidly multiply and are released into the bloodstream. Causes high red cell count (hematocrit) and Hemoglobin and increased blood volume and viscosity; can cause thrombosis, migraines, strokes. White cells and platelets may also increase.

Primary Myelofibrosis: A disorder of the bone marrow that disrupts normal production of blood cells. It causes excessive scarring in the bone marrow. Symptoms include enlarged spleen and anemia. Myelofibrosis is one of the Myeloproliferative Neoplasms (MPNs).

Proliferative: Takes part in rapid and repeated production of offspring (e.g., new cells).

Pruritus: Severe itching.

Pulmonary Embolism: The obstruction of one or more of the pulmonary arteries in the lungs, caused by a blood clot that has traveled from somewhere else in the body.

Purpura: Patches of purple/blue discoloration of skin (Bruises) when blood enters the skin and mucous membranes; can occur as petecchiae, ecchymosis, and hematomas.

Radiation Therapy: A type of treatment that uses high energy to kill cancer cells.

Red Blood Cells (RBCs): Cells that carry oxygen through the body.

Reynaud's Syndrome: A disorder in which the fingers or toes experience decreased blood circulation and the skin color.

Secondary Myelofibrosis: Myelofibrosis occurring as a progression from another of the MPNs, generally following PV, ET, or CML.

Spleen: An organ located on the left side of the abdomen that is part of the lymphatic system. The spleen makes lymphocytes, filters the blood, stores blood cells, and destroys old blood cells.

Splenectomy: Surgical removal part or the entirety of the spleen.

Splenomegaly: Enlargement of the spleen.

Stem Cell: A cell that can become a more mature type of blood cell.

Stem Cell Transplant: A procedure in which healthy bone marrow stem cells are use to replace diseased or damaged bone marrow. Also called a bone marrow transplant.

Stroke: Rapid loss of brain function due to a disturbance of blood flow to the brain such as a blockage or hemorrhage.

Thrombocythemia/ Thrombocytosis: A higher than normal number of platelets in the blood.

Thrombopoietin: Hormone that regulates megakaryocyte (platelet precursors) production, and thus platelets; it operates through its receptor (Mpl) to stimulate production.

Thrombosis/Thrombus: The formation of a blood clot in a blood vessel causing a total or partial obstruction of a vein or artery.

Transfusion: Procedure in which a patient receives blood products reds, platelets, or stem cells intravenously.

Ultrasound: High frequency sound waves used to look at organs and structures inside the body.

Vascular: Pertaining to vessels that carry/circulate fluid; usually referring to blood vessels (veins and arteries).

White Blood Cells (WBCs): Blood cells that fight infection and anything it perceives as foreign.

For a comprehensive list of terms and definitions visit our website: **mpnadvocacy.com/resources/glossary-of-terms/**

QUESTIONS FOR YOUR CHILD'S HEMATOLOGIST...

(CUT OUT AND TAKE WITH YOU)

- ❑ How many pediatric MPN patients do you see? How many adult?
- ❑ How long have you been seeing pediatric MPN patients?
- ❑ What kinds of treatments are available?
- ❑ What will the treatment you prescribe do?
- ❑ Will my child be in remission?
- ❑ Will treatments just address their symptoms?
- ❑ Are there clinical trials currently?
- ❑ Are they a candidate for a clinical trial?
- ❑ What should they expect if they want to have children someday?
- ❑ Do males and females with MPNs have different issues?
- ❑ What side effects are there with treatments?
- ❑ How long will they have to take drugs?
- ❑ What should their primary care physician know?
- ❑ What are red flags over the course of their treatment?
- ❑ Will they convert to another blood cancer?
- ❑ How do you feel about alternative treatments?
- ❑ What information should we share with family/caregiver?
- ❑ Is depression as prevalent with an MPN as other cancers?
- ❑ What life changes or health changes should they make?
- ❑ If their treatment is not covered by my insurance, what alternatives do we have?
- ❑ Will this diagnosis and treatment affect future intimate relationships?
- ❑ Are you open to our choices and input in caring for their cancer?
- ❑ Do you get updates on clinical trials and other up to date information about MPN research, etc.?
- ❑ I am tracking their lab reports and how they feel at each visit. Can we have copies of the lab reports?

MPN ADVOCACY & EDUCATION INTERNATIONAL

Sign up for our Free Monthly Newsletter *MPN Community Connection*

Stay informed and updated on issues related to myelofibrosis, polycythemia vera and essential thrombocythemia. Sign up online at: mpnadvocacy.com/blood-cancer-newsletter/

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