Chronic Myeloproliferative Neoplasms Treatment

Myelodysplastic syndromes are cancers in which immature blood cells in the bone marrow do not mature or become healthy blood cells.

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General Information About Chronic Myeloproliferative Neoplasms

KEY POINTS

- Myeloproliferative neoplasms are a group of diseases in which the bone marrow makes too many red blood cells, white blood cells, or platelets.
- There are 6 types of chronic myeloproliferative neoplasms.
- Tests that examine the blood and bone marrow are used to detect (find) and diagnose chronic myeloproliferative neoplasms.

Myeloproliferative neoplasms are a group of diseases in which the bone marrow makes too many red blood cells, white blood cells, or platelets.

Normally, the bone marrow makes blood stem cells (immature cells) that become mature blood cells over time.

A blood stem cell may become a myeloid stem cell or a lymphoid stem cell. A lymphoid stem cell becomes a white blood cell. A myeloid stem cell becomes one of three types of mature blood cells:

- Red blood cells that carry oxygen and other substances to all tissues of the body.
- White blood cells that fight infectionand disease.
- Platelets that form blood clots to stop bleeding.

In myeloproliferative neoplasms, too many blood stem cells become one or more types of blood cells. The neoplasms usually get worse slowly as the number of extra blood cells increases.

There are 6 types of chronic myeloproliferative neoplasms.

The type of myeloproliferative neoplasm is based on whether too many red blood cells, white blood cells, or platelets are being made. Sometimes the body will make too many of more than one type of blood cell, but usually one type of blood cell is affected more than the others are. Chronic myeloproliferative neoplasms include the following 6 types:

- Chronic myelogenous leukemia
- Polycythemia vera
- Primary myelofibrosis(also called chronic idiopathic myelofibrosis)
- Essential thrombocythemia
- Chronic neutrophilic leukemia
- Chronic eosinophilic leukemia

These types are described below. Chronic myeloproliferative neoplasms sometimes become acute leukemia, in which too many abnormal white blood cells are made.

Tests that examine the blood and bone marrow are used to detect (find) and diagnose chronic myeloproliferative neoplasms.

The following tests and procedures may be used:

- Physical examand history : An exam of the body to check general signs of health, including checking for signs of disease, such as lumps or anything else that seems unusual. A history of the patient's health habits and past illnesses and treatments will also be taken.
- Complete blood count (CBC) with differential: A procedure in which a sample of blood is drawn and checked for the following:
  - The number of red blood cells and platelets.
  - The number and type of white blood cells.
  - The amount of hemoglobin(the protein that carries oxygen) in the red blood cells.
  - The portion of the blood sample made up of red blood cells.

- Peripheral blood smear: A procedure in which a sample of blood is checked for the following:
  - Whether there are red blood cells shaped like teardrops.
  - The number and kinds of white blood cells.

    - The number of platelets.
    - Whether there are blast

- Blood chemistry studies: A procedure in which a blood sample is checked to measure the amounts of certain substances released into the blood by organs and tissues in the body. An unusual (higher or lower than normal) amount of a substance can be a sign of disease.
- Bone marrow aspiration and biopsy: The removal of bone marrow, blood, and a small piece of bone by inserting a hollow needle into the hipbone or breastbone. A pathologist views the bone marrow, blood, and bone under a microscope to look for abnormal cells.
- Cytogenetic analysis: A test in which cells in a sample of blood or bone marrow are viewed under a microscope to look for certain changes in the chromosomes. Certain diseases or disorders may be diagnosed or ruled out based on the chromosomal changes.
- Genemutation test: A laboratory test done on a bone marrow or blood sample to check for mutations in JAK2 , MPL , or CALR A JAK2 gene mutation is often found in patients with polycythemia vera, essential thrombocythemia, or primary myelofibrosis. MPL or CALR gene mutations are found in patients with essential thrombocythemia or primary myelofibrosis.

Chronic Myelogenous Leukemia

Chronic myelogenous leukemia is a disease in which too many white blood cells are made in the bone marrow. See the PDQ summary on Chronic Myelogenous Leukemia Treatment for information on diagnosis, staging, and treatment.

Polycythemia Vera

KEY POINTS

- Polycythemia vera is a disease in which too many red blood cells are made in the bone marrow.
- Symptoms of polycythemia vera include headaches and a feeling of fullness below the ribs on the left side.
- Special blood tests are used to diagnose polycythemia vera.

Polycythemia vera is a disease in which too many red blood cells are made in the bone marrow.

In polycythemia vera, the blood becomes thickened with too many red blood cells. The number

of white blood cells and platelets may also increase. These extra blood cells may collect in the spleen and cause it to swell. The increased number of red blood cells, white blood cells, or platelets in the blood can cause bleeding problems and make clots form in blood vessels. This can increase the risk of stroke or heart attack. In patients who are older than 65 years or who have a history of blood clots, the risk of stroke or heart attack is higher. Patients also have an increased risk of acute myeloid leukemia or primary myelofibrosis.

Symptoms of polycythemia vera include headaches and a feeling of fullness below the ribs on the left side.

Polycythemia vera often does not cause early signs or symptoms. It may be found during a routine blood test. Signs and symptoms may occur as the number of blood cells increases. Other conditions may cause the same signs and symptoms. Check with your doctor if you have any of the following:

- A feeling of pressure or fullness below the ribs on the left side.
- Double vision or seeing dark or blind spots that come and go.
- Itching all over the body, especially after being in warm or hot water.
- Reddened face that looks like a blush or sunburn.
- Weight loss for no known reason.

Special blood tests are used to diagnose polycythemia vera.

In addition to a complete blood count, bone marrow aspiration and biopsy, and cytogenetic analysis, a serum erythropoietin test is used to diagnose polycythemia vera. In this test, a sample of blood is checked for the level of erythropoietin (a hormone that stimulates new red blood cells to be made). In polycythemia vera, the erythropoietin level would be lower than normal because the body does not need to make more red blood cells.

Primary Myelofibrosis

KEY POINTS

- Primary myelofibrosis is a disease in which abnormal blood cells and fibers build up inside the bone marrow.
- Symptoms of primary myelofibrosis include pain below the ribs on the left side and feeling very tired.
- Certain factors affect prognosis (chance of recovery) and treatment options for primary myelofibrosis.

Primary myelofibrosis is a disease in which abnormal blood cells and fibers build up inside the bone marrow.

The bone marrow is made of tissues that make blood cells (red blood cells, white blood cells, and platelets) and a web of fibers that support the blood-forming tissues. In primary myelofibrosis (also called chronic idiopathic myelofibrosis), large numbers of blood stem cells become blood cells that do not mature properly (blasts). The web of fibers inside the bone marrow also becomes very thick (like scar tissue) and slows the blood-forming tissue's ability to make blood cells. This causes the blood-forming tissues to make fewer and fewer blood cells. In order to make up for the low number of blood cells made in the bone marrow, the liverand spleen begin to make the blood cells.

Symptoms of primary myelofibrosis include pain below the ribs on the left side and feeling very tired.

Primary myelofibrosis often does not cause early signs or symptoms. It may be found during a routine blood test. Signs and symptoms may be caused by primary myelofibrosis or by other conditions. Check with your doctor if you have any of the following:

- Feeling pain or fullness below the ribs on the left side.
- Feeling full sooner than normal when eating.
- Feeling very tired.
- Shortness of breath.
- Easy bruising or bleeding.
- Petechiae(flat, red, pinpoint spots under the skin that are caused by bleeding).
- Fever.
- Night sweats.
- Weight loss.

Certain factors affect prognosis (chance of recovery) and treatment options for primary myelofibrosis.

Prognosis (chance of recovery) depends on the following:

- The age of the patient.
- The number of abnormalred blood cells and white blood cells.
- The number of blasts in the blood.
- Whether there are certain changes in the chromosomes.

- Whether the patient has signs such as fever, night sweats, or weight loss.

Essential Thrombocythemia

KEY POINTS

- Essential thrombocythemia is a disease in which too many platelets are made in the bone marrow.
- Patients with essential thrombocythemia may have no signs or symptoms.
- Certain factors affect prognosis (chance of recovery) and treatment options for essential thrombocythemia.

Essential thrombocythemia is a disease in which too many platelets are made in the bone marrow.

Essential thrombocythemia causes an abnormal increase in the number of platelets made in the blood and bone marrow.

Patients with essential thrombocythemia may have no signs or symptoms.

Essential thrombocythemia often does not cause early signs or symptoms. It may be found during a routine blood test. Signs and symptoms may be caused by essential thrombocytopenia or by other conditions. Check with your doctor if you have any of the following:

- Burning or tingling in the hands or feet.
- Redness and warmth of the hands or feet.
- Vision or hearing problems.

Platelets are sticky. When there are too many platelets, they may clump together and make it hard for the blood to flow. Clots may form in blood vessels and there may also be increased bleeding. These can cause serious health problems such as stroke or heart attack.

Certain factors affect prognosis (chance of recovery) and treatment options for essential thrombocythemia.

Prognosis (chance of recovery) and treatment options depend on the following:

- The age of the patient.
- Whether the patient has signs or symptoms or other problems related to essential thrombocythemia.

Chronic Neutrophilic Leukemia

Chronic neutrophilic leukemia is a disease in which too many blood stem cells become a type of white blood cell called neutrophils. Neutrophils are infection -fighting blood cells that surround and destroy dead cells and foreign substances (such as bacteria). The spleen and liver may swell because of the extra neutrophils. Chronic neutrophilic leukemia may stay the same or it may progress quickly to acute leukemia.

Chronic Eosinophilic Leukemia

KEY POINTS

- Chronic eosinophilic leukemia is a disease in which too many white blood cells (eosinophils) are made in the bone marrow.
- Signs and symptoms of chronic eosinophilic leukemia include fever and feeling very tired.

Chronic eosinophilic leukemia is a disease in which too many white blood cells (eosinophils) are made in the bone marrow.

Eosinophils are white blood cells that react to allergens (substances that cause an allergic response) and help fight infections caused by certain parasites. In chronic eosinophilic leukemia, there are too many eosinophils in the blood, bone marrow, and other tissues. Chronic eosinophilic leukemia may stay the same for many years or it may progress quickly to acute leukemia.

Signs and symptoms of chronic eosinophilic leukemia include fever and feeling very tired.

Chronic eosinophilic leukemia may not cause early signs or symptoms. It may be found during a routine blood test. Signs and symptoms may be caused by chronic eosinophilic leukemia or by other conditions. Check with your doctor if you have any of the following:

- Fever.
- Feeling very tired.
- Swelling under the skin around the eyes and lips, in the throat, or on the hands and feet.
- Muscle pain.
- Diarrhea.

Stages of Chronic Myeloproliferative Neoplasms

KEY POINTS

- There is no standard staging system for chronic myeloproliferative neoplasms.

There is no standard staging system for chronic myeloproliferative neoplasms.

Staging is the process used to find out how far the cancer has spread. There is no standard staging system for chronic myeloproliferative neoplasms. Treatment is based on the type of myeloproliferative neoplasm the patient has. It is important to know the type in order to plan treatment.

Treatment Option Overview

KEY POINTS

- There are different types of treatment for patients with chronic myeloproliferative neoplasms.
- Eleven types of standard treatment are used:
  - Watchful waiting
  - Phlebotomy
  - Platelet apheresis
  - Transfusion therapy
  - Chemotherapy
  - Radiation therapy
  - Other drug therapy
  - Surgery
  - Biologic therapy
  - Targeted therapy
  - High-dose chemotherapy with stem cell transplant
- New types of treatment are being tested in clinical trials.
- Patients may want to think about taking part in a clinical trial.
- Patients can enter clinical trials before, during, or after starting their cancer treatment.
- Follow-up tests may be needed.

There are different types of treatment for patients with chronic myeloproliferative neoplasms.

Different types of treatments are available for patients with chronic myeloproliferative neoplasms. Some treatments are standard (the currently used treatment), and some are being tested in clinical trials. A treatment clinical trial is a research study meant to help improve current treatments or obtain information on new treatments. When clinical trials show that a new treatment is better than the standard treatment, the new treatment may become the standard

treatment. Patients may want to think about taking part in a clinical trial. Some clinical trials are open only to patients who have not started treatment.

Eleven types of standard treatment are used:

Watchful waiting

Watchful waiting is closely monitoring a patient's condition without giving any treatment until signs or symptoms appear or change.

Phlebotomy

Phlebotomy is a procedure in which blood is taken from a vein. A sample of blood may be taken for tests such as a CBC or blood chemistry. Sometimes phlebotomy is used as a treatment and blood is taken from the body to remove extra red blood cells. Phlebotomy is used in this way to treat some chronic myeloproliferative neoplasms.

Platelet apheresis

Platelet apheresis is a treatment that uses a special machine to remove platelets from the blood. Blood is taken from the patient and put through a blood cell separator where the platelets are removed. The rest of the blood is then returned to the patient's bloodstream.

Transfusion therapy

Transfusion therapy (blood transfusion) is a method of giving red blood cells, white blood cells, or platelets to replace blood cells destroyed by disease or cancer treatment.

Chemotherapy

Chemotherapy is a cancer treatment that uses drugs to stop the growth of cancer cells, either by killing the cells or by stopping them from dividing. When chemotherapy is taken by mouth or injected into a vein or muscle, the drugs enter the bloodstream and can reach cancer cells throughout the body (systemic chemotherapy). When chemotherapy is placed directly into the cerebrospinal fluid, an organ, or a body cavity such as the abdomen, the drugs mainly affect cancer cells in those areas (regional chemotherapy). The way the chemotherapy is given depends on the type and stage of the cancer being treated.

See Drugs Approved for Myeloproliferative Neoplasms for more information.

Radiation therapy

Radiation therapy is a cancer treatment that uses high-energy x-rays or other types of radiation to kill cancer cells or keep them from growing. There are two types of radiation therapy:

- External radiation therapyuses a machine outside the body to send radiation toward the cancer.
- Internal radiation therapyuses a radioactive substance sealed in needles, seeds, wires,

or catheters that are placed directly into or near the cancer.

The way the radiation therapy is given depends on the type of cancer being treated. External radiation therapy is used to treat chronic myeloproliferative neoplasms, and is usually directed at the spleen.

Other drug therapy

Prednisone and danazol are drugs that may be used to treat anemia in patients with primary myelofibrosis.

Anagrelide therapy is used to reduce the risk of blood clots in patients who have too many platelets in their blood. Low-dose aspirin may also be used to reduce the risk of blood clots.

Thalidomide, lenalidomide, and pomalidomide are drugs that prevent blood vessels from growing into areas of tumor cells.

See Drugs Approved for Myeloproliferative Neoplasms for more information.

Surgery

Splenectomy (surgery to remove the spleen) may be done if the spleen is enlarged.

Biologic therapy

Biologic therapy is a treatment that uses the patient's immune system to fight cancer or other diseases. Substances made by the body or made in a laboratory are used to boost, direct, or restore the body's natural defenses against disease. This type of treatment is also called biotherapy or immunotherapy. Interferon alfa and pegylated interferon alpha are biologic agents commonly used to treat some chronic myeloproliferative neoplasms.

Erythropoietic growth factors are also biologic agents. They are used to stimulate the bone marrow to make red blood cells.

Targeted therapy

Targeted therapy is a type of treatment that uses drugs or other substances to identify and attack specific cancer cells without harming normal cells. Tyrosine kinase inhibitors are targeted therapy drugs that block signals needed for tumors to grow.

Ruxolitinib is a tyrosine kinase inhibitor used to treat certain types of myelofibrosis.

See Drugs Approved for Myeloproliferative Neoplasms for more information.

Other types of targeted therapies are being studied in clinical trials.

High-dose chemotherapy with stem cell transplant

High-dose chemotherapy with stem cell transplant is a method of giving high doses of

chemotherapy and replacing blood-forming cells destroyed by the cancer treatment. Stem cells (immature blood cells) are removed from the blood or bone marrow of the patient or a donor and are frozen and stored. After the chemotherapy is completed, the stored stem cells are thawed and given back to the patient through an infusion. These reinfused stem cells grow into (and restore) the body's blood cells.

New types of treatment are being tested in clinical trials.

Information about clinical trials is available from the NCI website.

Patients may want to think about taking part in a clinical trial.

For some patients, taking part in a clinical trial may be the best treatment choice. Clinical trials are part of the cancer research process. Clinical trials are done to find out if new cancer treatments are safe and effective or better than the standard treatment.

Many of today's standard treatments for cancer are based on earlier clinical trials. Patients who take part in a clinical trial may receive the standard treatment or be among the first to receive a new treatment.

Patients who take part in clinical trials also help improve the way cancer will be treated in the future. Even when clinical trials do not lead to effective new treatments, they often answer important questions and help move research forward.

Patients can enter clinical trials before, during, or after starting their cancer treatment.

Some clinical trials only include patients who have not yet received treatment. Other trials test treatments for patients whose cancer has not gotten better. There are also clinical trials that test new ways to stop cancer from recurring (coming back) or reduce the side effects of cancer treatment.

Clinical trials are taking place in many parts of the country. Information about clinical trials supported by NCI can be found on NCI's clinical trials search webpage. Clinical trials supported by other organizations can be found on the ClinicalTrials.gov website.

Follow-up tests may be needed.

Some of the tests that were done to diagnose the cancer or to find out the stage of the cancer may be repeated. Some tests will be repeated in order to see how well the treatment is working. Decisions about whether to continue, change, or stop treatment may be based on the results of these tests.

Some of the tests will continue to be done from time to time after treatment has ended. The results of these tests can show if your condition has changed or if the cancer has recurred (come back). These tests are sometimes called follow-up tests or check-ups.

Treatment Options for Chronic Myeloproliferative Neoplasms

- Chronic Myelogenous Leukemia

- Polycythemia Vera
- Primary Myelofibrosis
- Essential Thrombocythemia
- Chronic Neutrophilic Leukemia
- Chronic Eosinophilic Leukemia

Chronic Myelogenous Leukemia

See the PDQ summary about Chronic Myelogenous Leukemia Treatmentfor information.

Polycythemia Vera

The purpose of treatment for polycythemia vera is to reduce the number of extra blood cells. Treatment of polycythemia vera may include the following:

- Phlebotomy.
- Chemotherapywith or without phlebotomy.
- Biologic therapyusing interferon alfa or pegylated interferon alpha.
- Low-doseaspirin.

Use our clinical trial search to find NCI-supported cancer clinical trials that are accepting patients. You can search for trials based on the type of cancer, the age of the patient, and where the trials are being done. General information about clinical trials is also available.

Primary Myelofibrosis

Treatment of primary myelofibrosis in patients without signs or symptoms is usually watchful waiting.

Patients with primary myelofibrosis may have signs or symptoms of anemia. Anemia is usually treated with transfusion of red blood cells to relieve symptoms and improve quality of life. In addition, anemia may be treated with:

- Erythropoieticgrowth factors.
- Prednisone.
- Danazol.
- Thalidomide, lenalidomide, or pomalidomide, with or without prednisone.

Treatment of primary myelofibrosis in patients with other signs or symptoms may include the following:

- Targeted therapywith ruxolitinib.
- Chemotherapy.
- Donorstem cell transplant.
- Thalidomide, lenalidomide, or pomalidomide.
- Splenectomy.
- Radiation therapyto the spleen, lymph nodes, or other areas outside the bone marrowwhere blood cells are forming.
- Biologic therapyusing interferon alfa or erythropoietic growth factors.
- A clinical trialof other targeted therapy drugs.

Use our clinical trial search to find NCI-supported cancer clinical trials that are accepting patients. You can search for trials based on the type of cancer, the age of the patient, and where the trials are being done. General information about clinical trials is also available.

Essential Thrombocythemia

Treatment of essential thrombocythemia in patients younger than 60 years who have no signs or symptoms and an acceptable platelet count is usually watchful waiting. Treatment of other patients may include the following:

- Chemotherapy.
- Anagrelidetherapy.
- Biologic therapyusing interferon alfa or pegylated interferon alpha.
- Platelet apheresis.
- A clinical trialof a new treatment.

Use our clinical trial search to find NCI-supported cancer clinical trials that are accepting patients. You can search for trials based on the type of cancer, the age of the patient, and where the trials are being done. General information about clinical trials is also available.

Chronic Neutrophilic Leukemia

Treatment of chronic neutrophilic leukemia may include the following:

- Donorbone marrow transplant.
- Chemotherapy.
- Biologic therapyusing interferon alfa.

- A clinical trialof a new treatment.

Use our clinical trial search to find NCI-supported cancer clinical trials that are accepting patients. You can search for trials based on the type of cancer, the age of the patient, and where the trials are being done. General information about clinical trials is also available.

Chronic Eosinophilic Leukemia

Treatment of chronic eosinophilic leukemia may include the following:

- Bone marrow transplant.
- Biologic therapyusing interferon alfa.
- A clinical trialof a new treatment.

Use our clinical trial search to find NCI-supported cancer clinical trials that are accepting patients. You can search for trials based on the type of cancer, the age of the patient, and where the trials are being done. General information about clinical trials is also available.

To Learn More About Chronic Myeloproliferative Neoplasms

For more information from the National Cancer Institute about chronic myeloproliferative neoplasms, see the following:

- Myeloproliferative Neoplasms Home Page
- Drugs Approved for Myeloproliferative Neoplasms
- Biological Therapies for Cancer
- Blood-Forming Stem Cell Transplants
- Targeted Cancer Therapies

For general cancer information and other resources from the National Cancer Institute, see the following:

- About Cancer
- Staging
- Chemotherapy and You: Support for People With Cancer
- Radiation Therapy and You: Support for People With Cancer
- Coping with Cancer
- Questions to Ask Your Doctor about Cancer

- For Survivors and Caregivers

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