Treating Myelodysplastic Syndromes

If you’ve been diagnosed with a myelodysplastic syndrome (MDS), your treatment team will discuss your options with you. It’s important to weigh the benefits of each treatment option against the possible risks and side effects.

Which treatments are used for MDS?

The main types of treatment for MDS are:

- **Supportive Therapy for Myelodysplastic Syndromes**
- **Growth Factors for Myelodysplastic Syndromes**
- **Chemotherapy for Myelodysplastic Syndromes**
- **Stem Cell Transplant for Myelodysplastic Syndrome**

Common treatment approaches

Treatment is based on the type of MDS, MDS risk group and other factors, as well as your age and overall health. Often more than one type of treatment is used. Doctors plan each person’s treatment individually to give them the best chance of treating the tumor while limiting the side effects as much as possible.

- **General Approach to Treatment of Myelodysplastic Syndromes**

Who treats MDS?

Based on your treatment options, you may have different types of doctors on your treatment team. These doctors could include:

- A **hematologist**: a doctor who treats disorders of the blood
- A **medical oncologist**: a doctor who treats cancer with medicines

Many other specialists might be part of your treatment team as well, including physician
assistants (PAs), nurse practitioners (NPs), nurses, nutrition specialists, social workers, and other health professionals.

- Health Professionals Associated With Cancer Care

Making treatment decisions

It’s important to discuss all treatment options, including their goals and possible side effects, with your doctors to help make the decision that best fits your needs. You may feel that you need to make a decision quickly, but it’s important to give yourself time to absorb the information you have learned. Ask your cancer care team questions.

If time permits, it is often a good idea to seek a second opinion. A second opinion can give you more information and help you feel more confident about the treatment plan you choose.

- Questions to Ask Your Doctor About Myelodysplastic Syndromes
- Seeking a Second Opinion

Thinking about taking part in a clinical trial

Clinical trials are carefully controlled research studies that are done to get a closer look at promising new treatments or procedures. Clinical trials are one way to get state-of-the-art cancer treatment. In some cases they may be the only way to get access to newer treatments. They are also the best way for doctors to learn better methods to treat cancer. Still, they’re not right for everyone.

If you would like to learn more about clinical trials that might be right for you, start by asking your doctor if your clinic or hospital conducts clinical trials.

- Clinical Trials

Considering complementary and alternative methods

You may hear about alternative or complementary methods that your doctor hasn’t mentioned to treat your cancer or relieve symptoms. These methods can include vitamins, herbs, and special diets, or other methods such as acupuncture or massage, to name a few.

Complementary methods refer to treatments that are used along with your regular medical care. Alternative treatments are used instead of a doctor’s medical treatment. Although some of these methods might be helpful in relieving symptoms or helping you feel better, many have not been proven to work. Some might even be dangerous.
Be sure to talk to your cancer care team about any method you are thinking about using. They can help you learn what is known (or not known) about the method, which can help you make an informed decision.

- Complementary and Alternative Medicine

Help getting through cancer treatment

Your cancer care team will be your first source of information and support, but there are other resources for help when you need it. Hospital- or clinic-based support services are an important part of your care. These might include nursing or social work services, financial aid, nutritional advice, rehab, or spiritual help.

The American Cancer Society also has programs and services – including rides to treatment, lodging, and more – to help you get through treatment. Call our National Cancer Information Center at 1-800-227-2345 and speak with one of our trained specialists.

- Find Support Programs and Services in Your Area

Choosing to stop treatment or choosing no treatment at all

For some people, when treatments have been tried and are no longer controlling the cancer, it could be time to weigh the benefits and risks of continuing to try new treatments. Whether or not you continue treatment, there are still things you can do to help maintain or improve your quality of life.

Some people, especially if the cancer is advanced, might not want to be treated at all. There are many reasons you might decide not to get cancer treatment, but it’s important to talk to your doctors and you make that decision. Remember that even if you choose not to treat the cancer, you can still get supportive care to help with pain or other symptoms.

- If Cancer Treatments Stop Working
- Palliative or Supportive Care

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Supportive Therapy for Myelodysplastic Syndromes

Supportive therapies are treatments that help treat (or prevent) the symptoms or complications of myelodysplastic syndromes (MDS), as opposed to treating the MDS directly. Supportive therapy might be used alone or along with other treatments for MDS.

For example, for many patients with MDS, one of the main goals of treatment is to prevent the problems caused by low blood cell counts.

Treating low red blood cell counts (anemia)

Low red blood cell counts (anemia) can cause severe fatigue and other symptoms. Patients with MDS and anemia that’s causing symptoms might benefit from getting injections of a manmade version of the growth factor erythropoietin, which can sometimes help the bone marrow make new red blood cells.

If this isn't helpful, red blood cell transfusions might be needed. Some people are concerned about a slight risk of infections (such as hepatitis or HIV) spread by blood transfusion, but this possibility is very unlikely, and the benefits of the transfused cells greatly outweigh this risk.

Treating iron build up from blood transfusions

Blood transfusions can cause excess iron to build up in the body. The iron can build up over time in the liver, heart, and other organs, affecting how they function. This is usually seen only in people who receive many transfusions over a period of years. Patients at risk for iron overload are often advised to avoid taking iron supplements or multivitamins that contain iron.

Drugs called chelating agents, which bind with the iron so that the body can get rid of it, can be used in patients who develop iron overload from red blood cell transfusions (unless they have poor kidney function).

- Deferoxamine (Desferal) is usually given as an infusion under the skin, using a small, portable pump. This can be inconvenient because the infusion must be done slowly (over at least 8 hours) each day or on most days of the week.
• **Deferasirox** (Exjade, Jadenu) is a newer drug that is taken by mouth (as a tablet, dissolved in juice or water, or sprinkled on food) once a day.

For more information about red blood cell transfusions, see [Blood Transfusion and Donation](#).

## Treating low platelet counts

MDS patients with low platelet counts might have problems with bleeding or bruising easily. The options for treating a shortage of platelets might include **platelet transfusions** or treatment with certain **growth factor** drugs. If bleeding is not helped by these treatments, another option might be treatment with a drug called an **antifibrinolytic agent**, such as aminocaproic acid (Amicar).

For more information about platelet transfusions, see [Blood Transfusion and Donation](#).

## Treating low white blood cell counts

Patients with low white blood cell counts are more likely to get infections, and the infections are more likely to be serious. It's important to avoid cuts and scrapes, and take care of them right away if they do happen. Patients should tell their doctors right away about any possible signs of infection, such as fever, signs of pneumonia (cough, shortness of breath), or urinary infection (burning when urinating).

Doctors typically treat known or suspected bacterial infections with **antibiotics**. For serious infections, a white blood cell **growth factor** may also be used. This drug can raise the white blood cell count to help fight the infection.

See [Infections in People With Cancer](#) for more detailed information about infections and how to lower your risk.

- **References**


Growth Factors for Myelodysplastic Syndromes

Shortages of blood cells (red blood cells, white blood cells, or platelets) cause most of the symptoms in people with myelodysplastic syndromes (MDS). Hematopoietic growth factors can often help bring the blood counts closer to normal.

Hematopoietic growth factors are hormone-like substances that help bone marrow make new blood cells. These substances occur naturally in the body, but scientists have found ways to make large amounts of them in the lab. Patients can get these factors in larger doses than would be made by their own body.

Patients usually receive the growth factors through subcutaneous (under the skin) injections. Your health care team can give the injections, or you or your family members can learn to give them.

Red blood cell growth factors

- **Epoetin** (Epogen or Procrit) is a manmade version of the growth factor erythropoietin, which promotes red blood cell production. It can help some patients avoid red blood cell transfusions. Giving some patients both epoetin and G-CSF (see "White blood cell growth factors") can improve their response to the epoetin.
- **Darbepoetin alfa** (Aranesp) is a long-acting form of erythropoietin. It works in the same way but was designed to be given less often.

White blood cell growth factors

- **Granulocyte colony stimulating factor** (G-CSF, fligrastim, or Neupogen) and **granulocyte macrophage-colony stimulating factor** (GM-CSF, sargramostim, or...
Leukine) can improve white blood cell production. These are not used routinely to prevent infections, but they can help some MDS patients whose main problem is a shortage of white blood cells and who have frequent infections.

- **Pegfilgrastim** (Neulasta) is a long-acting form of G-CSF. It works in the same way but can be given less often.

**Platelet growth factors**

- Drugs called *thrombopoietin-receptor agonists*, such as **romiplostim** (Nplate) and **eltrombopag** (Promacta) might help some people with MDS who have very low platelet levels, although this is still being studied.
- A drug called **oprelvekin** (interleukin-11, IL-11, or Neumega) can be used to raise platelet counts after chemotherapy and in some other diseases. But for most MDS patients, this drug has not been found to be very helpful.

Studies are under way to find the best way to predict which patients will be helped by growth factors and the best way to combine growth factors with each other and with other treatments, such as chemotherapy.

**References**


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Chemotherapy (chemo) is the use of drugs for treating a disease such as cancer. Some chemo drugs can be swallowed as pills, while others are injected by needle into a vein or muscle. These drugs are considered systemic treatment because they enter the bloodstream and reach most areas of the body. This type of treatment is useful for diseases such as myelodysplastic syndrome (MDS) that are not only in one part of the body. The purpose of the chemo is to kill the abnormal stem cells and allow normal ones to grow back.

**Hypomethylating agents**

These types of chemo drugs affect the way certain genes inside a cell are controlled. These drugs activate some genes that help cell mature. They also kill cells that are dividing rapidly. Examples of this type of drug include:

- **Azacitidine (Vidaza)**
- **Decitabine (Dacogen)**

In some MDS patients, using one of these drugs can often improve blood counts (sometimes enough so that blood transfusions aren't needed), improve quality of life, lower the chance of getting leukemia, and even help a person live longer.

Azacitidine can be injected under the skin or into a vein (IV), often for 7 days in a row each month. Decitabine is often injected into a vein (IV) over 3 hours every 8 hours for 3 days. This is repeated every 6 weeks. Decitabine can also be given by IV over an hour, each day for 5 days in a row, and repeated every 4 weeks.

These drugs can have some of the same side effects as standard chemo drugs (see below), but these side effects are usually milder. A major side effect of these drugs is usually an early drop in blood cell counts, which tends to get better as the drug begins to work. Other side effects can include:

- Fever
- Nausea/vomiting
- Diarrhea or constipation
- Fatigue and weakness

**Standard chemotherapy drugs**

Standard chemo drugs are less useful for MDS than the hypomethylating agents, so they are not used often. But higher-risk MDS is more likely to progress to acute myeloid
leukemia (AML), so some patients with these types of MDS may receive the same chemotherapy treatment as AML patients.

The chemo drug most often used for MDS is called **cytarabine** (ara-C). It can be given by itself at a low-dose, which can often help control the disease, but doesn’t often put it into remission.

Another option is to give the same, intense type of chemo that is used for younger patients with AML. This means giving cytarabine at a higher dose along with other chemo drugs. This is more often used in younger, healthier patients with higher-risk forms of MDS (like MDS with excess blasts). Some of the chemo drugs that can be combined with cytarabine are:

- **Idarubicin**
- **Daunorubicin**

Other chemo drugs might be used as well.

Patients who get the higher dose treatment are more likely to go into remission, but they can also have more severe, even life-threatening side effects, so this treatment is typically given in the hospital. Still, this treatment may be an option for some patients with advanced MDS.

Chemo drugs can cause many **side effects**. These depend on the type and dose of the drugs that are given and how long they are taken. Common side effects include:

- Hair loss
- Mouth sores
- Loss of appetite
- Nausea and vomiting
- Low blood counts

MDS patients already have low blood counts, which often become even worse for a time before they get better.

- Low white blood cell counts lead to an increased risk of serious infections.
- Low platelet counts can lead to problems with easy bruising and can have serious bleeding, including bleeding into the brain or the intestine.
- Low red blood cell counts (or anemia) can lead to fatigue and shortness of breath.
  
  In people with heart problems, severe anemia can lead to a heart attack.

If a patient’s blood cell counts become too low, they may need **supportive therapy** (including transfusions) or **growth factors** to help prevent or treat serious side effects.
Most side effects from chemo will go away after treatment is finished. Your health care team can often suggest ways to lessen side effects. For example, drugs can be given to help prevent or reduce nausea and vomiting.

Chemo drugs can also affect other organs. For example:

- Idarubicin and daunorubicin can damage the heart, so they are often not given to patients who already have heart problems.
- Cytarabine can affect the brain and cause balance problems, sleepiness, and confusion. This is more common with higher doses.

If serious side effects occur, the chemo treatments may have to be reduced or stopped, at least temporarily. It’s important to carefully monitor and adjust drug doses, because some of these side effects can be permanent.

## Immune treatments

### Immunomodulating drugs (IMiDs)

**Lenalidomide (Revlimid)** belongs to a class of drugs known as immunomodulating drugs (IMiDs). It seems to work well in low-grade MDS, often eliminating the need for blood transfusions, at least for a time. The drug seems to work best in people whose MDS cells are missing a part of chromosome 5 (MDS-del(5q)). But it can also help some MDS patients that do not have this abnormal chromosome.

Side effects can include:

- Lowered blood counts (most often the white cell count and platelet count)
- Diarrhea or constipation
- Fatigue and weakness

This drug can also increase the risk of serious blood clots that start in the veins in the legs (called a deep vein thrombosis, or DVT). Sometimes, part of a DVT can break off and travel to the lungs (called a **pulmonary embolus** or PE), where it can cause breathing problems or even death.

This drug might also cause serious birth defects if given to pregnant women. Because of this, it’s only available through a special program by the drug company.

### Immune system suppression
Drugs that suppress the immune system can help some patients with lower-risk MDS. These drugs are most helpful for people with low numbers of cells in the bone marrow (called *hypocellular* bone marrow).

**Anti-thymocyte globulin (ATG)** is an antibody against a type of white blood cell called the *T-lymphocyte*, which helps control immune reactions. For some patients with MDS, T-lymphocytes interfere with normal blood cell production, so ATG can be helpful. ATG is given as an infusion through a vein. It must be given in the hospital because it can sometimes cause severe allergic reactions leading to low blood pressure and problems breathing.

**Cyclosporine** is another drug that can suppress the immune system. It can be used along with ATG to help some patients with MDS. Side effects of cyclosporine can include loss of appetite and kidney damage.

**References**


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**Stem Cell Transplant for Myelodysplastic Syndrome**

A stem cell transplant (SCT) currently offers the only realistic chance to cure
myelodysplastic syndrome (MDS), although many patients with MDS might not be eligible to have one. In this treatment, the patient receives high-dose chemotherapy and/or total body irradiation to kill the cells in the bone marrow (including the abnormal bone marrow cells). Then the patient gets new blood-forming stem cells.

There are 2 main types of SCT:

- For an allogeneic stem cell transplant, after the bone marrow is destroyed, the patient receives blood-forming stem cells from another person -- the donor. This is the type of transplant typically used for MDS. The results of this treatment tend to be best when when the donor’s cell type (also known as the HLA type) is closely matched to the patient’s cell type and the donor is closely related to the patient, such as a brother or sister. Less often, the donor is matched to the patient, but is not related.
- In an autologous stem cell transplant, the patient gets back their own stem cells (which were removed before treatment). This type of transplant is not typically used for patients with MDS because the patient's bone marrow contains abnormal stem cells.

Allogeneic SCTs can have serious, even life-threatening, side effects, so they are typically done in younger patients who are in relatively good health. Patients in their 60s or even 70s have been transplanted successfully, but in older patients the SCT is generally done using less intensive (reduced intensity) chemotherapy and/or radiation. The lower doses may not kill all the bone marrow cells, but they are just enough to allow the donor cells to take hold and grow in the bone marrow. The lower doses also cause fewer side effects, which makes this type of transplant easier for older patients to tolerate. Still, some serious side effects are still possible.

**Side effects**

The early side effects from a SCT are similar to the side effects expected from chemotherapy and radiation, only more severe. One of the most serious side effects is low blood counts, which can lead to risks of serious infections and bleeding.

Another possible serious side effect from allogeneic transplants is graft-versus-host disease (GVHD). This occurs when the new immune cells (from the donor) see the patient’s tissues as foreign and attack them. GVHD can affect any part of the body and can be life threatening.

Although allogeneic SCT is currently the only treatment that can cure some people with MDS, not everyone who gets a transplant is cured. In addition, some people may die
from complications of this treatment. Your chance for cure is higher if you are young and your MDS hasn’t begun to transform into leukemia. Still, doctors often recommend waiting until the MDS develops into a more advanced stage before considering a stem cell transplant.

For more information about stem cell transplants, see Stem Cell Transplant for Cancer.

- References


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General Approach to Treatment of Myelodysplastic Syndromes

The treatment approach for myelodysplastic syndromes (MDS) depends on a number of factors, such as:

- The type of MDS
- The prognostic score (risk group) of the MDS
- A person’s age, overall health, and preferences

Some people with MDS who don’t have very low blood cell counts or bothersome symptoms might not need to be treated right away.
If treatment is needed, a **stem cell transplant** (SCT) is usually considered the only way to potentially cure MDS, so it may be the treatment of choice for younger, relatively healthy patients if a matched donor is available. Unfortunately, many people with MDS are older or in poor health and might not be good candidates for a SCT.

When SCT is not an option, MDS is very unlikely to be cured, but it can often still be treated. The main goal of treatment is to relieve symptoms and avoid complications and side effects.

If low blood counts are causing problems, **supportive care** treatments such as **transfusions** or **blood cell growth factors** may be helpful. Careful general medical care and measures to prevent and treat infections are also very important. Supportive care is important regardless of whether a person is getting other treatments for MDS.

If other treatment is needed, a **chemotherapy** drug such as azacitidine (Vidaza) or decitabine (Dacogen) is often the first choice, especially for patients with **lower-risk** forms of MDS. These drugs can often improve blood counts, and many patients need fewer transfusions and have a better quality of life, with less fatigue. These drugs can also help some people live longer. Another option for some people might be medicines to suppress the immune system, such as ATG and cyclosporine.

If a person has the del(5q) type of MDS (where the cells are missing part of chromosome 5), lenalidomide (Revlimid) is often used as the first treatment. If this drug doesn’t help, treatment with azacitidine or decitabine is often the next option.

For some patients with more advanced MDS, such as those whose **prognostic scores** are high risk or higher, or those whose MDS looks like it is becoming **acute myeloid leukemia (AML)**, standard **chemotherapy** drugs might be an option. Unfortunately, this treatment can often be too toxic for patients who are elderly or who have many other medical problems. For young and healthy patients, though, the treatment is similar to treatment for AML.

If one type of treatment doesn’t work (or if it stops working), another one might be tried. Many new medicines to treat MDS are also being studied in **clinical trials**. Because the best options to treat MDS aren’t clear, and because MDS often becomes hard to treat over time, taking part in a clinical trial might be a good option at some point. Talk to your health care team to learn more about clinical trials that might be right for you.

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