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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 and Similar Medicines for Myelodysplastic Syndromes](#)
- [Drug Therapy 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

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 harmful.

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 Integrative Medicine](#)

Help getting through cancer treatment

People with cancer need support and information, no matter what stage of illness they may be in. Knowing all of your options and finding the resources you need will help you make informed decisions about your care.

Whether you are thinking about treatment, getting treatment, or not being treated at all, you can still get supportive care to help with pain or other symptoms. Communicating with your cancer care team is important so you understand your diagnosis, what treatment is recommended, and ways to maintain or improve your quality of life.

Different types of programs and support services may be helpful, and can be 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.

- [Palliative Care](#)
- [Programs & Services](#)

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](#)

The treatment information given here is not official policy of the American Cancer Society and is not intended as medical advice to replace the expertise and judgment of your cancer care team. It is intended to help you and your family make informed decisions, together with your doctor. Your doctor may have reasons for suggesting a treatment plan different from these general treatment options. Don't hesitate to ask your cancer care team any questions you may have about your treatment options.

Supportive Therapy for Myelodysplastic Syndromes

- [Treating low red blood cell counts \(anemia\)](#)
- [Treating low platelet counts](#)
- [Treating low white blood cell counts](#)
- [More information about palliative care](#)

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. The main purpose of this type of treatment is to improve the comfort and quality of life for someone diagnosed with cancer no matter what stage the cancer or the goal of treatment might be. You might also hear supportive care referred to as palliative care, symptom management, or comfort care.

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.

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](#)².

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 tract 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](#)³ for more detailed information about infections and how to lower your risk.

More information about palliative care

To learn more about how palliative care can be used to help control or reduce symptoms caused by cancer, see [Palliative Care](#)⁴.

To learn about some of the side effects of cancer or treatment and how to manage them, see [Managing Cancer-related Side Effects](#)⁵.

Hyperlinks

1. www.cancer.org/cancer/managing-cancer/treatment-types/blood-transfusion-and-donation.html
2. www.cancer.org/cancer/managing-cancer/treatment-types/blood-transfusion-and-donation.html

3. www.cancer.org/cancer/managing-cancer/side-effects/low-blood-counts/infections.html
4. www.cancer.org/cancer/managing-cancer/palliative-care.html
5. www.cancer.org/cancer/managing-cancer/side-effects.html

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Estey EH, Schrier SL. Management of complications of the myelodysplastic syndromes. UpToDate. 2017. Accessed at <https://www.uptodate.com/contents/management-of-the-complications-of-the-myelodysplastic-syndromes> on October 12, 2017.

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Growth Factors and Similar Medicines for Myelodysplastic Syndromes

- [Red blood cell growth factors](#)
- [White blood cell growth factors](#)
- [Platelet growth factors](#)

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.

Other medicines that raise blood cell counts in different ways might also be helpful for some people.

Patients usually receive growth factors and similar drugs through subcutaneous (under the skin) injections. Your health care team can give the injections, or you or your family members might be able to learn to give them at home.

Red blood cell growth factors

Epoetin (Epoen or Procrit) is a manmade version of the growth factor

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 similar drugs, as well as the best way to combine growth factors with each other and with other treatments, such as [chemotherapy](#).

Hyperlinks

1. www.cancer.org/cancer/managing-cancer/treatment-types/blood-transfusion-and-donation.html

References

Estey EH, Schrier SL. Management of complications of the myelodysplastic syndromes. *J Clin Oncol*. 1998;16(12):3611-3621.

Drug Therapy for Myelodysplastic Syndromes

- [Immune therapy](#)
- [Targeted therapy](#)

Different types of drugs might be used to treat myelodysplastic syndrome (MDS).

Chemotherapy

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 MDS that are not only in one part of the body. The purpose of the chemo is to kill the abnormal stem cells in the bone marrow 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 cells 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 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, once a 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.

A newer form of this drug, known as **Inqovi**, combines decitabine with cedazuridine, which helps stop the decitabine from being broken down in the digestive system. This allows the drug to be taken by mouth as a tablet, typically once a day for 5 days in a row, which is repeated every 4 weeks.

Hypomethylating agents 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](#)¹ MDS is more likely to progress to [acute myeloid leukemia](#)² (AML), so some patients with these types of MDS may receive the same chemo treatment as AML patients.

The chemo drug most often used for MDS is **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 have their MDS 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](#)³. These depend on the type and dose of the drugs given and how long they are taken. Common side effects include:

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

Most patients with leukemia 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 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

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.

Targeted therapy

IDH inhibitor

In some people with MDS, the cancer cells have a change (mutation) in the IDH1 gene, which normally helps cells make the IDH1 protein. Mutations in this gene can lead to an abnormal IDH1 protein, which can stop MDS cells from maturing the way they normally would.

Ivosidenib (Tibsovo) is an IDH1 inhibitor. It blocks the abnormal IDH1 protein, which seems to help the cancer cells mature into more normal cells. This drug can be used in people with advanced, previously treated MDS, if the cancer cells are found to have an IDH1 mutation. Your doctor can test your cancer cells to see if they have an IDH1 mutation.

This drug is taken by mouth, once a day.

Possible side effects of ivosidenib

Common side effects can include fatigue, nausea, vomiting, abdominal (belly) pain or swelling, diarrhea, loss of appetite, cough, low red blood cell counts (anemia), rash, and changes in lab tests showing the drug is affecting the liver.

Less common but more serious side effects can include changes in heart rhythm, pneumonia, and jaundice (yellowing of the eyes and skin).

More information about targeted therapy

To learn more about how targeted drugs are used to treat cancer, see [Targeted Cancer Therapy](#)⁴. To learn about some of the side effects listed here and how to manage them, see [Managing Cancer-related Side Effects](#)⁵.

Hyperlinks

1. www.cancer.org/cancer/types/myelodysplastic-syndrome/detection-diagnosis-staging/staging.html
2. www.cancer.org/cancer/types/acute-myeloid-leukemia.html
3. www.cancer.org/cancer/managing-cancer/side-effects.html
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Stem Cell Transplant for Myelodysplastic Syndrome

- [Side effects](#)
- [More information about stem cell transplant](#)

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 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

Hyperlinks

1. www.cancer.org/cancer/managing-cancer/treatment-types/stem-cell-transplant.html
2. www.cancer.org/cancer/managing-cancer/side-effects.html

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