Myelodysplastic syndromes (MDS) are related diseases. The different types of MDS vary in their prognosis and response to treatment. Treatment is based on the type of MDS, as well as the patient’s age and health. Patients with these diseases are treated by specialists, such as a hematologist or an oncologist.

The main types of treatment for MDS are:

- **Supportive therapy**
- **Growth factors**
- **Chemotherapy** (including hypomethylating drugs)
- **Stem cell transplant**

Often, a combination of these are used. It is important to discuss all of your treatment options, including their goals and possible side effects, with your doctors to help make the decision that best fits your needs. It’s also very important to ask questions if there is anything you’re not sure about. You can find some good questions to ask in [What Should You Ask Your Doctor About Myelodysplastic Syndromes?](#)

*This information represents the views of the doctors and nurses serving on the American Cancer Society’s Cancer Information Database Editorial Board. These views are based on their interpretation of studies published in medical journals, as well as their own professional experience. This treatment information is not official policy of the 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 him or her questions about your treatment options.*

### Supportive Therapy for Myelodysplastic Syndromes
For many patients with a myelodysplastic syndrome (MDS) the main goal of treatment is to prevent the problems caused by low blood cell counts. For example, low red blood cell counts (anemia) can cause severe fatigue. Patients with MDS and anemia often benefit from receiving red blood cell transfusions if erythropoietin isn’t helping them.

Some people are concerned about a slight risk of infection (hepatitis or HIV) spread by blood transfusion, but this possibility is very unlikely, and the benefits of the transfused cells greatly outweigh this risk.

Blood transfusions can cause excess iron to build up in the body. This extra iron can deposit in the liver and heart, making the organs function poorly. Iron build up is usually seen only in people who receive many transfusions over a period of years. Drugs called chelating agents (substances that bind with metal so that the body can get rid of it) can be used in patients who develop iron overload from transfusions.

The most commonly used drug is desferoxamine. This drug helps treat and prevent iron overload. It is given intravenously or injected under the skin. This can be inconvenient because the injection must be given slowly (over several hours) 5 to 7 times per week. In some patients, treatment continues for years.

Deferasirox (Exjade®) is a newer drug that is taken by mouth once a day to treat iron overload. It has been used more for patients with certain congenital anemias (like thalassemia), but it can also help some MDS patients. Patients with poor kidney function should not take this drug.

MDS patients with bleeding problems resulting from a shortage of platelets may benefit from platelet transfusions.

For more information about transfusions, see Blood Transfusion and Donation.

Patients with low white blood cell counts are very susceptible to infections. They should especially avoid cuts and scrapes or take care of them right away. They should tell their doctors immediately about any fever, signs of pneumonia (cough, shortness of breath), or urinary infection (burning when urinating). Doctors will treat known or suspected infections with antibiotics. For serious infections, a white blood cell growth factor may also be used. This drug can help raise the white blood cell count to fight the infection. See Infections in People With Cancer for more detailed information about infections and how to lower your risk.

- References
See all references for Myelodysplastic Syndromes
Growth Factors for Myelodysplastic Syndrome

Hematopoietic growth factors are hormone-like substances that stimulate bone marrow to produce blood cells. These substances occur naturally in the body, but scientists have found a way to make them outside of the body in large amounts. This allows patients to get these factors in larger doses than would be produced by their own body.

Shortages of blood cells cause most of the symptoms in people with myelodysplastic syndromes (MDS), and growth factors can help the blood counts to become more normal. The growth factors granulocyte colony stimulating factor (G-CSF, Neupogen®, or filgrastim) and granulocyte macrophage-colony stimulating factor (GM-CSF, Leukine®, or sargramostim) can improve white blood cell production. These can benefit some MDS patients whose main problem is a shortage of white blood cells and who suffer from frequent infections. Pegfilgrastim (Neulasta®) is a long-acting form of G-CSF. It works in the same way but can be given less often.

Erythropoietin (Epo® or Procrit®), a growth factor that promotes red blood cell production, can help avoid red blood cell transfusions in some patients. Giving some patients both erythropoietin and G-CSF improves their response to the erythropoietin. Darbepoetin alfa (Aranesp®) is a long-acting form of erythropoietin. It works in the same way but was designed to be given less often.

A drug called oprelvekin (Neumega®, interleukin-11, or IL-11) can be used to stimulate platelet production after chemotherapy and in some other diseases. This drug can help increase the platelet counts of some MDS patients for a time, but then the counts go back down again. For most MDS patients, this drug is not very helpful.

More studies are under way to find the best way to predict which patients will benefit from growth factors and the best way to combine growth factors with each other and with other treatments, such as chemotherapy or hormones. 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.

*Androgens,* or male hormones, can boost blood cell production that is abnormally low due to certain diseases. A few people with MDS may be helped by androgens but most do not improve. If no other treatment options are appropriate for a patient, some doctors recommend trying androgens. However, these hormones can cause side effects, such as liver problems or muscle cramps. In women, androgens can produce male features such as growth of facial and body hair and can increase the sex drive.

- References

See all references for Myelodysplastic Syndromes

Chemotherapy for Myelodysplastic Syndromes

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

Conventional chemotherapy

Because MDS can progress to *acute myeloid leukemia* (AML), patients with MDS may receive the same treatment as AML patients. The chemo drug most often used for MDS is called cytarabine (ara-C). It can be given by itself in a low-dose, which can help control the disease, but doesn’t often put it into remission. This treatment is also used for older patients with AML.

Another option is to give the same chemo that is used in younger patients with AML.
This means giving cytarabine at a higher dose along with other chemo drugs. This is more often used for advanced MDS (like refractory anemia with excess blasts). For the treatment of MDS, the chemo drugs most often combined with cytarabine are:

- Idarubicin
- Topotecan
- Fludarabine

Patients treated with the higher dose treatment are more likely to go into remission, but they have more severe side effects. These can lead to death. Still, this treatment may be an option for some patients with advanced MDS.

Using cytarabine by itself at a low dose has a lower chance of serious side effects (including death).

Chemo drugs can cause many side effects. The side effects 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

Chemo often slows blood production, leading to 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. When platelet counts get low, patients have 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.

At times when their white blood cell counts are very low, patients may need to take steps to reduce their risk of infection, such as avoiding exposure crowds and being very careful about washing their hands. Some patients need to take antibiotics, which may be given before signs of infection or at the earliest sign that an infection may be developing. For more information about infections and ways to protect against them, see Infections in People With Cancer.

While their platelet counts are low, patients may receive platelet transfusions as to prevent or treat bleeding. Likewise, low red blood cell counts can be treated with red blood cell transfusions or with growth factors, such as erythropoietin (discussed below),
to raise red blood cell counts. More information about transfusions can be found in Blood Transfusion and Donation.

Most side effects are temporary and will go away after treatment is finished. Your health care team often can suggest ways to lessen side effects. For example, other drugs can be given along with the chemo to prevent or reduce nausea and vomiting.

Chemo drugs can also affect other organs such as the kidneys, liver, testicles, ovaries, brain, heart, and lungs. For example, drugs like idarubicin can damage the heart, and so 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 of this drug. If serious side effects occur, the chemo treatments may have to be reduced or stopped, at least temporarily.

Carefully monitoring and adjusting drug doses are important because some of these side effects can be permanent.

Hypomethylating agents

These drugs are actually a form of chemo that affect the way genes are controlled. They help in MDS by slowing down genes that promote cell growth. They also kill cells that are dividing rapidly. Examples of this type of drug include azacitidine (Vidaza®) and decitabine (Dacogen®). In some MDS patients, these drugs improve blood counts, lower the chance of getting leukemia, and even prolong life. Red blood cell counts may improve enough to stop transfusions.

These drugs have some of the same side effects as regular chemo, but these side effects are usually mild. They include:

- Nausea/vomiting
- Diarrhea or constipation
- Fatigue and weakness
- Low blood counts (most often the white blood cells or platelets)

Immune treatments

Immune modulating drugs: The drugs thalidomide and lenalidomide (Revlimid®) belong to the class of drugs known as immunomodulating drugs (or IMiDs). Thalidomide was used first in treating MDS. It helped some patients, but many people stopped taking the drug because of side effects. Lenalidomide is a newer drug related to thalidomide that has fewer side effects. It seems to work well in low-grade MDS, eliminating the
need for transfusions in about half the patients treated. The drug seems to work best in people whose MDS cells are missing a part of chromosome number 5 (this is called del(5q) or 5q-) and is approved by the FDA to treat these patients. It can also help MDS patients that do not have this abnormal chromosome.

Side effects include:

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

Both of these drugs can also increase the risk of serious blood clots that start in the veins in the legs (called deep venous thrombosis or DVT). Part of a DVT can break off and travel to the lungs (called a pulmonary embolus or PE), where it can cause problems with breathing or even death. Many experts feel that patients getting this drug should also get some kind of treatment to prevent blood clots.

When thalidomide was first released in the 1960s, it caused serious birth defects when given to pregnant women. This led to the drug being taken off the market for many years. Now, it is only available through a special program of the drug company. Lenalidomide hasn’t been shown to cause birth defects, but concern about this risk has limited the availability of this drug as well. It is also only available through a program from the company that makes it.

**Immunosuppression:** Drugs that suppress the immune system can help some patients with MDS. These drugs are used more often in patients with aplastic anemia, a condition where the immune system attacks the bone marrow, leading to low blood counts. In MDS, these drugs are most helpful in patients with low numbers of cells in the bone marrow (called hypocellular bone marrow).

A drug called *anti-thymocyte globulin* (ATG) has helped some people, usually younger ones, with MDS. The drug is an antibody against a type of white blood cell called the T-lymphocyte. T-lymphocytes help control immune reactions. In some patients with MDS, T-lymphocytes interfere with normal blood cell production. 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.

Another drug that works by suppressing the immune system is called *cyclosporine*. It was first used to block immune responses in people who have had organ or bone marrow transplants, but it has helped some patients with MDS. Side effects of cyclosporine include loss of appetite and kidney damage.
Stem Cell Transplant for Myelodysplastic Syndrome

Stem cell transplant (SCT) is the only treatment that can cure myelodysplastic syndrome (MDS). 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 receives new, healthy blood-forming stem cells. There are 2 main types of SCT: **allogeneic** and **autologous**.

In an autologous stem cell transplant, after the bone marrow is destroyed, the patient gets back their own stem cells. This type of transplant is not a standard treatment for patients with MDS because their bone marrow contains abnormal stem cells.

For an allogeneic stem cell transplant, the patient receives blood-forming stem cells from another person -- the donor. The best treatment results occur when the donor’s cells are 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.

Allogeneic stem cell transplant can have serious, even fatal, side effects and so is rarely used in elderly patients. Because of these side effects, some doctors restrict this treatment to people younger than a certain age.

A special type of allogeneic transplant, called **non-myeloablative allogeneic stem cell transplant** may be an option for older patients. This type of transplant is sometimes called a **mini-transplant** or a **mini-allo**. For this kind of transplant, the doses of chemotherapy and/or radiation that are given are lower than those used for a standard allogeneic transplant. These doses are not high enough to 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 of chemotherapy and/or radiation cause fewer side effects, which makes this type of transplant easier for older patients to tolerate. Still, some serious side effects remain.

**Side effects**

The early side effects from a stem cell transplant (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.

The most serious side effect from allogeneic transplants is called *graft-versus-host disease* (or GVHD). This occurs when the new immune cells (from the donor) see the patient’s tissues as foreign and so 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 patients with MDS, not all patients who get a transplant are cured. In addition, patients 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 recommend waiting until the MDS develops into a more advanced stage before considering transplant.

For more information about stem cell transplants, see [Stem Cell Transplant for Cancer](#).

- **References**
- See all references for Myelodysplastic Syndromes

Last Medical Review: February 10, 2014 Last Revised: July 2, 2015

American Cancer Society medical information is copyrighted material. For reprint requests, please see our [Content Usage Policy](#).

**General Approach to Treatment of Myelodysplastic Syndromes**

Stem cell transplant (SCT) is usually considered the only curative option for patients
with myelodysplastic syndrome (MDS), and may be the treatment of choice for younger patients when a matched donor is available. This is the recommended treatment for nearly all children. For older patients, either the high-dose or low-dose approach can be used. For either of these options, it appears best to wait until the disease is advanced before performing the SCT.

When SCT is not an option, MDS is not considered curable. In that case, the goal is to relieve symptoms and avoid complications and side effects of treatment. Patients with mild low blood counts and few symptoms may be carefully watched without treatment for a while. If low blood counts are causing problems, supportive care treatments such as transfusions, blood cell growth factors, and possibly androgens may be helpful.

If a person has the 5q- type of MDS, then lenalidomide (Revlimid) is often used as the first treatment. If this drug doesn’t help, treatment with azacitidine (Vidaza) or decitabine (Dacogen) is often the next option.

Treatment with azacitidine or decitabine is often the first choice for patients with MDS without the 5q- chromosome problem. Azacitidine can be injected under the skin, often for 7 consecutive days every month. The standard dosing of decitabine is to inject it into a vein (IV) every 8 hours for 3 days every 6 weeks. Since this means that the patient has to stay in the hospital for treatment, studies were done to see if the drug would still work if given on a different schedule. One option that seems to work well is to give the drug IV daily for 5 days every 4 weeks. This allows it to be given in an outpatient clinic. The major side effect of these drugs is an early drop in blood counts, as seen with most chemotherapy drugs. If the drug is successful, blood counts will improve to levels that are better than those seen before treatment was started.

A major benefit for patients receiving azacitidine or decitabine is that they need fewer transfusions and have a better quality of life. In particular, if they respond, they have less fatigue and are able to function more normally. Finally, these drugs can increase life span in some patients.

Other drugs, such as those mentioned previously, have helped some patients. It may be worth joining a clinical trial or receiving these agents outside a trial, if none is available.

Careful general medical care and measures to prevent and treat infections are very important. Patients should think about taking part in clinical trials of new treatments.

Conventional chemotherapy is an option for some patients with more advanced MDS, such as those whose prognostic scores are high risk or higher (discussed in the staging section), or those whose MDS looks like it is becoming an acute leukemia. Unfortunately, this treatment is too toxic for patients who are elderly or who have many
other medical problems and in these patients it can shorten their lives. For young and healthy patients, though, the treatment is similar to treatment for acute myeloid leukemia.

- References
  See all references for Myelodysplastic Syndromes

Last Medical Review: February 10, 2014 Last Revised: July 2, 2015

American Cancer Society medical information is copyrighted material. For reprint requests, please see our Content Usage Policy.

2016 Copyright American Cancer Society

For additional assistance please contact your American Cancer Society
1-800-227-2345 or www.cancer.org