

Treating and Living with Myelodysplastic Syndromes (MDS)

Treating Lower-risk MDS

The treatment options for people with lower-risk MDS consist of wait and watch, supportive care, immunosuppressive therapy, and medicines recently approved to treat MDS. This reference summary has more details about these treatment options.

Wait and Watch

If you have early stage or very low-risk MDS with minimal symptoms and you don't have a current need for blood transfusions, your doctor may suggest that the best treatment plan is to simply wait and watch. This will involve checking blood counts on a regular basis and keeping an eye on your symptoms.

Supportive Care

Supportive care is often the best treatment for lower-risk MDS patients. A patient with lower-risk MDS:

- May not need blood transfusions
- Usually does not have an excess of blasts in the bone marrow



Supportive care can increase blood counts. It can reduce problems caused by infection and too much bleeding. This type of care includes the use of:

- **Red blood cell transfusions:** If you don't have enough healthy red blood cells, your doctor may recommend you get a red blood cell transfusion. Some patients require transfusions only once in a while, and some may need a transfusion as often as every week or two. You can have transfusions as often as needed.
- **Platelet transfusions:** Patients with a low platelet count tend to bruise and bleed easily. This condition is called thrombocytopenia. If you don't have enough healthy platelets in your blood, you may get a platelet transfusion. Unfortunately, platelet transfusions don't last very long. Over time platelet transfusions can become less effective. For this reason, your doctor may recommend other

strategies to avoid platelet transfusions. Platelets live just 8 to 10 days. So transfusion helps for only a short time. Some patients need transfusions a couple of times a week to prevent uncontrolled bleeding.

- Red cell growth factors: If you don't have enough healthy red blood cells, your doctor may ask you to take a red blood cell growth factor. It causes your bone marrow to make more red blood cells.
- Your kidneys make a red blood cell growth factor called EPO, or erythropoietin. If you have anemia and your kidneys aren't making enough natural EPO, your doctor may recommend you take a form of EPO that is made in a lab. Most experts recommend that your doctor check your blood EPO level before you take a man-made form of EPO.
- White cell growth factors: If you don't have enough healthy white cells in your blood, your doctor may ask you to take a white blood cell growth factor to improve your immune system, especially if you are having an infection. White blood cell growth factors cause your bone marrow to make white blood cells. Doctors are careful about giving white cell growth factors to MDS patients. White cell growth factors have not shown any benefit to MDS patients who are not having infections. The long-term safety of taking white cell growth factors is not clear. For this reason, the use of these growth factors for MDS patients is often limited to the treatment of infections, along with antibiotics.
- Antibiotics: The most common white blood cells are called neutrophils. They fight infection. Patients who don't have enough healthy neutrophils are said to have neutropenia. Because of their low white count, they may get infections easily and have trouble getting rid of infections. Doctors may give patients with neutropenia antibiotics to prevent and fight infections.
- Iron chelation to treat iron overload: If you have iron overload, your doctor may ask you to take an iron chelator. This is a medicine that will help take excess iron out of your body. You should get your blood iron level checked regularly if you get red blood cell transfusions often. That's because the transfusions can cause you to have too much iron in your body. This can lead to a condition called iron overload. Iron can build up in your heart and other vital organs and damage them. Iron overload can start to become a problem after as few as 10 blood transfusions (20 units). If your blood iron level, or ferritin, is between 1,000 and 2,000, this is considered high and treatment may be necessary.

Your doctor may also ask you to take a white cell growth factor along with EPO. This combination can improve red cell counts in some patients.

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Medicines Approved by the by FDA to Treat MDS

The U.S. Food and Drug Administration, or FDA, has approved 3 medicines to treat MDS. They are:

- Azacitidine (Vidaza®)
- Decitabine (Dacogen®)
- Lenalidomide (Revlimid®)

Azacitidine (Vidaza®)

Azacitidine was the first medicine approved specifically to treat MDS. It can be used by both low- and high-risk patients with all subtypes of MDS. In some patients it can prevent the growth of abnormal bone marrow stem cells and improve blood counts.

It can be given as an injection under the skin or as an IV, where it slowly drips into your vein. It is given in a clinic or hospital. In high-risk patients, azacitidine has been shown to improve two year survival compared to a group of patients getting supportive care or chemotherapy.

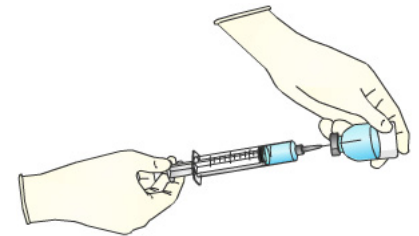


Given Through an IV

Decitabine (Dacogen®)

Decitabine and azacitidine are similar drugs. Like azacitidine, decitabine can be used by low- and high-risk patients with all subtypes of MDS. In some patients this drug can prevent the growth of abnormal bone marrow stem cells and improve blood counts.

Decitabine is taken as an injection in the vein in a clinic or hospital. Research has not shown that this drug provides the same long-term survival benefits as azacitidine in high-risk patients.



Given by Injection

Lenalidomide (Revlimid®)

Lenalidomide is approved for treating lower-risk transfusion-dependent MDS in patients with isolated deletion 5q whether or not tests show they have other abnormal chromosomes. It is a capsule you take by mouth and it can be taken at home. Researchers are not sure exactly how lenalidomide treats MDS.



Capsule Taken
by Mouth

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If you have deletion 5q MDS you are missing a section of chromosome 5 in one of your bone marrow stem cells. This type of MDS affects between 5% and 15% of MDS patients.

Low-risk patients with deletion 5q have about a 75% (75 out of every 100) chance of responding to lenalidomide. Many of these patients no longer need transfusions. This drug can also improve anemia in about 25 out of 100 low-risk patients who don't have deletion 5q.

Immunosuppressive Therapy

With some types of MDS, the immune system may attack the blood cells or the bone marrow cells which make blood cells. Immunosuppressive therapy uses medicines to keep the immune system from attacking the bone marrow. This therapy helps some MDS patients, especially low-risk patients.

The immunosuppressive medicines most often given include antithymocyte globulin, or ATG for short, and cyclosporine. ATG is given by IV, typically for 8 to 12 hours a day, for 4 days. It must be given in a hospital or other inpatient treatment facility. Cyclosporine comes in a liquid or pill form and is taken at home as an outpatient.

Positive responses to ATG may last for longer than 2 years. As many as 30 in every 100 MDS patients may benefit from ATG, but doctors are still not sure how to best select patients for this therapy. As with any drug used to treat MDS, the beneficial effects of ATG are temporary.

Treating Higher-Risk MDS

In patients with higher-risk MDS, blast cells will often fill the bone marrow, not allowing other cells to grow. Higher-risk MDS is strongly linked to the development of acute myeloid leukemia or AML.

AML stands for acute myelogenous leukemia and is a type of cancer of the white blood cells. In AML, abnormal white blood cells reproduce quickly and build up in the blood and bone marrow.

A person can develop higher-risk MDS at any point in their disease. However, higher-risk MDS is more commonly a progression from lower risk stages of the disease.



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People are defined as having higher-risk MDS when they have an International Prognostic Scoring System score of 1.5 or more.

Most patients who progress to higher-risk MDS will later develop AML if left untreated.

In addition to the supportive care treatments, there are a number of options available to treat higher-risk MDS.

They include:

- Azacitidine and decitabine are approved by the FDA specifically to treat higher-risk MDS patients. Azacitidine and decitabine can be used to treat both low and high-risk patients with all subtypes of MDS. These drugs help prevent the growth of abnormal bone marrow stem cells.
- Intensive chemotherapy drugs similar to those used to treat AML: Intensive chemotherapy for MDS patients involves the use of medicines to kill abnormal cells. Chemotherapy hurts healthy cells along with abnormal ones. So you may need to stay in the hospital for a few weeks after treatment. During this time, you will get transfusions of red blood cells and platelets. You will also take medicine to fight infection. If the chemo works in controlling the abnormal cells, then relatively normal blood cells will start to grow again within several weeks. As normal cells grow, you will have less need for transfusions and antibiotics – and maybe even no need at all. As with other treatments used for MDS, the beneficial effects of intensive chemotherapy are temporary.
- Stem cell transplantation, also called a bone marrow transplantation



Stem Cell Transplantation

A possible cure for MDS patients is allogeneic stem cell transplantation (also called allogeneic bone marrow transplantation). Not all MDS patients are candidates for this treatment option.

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Candidates for stem cell transplantation must be carefully screened. Some of the things your doctor will look at to learn whether you are a good candidate include:

- Your general health
- Your age
- Other medical problems you may have
- The types of donors available to you

For a donor to be a match for you, they need to share some or all of your white blood cell proteins called HLA proteins.

If stem cell transplantation is an option for you, start looking for a donor right away. The first step is to get family members tested to see if any of them are a good match for you.



If you do not have a matched donor who is a close relative, your doctor can help you search for other kinds of donors.

After a stem cell transplantation patients may require hospitalization for several weeks. Doctors will also require close monitoring and regular follow ups with the patient for several months or even years.

Clinical Trials

Scientists are always looking for new and better ways to treat MDS. Clinical trials are controlled research studies of new drugs or treatment approaches.

Clinical trials, also called research studies, can be a good option for some patients, even those that are just starting treatment. So before you start any treatment, you may want to talk to your doctor about whether you may be eligible for a clinical trial.

Good candidates for a clinical trial often include:

- Patients who have tried standard therapies but it has not made their MDS better.
- Patients who have had MDS return after standard therapies.
- Patients who have not started any treatment yet.



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Before considering a clinical trial discuss the possible risks and benefits with your doctor.

Clinical trials have several objectives. They may:

- Compare new treatments or new combinations of treatments with standard treatments
- Help scientists learn more about standard treatments
- Test the safety and effectiveness of new treatments

Living with MDS

In addition to medical treatments you get, your lifestyle plays a key role in managing your MDS. By taking good care of your body and mind, you allow yourself to be as healthy as possible. Here are some tips.

1. *Eat a healthy and well-balanced diet*

Although there is no specific diet that is best for people with MDS, getting proper nutrition is important for healthy blood production. Because your nutritional needs can be affected by your disease and medications you take, you should check with your doctor about your individual nutritional needs and the best diet for you.



2. *Get the right amount of exercise.*

It's good for your body to get some form of regular exercise. But if you have MDS, you may not have much energy to stay active. Plus, you may need to take special precautions. Your doctor can help you find the best fitness plan for you.



If you have a low platelet count:

- You should avoid any activities that could cause bleeding.
- Ask your doctor about safe ways to stay active.
- Tell your doctor if you get a bad headache or pain - that could mean you are bleeding.

If you have a low red count:

- You should ask your doctor about what amount of exercise and what activities will work for you.
- Tell your doctor if you have chest pains or shortness of breath when you exert yourself.

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- Tell your doctor if you are going on an airplane or traveling in the mountains. You may need blood cell transfusions to make sure you get enough oxygen.

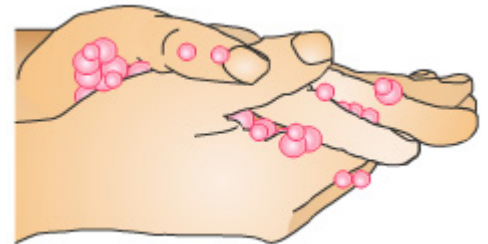
3. *Protect yourself from getting infected by germs*

- Brush your teeth often to prevent the need for dental work. Dental work can cause infection. Check with your doctor before flossing.
- Keep minor infections from getting serious. Tell your doctor if you have a fever or feel very tired. These can be early signs of infection.
- Make sure your food is not too hot. Burns can cause infection in your mouth.



If you have a very low white count you might take these extra steps to protect yourself from infection:

- Wash your hands often.
- Carry a lotion that kills bacteria (anti-bacterial) when you go out. Use it if you can't find soap and water.
- Stay away from crowds and sick people.



Wash Your Hands

Your doctor may recommend a diet to keep germs out of your food and help protect you from infection. Here are some of the things you need to do if you follow this diet:

- Do not eat any cheeses that say “aged” on the label.
- Only drink milk that has been pasteurized to kill germs. Milk you buy from the supermarket is fine to drink.
- Stay away from homemade fermented drinks. This includes homemade wine, cider, root beer, ale and vinegar.
- Stay away from buffets, salad bars, and crowded restaurants. Germs could end up on your food.
- Wash and peel fresh fruit and vegetables very well before eating them.



Wash Fruits
and Vegetables

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4. Relax and reduce stress

Different people react in different ways to having MDS. Here are some other ways to take care of your mind:

- Join a support group.
- Learn more about MDS.
- Speak with a Chronic Illness Counselor.
- Take an anti-depressant medicine, if your doctor says it will help you.

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