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Myelodysplastic Syndromes - MDS - Treatment Options [1]

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ON THIS PAGE: You will learn about the different ways doctors use to treat people with MDS. To see other pages, use the menu on the side of your screen.

This section outlines treatments that are the standard of care (the best proven treatments available) for this specific disease. When making treatment plan decisions, patients are also encouraged to consider clinical trials as an option. A clinical trial is a research study to test a new approach to treatment to evaluate whether it is safe, effective, and possibly better than the standard treatment. Clinical trials may test such approaches as a new drug, a new combination of standard treatments, or new doses of current therapies. Your doctor can help you review all treatment options. For more information, see the [Clinical Trials](#) [3] and [Latest Research](#) [4] sections.

Treatment overview

For MDS, different types of doctors often work together to create a patient's overall treatment plan that combines different types of treatments. This is called a [multidisciplinary team](#) [5].

Descriptions of the most common treatment options for MDS are listed below. The goal of treatment is to bring about a remission and to bring blood counts back to normal. Treatment options and recommendations depend on several factors, including the subtype and IPSS-R score of MDS, the risk of developing [AML](#) [6], possible side effects, and the patient's preferences, age, and overall health. As explained in the [Subtypes and Classification](#) [7] section, a patient's IPSS-R score and MDS subtype help doctors determine when treatment should be begin, but it is important to note that the treatments may not be equally effective for every patient. Your care plan may also include treatment for symptoms and side effects, an important part of your medical care. Take time to learn about all of your treatment options and be sure to ask questions about things that are unclear. Also, talk about the goals of each treatment with your doctor and what you can expect while receiving the treatment. Learn more about [making treatment decisions](#) [8].

Chemotherapy

Chemotherapy is the use of drugs to destroy cancer cells, usually by stopping the cancer cells' ability to grow and divide. Chemotherapy is given by a medical oncologist, a doctor who specializes in treating cancer with medication, or by a hematologist, a doctor who specializes in treating blood disorders.

Systemic chemotherapy is delivered through the bloodstream to reach cancer cells throughout the body. Common ways to give chemotherapy include an intravenous (IV) tube placed into a vein using a needle or in a pill or capsule that is swallowed (orally). A chemotherapy regimen, or schedule, usually consists of a specific number of cycles given over a set period of time. A patient may receive one drug at a time or combinations of different drugs at the same time.

Common drugs for MDS include:

- Azacitidine (Vidaza)
- Cytarabine (Cytosar-U)
- Daunorubicin (Cerubidine)
- Decitabine (Dacogen)
- Idarubicin (Idamycin)
- Lenalidomide (Revlimid)

People with high-risk subtypes of MDS who have an increased risk of developing AML may benefit from chemotherapy. Medications that are used to treat AML, such as cytarabine, idarubicin or daunorubicin, are also sometimes considered, particularly if the MDS has turned into AML. Overall, 30 to 40% of patients may benefit from chemotherapy for MDS. Factors such as the patient's age and medical condition are evaluated before starting chemotherapy.

Lenalidomide is taken by mouth, or an oral, medication that is very effective when given to patients with low-risk MDS and 5q chromosomal abnormalities (See [Subtypes and Classification](#) [7]). Therefore, it is very important for doctors to find out whether a patient has this chromosomal change.

Both decitabine and azacitidine are approved by the U.S. Food and Drug Administration, or FDA, to treat all types of MDS, although they are used most often for patients with higher IPSS-R scores. Both can be given in the doctor's office or clinic, and patients often need more than one round of treatment given monthly before it starts improving a patient's condition.

The side effects of chemotherapy depend on the individual and the dose used, but they can include fatigue, risk of infection, nausea and vomiting, hair loss, loss of appetite, and diarrhea. These side effects usually go away once treatment is finished.

Learn more about [chemotherapy](#) [9] and [preparing for treatment](#) [10]. The medications used to treat cancer are continually being evaluated. Talking with your doctor is often the best way to learn about the medications prescribed for you, their purpose, and their potential side effects or interactions with other medications. Learn more about your prescriptions by using [searchable drug databases](#) [11].

Immunotherapy

Immunotherapy, also called biologic therapy, is designed to boost the body's natural defenses to fight the cancer. It uses materials made either by the body or in a laboratory to improve, target, or restore immune system function. Although this is rarely used for MDS, it may be an option for some patients. One type of immunotherapy for MDS is anti-thymocyte globulin (ATGAM, Thymoglobulin), which is given after chemotherapy. Learn more about [immunotherapy](#) [12]

Stem cell transplantation/bone marrow transplantation

High-dose chemotherapy with stem cell/bone marrow transplantation is the only current treatment that can produce a long-term remission. However, transplantation is a higher-risk treatment and may not be recommended for patients who are older or have other medical problems. However, for patients ages 50 to 75, an ALLO transplant may be an option after reduced intensity treatment. Before recommending transplantation, your doctor will talk with you about the risks of this treatment and consider several other factors, such as the subtype of MDS, results of any previous treatment, and your age and general health.

A stem cell transplant is a medical procedure in which bone marrow that contains unhealthy cells is replaced by highly specialized cells, called hematopoietic stem cells, that develop into healthy bone marrow. Hematopoietic stem cells are blood-forming cells found both in the bloodstream and in the bone marrow. Today, this procedure is more commonly called a stem cell transplant, rather than bone marrow transplant, because it is the stem cells in the blood that are typically being transplanted, not the actual bone marrow tissue.

There are two types of stem cell transplantation depending on the source of the replacement blood stem cells: allogeneic (ALLO) and autologous (AUTO). ALLO uses donated stem cells, while AUTO uses the patient's own stem cells. In both types, the goal is to destroy all of the unhealthy cells in the marrow, blood, and other parts of the body using high doses of chemotherapy and/or radiation therapy and then allow replacement blood stem cells to create healthy bone marrow. Learn more about [stem cell and bone marrow transplantation](#) [13].

Getting care for symptoms and side effects

MDS and its treatment often cause side effects. In addition to treatment for MDS, an important part of care is relieving a person's symptoms and side effects. This approach is called palliative or supportive care, and it includes supporting the patient with his or her physical, emotional, and social needs.

Palliative care can help a person at any stage of illness. People often receive treatment for MDS and treatment to ease side effects at the same time. In fact, patients who receive both often have less severe symptoms, better quality of life, and report they are more satisfied with treatment.

Palliative treatments vary widely and often include medication, nutritional changes, relaxation techniques, and other therapies. You may also receive palliative treatments similar to those meant to treat the MDS, such as chemotherapy (see above). Talk with your doctor about the goals of each treatment in the treatment plan.

Some people with MDS who do not have any symptoms may only need close monitoring, including measuring blood counts, to watch for any symptoms. People with low-risk MDS, meaning those with RA and RARS subtypes, usually receive supportive care to control symptoms and improve quality of life. Supportive care may include:

- Transfusions to treat [anemia](#) [14] and [thrombocytopenia](#) [15]
- Use of [growth factors](#) [16] that help cells mature, such as epoetin (Epogen, Eprex, Procrit), a red blood cell growth factor, and filgrastim (Neupogen), a white blood cell growth factor
- Antibiotics to fight [infection](#) [17], a common side effect of low white blood cell counts

Before treatment begins, talk with your health care team about the possible side effects of your specific treatment plan and supportive care options. And during and after treatment, be sure to tell your doctor or another health care team member if you are experiencing a problem so it is addressed as quickly as possible. Learn more about [palliative care](#) [18].

If MDS worsens

If MDS continues to worsen despite treatment, patients are encouraged to talk with doctors who are experienced in treating this disease because there can be different opinions about the best treatment plan. Learn more about seeking a [second opinion](#) [19] before starting treatment, so you are comfortable with the treatment plan chosen. This discussion may include [clinical trials](#) [3] studying new treatments.

Your health care team may recommend a treatment plan that includes a combination of the treatments discussed above. Supportive care will also be important to help relieve symptoms and side effects.

For most patients, worsening MDS is very stressful and, at times, difficult to bear. Patients and their families are encouraged to talk about the way they are feeling with doctors, nurses, social workers, or other members of the health care team. It may also be helpful to talk with other patients, including through a support group.

Remission and the chance of recurrence

A remission is when MDS cannot be detected in the body and there are no symptoms. This may also be called "no evidence of disease" or NED.

A remission can be temporary or permanent. This uncertainty leads to many survivors feeling worried or anxious that the disease will come back. While many remissions are long-lasting, it is important to talk with your doctor about the possibility of the disease returning. Understanding the risk of recurrence and the treatment options may help you feel more prepared if the disease does return. Learn more about [coping with the fear of recurrence](#) [20].

If MDS does return after the original treatment, it is called recurrent MDS. When this occurs, a cycle of testing will begin again to learn as much as possible about the recurrence, including whether the subtype or classification has changed. After testing is done, you and your doctor will talk about your treatment options. Often the treatment plan will include the therapies described above, such as chemotherapy and stem cell transplantation, but they may be used in a different combination or given at a different pace. Your doctor may also suggest clinical trials that are studying new ways to treat this type of recurrent disease.

People with recurrent MDS often experience emotions such as disbelief or fear. Patients are encouraged to talk with their health care team about these feelings and ask about support services to help them cope. Learn more about [dealing with a recurrence](#) [21].

If treatment fails

Recovery from MDS is not always possible. If treatment is not successful, the disease may be called advanced or terminal disease.

This diagnosis is stressful, and this is difficult to discuss for many people. However, it is important to have open and honest conversations with your doctor and health care team to express your feelings, preferences, and concerns. The health care team is there to help, and many team members have special skills, experience, and knowledge to support patients and their families. Making sure a person is physically comfortable and free from pain is extremely important.

Patients who have advanced disease and who are expected to live less than six months may want to consider a type of palliative care called hospice care. Hospice care is designed to provide the best possible quality of life for people who are near the end of life. You and your family are encouraged to think about where you would be most comfortable: at home, in the hospital, or in a hospice environment. Nursing care and special equipment can make staying at home a workable alternative for many families. Learn more about [advanced care planning](#) [22].

After the death of a loved one, many people need support to help them cope with the loss. Learn more about [grief and loss](#) [23].

The next section helps explain clinical trials, which are research studies. Use the menu on the side of your screen to select About Clinical Trials, or you can select another section, to continue reading this guide.

Links:

[1] <http://www.cancer.net/cancer-types/myelodysplastic-syndromes-mds/treatment-options>

[2] <http://www.cancer.net/about-us>

[3] <http://www.cancer.net/node/19388>

- [4] <http://www.cancer.net/node/19391>
- [5] <http://www.cancer.net/node/25356>
- [6] <http://www.cancer.net/cancer-types/leukemia-acute-myeloid-aml>
- [7] <http://www.cancer.net/node/19386>
- [8] <http://www.cancer.net/node/24582>
- [9] <http://www.cancer.net/node/24723>
- [10] <http://www.cancer.net/node/24473>
- [11] <http://www.cancer.net/node/25369>
- [12] <http://www.cancer.net/node/24726>
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- [20] <http://www.cancer.net/node/25241>
- [21] <http://www.cancer.net/node/25042>
- [22] <http://www.cancer.net/node/25113>
- [23] <http://www.cancer.net/node/25111>