



Transplant and Myelodysplastic Syndromes (MDS)

Learning more about your disease and treatment options can help you make informed decisions about your health care. Be The Match® can help you understand how transplant may be used to treat MDS.

To get started, read on to learn about:

- How transplant can treat MDS
- If transplant can help your MDS
- If transplant is right for you
- Questions to ask your doctor
- Transplant outcomes for MDS
- Initial treatment of MDS
- Making treatment decisions

About Myelodysplastic Syndromes (MDS)

Myelodysplastic syndromes (MDS) are a group of diseases that affect the bone marrow and blood. In MDS, the blood-forming cells in the marrow slow down, or even stop, making the three types of blood cells. Most patients with MDS will develop anemia (low numbers of red blood cells) and may need blood transfusions. Some patients also have low numbers of white blood cells (which fight infections) and platelets (which help blood clot when you bruise or get a cut).

Some types of MDS are mild and easily managed, while other types are severe and life-threatening. Mild MDS can grow more severe over time. It can also develop into a fast-growing, severe leukemia called acute myelogenous leukemia (AML).

About 19,000 people are diagnosed with MDS in the United States each year.¹ Although MDS can affect people of any age, the majority are older than 60 years. Exposure to certain industrial chemicals or radiation can increase the risk of developing MDS. In some cases, MDS is caused by chemotherapy used to treat a different disease. This is called secondary MDS. In most cases, the cause of MDS is unknown.

There are many different types of MDS. Doctors look at cells in the blood and in the bone marrow to determine what type of MDS a patient has. Long-term survival and the risk that MDS might turn into AML are different for each type of MDS.

Key points:

- Some types of MDS are mild and easily managed; other types are severe and life-threatening
- MDS can sometimes turn into a fast-growing type of leukemia called AML
- There are many types of MDS with different treatment plans, so it is important to know what type you have

How transplant can treat MDS

A bone marrow or cord blood transplant begins with chemotherapy, with or without radiation, to destroy the diseased cells and marrow. The transplant replaces diseased blood-forming cells with healthy ones. The type of transplant used for MDS is an **allogeneic** transplant, which uses healthy blood-forming cells from a family member, unrelated donor, or umbilical cord blood unit.

At the start of the transplant process, a patient gets chemotherapy to prepare his or her body for the treatment. Then the replacement cells are infused into the patient's bloodstream. From there, the cells find their way into the bone marrow, where they start making healthy red blood cells, white blood cells, and platelets. The entire process, from the start of chemotherapy or radiation, until hospital discharge, can last weeks to months, followed by many months of recovery at home.

Allogeneic transplant is the only cure for MDS at this time. If transplant is an option for you, your doctor can talk with you about your risks and your chances of remaining disease-free with transplant.

Key point:

- Allogeneic transplant is the only cure for MDS at this time

Understanding if transplant would help your MDS

Whether a transplant is right for you depends on several things, including your age and overall health, what type of MDS you have, and the risk score for your MDS.

Your risk score is based on several aspects of your disease, including how many blasts (immature white blood cells) are in your marrow, whether you have low blood counts, and **cytogenetic testing**.

Cytogenetic testing is when a doctor looks at the **chromosomes** in the diseased cells. Chromosomes are thread-like strands of DNA that carry genetic information about your body. Certain changes in the chromosomes can predict whether you have a lower-risk or higher-risk disease. Your doctor can tell you what risk score your MDS has.

In the newest international scoring system, MDS risk scores are grouped into five categories: Very Low, Low, Intermediate, High and Very High. Patients in the low and very low risk groups have mild disease and may not receive any treatment at all ("watch and wait"). But, they will likely have frequent blood tests to see if the MDS is stable or becoming worse.

Patients in all other categories will likely receive one or more drugs that can help treat the MDS to improve blood counts. These drugs cannot cure MDS, but they help increase blood counts for some patients and improve a patient's quality of life. Sometimes, these drugs are used to keep patients stable until they are ready for an allogeneic transplant.

There are medical guidelines for when someone should be referred for a transplant consultation, whether or not you might need a transplant at that time. An allogeneic transplant is an option for MDS patients in the Intermediate, High or Very High risk

categories. Talking to a transplant doctor is especially recommended if your disease has any of the following features.²

- MDS caused by another treatment, such as chemotherapy for another cancer
- Low blood cell counts that do not improve with drugs
- Changes in the chromosomes of the cells in the marrow that predict a poor result
- Dependence on red blood cell or platelet transfusions

Key points:

- Ask your doctor about your specific risk score
- If you have an Intermediate, High, or Very High risk score, ask for a referral to a transplant doctor to find out if transplant is right for you

How a transplant doctor helps you decide if transplant is right for you

To find out if transplant is right for you, you will need a physical check-up by your transplant doctor. During the check-up, your lungs, heart, liver, kidneys, and nervous system will be checked. The transplant doctor will also review your health history, including how much chemotherapy or radiation therapy you've had in the past and any past or current infections.

You will also meet with other members of the health care team. A social worker or other professional will meet with you to talk about your concerns related to transplant (for example: emotional, financial, travel, lodging, work and/or school). The social worker can help you find resources to support you during your transplant journey. Most transplant centers (hospitals that do transplants) require you to have a dedicated caregiver to help you through the recovery process.

Key points:

- A transplant doctor will look at many things including your health history, disease status and the risks and

benefits of transplant before recommending a transplant

- A transplant social worker is available to help you and your family with emotional and practical support

Questions to ask your doctor

It is important to ask questions so you are comfortable with the treatments that your doctors recommend and so you can make decisions about your treatment. Questions you may want to ask your doctor include:

- What are my chances of living disease-free if I get a transplant? If I don't get a transplant?
- What are the risks of waiting or trying other treatments before a transplant?
- Do I have any risk factors that might affect my transplant outcomes?
- How much does my age influence my risk?
- What are the possible side effects of transplant? How can they be reduced?
- What can you tell me about my quality of life if I get a transplant? If I don't?
- How might my quality of life change over time, with or without transplant?

Key point:

- Don't be afraid to ask questions so you understand which treatments are right for you

Transplant outcomes for MDS

Outcomes data (information on how patients have done after their transplant) is used to estimate transplant outcomes. Outcomes data only show how other patients have done as a group. This information can't tell how you will do for sure. It can only give you an idea of how other patients have done with a similar disease and treatment. No two people are exactly the same, and you may respond differently to your transplant than someone else. Talk to your transplant doctor about how outcomes data may apply to your specific situation. Fortunately,

transplant outcomes have continued to improve over time.

Key point:

- Transplant outcomes overall are useful but only your transplant team can tell you what your chances are of doing well

Initial treatment of MDS

The best treatment for a person with MDS depends on the type of MDS, risk score, age, overall health, and his or her own preferences. In addition to transplant, other treatment options include:

- Supportive care, including blood transfusions and medications to control iron accumulation from red cell transfusions
- Medications to improve marrow function
- Chemotherapy

Supportive care

Patients with mild disease may receive supportive care. In this "watch and wait" situation, doctors will do frequent blood tests to check for signs that the MDS is becoming worse. Some patients may get platelet or red blood cell transfusions, or both. After many red blood cell transfusions, however, iron builds up in the body, which can cause organ damage. If this happens, patients need additional treatment to remove iron from the body. This is called **iron chelation therapy**.

Medications to improve marrow function

Some patients may also take medicines, such as G-CSF or erythropoietin, which stimulate the marrow to make more white cells and red blood cells, respectively.

There are other medicines, called **hypomethylating agents**, which are now being used to treat MDS. Doctors use these drugs to help MDS patients achieve a long-term remission of their disease and to extend their lives.

Sometimes, these medications are used to lessen symptoms and/or the need for

transfusions until a patient can have a transplant. Although these medicines can help many patients have a higher quality of life and a longer life, they cannot cure MDS.

Chemotherapy

Chemotherapy is a treatment that uses a group of medicines that destroy diseased cells or stop them from growing. A treatment option for some people with severe MDS is **induction chemotherapy**. Induction chemotherapy is very intense. The goal is to bring the disease into remission (no more signs of disease).

Induction chemotherapy may be an option for patients in the High or Very High risk categories who are in good health, but do not have a matched donor for an allogeneic transplant. It is also sometimes used to bring a patient with MDS into remission before he or she has a transplant.

Key points:

- Initial therapy for MDS includes supportive care, chemotherapy, and other drug therapies
- Drug therapies can lessen MDS symptoms and/or the need for transfusions, improve quality of life, and help patients live longer

Making treatment decisions

It is important to know your treatment options so you can make the best decision for yourself. Soon after your diagnosis, ask your doctor if a consultation with a transplant doctor is right for you. When you meet with a transplant doctor, there are two main decisions to make. The first decision is whether to have a transplant. A transplant

doctor can help you understand the risks and benefits of transplant for your specific situation.

If a transplant is the best treatment option, the second decision is when to have a transplant. Getting a transplant at the right time in the course of your disease may offer the best chance of a cure. The transplant doctor will work with you to decide what timing for the transplant is best for you.

Other resources to help you learn more

Be The Match has a variety of free resources to help you learn about transplant. Visit BeTheMatch.org/patient-learn and choose the resources that best meet your needs. Here are just a few that you might find helpful:

- Webcast: *An Introduction to Marrow and Cord Blood Transplant*
- Booklet: *An Introduction to Marrow and Cord Blood Transplant*
- Brochure: *Understanding Transplant Outcomes Data*

Be The Match has a team dedicated to providing information and support to you before, during, and after transplant. You can contact us to ask questions you may have about transplant, to request professional or peer support, or to receive free patient education materials.

Call: 1-888-999-6743

Email: patientinfo@nmdp.org

Web: BeTheMatch.org/patient-learn

Reference

1. Howlader N, Noone AM, Krapcho M, Garshell J, Neyman N, Altekruse SF, Kosary CL, Yu M, Ruhl J, Tatalovich Z, Cho H, Mariotto A, Lewis DR, Chen HS, Feuer EJ, Cronin KA (eds). SEER Cancer Statistics Review, 1975-2010, National Cancer Institute. Bethesda, MD, http://seer.cancer.gov/csr/1975_2010/, based on November 2012 SEER data submission, posted to the SEER web site, April 2013.

2. *Recommended Timing for Transplant Consultation*. Guidelines developed jointly by National Marrow Donor Program/Be The Match and the American Society for Blood and Marrow Transplantation (ASBMT). Available at: marrow.org/md-guidelines

Most recent medical review completed 7/2013

Be The Match® has a team dedicated to supporting patients, caregivers and families before, during and after transplant.

We offer you confidential one-on-one support, financial guidance and free educational resources—DVDs, booklets, online tools and more. Our goal is to get you what you need, when you need it.

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Every individual's medical situation, transplant experience, and recovery is unique. You should always consult with your own transplant team or family doctor regarding your situation. This information is not intended to replace, and should not replace, a physician's medical judgment or advice.

