

YOUR GUIDE TO

Understanding MDS

(Myelodysplastic Syndromes)



AA•MDS

INTERNATIONAL FOUNDATION

www.aamds.org

The Aplastic Anemia and MDS International Foundation is a nonprofit health organization dedicated to supporting patients, families and caregivers coping with:

- Aplastic anemia
- Myelodysplastic syndromes (MDS)
- Paroxysmal nocturnal hemoglobinuria (PNH)
- Related bone marrow failure diseases

This guide is based on the most up-to-date research and has been carefully reviewed by independent experts. It provides important information about MDS and treatment options. But remember, this guide isn't a replacement for the advice of your doctor. If you have MDS, you need to work closely with a medical specialist who knows how to evaluate the type of MDS you have and select the best possible treatment for you.

For more information about MDS or our Foundation, call (800) 747-2820 or go to www.aamds.org.

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JUST LEARNING ABOUT MDS?

If you or a loved one was just diagnosed, you may feel a little overwhelmed right now. MDS is a group of rare blood and bone marrow cancers, and many people have never heard of it before.

But there's good news – there are excellent treatments that can help people feel better and live longer.

Right now, you need to learn as much as you can about MDS, and this guide is the right place to start. It answers the basic questions you and your family are likely to have about the disease, the treatments, and what you can expect.

This guide will help you become an informed and effective member of your health care team.

The more you understand about your condition, the more in control you will feel and the more prepared you will be to work with your doctor and decide on your next steps.



WHAT IS MDS?

MDS (myelodysplastic syndromes) is the name for a group of different cancers that affect your blood and bone marrow. Bone marrow is inside some of your bones. It's where your body makes all of your blood cells.

MDS can stop your marrow from making enough healthy blood cells. That can cause symptoms and make you sick.

Types of MDS

MDS is a group of diseases, not just one disease. That's why MDS patients can have very different symptoms and need different treatments.

Doctors often describe 2 main categories of MDS. **Lower-risk MDS** tends to develop more slowly while **higher-risk MDS** develops faster.

MDS — especially higher-risk MDS — can progress into a type of cancer called AML (acute myeloid leukemia) that is hard to treat. While most people with MDS never get AML, 3 out of 10 people do



If you have MDS, you're not alone. In the United States:

- More than 15,000 people are diagnosed with MDS every year
- About 50,000 to 170,000 people are currently living with MDS

WHAT CAUSES MDS?

Most of the time, doctors don't know what causes MDS. Nine out of 10 people with MDS have de novo MDS, meaning there is no explanation for why it developed.

Other cases of MDS can have a clear cause. These types are called treatment-related MDS (t-MDS) or secondary MDS. People who have had radiation or chemotherapy for cancer or another problem can later develop t-MDS. People who are born with certain genetic bone marrow disorders or who had aplastic anemia (a different kind of bone marrow problem) in the past can develop secondary MDS. These types of MDS can be harder to treat.

Risk factors for MDS

We do know that some people are more likely to develop MDS than others. This includes:

- **Men** — MDS is more common in men than women MDS is more common in men than women
- **Age 60 or older** — MDS is not common in people under age 60
- **White** — MDS is a little less common among African-Americans, Native Americans, Asians, Inuits and Pacific Islanders than among people whose ancestors are from Europe

We also know a few things that can increase the risk of getting MDS:

- Having had regular contact with benzene or other harmful chemicals (organic solvents)
- Smoking, or having been a smoker
- Getting chemotherapy or radiation for other diseases
- Having another bone marrow failure disorder, such as aplastic anemia

Even though MDS can cause changes in your chromosomes (the part of a cell that carries genes), it can't be passed on to your children and it doesn't typically run in families. You also can't catch it from someone else.

HOW DOES MDS AFFECT BLOOD CELLS?

MDS stops your body from making healthy blood cells. Instead, your body makes cells that aren't normal. To better understand MDS, it helps to know a little bit about the different kinds of blood cells and how your body makes them.

Healthy blood cells

Your blood carries 3 different kinds of blood cells. Each one has an important role to play.

- **Red blood cells** carry oxygen from your lungs to all the cells in your body
- **White blood cells** protect you by attacking germs There are many different types of white cells to fight different germs
- **Platelets** help your blood clot and stop bleeding

Your bone marrow — a spongy tissue inside some of your bones — is like a factory that makes all of these blood cells. Normally, special cells inside your bone marrow (called blood-forming stem cells) grow into all the healthy red blood cells, white blood cells, and platelets your body needs.



People with MDS always have:

- A low level of at least 1 of the 3 types of blood cells — red blood cells, white blood cells, or platelets
- Cells that aren't normal (dysplastic) in their bone marrow or blood

MDS and damaged blood cells

If you have MDS, the stem cells in your bone marrow don't work normally.

They don't turn into healthy blood cells. Instead, the cells may not be a normal shape or size (dysplastic). They may also be immature (not full-grown) and stay inside the marrow instead of going out in the blood. These immature cells are called blasts.

People with MDS often have too many blasts or dysplastic cells in their bone marrow and don't have enough healthy blood cells in their blood. This can make them feel sick with a variety of symptoms. Learn more about these symptoms in the next chapter.



REVIEW

- MDS is the name for a group of cancers that affects blood cells and bone marrow
- In 9 out of 10 cases, we don't know why people get MDS
- MDS makes you sick because it stops your body from making healthy red blood cells, white blood cells, and platelets
- Instead, your body makes cells that aren't normal — including blasts, which are cells that are immature (not full-grown)

Know the Symptoms

The symptoms of MDS can be very different from person to person. Some people have very bad (severe) symptoms. Others have mild symptoms or no symptoms at all — especially if they have lower-risk MDS.

Remember that MDS causes low blood cell levels. So your symptoms depend on whether you are low on red blood cells, white blood cells, or platelets.

Keep in mind that all of these symptoms can be caused by other conditions too.

“I just joined a support group for people with MDS. I was surprised to find out that people can have such different symptoms.

Most people have a low red cell count like me, but other folks had a low white cell count or a low platelet count.”

—Samuel



LOW RED BLOOD CELL LEVELS (ANEMIA)

Red blood cells carry oxygen to your cells. Having a low level can make you feel tired and rundown. You may notice changes in how you feel, like:

- Getting more tired when you're active or having trouble staying active
- Not being as hungry as usual
- Having a harder time thinking clearly or concentrating

You may notice other symptoms like:

- Headaches
- Pale skin
- Trouble breathing, especially when you're physically active
- Fast heartbeat or chest pain
- Weight loss
- Swelling in your ankles and legs

LOW WHITE BLOOD CELL LEVELS (LEUKOPENIA OR NEUTROPENIA)

If you don't have enough white blood cells to fight germs, you may get sick more often and have more infections. It can also take you longer to feel better. Symptoms include:

- Mouth sores
- Stuffy nose and sinus infections
- Throat infections
- Skin infections
- Lung infections including pneumonia or bronchitis
- Bladder infections that may make it hurt to urinate or make you urinate more often

LOW PLATELET LEVELS (THROMBOCYTOPENIA)

Platelets help your blood clot so you stop bleeding. When you have low platelet levels you may:

- Bruise or bleed more easily, even from small scrapes and bumps
- Have bleeding gums, especially after brushing your teeth or seeing the dentist
- Get nose bleeds
- Bleed under the skin, causing tiny, flat, red spots — called petechiae — most often on your lower legs

If you have low platelet levels, tell your doctor or dentist before you get an operation, tooth cleaning, or any other procedures.



REVIEW

- Low red blood cell levels can make you feel tired and run down
- Low white blood cell levels raise your risk of getting infections
- Low platelet levels can make you bruise or bleed easily

Discover Your Diagnosis

Your doctor has a few different ways of figuring out if you have MDS, and if you do, what type it is.

They include taking a medical history, blood tests, and bone marrow tests. Getting the right diagnosis is key because it helps your doctor decide which treatments will work best.

If you have MDS, your doctor will want to do regular tests of your blood and may also need to test your bone marrow. These tests are an important way to see whether your MDS has gotten better or worse since your last check-up. It's also a good way to find out if treatment is working.

"I was feeling run down. At first my doctor thought I might have the flu. After a month of feeling too tired to get off the couch and lots more tests, my doctor diagnosed me with MDS."

—Lori



MEDICAL HISTORY

This is usually the first step in getting a diagnosis. Your doctor may ask questions about how you're feeling now like:

- Have you noticed any symptoms?
- Are you feeling tired?
- Have you had a fever or any infections recently?
- Have you had bruises or cuts that take longer to stop bleeding?

Your doctor may also ask questions to try to understand why you may have developed MDS, like:

- Have you ever had chemotherapy or radiation treatments for cancer?
- Have you ever had regular contact with harmful chemicals, like benzene?
- Do you smoke now or have you been a smoker?

BLOOD TESTS

Your doctor will want to test your blood to check for signs of MDS. You'll give a small sample of blood that will get sent to a lab. The lab will test the sample in different ways.

Complete blood count (CBC)

This test counts the number of blood cells in your sample. Blood counts are a way of finding out if you're low on red blood cells, white blood cells, or platelets.

When you get your CBC results back from your doctor, you may see a lot of different terms and numbers.



Check out **Appendix A** to learn more about how to read and understand your CBC results.

Other blood tests

Your doctor may also want to do other tests on your blood sample:

- Blood smear to check for cells that don't look normal under a microscope
- Erythropoietin (EPO) level, because a very high level can be a sign that one common treatment will be less likely to work
- Iron level, because a low level can cause low red blood cell levels
- Ferritin, because a high level can be a sign you have too much iron (which can happen if you get a lot of blood transfusions). Doctors call this iron overload
- Vitamin B12 and folate levels, because low levels of these vitamins can cause blood cells that aren't normal and low blood cell levels

BONE MARROW TESTS

You need to have bone marrow tests to confirm that you have MDS. Your doctor will take a sample of liquid bone marrow (aspirate) and a small piece of bone and solid marrow (core).

Bone marrow tests show:

- The type of cells your bone marrow is making and how many
- Levels of blast cells and other cells that aren't normal (see Appendix A)
- Signs of damage to your bone marrow (fibrosis, a kind of scar tissue)
- Signs of too much iron in certain types of cells that make red blood cells (ring sideroblasts)

Your doctor may run some other special tests on your bone marrow samples, like:

- Cytogenetics or karyotyping and FISH (fluorescence in situ hybridization), that check for problems in the chromosomes in your bone marrow
- Flow cytometry, that shows the types and amounts of different cells in your bone marrow, including blasts
- Genomic analysis, or sequencing of genes in the bone marrow cells, to check for changes (mutations) in your genes

What to expect from a bone marrow test

Your doctor will probably take bone marrow samples from the back of your hipbone. Here's what usually happens:

- You lie down on an exam table on your belly or on one side
- You may get some medicine to help you feel relaxed
- You'll get a shot of medicine in the skin (local anesthetic) to make the area numb
- Your doctor will put a needle through your skin into the bone to get samples of your bone marrow

The test can hurt, but usually just for a few moments. Afterward, you may be sore for a few days and bruised and notice a little bleeding. Your doctor may suggest you take acetaminophen (Tylenol®) for pain. Aspirin and non-steroidal anti-inflammatory drugs (NSAIDs) — like Advil® — may not be safe, because they can make bleeding worse.

Infections after a bone marrow test are not common, but they can happen. Ask your doctor for signs of infection you should watch for. They can include:

- Fever
- Redness or swelling at the site of the test

CLASSIFYING AND SCORING MDS

Doctors use all your blood and bone marrow test results to classify and score your MDS. This helps your doctor see how you're doing and decide what kinds of treatment you may need.

Classifying your MDS means figuring out what kind — or subtype — of MDS you have. This helps your doctor decide on the best treatment options. It also shows how your MDS might progress over time and if it's likely to become acute myeloid leukemia (AML).

Scoring your MDS helps show how serious (or severe) your MDS is. There are three main prognostic scoring systems for MDS:

- WHO Classification System
- IPSS-R - Revised International Prognostic Scoring System
- IPSS-M- International Prognostic Scoring System Molecular

IPSS-R looks at 3 things:

- Changes in your chromosomes (cytogenetics)
- Percentage of blasts in your bone marrow (a normal level is 2 to 3 percent)
- How many blood cell levels are low and how bad (severe) they are

IPSS-R - classifies MDS into 5 risk groups: very low risk, low risk, intermediate risk, high risk and very high risk. These 5 groups are divided into two basic risk categories: **LOWER RISK MDS** (very low, low and intermediate) and **HIGHER RISK MDS** (intermediate, high and very high) IPSS-R - classifies MDS into 5 risk groups: very low risk, low risk, intermediate risk, high risk and very high risk. These 5 groups are divided into two basic risk categories: LOWER RISK MDS (very low, low and intermediate) and HIGHER RISK MDS (intermediate, high and very high).

IPSS-M looks at 5 things:

- Blood counts
- Blast percentage
- IPSS-R risk categories
- Genetics
- Molecular sequencing results

IPSS-M classifies MDS into 6 groups: very low, low, moderate low, moderate high, high and very high. These 6 groups are divided into two risk categories - **LOWER RISK MDS** (very low, low or moderate low) and **HIGHER RISK MDS** (moderate high, high and very high)

The scoring systems do not predict how MDS will respond to treatment and only help predict how MDS will progress over time without treatment.



Understanding your classification and score can be a little confusing at first. Ask your doctor what your results mean. You can also learn more about how MDS is classified and scored in **Appendix B**.



REVIEW

- Doctors use blood tests and bone marrow tests to diagnose MDS
- They also use test results to figure out what type of MDS you have and how to treat it
- People who have MDS need regular testing, so their doctors know how they're doing and if treatment is working



Understand Your Treatment Options

MDS is a complex disease, and there are a lot of different types of treatment. It's normal to feel confused. But you and your doctor will work together to figure out the best treatment for you.

Most people with MDS need different treatments at different times. It depends on what kinds of symptoms they have and what their blood and bone marrow tests show.

The main goal of treatment is to help you live longer. Treatment can also:

- Make your symptoms better
- Improve your quality of life
- Boost your healthy blood cell levels
- Cut down on the number of transfusions you need
- Make it less likely you'll need to stay in the hospital

This chapter breaks down your treatment options into 6 different types:

- Watching and waiting
- Treating symptoms
- Medicines for MDS
- Immunosuppressive therapy
- Chemotherapy
- Stem cell transplants



You can also see a chart of all your treatment options in Appendix C.

“Until recently, there weren’t many ways to treat MDS. It’s really different now — there are lots of options to choose from. It’s good to know I have choices!”

—David



WATCHING AND WAITING

Watching and waiting means that you don’t get any treatment right now. Instead, your doctor will keep a close eye on your blood cell levels and symptoms. This approach is also called active surveillance.

If you have lower-risk MDS and don’t have many (or any) symptoms, your doctor may suggest watching and waiting. If your blood cell levels go down or you start to have symptoms, then your doctor may decide to start treatment.

TREATING SYMPTOMS

This approach is also called supportive care. It can be good if you have lower-risk MDS. You only treat the symptoms of MDS — and not the MDS itself — so you feel better. Supportive care is often still needed after you start other treatments.

Supportive care can help treat low red blood cell levels (anemia), prevent infections, and stop or prevent bleeding. Doctors use different types of supportive care, depending on your blood cell levels and symptoms.

Blood Transfusions

MDS causes low levels of blood cells. Adding healthy blood cells from a donor to your blood can help. This is called a transfusion. It can help get your blood cell levels back to healthier levels.

During a transfusion, your doctor will put donated blood cells into your body through a needle (IV). There are 2 different types of transfusions you may need.

- Red blood cell transfusions can help if you have low red blood cell levels (anemia). Some people only need these rarely, and others need them every 1 to 2 weeks.
- Platelet transfusions can help if you don't have enough platelets, but the effects don't last long. Also, the more often you get platelet transfusions, the less they may help. So your doctor may want to try other treatments to improve your platelet levels.



Getting donated blood from a family member could be a bad idea because:

- At some point, you may need a stem cell transplant from someone in your family
- If you get donated stem cells from a person who donated blood to you in the past, the transplant is less likely to work
- That's why doctors recommend using blood donated by someone outside of your family — or at least testing the family member first to make sure they are not a stem cell match.

Iron chelation therapy

People who get a lot of red blood cell transfusions can get too much iron in their blood. Over time, that extra iron can build up in their heart, liver and other organs and damage them. This is called iron overload.

Using special medicines to get some of the extra iron out of your body is called chelation. These medicines include:

- Deferasirox (Exjade® or Jadenu®), which is given as a pill
- Deferoxamine (Desferal®), which is given as a shot or through an IV
- Deferiprone (Ferroprox®), which is given as a pill. However, it's not often used with MDS and is more common outside the United States

Antibiotics

If you have low levels of one type of white blood cell — neutrophils — you may get infections easily and have trouble getting better. Antibiotic medicines can help prevent and fight these infections.

Red blood cell growth factors

If you don't have enough red blood cells, certain medicines can help your bone marrow grow more of them. They are called erythropoiesis stimulating agents (ESA) and are given as a shot. Types of ESA include epoetin (Procrit® and Epogen®) and darbepoetin (Aranesp®).

White blood cell growth factors

If you have an infection because of low white blood cell levels, these medicines may help your bone marrow make more. They can also help some people who are also getting red blood cell growth factor. However, they may not be safe if you have higher-risk MDS, because they can cause an increase in MDS blast cells in some people. Studies haven't shown that they help people live longer.

These medicines are given as a shot. There are 2 types:

- G-CSF (granulocyte colony-stimulating factor) sold as Filgrastim® and Neupogen® and Granix® (tbo-filgrastim) and others
- GM-CSF (granulocyte macrophage colony-stimulating factor) sold as Leukine® and Sargramostim®

MEDICINES FOR MDS

Hypomethylating Agents (HMA)

A hypomethylating agent is a medicine that affects 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. These medicines are sometimes called chemotherapy. But they're different from other types of chemotherapy that make you feel very sick and cause symptoms like losing your hair. These medicines are sometimes called chemotherapy. But they're different from other types of chemotherapy that make you feel very sick and cause symptoms like losing your hair.

Here's an overview of the 3 options.

Azacitidine (Vidaza®)

What is it? Azacitidine was the first medicine (hypomethylating agent) approved to treat MDS. It helps stop abnormal bone marrow cells from growing.

Studies show that in patients with higher-risk MDS, azacitidine can help improve red blood cell levels, lower the need for transfusions, help people live longer, and improve quality of life.

Who is it for? It's usually for people with higher-risk MDS, but can also help with lower-risk MDS — especially those who have low platelet and white blood cell levels or who have anemia that hasn't been helped by other treatments.

How is it given? You can get azacitidine as a shot under the skin or through an IV. Normally, people get it once a day for 5 to 7 days, followed by 23 or 21 days without it. Then, you'll repeat this cycle, typically for 6 months or more. Your doctor may keep you on this medicine as long as it helps and as long as you're feeling okay.

What are the side effects?

- Feeling sick to your stomach or throwing up
- Diarrhea or constipation
- Pain or redness where you get the shot (if you get it as a shot under the skin)
- Mouth sores
- Low blood counts (blood counts often get lower in the first few months before they get better later on)

Decitabine (Dacogen®)

What is it? Just like azacitidine, decitabine is a hypomethylating agent that helps stop abnormal stem cells from growing. Most doctors think that it works as well as azacitidine in people with higher-risk MDS.

Studies show decitabine can improve your blood cell levels and lower the number of blasts in your blood. However, we don't have clinical research evidence yet about whether it helps people live longer.

Who is it for? It's usually for people with higher-risk MDS, but it can help with lower-risk MDS too.

How is it given? Most doctors give decitabine as a shot once a day for 5 days in a row. The cycle is then repeated every 4 weeks. Sometimes, people get decitabine in the hospital through an IV. This cycle is then repeated for at least 4 months. Your doctor may keep you on this medicine as long as it helps and as long as you are feeling okay.

What are the side effects?

- Feeling sick to your stomach
- Feeling tired
- Diarrhea or constipation
- Mouth sores (like cold sores)
- Low blood counts (blood cell levels often get lower in the first few months of treatment before they get better later on)

Decitabine and Cedazuridine (Inqovi®)

What is it? Decitabine and Cedazuridine is a prescribed oral tablet combination approved for adult patients with myelodysplastic syndromes (MDS).

The approval was based on clinical trial results which showed similar drug concentrations between intravenous decitabine and INQOVI®. The complete response (CR) rate in the landmark phase 3 ASCERTAIN study was 21%. Additionally, about half of the patients who were formerly dependent on transfusions were able to no longer require transfusions during an 8-week period. The safety profile of INQOVI® was also similar to intravenous decitabine.

Who is for? MDS patients previously treated and untreated, de novo and secondary MDS with the following French - American British subtypes (refractory anemia, refractory anemia with ringed sideroblasts, refractory anemia with excess blasts, and CMML) and intermediate-1, intermediate-2, and high-risk International Prognostic Scoring System groups.

How is it given? Take one tablet by mouth once daily for five consecutive days of each 28-day cycle. The tablet contains 35 mg decitabine and 100 mg cedazuridine.

What are the side effects:

- Low blood counts (blood cell levels often get lower in the first few months of treatment before they get better later on)
- Feeling tired
- Diarrhea or constipation
- Mouth sores
- Muscle or joint pain
- Shortness of breath
- Changes in liver function tests
- Decreased appetite

Immunomodulator

An immunomodulating agent is a medicine that changes your immune system so it works more effectively. They include treatments that increase or decrease your immune response.

Lenalidomide (Revlimid®)

What is it? Lenalidomide helps MDS in different ways. It kills abnormal cells and helps healthy cells grow. In some studies, people using this drug stopped having MDS symptoms and stopped needing red blood transfusions. On average, these benefits lasted 2 years.

Who is it for? Lenalidomide is for people with lower-risk MDS who are getting transfusions and who have a specific type of change to their chromosomes from MDS. This kind of MDS is called deletion (5q) subtype or del5q.

How is it given? You take it as a pill, usually every day or once a day for 21 out of 28 days. It will probably take about 1 to 2 months before you know if it's working. Your doctor may keep you on it as long as it helps.

What are the side effects?

- Feeling itchy
- Skin rashes
- Feeling very tired
- Pain in your bones or muscles
- Diarrhea
- Low blood counts (blood cell levels often get worse before they get better)

Lenalidomide can also cause serious birth defects in babies — your doctor will talk to you about the risks.

IDH1 inhibitors

IDH inhibitors have been developed to treat MDS patients with IDH1 mutations. An IDH1 mutation is a genetic change in the IDH1 gene, which codes for an enzyme involved in cell metabolism. A small percentage of MDS patients have an IDH1 mutation, which can be detected through genetic testing.

Ivosidenib (Tibsovo®)

What is it? Ivosidenib is the first in a class of drugs known as IDH1 inhibitors.

Who is it for? It is a prescribed medication approved to treat adult patients with myelodysplastic syndromes (MDS) with an isocitrate dehydrogenase-1 (IDH1) mutation either when the disease has relapsed or has not improved after previous treatments.

How is it given? Ivosidenib is an oral medication you can take at home. Patients typically take 2 tablets once a day.

What are the side Effects:

- Changes in kidney function
- Changes in blood counts
- Decreased phosphorus and sodium levels in blood
- Joint, muscle, back and neck pain
- Tiredness
- Diarrhea
- Sores in mouth
- Decreased appetite
- Itchy skin or rash

Erythroid Maturation Agent (EMA)

An erythroid maturation agent (EMA) is a medicine that helps immature red blood cells (RBCs) develop into mature, working RBCs. EMAs can be used to treat anemia caused by MDS, and can help reduce or stop the need for RBC transfusions.

Luspatercept (Reblozyl®)

What is it? Luspatercept is the first erythroid maturation agent (EMA) approved for the treatment of anemia in adult MDS patients.

Who is it for? Patients who have been failed an erythropoiesis stimulating agent (ESA) or are unlikely to respond to an ESA and are requiring transfusions of 2 or more red blood cell (RBC) units over 8 weeks. It is also approved to treat very low to intermediate risk MDS patients who have not previously been treated with an erythropoiesis stimulating agent (ESA) but may require regular red blood cell (RBC) transfusions.

How is it given? It is given as an injection under your skin (subcutaneous) in the upper arm, thigh, or stomach (abdomen). Your healthcare provider will do regular blood tests to check your hemoglobin to monitor if your anemia is getting better before each injection.

What are the side effects:

- Blood clots
- High blood pressure
- Feeling Tired
- Joint or muscle pain
- Trouble breathing
- Diarrhea
- Stomach pain
- Dizziness
- Headache

Imetelstat (Rytelo®)

What is it? Imetelstat is a medicine used to treat anemia in adult MDS patients.

Who is it for? Patients with low to intermediate-1 myelodysplastic syndromes with transfusion dependent anemia requiring four or more red blood cell units over 8 weeks who have not responded/lost response to or are ineligible for erythropoiesis-stimulating agents (ESAs).

How is it given? It is given as an intravenous infusion into your vein over a period of 2 hours every 4 weeks. Your healthcare provider will do certain blood tests to check for side effects and to see how well you are responding to treatment. They may also change your dosage based upon how well you are responding to treatment.

What are the side effects:

- Low platelet count
- Low neutrophil count
- Increased liver enzymes
- Tiredness
- Joint, muscle and bone pain
- Headache
- Abnormal blood coagulation tests



Give your medicine time to work

MDS medicines may take months before they start working.

In the meantime, your blood cell levels may go down and you may feel worse. That's normal, unfortunately.

But don't give up and stop taking it! If the medicine is making you feel bad, talk to your doctor. Treating side effects can help

IMMUNOSUPPRESSIVE THERAPY

Your immune system helps keep you healthy by fighting germs. But in some people with MDS, the immune system doesn't work well. It starts to attack your own bone marrow, killing healthy cells.

Immunosuppressive therapy uses medicines that stop your immune system from attacking your bone marrow. These medicines include alemtuzumab (Campath®), anti-thymocyte globulin (ATG), cyclosporine, and tacrolimus.

You can take cyclosporine or tacrolimus as a pill or liquid at home. You need to get alemtuzumab or ATG by needle (IV) in a hospital — usually daily over a few hours for up to 10 days. This treatment may work best in younger people with lower-risk MDS. It may keep working for 2 years or longer.

Alemtuzumab and ATG often have mild side effects — like chills, fever, hives, and low blood pressure. These go away when you're done with treatment. Less often, ATG and alemtuzumab can cause:

- Allergic reactions, like hives, difficulty breathing, and swelling of your face, lips, tongue, or throat, which can be dangerous.
- Serum sickness, which can happen when your body has a strong reaction to proteins in ATG. It can cause fever, rash, joint pain, and muscle aches. Medicines like steroids can help prevent or treat serum sickness.

CHEMOTHERAPY

“Chemo” uses powerful chemicals to kill unhealthy blast cells in your bone marrow. This treatment is sometimes used for people with higher-risk MDS, especially if they’re considering a stem cell transplant and drugs like azacitidine or decitabine haven’t worked.

Types of chemotherapy for MDS

There are 2 basic types of chemotherapy.

Low-dose chemotherapy uses medicines that are less likely to cause side effects. Usually, you don’t need to stay overnight in a hospital to get low dose chemotherapy. Medicines include cytarabine (Ara-C®) and hydroxyurea (Hydrea®).

High-dose chemotherapy uses stronger medicines and higher doses. It’s more likely to cause bad side effects. You will most likely need to stay in the hospital for a month or more if you get this treatment. Medicines include Cytarabine (Ara-C®) used together with either daunorubicin (Cerubidine®), idarubicin (Idamycin®), hydroxyurea (Hydrea®), or mitoxantrone (Novantrone®).

Most people don’t get high-dose chemotherapy nowadays, because it only helps for a few months. However, it can be useful for people who are going to get a stem cell transplant.

How chemotherapy helps

After killing harmful blast cells, your normal cells should start to grow again within a few weeks. As healthy cells start growing, you may not need as many transfusions or antibiotics — or you may not need any at all.

Side effects and risks

The problem with chemotherapy is that it kills healthy cells as well as unhealthy ones. So during treatment, you may feel sicker and your blood cell levels may go down. You may need transfusions and antibiotics to prevent infections.

Some common side effects include:

- Feeling sick to your stomach or throwing up
- Mouth sores
- Diarrhea
- Losing your hair
- Heart or kidney damage (organ failure)
- Rashes
- Poor appetite
- Infection, which is the most common cause of death with this treatment



Tips for treatment side effects

For dizziness and headaches

What to do:

- Sit up in bed for a few minutes before getting out of bed
- Stand up slowly after you've been sitting down

For upset stomach

What to do:

- Eat dry foods, like toast, if you feel sick in the morning
- Eat more small meals instead of 3 big meals
- Drink cool, clear, unsweetened fruit juices, like apple and pear juice
- Avoid strong cooking smells by eating food cold or at room temperature

What not to do:

- Eat spicy, sweet, fried, or fatty foods
- Eat or drink milk, or other dairy products
- Drink alcohol or beverages with caffeine, such as colas, coffee, and black tea

STEM CELL TRANSPLANTATION

In this treatment, you replace all of your bone marrow stem cells damaged by MDS with healthy stem cells from a donor. This is the only MDS treatment that offers the possibility of a full cure. However, it has serious risks and it's usually only considered for people with higher-risk MDS.

Stem cell transplantation also works best in patients who:

- Are generally healthy and don't have other serious medical conditions
- Are younger than age 75

Finding a donor

To get a stem cell transplant, you need to find a donor who has cells that closely match your own. Usually, you would look for a donor in your family — most commonly a brother or sister. Your doctor will need to do special tests to make sure a donor is a full match. Age matters too — transplants with stem cells from younger donors (younger than age 55) have better results.

If you can't find a family member who is a match — or if your family members are older than age 55 — your doctor will look outside your family or an unrelated donor. To find an unrelated donor, your doctor will use a bone marrow donor registry managed by the National Marrow Donor Program. Learn more by visiting their website at www.bethematch.org.

If you can't find any donor, other possible options include:

- Cord blood transplantation, which uses stem cells taken (and frozen) from a baby's umbilical cord and placenta right after birth. Cord blood does not need to be fully matched for transplant.
- Half-matched donors. Usually, you would need a full-matched donor for a stem cell transplant. But some transplant centers are able to use half-matched donors. This makes it easier to find a possible donor. Your children or parents are usually half-matched. Doctors call this a haploidentical transplant.

What to expect

Stem cell transplantation is a serious medical procedure.

- You check into the hospital and get chemotherapy, immune suppressing therapy, and maybe radiation to destroy most of your bone marrow
- You will have to stay in the hospital to protect yourself from getting an infection during this time — this could be as short as a week to more than a month
- Your doctor will add healthy stem cells from your donor into your blood
- If it works, your healthy stem cells will move into your marrow and start copying themselves — this is called engraftment and it can take up to 1 month
- Your doctor will watch your blood cell levels to see if the new cells are working correctly

If everything goes well, the new, healthy cells will take over and start making all the red blood cells, white blood cells, and platelets you need. About 4 out of 10 people who get a stem cell transplant for MDS are potentially cured.

Side effects and risks

Stem cell transplants have serious risks and you need to talk them over with your doctor. You will have side effects from chemotherapy and radiation, like infections and low blood cell levels. In some cases, the body can start to attack the donated stem cells. This is called graft-versus-host disease (GVHD) and it can be life threatening.



REVIEW

- Watching and waiting (active surveillance) can be a good choice if you don't have many symptoms right now
- Treating symptoms (supportive care) helps you increase blood cell levels and prevent infections
- Medicines for MDS can attack the disease directly, helping healthy bone marrow cells grow and stopping abnormal cells
- Immunosuppressive therapy can improve blood cell levels by stopping your body from attacking healthy cells
- Chemotherapy uses powerful drugs to kill abnormal cells
- Stem cell transplantation replaces damaged stem cells in your bone marrow with healthy ones from a donor

Explore Clinical Trials

Clinical trials are another treatment option for some people with MDS.

Before people can use a new MDS treatment, it has to be tested. These tests are called research studies or clinical trials.

All of the current MDS treatments started in clinical trials. Clinical trials in hospitals and research centers are a key way for experts to learn:

- Whether a new treatment works and is safe
- How new treatments — or new combinations of treatments — compare with older ones

Think about joining a clinical trial if:

- Regular treatments have not made your MDS better
- Your MDS has come back after treatment
- You don't like your treatment options right now

Taking part in a clinical trial has benefits:

- You'll get excellent care from MDS experts
- You could get access to a new, cutting-edge treatment for MDS
- The results of the clinical trial could help other people who have MDS

But clinical trials also have risks:

- You don't know if the treatment you're getting will actually help
- You could have unexpected side effects or other problems

Clinical trials aren't right for everyone with MDS. Before you decide to take part in a clinical trial, think it over carefully. Talk it over with your family. Discuss the pros and cons with your doctor.

For more information about clinical trials:

- Visit www.aamds.org/clinicaltrials and www.clinicaltrials.gov
- Call (800) 747-2820 and ask to speak with our Information Specialist



REVIEW

- Clinical trials test out new treatments and approaches for MDS
- Taking part in a clinical trial has possible benefits and risks — so you need to talk them over with your family and doctor

Take Control of Your Care

Now that you've learned more about MDS, you're ready to take the next steps. Here are 6 things you can do to take charge of your care — and feel more confident about your future.

1. Find a doctor you trust

You and your MDS doctor need to be a team. You'll probably be working together for a long time.

So if you haven't already found an MDS expert, you need to. Two types of doctors specialize in treating MDS, and either one can be a good choice:

- Hematologists, who focus on treating problems with the blood and bone marrow
- Oncologists, who focus on treating cancer

To get the names of MDS doctors in your area, you can:

- Ask your regular doctor or insurance company for the names of experts in your area
- Call the nearest medical school with a teaching hospital
- Call the Aplastic Anemia and MDS International Foundation HelpLine at (800) 747-2820 or write to help@aamds.org

Use the “Find a Specialist” map on the AAMDS website, www.aamds.org, to locate a specialist.

When you first meet a doctor, imagine that you're interviewing him or her for a job. Make sure you're a good match and that the doctor makes you feel comfortable, really listens to you and answers all your questions.





Think about getting a second opinion. Seeing a second expert can help you understand all your choices and decide on a treatment plan. Some health plans actually require a second opinion.

2. Be prepared for doctor visits

It's easy to forget important questions when you're sitting in the doctor's office. So before every check-up, make sure that you:

- Take a list of questions you wrote down earlier. See page 59 at the end of this section for some ideas of what to ask.
- Take a notebook to write down what your doctor says, or ask a family member to take notes for you.
- If you don't understand something, ask your doctor to explain. Remember, the doctor is there to help you.

3. Keep track of your information

Keep all of your MDS information in one place — along with your lab results and medical records. You can store them all in a notebook, on your computer, or online using a phone or tablet app.

4. Work together on a treatment plan

Your treatment plan is like a roadmap. It shows what treatments you need now and what you may need in the future. Your doctor will make changes to your treatment plan over time.

The best treatment plan depends on a lot of different things like:

- Your symptoms
- Your age
- Your blood cell levels
- Your overall health
- Your MDS score and subtype (see Appendix B)



Take a good look at the treatment plan your doctor suggests. Make sure you understand it and that it seems like the right approach. Having a good treatment plan will make you feel more in control and more positive about the future.

5. Get support

Living with MDS and going through treatment can be hard. Sometimes you may feel tired, sick, or worried.

That's why it's so important to ask for help. See if family members or friends can take over some chores, like shopping or driving you to and from doctor's appointments. You may be surprised by how many people really want to help out if you ask.



Think about making an appointment with a counselor or therapist. Try to find someone with experience helping people dealing with health issues, like MDS.

You can also ask your doctor about support groups for people with MDS. Joining a support group can be a great way to share experiences and get tips on managing life with MDS. Meeting folks who truly understand what you're going through can make a big difference.

"If you have MDS, you can't go it alone! It took me a while to realize I needed to ask for help. Once I had a team of family and friends together, I felt a lot better about getting through treatment."

—Ken



6. Lower your stress levels

It's easy to get overwhelmed or feel down when living with a serious illness. You might stop doing things you used to love. You might isolate yourself from others. Medicines and other treatments can help your symptoms and blood cell levels. But they may not help how you feel emotionally.

That's why trying mind-body therapies — which focus on lowering stress and boosting your mood — is a great idea. These therapies can also help with pain and give you more energy. Look into treatments like:

- Relaxation techniques, like meditation, deep breathing exercises, and aromatherapy
- Gentle physical activity, like yoga, tai chi, and chi gong
- Massage therapy, acupuncture, reiki, or healing touch

Other simple things that can help are:

- Going on walks
- Sitting outside and enjoying nature
- Laughing more — by watching funny movies or reading funny books
- Writing in a journal — especially about what you feel grateful for
- Enjoying hobbies, like knitting, coloring, or gardening

Check with your doctor before trying a mind-body therapy — talk about which ones might be best for you. You can also find out if your hospital or medical center has integrative medicine specialists on staff. They're experts in using mind-body therapies to help people feel better.



Learn more about living with MDS.

The Aplastic Anemia and MDS International Foundation can help.

Contact us to:

- Get support from our Information Specialists
- Connect with other MDS patients through the Peer Support Network
- Join our virtual MDS support groups
- Find out more about our patient and family conferences at www.aamds.org/conferences
- Call us at (800) 747-2820 or visit www.aamds.org

Appendix A:

Understanding Key Lab Results



When you get your lab results back from the doctor, you may wonder what all those terms and numbers mean. This section will help you understand 2 types of results — your complete blood count and your blast count.

COMPLETE BLOOD COUNT (CBC)

Complete blood count is a way of checking your blood cell levels. It's one test, but it gives you many results and details about the health of your blood cells. Compare your CBC results with what you see below.

CBC Test Result	What is it for?	What's normal for adults?*
Red blood cell count (RBC)	Checks the number of red blood cells in your blood sample	Men: 4.4 to 5.8 million cells/mcL Women: 3.9 to 5.2 million cells/mcL
Hemoglobin (Hgb)	Checks the amount of a protein that carries oxygen in red blood cells — people with anemia have a low level	Men: 13.8 to 17.2 grams/dL Women: 12.0 to 15.6 grams/dL
Hematocrit (HCT)	Measures how much of a given volume of whole blood is made up of red blood cells — people with anemia have a low level	Men: 41 percent to 50 percent Women: 35 percent to 46 percent
Mean corpuscular volume (MCV)	Measures the average size of red blood cells — a high level means they're bigger than normal and a low level means that they're smaller than normal	76 to 100 cu μ m
White blood cell (WBC) count	Measures the number of white blood cells in your blood sample	4.5 to 10 thousand cells/mcL
Platelet (thrombocyte) count	Measures the number of platelets in your blood sample	150 to 450 thousand/mcL

**Varies in different clinics or hospitals and in different parts of the country.*

TESTING FOR BLASTS

Blasts can be a common sign of MDS. They’re blood cells that are immature — not fully grown. Doctors will check your bone marrow to find out how many of your blood cells (out of 100) are blasts.

Checking the number of blasts in your bone marrow will help your doctor keep track of how you’re doing and how well treatment is working. The more blasts you have, the more serious (severe) your disease is.

Number of blasts in 100 blood cells in bone marrow	What the results mean
Less than 5 percent	This level is normal. You probably have lower-risk MDS.
5 to 19 percent	You probably have higher-risk MDS.
20 to 100 percent	Your MDS has caused a second type of cancer. It’s called AML (acute myeloid leukemia). You will need treatment for this cancer as well.

Appendix B: MDS Classifications and Scoring Systems



Remember that MDS is not a single type of cancer. Instead, it's a group of related cancers. Doctors need to know exactly what kind of MDS you have so they can give you the right treatment.

Doctors look at all your test results and then use classification and scoring systems to figure out what kind (subtype) of MDS you have and how quickly or slowly it's progressing.

There are 2 systems your doctor may use:

- World Health Organization (WHO) Classification System, that shows which subtype of MDS you have
- Revised International Prognostic Scoring System (IPSS-R) that scores your MDS so you'll know how slowly or quickly your MDS is likely to progress
- International Prognostic Scoring System Molecular (IPSS-M) provides time estimates for survival and AML evolution using blast percentage, blood counts, IPSS-R scores, genetics and molecular sequencing.

"I finally found out yesterday exactly what type of MDS I have. The waiting and not knowing was hard.

Now, at least I know what to expect. And my doctor and I are working on a treatment plan that is right for me".

—Beth



WORLD HEALTH ORGANIZATION (WHO) CLASSIFICATION SYSTEM

The WHO system divides MDS into 8 subtypes based on blood and bone marrow tests. It was most recently updated in 2016. This chart reflects the most recent updates and terminology.

MDS Subtype	Blood testing results	Bone marrow testing results
MDS with single lineage dysplasia (MDS-SLD) Note: Considered the mildest type. MDS is limited to one cell line – red cells, white cells or platelets. There is little dysplasia.	Blood cell counts are low for one blood cell type and do not respond to treatment. You may still hear doctors use these terms: ■ RA (Refractory Anemia) for low red blood cell count ■ RN (Refractory Neutropenia for low white cell count ■ RT (Refractory Thrombocytopenia) for low platelet count	Less than 5 percent young blood cells (blasts) Red cell, white cell or platelet cell dysplasia (cells with an abnormal size, shape, or appearance)
MDS with ring sideroblasts (MDS-RS)		More than 15 percent red blood cells that contain ring-shaped iron deposits (ring sideroblasts) or a specific gene mutation called SF3B1 plus a few ring sideroblasts
MDS with multilineage dysplasia (MDS-MLD)	Low white blood cell count (neutropenia) or low platelet count (thrombocytopenia)	Dysplasia in more than one cell type. Less than 5 percent blasts, and less than 15 percent ringed sideroblasts Note: In patients with more than 15 percent ring sideroblasts, the subtype is called MDS-RS-MLD (MDS with multilineage dysplasia and ring sideroblasts)

MDS Subtype	Blood testing results	Bone marrow testing results
MDS with excess blasts, type 1 (MDS-EB-1)	The same as MDS-SLD.	5 percent to 9 percent blasts in marrow No blasts in blood
MDS with excess blasts, type 2 (MDS-EB-2)	The same as MDS-SLD.	Ten percent to 19 percent blasts in marrow May have blasts in blood
MDS with Isolated Del 5q (Del 5q)	The same as MDS-SLD plus normal or high platelet count	Deletion of the long arm of chromosome 5, called 5q, with no other chromosome abnormality Note: More common in women 65 and older who have mild to moderate anemia, low white blood cell counts, and normal to high platelet counts; life expectancy more than 5 years from time of diagnosis
MDS unclassified (MDS-U)	Low count for of either platelets or white blood cells	Unusual features, such as scarring (fibrosis) of the bone marrow Note: Just 1 to 2 percent of MDS patients have this subtype
Refractory cytopenia in childhood (RCC)	Often more than one low blood count.	Bone marrow is often empty and has low numbers of cells (hypocellular) This is rare.

REVISED INTERNATIONAL PROGNOSTIC SCORING SYSTEM (IPSS-R)

Your IPSS score helps your doctor answer 3 key questions:

- How serious is your case of MDS?
- How likely are you to progress to acute myeloid leukemia (AML)?
- How long are you likely to live?

Your score will also help your doctor decide on which medicines and treatment will work best for you.

The IPSS-R was developed in 2012. It’s different from the older original IPSS in important ways. You can learn more about the old IPSS on our website at www.aamds.org/diseases/mds/types.

Like the IPSS, the IPSS-R is based on 3 factors:

- Changes to chromosomes in your bone marrow cells. The IPSS-R added more categories for changes in chromosomes (cytogenetic abnormalities) than the IPSS (5 rather than 3)
- Number of low blood cell counts (cytopenias) you have and how low the counts are
- Percentage of blasts in your bone marrow. The IPSS-R includes more categories for percentage of blasts you have

These changes add more detail to a person’s score, so they may give a clearer picture of their health. Most doctors in the United States now use the IPSS-R.

IPSS-R Cytogenic Score		
Prognostic subgroups	Cytogenetic Abnormalities (Changes in chromosomes)	Median survival in years* (How long a person might live)
Very Good	-Y, del(11q)	5.4
Good	Normal, del(5q), del(12p), del(20q), double incl. del(5q)	4.8
Intermediate	del(7q), +8, +19, i(17q), any other single or double independent clones	2.7
Poor	-7, inv(3)/t(3q), del3q, double including -7/del(7q), Complex: 3 abnormalities	1.5
Very Poor	Complex: greater than 3 abnormalities	0.7

** These numbers are only estimates. There is no way to tell how long you will live. Survival rates are based on people who had MDS in the past. People may live longer now because we have better treatments and every case is unique.*

IPSS-R Prognostic Score Values							
Prognostic Variable	0	0.5	1	1.5	2	3	4
Cytogenetics	Very Good		Good		Intermediate	Poor	Very Poor
Bone Marrow Blasts (%)	≤ 2	—	>2 --<5		5-10	> 10	—
Hemoglobin	≥ 10	—	8--<10	< 8	—	—	—
Platelets	≥ 100	50-<100	<50	—	—	—	—
ANC	≥ 0.8	< 0.8	—	—	—	—	—

IPSS-R Prognostic Risk Categories/Scores and Estimated Survival		
Risk Category	Risk Score	Median survival in years* (How long a person might live)
Very Low	1.5 or lower	8.8
Low	More than 1.5 to 3	5.3
Intermediate	More than 3 to 4.5	3.0
High	More than 4.5 to 6	1.6
Very High	More than 6	0.8

** These numbers are only estimates. There is no way to tell how long you will live. Survival rates are based on people who had MDS in the past. People may live longer now because we have better treatments and every case is unique.*

International Prognostic Scoring System-Molecular (IPSS-M)

The International Prognostic Scoring System-Molecular (IPSS-M) is a clinical tool that uses genomic profiling to help predict the risk of myelodysplastic syndromes (MDS) and guide treatment decisions.

Like the IPSS-R, the IPSS-M considers blood counts, such as anemia and low platelet counts, the percentage of bone marrow blasts and the presence of certain chromosomal abnormalities. Unlike the IPSS-R, the IPSS-M also considers whether certain gene mutations are present and how many genes are mutated.

The IPSS-M classifies MDS into six risk groups:

- Very low (VL)
- Low (L)
- Moderate low (ML)
- Moderate high (MH)
- High (H)
- Very High (VH)

The IPSS-M's ability to consider gene mutations and how many are mutated can help to improve risk stratification and treatment decisions.

Appendix C:

MDS Treatment Overview Chart



There are lots of different treatments for MDS. This chart gives you an at-a-glance overview of the different types and how they can help.

Treatment Category	Type of Treatment	Medicine Name	Why It Can Help	Who It Can Help
Watch and Wait (Active Surveillance)	You and your doctor keep an eye on your MDS with regular lab tests		You don't start treatment until you really need it	People with lower-risk MDS who don't need blood transfusions
Treating Symptoms (Supportive Care)	Red blood cell and platelet transfusions		Improves blood cell levels	People with any type of MDS May be used along with other treatments
Treating Symptoms (Supportive Care)	Antibiotics	Medicine depends on the type of infection	Treats an infection or lowers your risk of getting one	People with any type of MDS May be used along with other treatments
Treating Symptoms (Supportive Care)	Red blood cell growth factors	Erythropoiesis stimulating agents (ESAs): Epoetin (Procrit®, Epogen®) Darbepoetin (Aranesp®)	Improves red blood cell levels Helps make more healthy red blood cells grow	Most useful for people with lower-risk MDS, depending on EPO levels
Treating Symptoms (Supportive Care)	White blood cell growth factors	Granulocyte Colony Stimulating Factor or G-CSF (Filgrastim®, Neupogen®, Granix®, or others) Granulocyte macrophage colony-stimulating factors or GM-CSF (Leukine®)	Helps make more healthy white blood cells	Most useful for people with lower-risk MDS with low white blood cell levels Can be risky in people with higher-risk MDS

Appendix C: MDS Treatment Overview Chart

Treatment Category	Type of Treatment	Medicine Name	Why It Can Help	Who It Can Help
Medicines for MDS	Immuno-modulatory agents	Lenalidomide (Revlimid®)	Helps slow down the growth of MDS cells	FDA approved to treat people with lower-risk MDS who need transfusions and have a change in chromosome 5 called deletion of 5q or del (5q)
Medicines for MDS	Hypomethylating agents	5-azacitidine (Vidaza®) Decitabine (Dacogen®) Decitabine and Cedazuridine (Inqovi®)	Prevents growth of abnormal bone marrow cells	FDA approved to treat all MDS subtypes Most effective in higher-risk MDS
Medicines for MDS	Erythroid Maturation agent (EMA)	Luspatercept (Reblozyl®) Imetelstat (Rytelo®)	Helps immature red blood cells (RBCs) develop into mature, working RBC	FDA approved to treat very low to intermediate risk MDS
Medicines for MDS	IDH1 inhibitors	Ivosidenib (Tibsovo®)	Helps patients achieve remission who have relapsed, or other treatments have not worked	FDA approved to treat adult MDS patients with an isocitrate dehydrogenase-1 (IDH1) mutation either when the disease has relapsed or has not improved after previous treatments.

Appendix C: MDS Treatment Overview Chart

Treatment Category	Type of Treatment	Medicine Name	Why It Can Help	Who It Can Help
Immuno-suppressive therapy	Immuno-suppressive agents	Anti-thymocyte globulin or ATG (Thymoglobulin®, Lymoglobulin®, Atgam®), Cyclosporine or tacrolimus	Stops the immune system from attacking healthy bone marrow cells	Younger people with lower-risk MDS
Chemotherapy	Low-dose chemotherapy	Cytarabine (Ara-C®) Hydroxyurea (Hydrea®)	Stops growth of harmful cells	Some people with higher-risk MDS
Chemotherapy	High-dose chemotherapy	Cytarabine (Ara-C®) Daunorubicin (Cerubidine®) Idarubicin (Idamycin®) Mitoxantrone (Novantrone®)	Kills harmful cells	May be used to treat some patients with higher-risk MDS
Stem cell transplants	Stem cells from a donor	Various chemotherapy drugs Possibly radiation	Chemotherapy destroys all bone marrow cells Transplantation replaces them with donor cells	Only potential cure for MDS May be used to treat patients with higher-risk MDS who have a transplant donor
Clinical trials	Research studies to find new treatments	Various medicines and other treatments	Offers a chance at new treatments before they become standard	MDS patients who qualify and may benefit from being in a research study To learn more, go to www.clinicaltrials.gov

Notes

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Questions to Ask Your MDS Expert

NEWLY DIAGNOSED PATIENTS (FIRST 30 DAYS)

- Is MDS a cancer?
- How long will it take to get a confirmation that I have this disease?
- Why type of MDS do I have? Is it Low Risk? High Risk? What is an IPSS-R and IPSS-M score?
- What is my prognosis? How long do I have to live?
- Am I at risk for developing acute myeloid leukemia (AML)?
- What caused my diagnosis?
- Is MDS hereditary? Do I need genetic testing?
- Is MDS curable?
- Will I need more tests? How often do I need to get a bone marrow biopsy?
- How will you monitor my disease?
- What impact will MDS have on my quality of life?
- Do I have any dietary restrictions? Will diet help my condition?
- What is your experience in treating MDS?
- How do I get a second opinion?
- Who should be part of my medical team – additional specialists?
- How do I reach you should I have any questions?
- Do you have written information about MDS and AML?
- How many MDS patients do you have?
- Could this be something other than MDS?

DIAGNOSIS AND TREATMENT DECISION PHASE (30-60 DAYS)

- Why are we not treating my disease? Is “watch and wait” a real treatment option?
- How quickly will I start treatment?
- What is the goal of my treatment?
- What are my MDS treatment options?
- How long can I be treated with blood transfusions? Side effects? What about Iron Overload?
- Do I need more tests before starting treatment?
- How long will I be treated?
- What are the risks and benefits of each treatment?
- Will I be hospitalized for treatment?
- How will treatment be administered?
- When should I expect to see a response from treatment?
- What are the side effects of each medication?
- When are side effects likely to happen?
- How will you treat any side effects?
- What are the long-term side effects of my treatment and disease?
- How will I be monitored during and after treatment?
- Why do you think the treatment you are recommending is the best option for me?

- Are there different treatment options for different kinds of MDS?
- How do mutations affect treatment decisions?
- What are my options if treatment fails?
- What happens if I choose not to treat my disease?
- Is the treatment you are suggesting covered under my health insurance plan?
- Are there clinical trials I should consider?
- Do you have written information about the treatments you are suggesting?

MDS: TREATMENT PHASE (90 DAYS+)

- What if the blood transfusions and ESA don't work? What's next?
- What are the possible side effects that you have seen in patients in your practice?
- What if my insurance won't cover the treatment?
- I've heard a lot about joining a clinical trial for MDS. Can you help me understand what might be right for me?
- My caregivers are really struggling to help me and take care of our family. What resources are out there to help them?
- What are the signs and symptoms my disease is relapsing?
- How would we treat relapsed disease?
- How often will I need follow up exams and tests?
- What happens if I develop AML? Prognosis? Treatment Options?
- Is it safe to receive the COVID, flu, shingles and pneumonia vaccine?
- Can I resume normal activities such as exercise?
- Am I always considered immunocompromised without a transplant?
- Should I be monitored lifelong for other diseases such as acute myeloid leukemia?
- What are the longer-term effects that I should know about with MDS?
- Are there any supplements that I should take to help my immune system?
- What are the signs and symptoms that my disease is relapsing? How will we treat relapsed disease?
- How often will I need follow up exams and tests?
- When can I resume normal activities such as exercise and work?
- Should I be monitored throughout my life for other bone marrow failure diseases like aplastic anemia and/or PNH?

How AAMDSIF Can Help You ...

Many FREE services and programs are available to anyone impacted by, or just interested in, bone marrow failure diseases:

- **Personalized Support** from Information Specialists at (800) 747-2820 or help@aamds.org
- **Educational Materials** on diseases and treatments
- **Global Educational Materials**
- **Webinars** providing live and recorded education
- **Patient and Family Conferences** connecting patients with experts and building community with each other
- **Podcasts** interviews with patients and healthcare providers
- **Clinical Trials Information**
- **Peer Support Network** staffed by specially-trained volunteers who listen and offer guidance
- **Support Groups** bring together patients and caregivers virtually for support

AAMDSIF relies on its growing team of volunteers who contribute their time and talent in many ways, such as becoming:

- **Online Supporters** who hold digital fundraisers in their community or workplaces
- **Event Organizers** who plan "March for Marrow" fundraising walks or other events
- **Awareness Campaigners** who teach their community about bone marrow failure
- **Support Group** leaders who coordinate patient support groups

Learn more about
volunteering at
development@aamds.org
or (301) 279-7202 x 105.



www.aamds.org



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