



X-Plain *Myelodysplastic Syndromes or* *MDS – Overview*

Reference Summary

Introduction

MDS is a group of disorders where the bone marrow does not work well and the bone marrow cells fail to make enough healthy blood cells.

About ten to fifteen thousand persons are diagnosed with MDS in the USA every year.

MDS stands for myelodysplastic syndromes. “Myeloid” stands for “blood cells” and dysplastic means “funny looking” or misshapen.

This reference summary explains MDS. It first discusses its symptoms and causes and how it is diagnosed and treated. It also includes tips for coping with MDS.

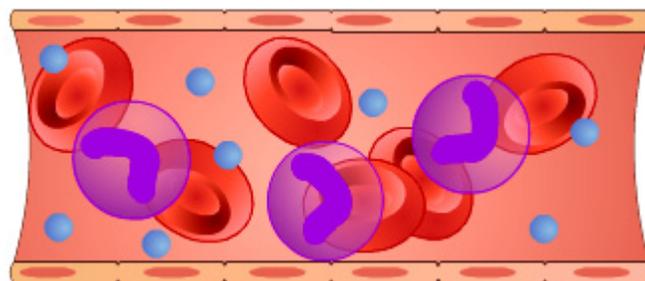
MDS (Myelodysplastic Syndromes)

MDS is a group of disorders where your bone marrow does not work well, and the bone marrow cells fail to make enough healthy blood cells. People with MDS may not have the right amount of red blood cells, white blood cells, and platelets.

In patients with this disease, many bone marrow cells do not develop into working blood cells. Instead, many of these cells die off in the bone marrow. This is why blood counts tend to be low in patients with MDS.

MDS is a disease of the blood. To understand MDS, it is important to first understand how blood cells are made.

Blood consists of blood cells floating in plasma. Plasma is mostly made of water with chemicals in it. These chemicals include proteins, hormones, minerals, and vitamins.



Blood

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There are three basic types of blood cells: Platelets, red blood cells, and white blood cells.

Platelets are also called thrombocytes. They are small pieces of cell that help blood clot and stop bleeding.

Red blood cells are also called erythrocytes. They make up almost half of blood. Red blood cells are filled with hemoglobin. That's a protein that picks up oxygen in the lungs and delivers it to cells all around the body.

White blood cells are also called leukocytes. They fight disease and infection by attacking and killing germs that get into the body. There are several kinds of white blood cells. Each kind fights different kinds of germs.

Blood cells are made in the bone marrow. That's a spongy tissue located inside some bones. It contains young parent cells called stem cells.

Stem cells can grow into all 3 types of blood cells. They make copies of themselves all the time. These cloned stem cells eventually become mature blood cells.

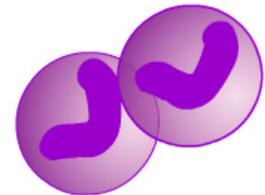
When blood cells are fully formed and functional, they leave the bone marrow and enter the blood. Healthy people have enough stem cells to keep making all the blood cells the body needs every day.



Platelets

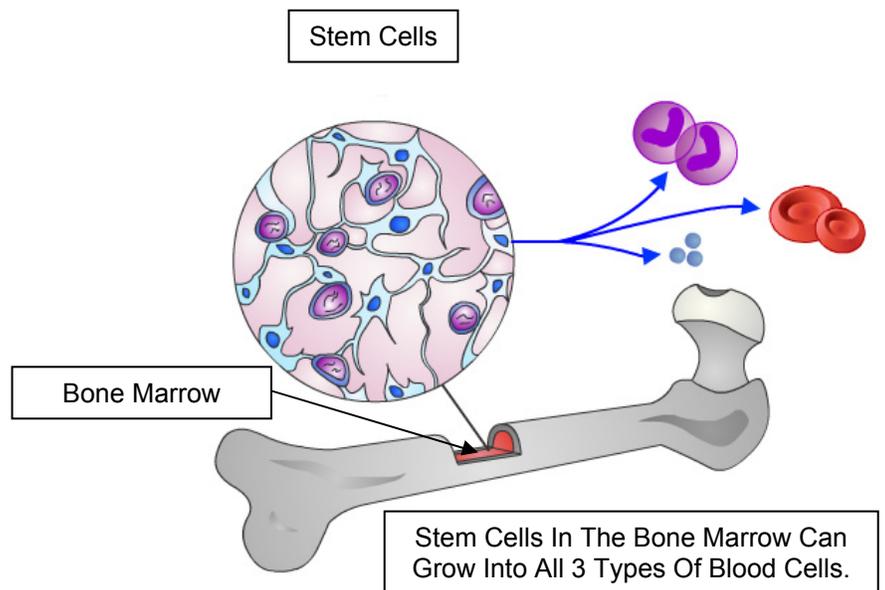


Red Blood Cells



White Blood Cells

Stem Cells

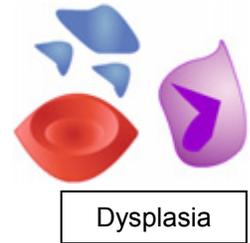


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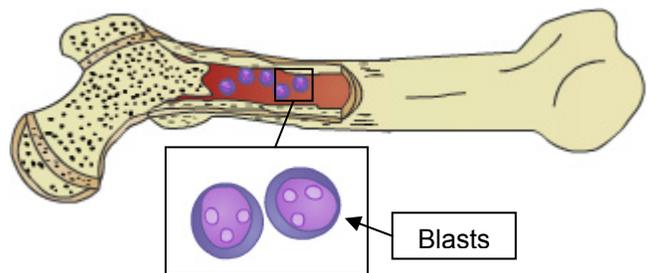
People with MDS have an abnormal stem cell in their bone marrow. All the cells that come from this parent cell have the same defect. Many experts believe that MDS should be considered a chronic or low-grade form of leukemia, a cancer of the blood and bone marrow.

Defects in stem cells can cause many problems:

- Blood cells may have an abnormal shape, size, or look that is not normal. This is called dysplasia.
- Blood cells may not mature fully or live as long as they should. This may result in too many young cells in the bone marrow and blood. It may also lead to not enough mature cells in the blood.



People with MDS may have too many blasts in their bone marrow. Blasts are the youngest or most immature white blood cells. In normal bone marrow, no more than 5 out of 100 white cells are blasts.



The number of blasts in the blood and bone marrow is some of the information used to tell how severe the MDS is.

Types of MDS

The symptoms and the course of MDS may vary greatly from person to person. These differences depend on which blood cells are affected.

All people with MDS have 2 things in common:

- They have a low blood cell count for at least 1 blood cell type.
- Their bone marrow and blood contain blood cells with an abnormal shape, size, or look.

Doctors use two systems to classify the types of MDS. These systems give you and your doctor important information about your specific case.



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The 2 classification systems are:

1. French-American-British (FAB) Classification System
2. World Health Organization (WHO) Classification System

Doctors have also created a scoring system to help them understand how a patient's disease may progress over time.

International Prognostic Scoring System

In 1997, the International Prognostic Scoring System or IPSS was launched. This system turns patient blood test and bone marrow biopsy data into a score. The score tells how quickly an MDS case is likely to progress and helps predict what may happen with the patient's MDS in the future.

Together, the WHO and FAB classification systems and the IPSS are important tools. Together they give you and your doctor key information about your specific case.

They tell:

- How severe your case is
- What types of medication or treatment might work best for you
- How your disease is likely to progress over time
- How long you are likely to live

See the program titled "Understanding the Different Types of MDS" for further information about these classification systems and the IPSS.

Symptoms & Complications

The symptoms of MDS may vary greatly from person to person. The symptoms depend on what blood cells are affected and how low the blood counts have fallen. People who have early-stage MDS may not have any symptoms. That's because their blood cell counts are still close to normal.

A person with MDS can have any of the following:

- A low red blood cell count is called anemia. Red blood cells carry oxygen from your lungs to the rest of your body.
- A low white blood cell count is called leucopenia. White blood cells fight infections in the body. The type of white cells which are most important for attacking and killing bacteria are called neutrophils. A low neutrophil count is called neutropenia.

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- A low platelet count is called thrombocytopenia. Platelets help blood to clot and stop bleeding.

If your MDS is in its early stages and your blood counts are not too low yet, you may have only mild symptoms. If your blood counts are very low, you will have more symptoms.

If you have a low red blood cell count, your symptoms may include:

- Tiredness
- Feeling less alert or having trouble concentrating
- Pale skin
- Trouble breathing
- Rapid heartbeat and chest pain

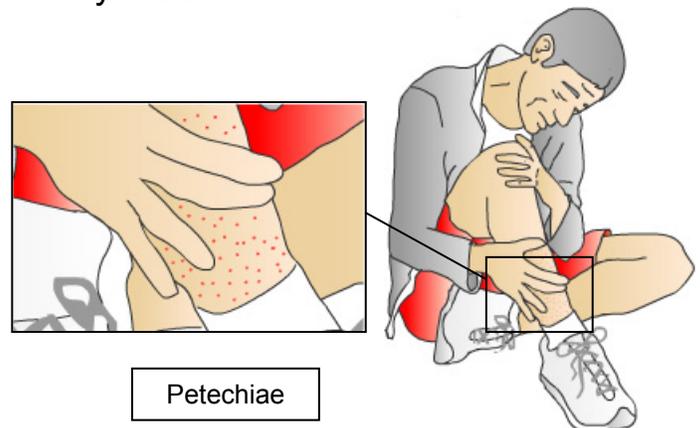


If you have a low white blood cell count, you are more likely to develop infections. These may include:

- Bladder infections that may make it painful to urinate or make you urinate more often
- Lung infections that may make breathing hard
- Mouth sores
- Sinus infections and a stuffy nose
- Skin infections

If you have a low platelet count, your symptoms may include:

- Bruising or bleeding more easily, even from minor scrapes and bumps.
- Nose bleeds.
- Tiny, flat red spots under your skin, which are caused by bleeding. These spots are called petechiae. These occur more often in the lower legs.
- Bleeding gums, especially after dental work or from brushing your teeth. Be sure to check with your doctor before getting any dental work.



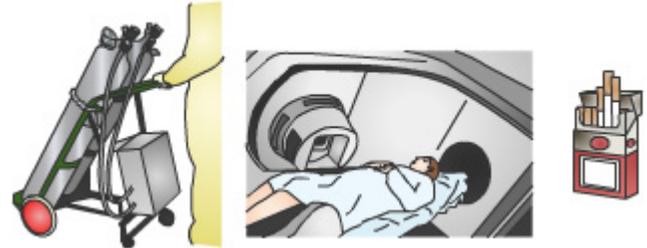
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Causes

Most of the time, we don't know the exact cause of MDS. We do know that certain lifestyle factors are linked to MDS. MDS cannot be passed down through the genes from parent to child. It cannot be passed through germs from person to person.

You may be more likely to develop MDS if you:

- Have been heavily exposed to certain chemicals, such as benzene.
- Have had chemotherapy or radiation treatments.
- Have been a smoker.



We know that people diagnosed with MDS are:

- More often men than women.
- Most often age 60 or older. The average age at diagnosis is 71.

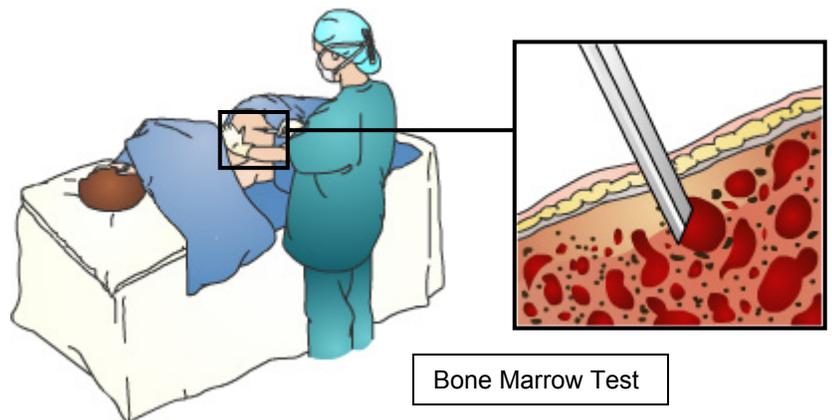
Diagnosis

MDS is a complex disease. So diagnosing it is a complex process. Doctors use a number of tests to figure out exactly what type of MDS you have. To understand what is causing your symptoms and low blood counts, your doctor will take a detailed medical history. Your doctor may ask you questions about your symptoms, past medical treatments and exposure to harmful chemicals.

When trying to figure out the cause of your symptoms, your doctor will ask you for blood samples and a sample of your bone marrow. These samples will be used in a number of tests.

The bone marrow tests show:

1. Exactly what types and amounts of cells your marrow is making.
2. Levels of bone marrow blasts, the immature white blood cells. If more than 5 out of every 100 white blood cells in your bone marrow are blast cells, this is not normal.
3. Damaged chromosomes in the cells of your bone marrow.



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Sometimes it is good to get a second opinion. If you are seeking a second opinion you should find a physician with expertise in treating MDS. One way to find an expert is by contacting a hospital affiliated with a medical university. Physicians at teaching hospitals are generally more familiar with rare diseases like MDS. Ask for the hematology or oncology department and for a consultation with an expert in MDS.

Treatment

The main goal of MDS treatment is to help patients live longer. One key to achieving this goal is increasing the number of healthy blood cells in your blood. This is known as your blood count.

When your blood counts go up:

- You are less likely to need a blood transfusion.
- Your quality of life gets better.
- Your symptoms are not as bad.



Your doctor will look at several issues in finding the best treatment plan for you. These include:

- Your symptoms
- Your age
- Which type of MDS you have
- Your disease risk score using the International Prognostic Scoring System
- Other serious conditions or diseases you have
- Whether someone -preferably a family member- is willing and able to donate matching bone marrow to you

Treatments for MDS have a variety of goals. They may:

- Help healthy cells mature
- Increase the number of healthy cells in your blood
- Kill abnormal bone marrow cells
- Reduce the number of abnormal blasts in your bone marrow

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The treatment options used for MDS include the following.

1. Wait and watch is an approach your doctor might suggest if your blood counts aren't too low and your symptoms aren't too bad.
2. Supportive care helps you manage the symptoms of your MDS. Its goals are to increase blood counts, treat infections, and treat iron overload. Getting many red blood cell transfusions can cause you to have too much iron in your body. This can lead to a condition called iron overload. Iron overload can hurt your heart and other organs.



Supportive care includes red cell and platelet transfusions, growth factors that can increase the number of red cells, white cells and platelets your body produces, antibiotics to help prevent or treat infection and iron chelation therapy to treat iron overload.

3. Drugs which lower the body's immune response (Immunosuppressive therapy) can improve blood counts in some patients.
4. Medicines recently approved to treat MDS can be used to stop abnormal cells from growing and stimulate the growth of healthy bone marrow cells. Medicines currently approved by the FDA to treat MDS include azacitidine (Vidaza ®), decitabine (Dacogen ®) and lenalidomide (Revlimid ®).
5. Intensive Chemotherapy treats MDS by using drugs that kill abnormal cells.
6. Stem cell transplantation which replaces damaged stem cells in your bone marrow with healthy stem cells provided by a donor. Stem cell transplantation is currently the only cure for MDS.

See the program titled "Treatment of MDS" for further information about treating the different types of MDS.

Conclusion

MDS is a group of disorders where your bone marrow does not work well, and the bone marrow cells fail to make enough healthy blood cells.

People with MDS have abnormal stem cells that turn into abnormal blood cells. They may have too many blasts in their bone marrow. The symptoms of MDS are caused by low blood counts.

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A person with MDS may have:

- A low red cell count that can cause you to feel tiredness and have low energy
- A low white cell count that can cause trouble fighting infections
- A low platelet count that can cause you to bruise or bleed more easily

In order to diagnose exactly what type of MDS you have, your doctor may take samples of your blood and bone marrow for tests.

Thanks to advances in medicine, many treatment options are available to treat MDS. They include:

- Supportive care, such as red blood cell and platelet transfusions, antibiotics, growth factors, and iron chelators
- Medication approved by the FDA to treat MDS such as azacitidine, decitabine and lenalidomide
- Immunosuppressive therapy
- Chemotherapy
- Stem Cell Transplantation

In addition to the choosing and sticking to your medical treatments, you can play a key role in managing your MDS. By eating healthy, exercising, preventing infections, and relaxing you allow yourself to be as healthy as possible in order to fight and treat this blood disease.

The good news is that scientists are making great strides in treating MDS and its symptoms. Learning about MDS and joining support groups will empower you to cope with this disease. Your doctor and healthcare providers can give you additional information. You can also check the resources available at the website of the Aplastic Anemia & MDS International Foundation, www.AAMDS.org.



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