About Myelodysplastic Syndromes

Overview and Types

If you have been diagnosed with a myelodysplastic syndrome or are worried about it, you likely have a lot of questions. Learning some basics is a good place to start.

- What Are Myelodysplastic Syndromes?
- Types of Myelodysplastic Syndromes

Research and Statistics

See the latest estimates for new cases of myelodysplastic syndromes in the US and what research is currently being done.

- What Are the Key Statistics About Myelodysplastic Syndromes?
- What's New in Myelodysplastic Syndrome Research and Treatment?

What Are Myelodysplastic Syndromes?

Myelodysplastic syndromes (MDS) are conditions that can occur when the blood-forming cells in the bone marrow are damaged. This damage leads to low numbers of one or more type of blood cells. MDS is considered a type of cancer.

Normal bone marrow

Bone marrow is found inside certain bones, including the skull, ribs, pelvis, and spine. It is made up of blood-forming cells, fat cells, and supporting tissues that help the blood-forming cells grow. A small fraction of the blood-forming cells are a special type of cell known as blood stem cells. Stem cells are needed to make new cells. When a stem cell divides it makes 2 cells: one cell that stays a stem cell, and another cell that can keep
changing and dividing to make blood cells. There are 3 types of blood cells: red blood cells, white blood cells, and platelets.

**Red blood cells** pick up oxygen in the lungs and carry it to the rest of the body. These cells also bring carbon dioxide back to the lungs. Having too few red blood cells is called anemia. It can make people feel tired and weak and look pale. Severe anemia can cause shortness of breath.

**White blood cells (also known as leukocytes)** are important in defending the body against infection. The 2 major types of white blood cells are lymphocytes and granulocytes.

Lymphocytes are immune cells that are found in the bone marrow, the blood, and in lymph nodes. They make the antibodies that help the body fight germs. They can also directly kill invading germs by producing toxic substances that damage the cells. Lymphocytes are not usually abnormal in MDS.

Granulocytes are white blood cells that destroy bacteria. They are called granulocytes because they have granules that can be seen under the microscope. These granules are made up of enzymes and other substances that can destroy germs that cause infections. In the bone marrow, granulocytes develop from young cells called myeloblasts. The most common type of granulocyte is the neutrophil; this cell is crucial in fighting bacteria. Other types of granulocytes are basophils and eosinophils. When the number of neutrophils in the blood is low, the condition is called neutropenia. This can lead to severe infections.

Monocytes, which are related to the granulocyte family, are also important in protecting the body against bacteria. The cells in the bone marrow that turn into monocytes are called monoblasts. Monocytes can leave the bloodstream to become macrophages in some of the body’s organs. Macrophages can destroy germs by surrounding and digesting them. They are also important in helping lymphocytes recognize germs and begin producing antibodies to fight them.

**Platelets** are thought of as a type of blood cell, but they are actually small pieces of a cell. They start as a large cell in the bone marrow cell called the megakaryocyte. Pieces of this cell break off and enter the bloodstream as platelets. You need platelets for your blood to clot. They plug up damaged areas of blood vessels caused by cuts or bruises. A shortage of platelets, called thrombocytopenia, can result in abnormal bleeding or bruising.

**Myelodysplastic syndromes**
In MDS, some of the cells in the bone marrow are damaged and have problems making new blood cells. Many of the blood cells formed by the damaged bone marrow cells are defective. Defective cells often die earlier than normal cells and the body also destroys some abnormal blood cells, leaving the patient with low blood counts because there aren’t enough normal blood cells.

In about one-third of patients, MDS can progress to a rapidly growing cancer of bone marrow cells called acute myeloid leukemia. Because most patients do not get leukemia, MDS was previously classified as a disease of low malignant potential. Now that doctors have learned more about MDS, it is considered to be a form of cancer.

In the past, MDS was referred to as pre-leukemia and smoldering leukemia. Since most MDS patients do not get leukemia, these terms are not accurate and are no longer used.

See Acute Myeloid (Myelogenous) Leukemia for more information about the leukemia that develops in some MDS patients.

References
See all references for Myelodysplastic Syndromes

Last Medical Review: February 10, 2014 Last Revised: July 2, 2015

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Types of Myelodysplastic Syndromes

Myelodysplastic syndromes (MDS) were originally classified more than 20 years ago at an international conference attended mostly by doctors from France, the United States, and Great Britain. This system was known as the French-American-British (FAB) classification.

The system used today is the World Health Organization (WHO) classification. This system seems to be more helpful than the FAB classification in predicting prognosis (outlook). The WHO system recognizes 7 types of MDS:

- Refractory cytopenia with unilineage dysplasia (RCUD)
- Refractory anemia with ringed sideroblasts (RARS)
- Refractory cytopenia with multilineage dysplasia (RCMD)
- Refractory anemia with excess blasts-1 (RAEB-1)
- Refractory anemia with excess blasts-2 (RAEB-2)
- Myelodysplastic syndrome, unclassified (MDS-U)
- Myelodysplastic syndrome associated with isolated del(5q)

Most of these types are determined by the the blood and the bone marrow look under the microscope. One type is defined by a certain chromosome change in the bone marrow cells. Because small differences in the way the cells look can change the diagnosis, doctors may sometimes disagree on a patient’s exact type of MDS.

Chronic myelomonocytic leukemia (CMML) was considered a type of MDS in the FAB classification, but the WHO classification includes it in another group of diseases. See Chronic Myelomonocytic Leukemia (CMML) for more information.

**Refractory cytopenia with unilineage dysplasia (RCUD)**

About 5% to 10% of all MDS patients have RCUD.

People with RCUD have low numbers of one type of blood cell, but normal numbers of the other 2 types. Examples of RCUD include refractory anemia (RA), refractory neutropenia (RN), and refractory thrombocytopenia (RT). Refractory anemia (RA) is the most common type of RCUD. People with RA have low numbers of red blood cells (anemia) but have normal numbers of white blood cells and platelets. In the bone marrow of RA patients, only the cells that grow to become red blood cells look abnormal. In the bone marrow of RCUD patients, at least 10% of the early cells of the affected cell type look abnormal (show dysplasia), but the other types of cells in the bone marrow look normal. There is a normal number (less than 5%) of very early cells called blasts in the bone marrow and blasts are rare (or absent) in the blood.

This type of MDS seldom, if ever, progresses to acute myeloid leukemia. Patients with this type of MDS can live a long time.

**Refractory anemia with ringed sideroblasts (RARS)**

About 10% to 15% of all people with MDS have this type.

This condition is similar to refractory anemia- the patient has low numbers of red blood cells but normal numbers of white blood cells and platelets. In the bone marrow, at least 10% of the early cells look abnormal. In RARS, though, 15% or more of the early red
blood cells in the bone marrow contain circles of iron deposits (rings) around the nucleus (these cells are called *ringed sideroblasts*).

This type rarely turns into leukemia, and the outcome for people with this type is generally the same as for those with refractory anemia.

**Refractory cytopenia with multilineage dysplasia (RCMD)**

About 40% of people with MDS have this type.

In this condition, the counts of at least 2 types of blood cells are low. In the bone marrow, those same types of cells look abnormal under the microscope (dysplasia). Ringed sideroblasts may or may not be present. The number of blasts (very early cells) in the bone marrow is less than 5% and none of the blasts contain Auer rods (an abnormality seen in some leukemia cells). Blasts are rare or absent in the blood.

RCMD changes into leukemia in about 10% of patients. Having this type of MDS will shorten a person’s life. One estimate is that half of patients will die within 2 years of diagnosis.

**Refractory anemia with excess blasts-1 (RAEB-1)**

One or more cell types are low in the blood and look abnormal in the bone marrow. The number of blasts in the bone marrow is increased; but is still less than 10%. The blasts do not contain Auer rods. Blasts may be present in the blood, but they make up less than 5% of the white blood cells.

The chance of RAEB-1 turning into *acute myeloid leukemia* is about 25%. This type of MDS has a poor outlook and most patients die within 2 years.

**Refractory anemia with excess blasts-2 (RAEB-2)**

This type of MDS is similar to RAEB-1 except the bone marrow contains more blasts – between 10% and 20% of the bone marrow cells are blasts. The blood also contains more blasts: between 5 and 19% of the white blood cells in the blood are blasts. The blasts may contain Auer rods. Any one (or more) of the cell types can be low in the blood and look abnormal in the bone marrow.

The chance of RAEB-2 turning into *acute myeloid leukemia* may be as high as 50%.
**Myelodysplastic syndrome, unclassified (MDS-U)**

This type of MDS is uncommon. For a case to be considered MDS-U, the findings in the blood and bone marrow can’t fit any other type of MDS. Numbers of any one of the cell types may be low in the blood but less than 10% of that type of cell looks abnormal in the bone marrow. The cells in the bone marrow have at least one certain chromosome abnormality that is only seen in MDS or leukemia. The number of blasts in the bone marrow is less than 5%.

Because this type is so rare, it has not been studied well enough to predict prognosis (outlook).

**Myelodysplastic syndrome associated with isolated del(5q)**

In this type of MDS, the chromosomes of the bone marrow cells are normal except they show that a part of chromosome number 5 is missing. In the blood, the red cell counts are low, but the white blood cell counts are normal. Often the platelet count is increased. The number of blasts in the bone marrow is less than 5%.

For unknown reasons, patients with this type of MDS have a very good prognosis (outlook). They often live a long-time and rarely go on to develop leukemia.

**Clinical classification of MDS**

The WHO system defines types of MDS based on the cells in the blood and bone marrow. This is called a **cellular classification system**. Cases of MDS can also be classified based on the underlying cause. This is known as a **clinical classification**. If no cause can be identified, it is called **primary MDS**. When the cause of the disease is known, it is called **secondary MDS**. Secondary MDS is often called **treatment-related MDS**, because the most common cause is prior cancer treatment. This is discussed further in [What Are the Risk Factors for Myelodysplastic Syndrome?](#) Identifying MDS as primary or secondary is important because the secondary type is much less likely to respond to treatment.

- References
  See all references for Myelodysplastic Syndromes

Last Medical Review: February 10, 2014 Last Revised: July 2, 2015
What Are the Key Statistics About Myelodysplastic Syndromes?

In the United States, myelodysplastic syndromes (MDS) occur at a rate of 4.8 cases for every 100,000 people. That works out to about 13,000 new cases of MDS each year. The number of new cases diagnosed each year seems to be increasing as the average age of the population increases.

• References
See all references for Myelodysplastic Syndromes

Last Medical Review: February 10, 2014 Last Revised: July 2, 2015

What's New in Myelodysplastic Syndrome Research and Treatment?

Genetics and biology of MDS

Research on the causes, diagnosis, and treatment of myelodysplastic syndromes (MDS) is being done at many cancer research centers. Scientists are making progress in understanding how a series of changes in a person’s DNA can cause normal bone marrow cells to develop into myelodysplastic cells.

Scientists are also learning how bone marrow stromal cells influence MDS cells. Bone marrow stromal cells are found in the bone marrow but do not develop into blood cells. Instead, they help support, nourish, and regulate the blood-forming cells. Recent studies suggest that although the stromal cells in MDS patients are not cancerous, they are not normal either, and seem to have a role in causing MDS. Scientists have identified some
of the chemical signals that are exchanged between stromal cells and MDS cells.

As more information from this research unfolds, it may be used to design new drugs or eventually in developing gene therapy. This approach replaces the abnormal DNA of cancer cells with normal DNA to restore normal control of cell growth.

**Chemotherapy**

Studies are being done to find drug combinations that work well without serious side effects. New drugs are continually being developed and tested. The drugs sapacitabine and clofarabine have both shown promise. Also, an oral (by mouth) form of azacitidine is being tested.

Research is also under way to see if there is a group of patients that may benefit from more intensive chemotherapy.

**Immune suppression**

Researchers are also looking at different ways to block patients’ immune systems. The drug alemtuzumab (Campath), which is more often used to treat lymphoma and a certain type of chronic leukemia, acts by attacking T-cells. This suppresses the immune system, and was helpful in a recent study in MDS.

**Targeted therapy**

Targeted therapy is a newer type of cancer treatment that uses drugs or other substances to identify and attack cancer cells while doing little damage to normal cells. These therapies attack the cancer cells’ inner workings – the programming and gene changes that make them different from normal, healthy cells. Each type of targeted therapy works differently, but all alter the way a cancer cell grows, divides, repairs itself, or interacts with other cells.

Some targeted therapy drugs, called *angiogenesis inhibitors*, work by preventing growth of new blood vessels. This type of drug has been helpful in treating some types of cancer that form tumors, but may also be helpful in cancers like leukemia and MDS that grow in the bone marrow. Other types of targeted therapy drugs target certain abnormal genes in cancer cells. Some drugs that have been studied in MDS include bevacizumab, aflibercept, everolimus, sorafenib, sunitinib, and midostaurin.
More information can be found in Targeted Therapy.

**Stem cell transplant**

Scientists continue to refine this procedure to increase its effectiveness, reduce complications, and determine which patients are likely to be helped by this treatment.

**Drugs to help blood counts**

Romiplostim (Nplate®) is a new drug that raises platelet counts. It is approved to treat patients who have a disease (called ITP) in which their immune system attacks and destroys their platelets but in more recent studies it has helped raise platelet counts in people with MDS.

- References
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