



## Frequently Asked Questions About MDS

### **What is MDS?**

MDS, or myelodysplastic syndromes, is a collection of disorders in which the bone marrow does not produce enough blood cells.

Normally, the bone marrow produces three major types of blood cells: red blood cells (which carry oxygen to the blood), white blood cells (which help the body fight infections), and platelets (which help blood clot).

In patients with MDS, this process breaks down. Blood cells do not develop properly, and as a result, there is a lack of healthy blood cells in the body.

### **Is MDS a type of cancer?**

MDS is a neoplastic hematologic disorder. And some forms of MDS can progress to acute myeloid leukemia (AML), another type of cancer

### **Why does MDS occur?**

Scientists have determined that the disease can develop due to exposure to certain chemicals or radiation. A rare, familial form may also develop in patients who have family members with MDS. When patients develop MDS that has no known cause, it is called de novo MDS.

### **How common is MDS?**

It is not known exactly how many people have MDS; however, about 7,000 to 12,000 new cases are diagnosed annually in the United States. It primarily occurs in people older than 60.

### **What are the symptoms of MDS?**

Symptoms vary depending on the individual and the extent of the disease. Typical symptoms include weakness, fatigue, frequent infections, easy bruising, bleeding, fever, weight loss, and a sense of feeling full.

### **How is MDS classified?**

MDS is often classified by risk for developing AML. Patients with low or intermediate-1 risk typically have refractory anemia (RA), refractory anemia with ringed sideroblasts (RARS), or refractory anemia with excessive blasts (RAEB). Patients with intermediate-2 or high risk usually have refractory anemia with excessive blasts in transformation (RAEBT) or chronic myelomonocytic leukemia (CMML).

### **What are the syndromes of MDS?**

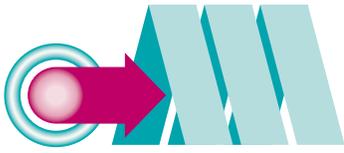
There are several MDS syndromes. Patients with 5q- syndrome have an abnormality in their DNA (DNA is the blueprint that dictates the function and behavior of every cell within the body). The 5q refers to the location of the chromosome, or DNA strand, where the abnormality is present. This syndrome is more frequently seen in older women. It rarely progresses to AML.

Hypoplastic MDS is a syndrome that is similar to a type of anemia, and patients with this syndrome have low numbers of blood cells in the bone marrow, where these cells are produced. Other syndromes include MDS with myelofibrosis (a condition in which bone marrow cells grow inside the spleen and liver and in which the bone marrow is replaced by fibrous tissue) and MDS with prominent eosinophilia or monocytosis (an excess of types of white blood cells).

### **What are the treatment options for MDS?**

There are three options for the treatment of MDS: Low-intensity therapy, high-intensity therapy, and supportive care.

- ▶ Most patients will receive supportive care, which includes red blood cell and platelet transfusions. Supportive care also includes close monitoring by everyone on the healthcare team.
- ▶ Low-intensity therapy includes chemotherapy, which is the use of chemicals to treat the disease. Chemotherapy is usually given as part of a clinical trial. Typically, no hospital stay is required. Chemotherapy is given in the doctor's office or clinic.
- ▶ High-intensity therapy includes intensive chemotherapy, bone marrow transplantation (healthy bone marrow replaces the diseased bone marrow), and peripheral blood stem cell transplantation (healthy immature blood-forming cells replace diseased cells). These treatments are usually given as part of a clinical trial.



***How do I know what treatment is right for me?***

With MDS, treatment is typically tailored according to the individual's age, general health, and type of disease. For instance, patients younger than 60 are considered better suited for intensive therapies such as bone marrow transplantation.

Patients are also separated into risk groups—low, intermediate-1, intermediate-2, and high—based on several factors related to the type of MDS the patient has. Your doctor will determine which type of therapy may be most appropriate.

***What is CC-5013?***

CC-5013 is an investigational immunomodulatory drug. It boosts the production of the body's natural disease-fighting cells, such as T-cells, and disease-fighting chemicals interleukin-2 and interferon- $\gamma$ .

***Has CC-5013 been used to treat other diseases?***

Researchers are evaluating CC-5013 in the treatment of patients with multiple myeloma, a type of cancer. CC-5013 may be safe and active against this cancer, so further clinical trials are being conducted.

***How is CC-5013 taken?***

CC-5013 comes in pill form and is taken by mouth.

***What are the potential side effects?***

Common side effects of CC-5013 include neutropenia, which is a reduction in the number of white blood cells, and thrombocytopenia, which is a reduction in the number of platelets. Symptoms of low numbers of white blood cells include fever, and symptoms of low numbers of platelets include bruising or excessive bleeding. The study physician can provide additional information to you.

***Is CC-5013 available to all patients with MDS?***

Currently CC-5013 is given only to MDS patients who enroll in clinical research trials.

***What is a clinical trial?***

A clinical trial is a study conducted by doctors to test investigational therapies or investigational medications. Doctors conduct these trials to see if these investigational treatments can improve on ones they currently use.