

What are Myelodysplastic Syndromes?

Myelodysplastic Syndromes (MDS) are disorders in which blood progenitor (stem) cells do not mature into healthy blood cells.¹ The disease is also known as a form of blood cancer.²

Failure of the bone marrow to produce mature healthy cells is a gradual process, and reduced blood cell and/or reduced platelet counts and/or reduced neutrophil may be accompanied by the loss of the body's ability to fight infections and control bleeding.

MDS shares clinical and pathologic features with acute myeloid leukemia (AML), but MDS has a lower percentage of blasts in peripheral blood and bone marrow (<20 percent).

Disease Burden: MDS by the Numbers

~16,000

New cases of MDS are reported each year in the U.S.³

0.8–8 years

Median overall survival range depending on clinical variables and risk stratification⁶

1 in 3

Patients with MDS eventually progress to AML⁴

3.6%

Patients with MDS have an isocitrate dehydrogenase 1 (IDH1) mutation, which are considered early "driver" mutations in the progression of MDS to AML⁸

71

Median age of diagnosis⁵

5

Risk categories for MDS (very low-, low-, intermediate-, high- and very high-risk) according to the revised International Prognostic Scoring System (IPSS-R)⁶

25–40%

Patients with MDS have high or very high risk disease^{6,7}



Risk Factors

While some cases of MDS are linked to known risk factors, the exact cause is unknown.⁹ Factors that may increase the risk of MDS include:⁹

- Age
- Sex
- Genetic syndromes
- Smoking
- Environmental toxin exposure (benzene and others)
- Prior chemotherapy
- Radiation exposure (therapeutic or occupational)

What are Myelodysplastic Syndromes (MDS)?



Signs and Symptoms^{10,11}

- Fatigue
- Fever
- Shortness of breath
- Unusual paleness due to a low red blood cell count (anemia)
- Easy or unusual bruising or bleeding
- Pinpoint-sized red spots just beneath your skin caused by bleeding (petechiae)
- Prone to infections due to low white blood cell count (neutropenia)

Patients with MDS may also be asymptomatic for years and may have incidental findings caught on routine blood panels.¹⁰



Treatment

The diagnosis and treatment approach for MDS depends on a variety of factors, including:⁹

- **Risk stratification:** As risk category increases, overall survival decreases and the risk of transformation to AML increases¹²
- **Cytogenetic features** (i.e., chromosomal abnormalities)
- **Eastern Cooperative Oncology Group (ECOG) Performance Status**
- **A person's age, overall health, and preferences**
- **Mutational status:** MDS patients can have multiple mutations and mutational status can change over the course of treatment, which is why it is important to retest as molecular classifications may re-stratify patients into a higher risk category^{13,14}

The most common treatment approaches for MDS are:⁹

- Supportive therapy
- Growth factors and similar medicines
- Chemotherapy
- Stem cell transplant

Patients living with high-risk MDS, especially those in the relapsed or refractory setting, are in urgent need of new treatment options.¹³

It's important to discuss your risk group, mutational testing, and different therapeutic options with your healthcare team to help you make informed decisions about a treatment plan.

Did You Know: Genes involved in DNA methylation and histone modification, including IDH1, are the second most common class of mutations in MDS?¹⁵ IDH1 mutations are considered early “driver” mutations in disease progression and have often been associated with worse overall outcomes.^{8,16}

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