Myelodysplastic Syndromes (MDS) FAQs for Nurses

Find answers to the most commonly asked questions about MDS from nurses and patients.

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www.MDSCenterforNurses.com
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Overview of MDS

What is MDS?
Myelodysplastic syndromes (MDS) refer to a heterogeneous group of clonal disorders characterized by damaged bone marrow stem cells, insufficient hematopoiesis, and bone marrow dysplasia.\(^1\)\(^-\)\(^3\) Formerly classified as myeloid leukemia, MDS were subsequently reclassified as forms of cancer separate from leukemia and lymphoma.\(^4\)

Patients with MDS experience significant cytopenias, anemia being the most common, affecting approximately 80% of patients at the time of diagnosis with MDS. Neutropenia is observed in nearly half of patients (46%), while thrombocytopenia is seen in approximately 37%.\(^1\),\(^5\) Development of acute myeloid leukemia (AML) is also a significant risk in MDS, with more than 25% of patients ultimately developing AML.\(^5\),\(^6\)

For additional information:
http://www.aamds.org/what-mds-ipad-app
http://www.mds-foundation.org/what-is-mds/
http://buildingblocksofhope.com/

How common are MDS?

The incidence of MDS is estimated at 4.8 cases per 100,000 people in the United States, producing approximately 13,000 new cases per year, although there is reason to believe the rate may be higher.\textsuperscript{1,2} The incidence of MDS is increasing each year as the population age increases, since MDS are far more prevalent in older people. MDS are rare among children/adolescents with an incidence rate of 0.2/100,000 people per year in those younger than 40 years old. The incidence rate increases to 29.6/100,000 people among individuals between the ages of 70-79. The rate increases to 55.8/100,000 among those 80 years of age and older.\textsuperscript{2}

\begin{table}[h]
\centering
\begin{tabular}{|c|c|}
\hline
Incidence per 100,000/y\textsuperscript{2} & \\
\hline
General Population & 4.8 \\
<40 Years & 0.2 \\
>70 Years & 29.6 \\
\geq 80 Years & 55.8 \\
\hline
\end{tabular}
\end{table}


\textsuperscript{2} Referenced with permission from the NCCN Clinical Practice Guidelines in Oncology (NCCN Guidelines\textsuperscript{®}) for Myelodysplastic Syndromes V.1.2016. © National Comprehensive Cancer Network, Inc 2015. All rights reserved. Accessed June 10, 2015. To view the most recent and complete version of the guideline, go online to NCCN.org. NATIONAL COMPREHENSIVE CANCER NETWORK®, NCCN®, NCCN GUIDELINES®, and all other NCCN Content are trademarks owned by the National Comprehensive Cancer Network, Inc.
What causes MDS?
DNA mutations and deletions—in some cases, resulting from exposure to radiation or certain chemicals—have been implicated in MDS etiology. The variety of genetic abnormalities observed in patients with MDS points to a number of different potential initiating mechanisms, and helps explain the heterogeneity of MDS’ clinical phenotype. Several milestones in the development of MDS have been identified:

1. A capacity for self-renewal in the disease-initiating cell (stem cell or myeloid progenitor)
2. Increased proliferation in disease-sustaining clone (or more differentiated progeny)
3. Impaired differentiation
4. Genetic and epigenetic instability
5. Resistance to apoptosis in disease-sustaining clone
6. Evasion of immune system
7. Suppression of hematopoiesis

What are the risk factors associated with developing MDS?
Apart from age, the primary MDS risk factors include exposure to chemotherapy drugs and to radiation therapy, which either separately or in combination can cause “secondary” or “treatment-related” MDS. Other risk factors for MDS include certain genetic syndromes (eg, Fanconi anemia), exposure to ionizing radiation, exposure to benzene, solvents, or pesticides, as well as smoking, obesity, and male gender.

For additional information:
http://www.cancer.net/cancer-types/myelodysplastic-syndromes-mds/risk-factors

Overview of MDS

What is the main pathophysiology of MDS?
The pathophysiology of MDS is, at present, not well understood in detail. It is clear that for patients with MDS, stem cells in the bone marrow fail to achieve normal maturity, increasing the number of blast cells and dysplastic cells, while the number of mature blood cells decreases, causing dysfunction of the bone marrow, resulting in anemia, neutropenia, and thrombocytopenia.\(^1,2\)

At the molecular level, mutations and epigenetic effects, resulting in altered gene expression, play a large role in driving MDS pathophysiology.\(^3\) At the same time, pathogenic factors in MDS may be understood to promote “hematopoietic senescence,” which is consistent with a disease that manifests primarily in older patients. These factors include not only those related to genetic/epigenetic dysfunction, but also exposure to toxic environmental agents, especially genotoxic cancer therapies, such as chemotherapy and radiation, which are strongly associated with MDS risk.\(^4\)

For additional information:
http://buildingblocksofhope.com/

Overview of MDS

What are the different types of MDS?
The World Health Organization (WHO) classification—which replaced the earlier French-American-British (FAB) classification system—specifies 7 types of MDS based on cellular classification:

- 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)

Chronic myelomonocytic leukemia (CMML) was considered a variety of MDS in the earlier FAB classification system but was reclassified as a myelodysplastic/myeloproliferative neoplasm (MDS/MPN) in the WHO system.

It should be noted that when no cause of the underlying disease can be identified, it is considered primary MDS. When the cause is known, it is called secondary MDS. Secondary MDS is often referred to as treatment-related MDS because the most common cause of secondary MDS is prior cancer treatment.

For additional information:
http://www.lls.org/diseaseinformation/myelodysplasticsyndromes/mdssubtypes
http://www.aamds.org/node/62/

What are the most common cytogenetic abnormalities?

Cytogenetic abnormalities are common features of MDS, and cytogenetic analysis of bone marrow has become an important part of clinical care. Cytogenetic evaluation can help confirm the diagnosis of MDS, aid in prognosis (see FAQs on prognosis on page 18), and estimate the risk of AML development. Cytogenetic analysis is also a widely available and standard technique for determining clonality in MDS.¹

Recurring chromosomal abnormalities are found in 40-70% of patients with primary MDS and in 95% of patients with therapy-related MDS. The most common cytogenetic abnormalities in MDS are del(5q), -7, and +8.¹

Relative Percentages of Various Cytogenetic Abnormalities in Primary MDS²

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Overview of MDS

What are the most common symptoms of MDS?
Roughly 80% of patients with MDS experience anemia, 46% neutropenia, and 37% thrombocytopenia.1–3 Symptoms of MDS secondary to cytopenias include fatigue, dyspnea, easy bruising or bleeding, pale skin, and petechiae. Weight loss, fever, and appetite loss are also common in MDS.1,4 For more information on managing these symptoms, see Supportive Care FAQs on pages 25-26.

For more information:
http://aamds.org/what-mds-ipad-app
http://www.mds-foundation.org/what-is-mds/

What are the risks and symptoms of anemia with MDS?
Anemia in MDS typically presents as hypoproducive (low reticulocyte count) macrocytic anemia and is associated with suboptimal elevation of serum erythropoietin (sEpo) levels.1 Anemia is present in more than 80% of newly diagnosed MDS patients.2 Symptoms include fatigue, dyspnea, dizziness, tachycardia, and palpitations.3 Older patients with severe anemia may experience chest pain due to a decrease in blood flow to the heart. These symptoms are rarely life-threatening, but often result in a decreased quality of life.4

For more information:
http://www.mds-foundation.org/what-is-mds/

Overview of MDS

What are the risks of thrombocytopenia and bleeding in patients with MDS?
At least one-third of patients with MDS have moderate or severe thrombocytopenia. Patients may experience petechiae, ecchymosis, epistaxis, hemoptysis, and hematuria. Serious bleeding is uncommon but may include gastrointestinal hemorrhage, macrohematuria, retinal hemorrhage, or central nervous system hemorrhage. In severe thrombocytopenia associated with MDS, platelet transfusion is used to avoid the potentially serious consequences of significant blood loss. For information on managing thrombocytopenia, see What supportive care measures are important for patients with MDS on page 25.

For more information:
http://www.mds-foundation.org/what-is-mds/

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What are the risks of neutropenia and infections with MDS?
Neutropenia affects more than half of all patients with MDS. Approximately one-third of patients experience recurrent infections, which may result in fever, cough, dysuria, abdominal pain, or diarrhea. Infections are usually bacterial and most often occur in the skin. MDS patients with neutropenia are also at increased risk of developing sepsis. For information on managing neutropenia, see What supportive care measures are important for patients with MDS on page 25.

For more information:
http://www.mds-foundation.org/what-is-mds/

Overview of MDS

How often do MDS transform to AML?
Transformation to AML occurs in more than 25% of patients with MDS.\textsuperscript{1,2} The majority of patients will die from complications of their MDS rather than transformation to AML.\textsuperscript{3} See survival by prognostic risk category on page 18.

Diagnosis and Prognosis
Diagnosis and Prognosis

How are MDS diagnosed?
Initial diagnostic evaluation for MDS consists of:

Examination
- Patient history and physical exam
- CBC, platelets, differential, reticulocyte count, examination of peripheral smear
- Bone marrow aspiration with iron stain and biopsy; cytogenetics by standard karyotyping
- Serum erythropoietin level
- Red blood cell folate levels; vitamin B₁₂
- Serum ferritin, iron, total iron-binding capacity
- Thyroid-stimulating hormone levels
- Transfusion history

Establishing diagnosis
- Use WHO classification system with IPSS or IPSS-R

Additional testing (helpful in some clinical situations)
- Flow cytometry
- Human leukocyte antigen (HLA) levels
- HLA & HLA-DR15 typing
- HIV testing
- Consider evaluation of 5q31-33 translocations
- Consider molecular & genetic testing
- Consider copper deficiency evaluation
### Classification of MDS

<table>
<thead>
<tr>
<th>Subtype</th>
<th>Blood</th>
<th>Bone marrow</th>
</tr>
</thead>
<tbody>
<tr>
<td>Refractory cytopenia with unilineage dysplasia (RCUD)</td>
<td>Single or bicytopenia</td>
<td>Dysplasia in ≥10% of one cell line, &lt;5% blasts</td>
</tr>
<tr>
<td>Refractory anemia with ring sideroblasts (RARS)</td>
<td>Anemia, no blasts</td>
<td>≥15% of erythroid precursors w/ring sideroblasts, erythroid dysplasia only, &lt;5% blasts</td>
</tr>
<tr>
<td>Refractory cytopenia with multilineage dysplasia (RCMD)</td>
<td>Cytopenia(s), &lt;1 x 10⁹/L monocytes</td>
<td>Dysplasia in ≥10% of cells in ≥2 hematopoietic lineages, ±15% ring sideroblasts, &lt;5% blasts</td>
</tr>
<tr>
<td>Refractory anemia with excess blasts-1 (RAEB-1)</td>
<td>Cytopenia(s) ≤2%-4% blasts, &lt;1 x 10⁹/L monocytes</td>
<td>Unilineage or multilineage dysplasia, 5%-9% blasts, no Auer rods</td>
</tr>
<tr>
<td>Refractory anemia with excess blasts-2 (RAEB-2)</td>
<td>Cytopenia(s) 5%-19% blasts, &lt;1 x 10⁹/L monocytes</td>
<td>Unilineage or multilineage dysplasia, 10%-19% blasts, ± Auer rods</td>
</tr>
<tr>
<td>Myelodysplastic syndrome, unclassified (MDS-U)</td>
<td>Cytopenias, ±1% blasts</td>
<td>Unilineage dysplasia or no dysplasia but characteristic MDS cytogenetics, &lt;5% blasts</td>
</tr>
<tr>
<td>MDS associated with isolated del(5q)</td>
<td>Anemia, platelets normal or increased</td>
<td>Unilineage erythroid dysplasia, isolated del(5q), &lt;5% blasts</td>
</tr>
<tr>
<td>Refractory anemia with excess blasts in transformation (RAEB-T)</td>
<td>Cytopenias, 5%-19% blasts</td>
<td>Multilineage dysplasia, 20%-30% blasts, ± Auer rods</td>
</tr>
</tbody>
</table>


This category encompasses refractory anemia (RA), refractory neutropenia (RN), and refractory thrombocytopenia (RT). Cases of RN and RT were previously classified as MDS, unclassified.

The limitations of the current diagnostic approach can be seen in diagnostic inconsistencies and inaccuracies. However, molecular genetic testing is becoming more prominent in MDS diagnosis and may soon play a primary role in the diagnostic process.

**For additional information:**
- [http://www.nccn.org/](http://www.nccn.org/)
- [http://www.aamds.org/node/61](http://www.aamds.org/node/61)

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What common comorbidities do patients with MDS face?

As MDS are cancers most commonly diagnosed in older patients, comorbidities are also more common. In a large, population-based study of 1,708 newly diagnosed patients with MDS, 51% had comorbid conditions. The most common nonhematologic comorbidities observed were diabetes, congestive heart failure (CHF), and chronic obstructive pulmonary disease (COPD). Patients with MDS and CHF or COPD had significantly shorter survival than patients without those conditions, whereas diabetes did not appear to have an impact on survival in this study.

For more information:
http://www.aamds.org/node/471


How is risk assessed in MDS?

There are currently 5 prognostic scoring systems employed in MDS\cite{1,5}:

- International Prognostic Scoring System (IPSS)
- World Health Organization (WHO) Prognostic Scoring System (WPSS)
- MD Anderson General Model
- MD Anderson Lower-Risk Prognostic Scoring System (LR-PSS)
- IPSS-Revised (IPSS-R)

The IPSS uses 3 factors—percentage of blasts in bone marrow, chromosome abnormalities, and number of cytopenias—to stratify patients with MDS into 4 risk groups: low risk, intermediate-1 risk (Int-1), intermediate-2 risk (Int-2), and high risk.\cite{1}
Diagnosis and Prognosis

The WPSS uses 3 criteria—MDS category (see page 13), chromosome abnormalities, and presence of severe anemia—to stratify patients into 5 risk categories: very low risk, low risk, intermediate risk, high risk, and very high risk. WPSS has been validated at time points other than diagnosis and can be used as a dynamic scoring system.²

Abbreviations: RCUD, refractory cytopenia with unilineage dysplasia; RARS, refractory anemia with ringed sideroblasts; RCMD, refractory cytopenia with multilineage dysplasia; RAEB-1, refractory anemia with excess blasts-1; RAEB-2, refractory anemia with excess blasts-2.

²In males, <9 g/dL; in females, <8 g/dL.
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The MD Anderson General Model was developed as an improvement upon the IPSS. While it uses some of the same criteria as the IPSS, the MD Anderson General Model gives more emphasis to the importance of thrombocytopenia and adds patient age, Eastern Cooperative Oncology Group (ECOG) performance, and prior red blood cell transfusion history as criteria.³
### Diagnosis and Prognosis

#### MD Anderson General Model

<table>
<thead>
<tr>
<th>MD Anderson General Model Prognostic Category</th>
<th>Score</th>
</tr>
</thead>
<tbody>
<tr>
<td>Performance status</td>
<td>1</td>
</tr>
<tr>
<td>Age (y)</td>
<td>2</td>
</tr>
<tr>
<td>Platelets (&gt;10^9/L)</td>
<td>3</td>
</tr>
<tr>
<td>Hemoglobin (g/dL)</td>
<td></td>
</tr>
<tr>
<td>Bone marrow blasts (%)</td>
<td></td>
</tr>
<tr>
<td>WBC (&gt;10^9/L)</td>
<td></td>
</tr>
<tr>
<td>Cytogenetics</td>
<td></td>
</tr>
<tr>
<td>Prior transfusion</td>
<td></td>
</tr>
</tbody>
</table>

#### MD Anderson General Model Risk Stratification by Score

<table>
<thead>
<tr>
<th>Score</th>
<th>MD Anderson General Model Risk Group</th>
</tr>
</thead>
<tbody>
<tr>
<td>0-4</td>
<td>Low</td>
</tr>
<tr>
<td>5-6</td>
<td>Int-1</td>
</tr>
<tr>
<td>7-8</td>
<td>Int-2</td>
</tr>
<tr>
<td>≥9</td>
<td>High</td>
</tr>
</tbody>
</table>

### MD Anderson Lower-Risk Prognostic Scoring System

<table>
<thead>
<tr>
<th>LR-PSS Prognostic Category</th>
<th>Score</th>
</tr>
</thead>
<tbody>
<tr>
<td>Cytogenetics</td>
<td>1</td>
</tr>
<tr>
<td>Age (y)</td>
<td>2</td>
</tr>
<tr>
<td>Hemoglobin (g/dL)</td>
<td></td>
</tr>
<tr>
<td>Platelets (&gt;10^9/L)</td>
<td></td>
</tr>
<tr>
<td>Bone marrow blasts (%)</td>
<td></td>
</tr>
</tbody>
</table>

#### MD Anderson Lower-Risk Prognostic Scoring System Risk Stratification by Score

<table>
<thead>
<tr>
<th>Score</th>
<th>LR-PSS Risk Group</th>
</tr>
</thead>
<tbody>
<tr>
<td>0-2</td>
<td>1</td>
</tr>
<tr>
<td>3-4</td>
<td>2</td>
</tr>
<tr>
<td>≥5</td>
<td>3</td>
</tr>
</tbody>
</table>

Abbreviations: WBC, white blood cell.

Adapted with permission from Kantarjian H, et al. Proposal for a new risk model in myelodysplastic syndrome that accounts for events not considered in the original international prognostic scoring system, *Cancer*, John Wiley and Sons. ©2008 American Cancer Society.

The same group that developed the MD Anderson General Model also developed a scoring system aimed at low-risk patients. The LR-PSS is a simpler system than the MD Anderson General Model and is intended to identify patients with lower-risk MDS and a poor prognosis who may benefit from early intervention.4

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4Favorable cytogenetics included diploid and del(5q). All others were unfavorable.
Diagnosis and Prognosis

A revised version of the IPSS (IPSS-R) has been released and varies from the original scoring system in several ways to produce a more precise risk stratification. These changes include addressing the degree of cytopenias individually, increasing the number of risk groups from 4 to 5, and putting less emphasis on blast percentage. An age-adjusted calculation of risk can also be used for a survival estimation.\(^5\)

<table>
<thead>
<tr>
<th>Revised International Prognostic Scoring System Risk Stratification(^1)</th>
<th>Score</th>
</tr>
</thead>
<tbody>
<tr>
<td>IPSS-R Prognostic Category</td>
<td>0</td>
</tr>
<tr>
<td>Cytogenetics(^a)</td>
<td>Very good</td>
</tr>
<tr>
<td>Bone marrow blasts (%)</td>
<td>≤2</td>
</tr>
<tr>
<td>Hemoglobin (g/dL)</td>
<td>≥10</td>
</tr>
<tr>
<td>Platelets (×10(^9)/L)</td>
<td>≥100</td>
</tr>
<tr>
<td>ANC (×10(^9)/L)</td>
<td>≥0.8</td>
</tr>
</tbody>
</table>

Abbreviations: ANC, absolute neutrophil count; Inter, intermediate.

\(^a\)Cytogenetic Subgroup Criteria: Very good: -Y, del(11q); Good: Normal, del(5q), del(12p), del(20q), double including del(5q); Intermediate: del(7q), +8, +19, i(17q), any other single or double independent clones; Poor: -7, inv(3)/t(3q)/del(3q), double including -7/del(7q), complex: 3 abnormalities; Very poor: Complex: >3 abnormalities.

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For more information:
http://www.mds-foundation.org/ipss-r-calculator/

Diagnosis and Prognosis

What is the prognosis for patients with MDS?
Displayed in the tables are prognostic data based on designated risk groups using the following systems: World Health Organization (WHO) Prognostic Scoring System (WPSS), International Prognostic Scoring System (IPSS), and Revised IPSS (IPSS-R).

<table>
<thead>
<tr>
<th>Prognostic Data Based on Designated Risk Groups per Scoring System</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>World Health Organization Prognostic Scoring System</strong>¹</td>
</tr>
<tr>
<td>WPSS Risk Group</td>
</tr>
<tr>
<td>Very low</td>
</tr>
<tr>
<td>Low</td>
</tr>
<tr>
<td>Intermediate</td>
</tr>
<tr>
<td>High</td>
</tr>
<tr>
<td>Very high</td>
</tr>
</tbody>
</table>

| **International Prognostic Scoring System**²                  |
| IPSS Risk Group     | Median Survival | Time to 25% of Patients Developing AML |
| Low                 | 5.7 years      | 9.4 years                    |
| Intermediate-1      | 3.5 years      | 3.3 years                    |
| Intermediate-2      | 1.2 years      | 1.1 years                    |
| High                | 0.4 years      | 0.2 years                    |

| **Revised International Prognostic Scoring System**³         |
| IPSS-R Risk Group  | Median Survival | Time to 25% of Patients Developing AML |
| Very low           | 8.8 years      | N/R*                         |
| Low                | 5.3 years      | 10.8                         |
| Intermediate       | 3.0 years      | 3.2                         |
| High               | 1.6 years      | 1.4                         |
| Very high          | 0.8 years      | 0.7                         |

Abbreviation: AML, acute myeloid leukemia.
*Not reached during study.

For more information:
http://www.mds-foundation.org/ipss-r-calculator/

How can disease progression be assessed?

In patients who have received treatment, the International Working Group (IWG) response criteria defines disease progression by a relative increase in blasts, decreases in granulocytes and platelets, reductions in hemoglobin, and transfusion dependence.

<table>
<thead>
<tr>
<th>Baseline Blast Status</th>
<th>Minimum Blast Increase</th>
<th>Disease Progression Threshold</th>
</tr>
</thead>
<tbody>
<tr>
<td>&lt;5%</td>
<td>≥50%</td>
<td>&gt;5% blasts</td>
</tr>
<tr>
<td>5%-10%</td>
<td>≥50%</td>
<td>&gt;10% blasts</td>
</tr>
<tr>
<td>10%-20%</td>
<td>≥50%</td>
<td>&gt;20% blasts</td>
</tr>
<tr>
<td>20%-30%</td>
<td>≥50%</td>
<td>&gt;30% blasts</td>
</tr>
</tbody>
</table>

Other Disease Progression Criteria

- ≥50% decrement from maximum remission/response in granulocytes or platelets
- Hemoglobin reduction ≥2 g/dL
- Transfusion dependence

Treatment
When is hematopoietic stem cell transplantation a treatment option for patients with MDS?

Stem cell transplantation is a preferred treatment option for certain patients with MDS.\(^1,2\) It is, however, a treatment associated with significant risk, including risk of death, and is reserved for candidates with a good chance of success—ie, very often younger patients and those with a good stem cell donor match (in cases of allogeneic as opposed autologous transplantation). Nonmyeloablative allogeneic stem cell transplant, which requires lower doses of chemotherapy and/or radiation than those used for standard stem cell transplants, may be used in some older patients.\(^1\)

The National Comprehensive Cancer Network\(^\circledast\) (NCCN\(^\circledast\)) Clinical Practice Guidelines in Oncology (NCCN Guidelines\(^\circledast\)) for MDS describe stem cell transplantation as a potential third-line treatment option in select patients at very low to intermediate risk scoring on the International Prognostic Scoring System (IPSS), the revised IPSS (IPSS-R), and the World Health Organization Prognostic Scoring System (WPSS). Patients with intermediate to very high risk scoring may be offered stem cell transplantation as first-line treatment if they are appropriate transplant candidates.\(^2\)

For more information:
http://www.aamds.org/about/MDS/treatment/transplantation

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What are the treatment goals for patients with low-risk MDS? High-risk MDS?
The National Comprehensive Cancer Network® (NCCN®) Clinical Practice Guidelines in Oncology (NCCN Guidelines®) divide treatment goals for primary MDS into 2 categories, along a low-risk/high-risk divide. The lower-risk patients include those categorized as low or intermediate-1 risk (according to the International Prognostic Scoring System [IPSS]), very low to intermediate risk (according to the Revised IPSS [IPSS-R]), or very low to intermediate risk (according to the World Health Organization [WHO] Prognostic Scoring System [WPSS]). (See How is risk assessed in MDS? on page 14) The treatment goal for lower-risk patients is to improve hematologic parameters, including a reduction in transfusion burden. Patients with intermediate risk who fail low-risk treatment should be considered for high-risk treatment. See NCCN Guidelines®.

A high-risk therapeutic approach is typically used among patients with IPSS intermediate-2 and high risk, IPSS-R intermediate to very high risk, or WPSS high or very high risk, as well as patients with secondary MDS. The goal of treatment for higher-risk patients is to alter the natural history of the disease and delay or avoid leukemic transformation. Improvement in quality of life and cytogenetic response are additional meaningful outcomes in MDS. All patients, regardless of risk group, should receive supportive care appropriate to their needs. See NCCN Guidelines®.

For more information:
http://www.nccn.org/

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How are patients followed during treatment?
Blood counts should be performed to monitor for adverse events that may develop during MDS treatments. All patients should receive supportive care including observation, clinical monitoring, and psychosocial support. See FAQs on Supportive Care on pages 25-26. For patients needing chronic RBC transfusions, serum ferritin levels and transfusion numbers should be tracked to assess for iron overload. Associated heart, liver, and pancreas dysfunction should also be monitored. See FAQ on RBC transfusions on page 25.

For additional information:
http://www.nccn.org/

How often do patients with MDS need to have blood counts assessed?
The need for blood counts will vary depending on the patient and the patient’s treatment. Please check the package insert of the medication your patient is receiving for more information.

What are the most common adverse events associated with MDS therapies?
The most common adverse events associated with MDS therapies relate primarily to pharmacologic treatment as well as transfusion and stem cell transplantation. Pharmacologic treatments are associated with myelosuppression (anemia, neutropenia, and thrombocytopenia) and its risks, such as elevated infection risk. Gastrointestinal adverse events are also seen with pharmacologic treatment, primarily in the form of nausea, vomiting, and diarrhea. The well-known adverse events associated with stem cell transplantation are significant and may result in death, particularly in an elderly patient population.

For more information:


How often should patients with MDS be seen for follow-up by their physician?
The frequency of follow-up visits will depend on a patient’s health status and treatment regimen.
Supportive Care

What supportive care measures are important for patients with MDS?
The key activities of supportive care applicable to MDS patients include:

- Clinical monitoring
- Transfusion support (red blood cells and/or platelets)
- Blood cell growth factor administration
- Antibiotics for bacterial infections
- Aminocaproic acid or other antifibrinolytic agents for bleeding refractory to platelet transfusions or profound thrombocytopenia
- Iron chelation therapy for patients at risk of transfusional iron overload.

Psychosocial support and assessment of patient quality of life are also fundamental to supportive care.

For additional information:
http://www.nccn.org/

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What are the risks and benefits associated with red blood cell (RBC) transfusions?
RBC transfusion is a common treatment for patients with MDS who have symptomatic anemia and for whom erythropoietin treatment is insufficiently effective.1,2 The primary benefit of RBC transfusion in MDS is to relieve anemia and the symptoms associated with anemia (eg, fatigue, dyspnea). Approximately 39% of patients designated low-risk or intermediate-1–risk (per International Prognostic Scoring System criteria) will require regular RBC transfusions.3

The primary concern surrounding the use of RBC transfusions is the risk of iron overload, which occurs when excess iron is accumulated in the body over the course of repeated RBC transfusions due to the body’s inability to clear the increased iron. The consequences of sustained iron overload are serious, including organ damage resulting in congestive heart failure, diabetes, liver dysfunction, as well as dysfunctional hematopoiesis.4 Dependency on RBC transfusions has been associated with decreased survival.5
Additionally, anemia has been shown to impact patient quality of life through persistent fatigue and through the exacerbation of comorbidities. RBC transfusions alleviate the symptoms of anemia, but require frequent trips to the hospital, which may also impact the patient’s quality of life.6

For more information:
http://www.aamds.org/about/MDS/treatment/blood-transfusions

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What is the role of erythropoiesis-stimulating agents (ESAs) in the treatment of MDS?
The National Comprehensive Cancer Network® (NCCN®) has identified ESAs as a possible treatment for symptomatic anemia. A model has been developed to help identify potential responders. See the NCCN Clinical Practice Guidelines in Oncology (NCCN Guidelines®).

For more information:
http://www.aamds.org/about/MDS/treatment/growth-factors

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What life modifications could be of benefit to patients with MDS?
Recommended modifications to lifestyle for patients with MDS are similar to lifestyle changes for other patients with cancer: eating a balanced diet, getting regular exercise (as tolerated in patients likely to experience fatigue), engaging in daily and social activities, limiting alcohol, and stopping tobacco use (if applicable).\(^1\,^2\)

It is also important to help patients live with MDS by encouraging them to keep a positive attitude and effectively manage stress while undergoing treatment. Patients can build a positive attitude by taking steps to make the best of life situations in areas under their control and not wasting energy over situations they can’t control. Patients can help manage stress by being an integral part of their healthcare team. Empower patients by educating them on disease and treatment options and encourage them to bring a list of questions to their office visits. Additionally, encourage patients to seek support from family, friends, and support groups, as having a network for emotional support is an integral part of coping with MDS.\(^3\)

For more information:
- [http://aamds.org/about/living-well](http://aamds.org/about/living-well)

How can caregivers help support patients with MDS?
Caregivers for patients with MDS can play a useful role by being attentive to the signs and symptoms exhibited by the patient that the patient may not notice and that healthcare providers may not be present to observe. For example, cognitive mood changes will often be unnoticed by the patient, while the emergence of symptoms, particularly those perceived as indicating disease progression, may be too difficult for the patient to acknowledge. Caregivers can provide clinicians with important details regarding the patient’s appetite, activities of daily living, energy and endurance, sleeping habits, and numerous other health-related variables that may be useful in the clinical setting.

For more information:
http://www.aamds.org/treating-mds-toolkit
http://www.mds-foundation.org/building-blocks-of-hope-downloads/


What are the most effective ways for patients with MDS to combat fatigue?
According to an internet survey, more than 90% of patients with MDS report excessive fatigue as a common symptom. Pharmacologic therapies for fatigue in MDS have had mixed success. At present, lifestyle changes may help with fatigue symptoms. These include regular exercise and a balanced diet as well as sufficient and undisturbed sleep. Reducing stress and engaging in activities and hobbies that promote relaxation may also help relieve fatigue symptoms.

For more information:
http://www.aamds.org/node/150

How should patients be educated on dealing with illnesses like MDS?

Patient education about MDS resembles that of education about cancers in general in that patients require information about the disease state, treatment options, treatment duration, adverse events, and healthy lifestyle choices. With regard to disease state, MDS differ from more familiar cancers, such as breast or lung cancer. Most people have some idea about the nature of those diseases, whereas MDS are likely to be unfamiliar. Thus, patients need to understand the disease for their own knowledge and so that they can communicate it to others. As MDS are illnesses without a clear beginning and end to treatment, patients must be educated to come to terms with the fact that treatment in MDS is “a marathon, not a sprint” (Sandra E. Kurtin, RN, MS, AOCN, ANP-C).

For more information:
http://aamds.org/about/living-well/emotional-health