

Lower-Risk Myelodysplastic Syndromes & The Need for More Effective Treatment Options

Myelodysplastic syndromes (MDS) are a group of cancers in which bone marrow does not produce enough healthy blood cells. There are several types of MDS. In some cases, MDS can progress to acute myeloid leukemia (AML). When AML is less likely to develop, healthcare providers will refer to the condition as lower-risk MDS (LR-MDS).¹⁻⁴



LR-MDS accounts for **~70%** of MDS cases.^{5,6}

Key Information About LR-MDS

LR-MDS is a blood cancer that often progresses to require increasingly intensified management of key symptoms such as anemia and resulting fatigue.⁷

Key drivers of **PATIENT BURDEN** and **POOR QUALITY OF LIFE** include:^{2,5,8-12}



Severe anemia

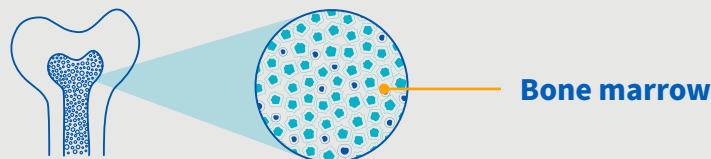


Transfusion dependence



Limited treatment options

Understanding MDS



In **HEALTHY INDIVIDUALS**, bone marrow produces blood stem cells that mature into various blood cell types that play essential roles in the body.¹³

Blood stem cell

Red blood cells:

carry oxygen to tissues and remove carbon dioxide

White blood cells:

protect the body from infection

Platelets:

form clots to stop or prevent bleeding

In **INDIVIDUALS WITH MDS**, blood stem cells may never mature or may develop into abnormal, nonfunctional blood cells.¹³

Blood stem cell

Anemia:

too few red blood cells (RBC)

~90% of patients with MDS experience anemia^{9,10}

Neutropenia:

too few white blood cells

Thrombocytopenia:

too few platelets

LR-MDS can introduce physical and emotional challenges.

Approximately 90% of patients with LR-MDS experience anemia.

Patients living with symptomatic anemia can experience.^{2,5,8,9}



Shortness of breath



Tachycardia



Severe fatigue



Dizziness

As the disease continues to progress and the severity of anemia increases, people with LR-MDS require RBC transfusions to manage symptomatic anemia and often become **transfusion dependent**.¹ These transfusions can provide short-term relief but also come with short- and long-term clinical consequences and negative impacts on patients' lives.^{2,11,12,14-17}

The burden of transfusion dependence may include:^{2,10-12}



Lengthy and frequent hospital visits



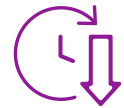
Symptom recurrence



Decreased quality of life



Psycho-emotional impact



Shortened survival

There is an unmet need for treatments that can provide extended and continuous red blood cell transfusion independence for select sub-populations.^{2,18}

One of the ways healthcare providers classify this blood cancer is based on the presence or absence of ring-shaped iron deposits in red blood cells, referred to as ring sideroblasts (RS), which can impact treatment options.¹⁹



Treatments are available for LR-MDS, including erythropoiesis stimulating agents (ESAs)—a first-line treatment for symptomatic or transfusion-dependent anemia in patients with LR-MDS. **However, current treatments for RBC transfusion-dependent patients ineligible for ESAs have significant limitations, are limited to select sub-populations and there is an unmet need for treatments that can provide extended and continuous transfusion independence.**^{2,8,9,20,21}



~1/10 patients who are transfusion-dependent are not eligible to receive ESAs, leaving them with limited treatment options.^{8,20,21}

Of patients who no longer benefit from or who are ineligible for ESA treatment:



RS- patients are particularly vulnerable to poor clinical outcomes.^{14,19}



RS+ patients experience high transfusion burden despite available therapies.¹⁹

These underserved groups are at greater risk for disease burden. This underscores the need for novel treatment options that provide durable transfusion independence.^{2,18}

RBC transfusion independence is a key therapeutic goal for patients and physicians alike.

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