



**Myelodysplastic Syndromes:  
Update from ASH, 2005**

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Myelodysplasia is the number one cause of anemia in the elderly population. There is an incidence of 20,000 cases per year, with a median age of 65 years in the US. More than 70% of the patients are older than 50 years of age on diagnosis. Myelodysplasia is a clonal disorder characterized by ineffective erythropoiesis and cytopenias of variable degree. Ultimately, most patients will succumb to infection and/or bleeding. There is a 35%-40% rate of transformation into acute leukemia. Average survival is between 3 to 4 years.

The disease was initially classified according to morphologic findings and percentage of blasts in the bone marrow. In the recent past, myelodysplasia has been better characterized, and now the classification system will include the number of cytopenias, the blast percentage in the blood and bone marrow, and the presence of cytogenetic abnormalities. An International Prognostic Scoring System (IPSS) has been developed to try to predict clinical course,

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transformation to acute leukemia, and survival.

While allogeneic bone marrow transplant remains the only curative option for the minority of patients with MDS, supportive care (including blood transfusions), with and without erythropoietic growth factors has been the standard treatment for the disease. The landscape for the treatment of myelodysplasia has dramatically changed in the past two years. Two drugs have been granted FDA approval for treatment of patients with MDS.

A multi-center Phase III trial comparing Five-Azacidine (Vidaza) versus supportive care demonstrated a 15% response rate across all sub-types of MDS. Responding patients appear to have a decreased rate of leukemia transformation and may enjoy improved survival. Vidaza became the first FDA agent approved for the treatment of high-risk patients with all sub-types of MDS.

In a multi-center Phase II trial, the thalidomide analog Lenalidomide (Revlimid) showed an impressive 60% response in transfusion requirements for patients with low-risk myelodysplastic syndrome and 5q minus cytogenetic abnormality. Based on those results the

