Myelodysplastic Syndromes | Niche and Rare Pharmacor | G7 | 2015

Myelodysplastic syndromes (MDS) consists of a group of hematological stem cell disorders that are heterogeneous in cause and manifestations but share the common features of aberrant hematopoiesis and deteriorating cytopenias. Patients with MDS most commonly display anemia, but suffer from additional cytopenias as well, with outcomes such as extreme fatigue, high rates of infections, excessive bleeding, and shortened life span. Lower-risk MDS patients are typically treated for anemia and associated issues, while higher-risk MDS patients are treated more aggressively in attempt to eliminate the clonal population and extend lifespan. This report provides an overview of the MDS landscape featuring a comprehensive analysis of patient populations, current therapies and medical practices, and opportunities for emerging therapies. The report identifies a very high level of unmet need for new, disease-modifying MDS therapies and provides expert insight on potential drug targets. At this time, the only potentially curative therapy for MDS is allogeneic hematopoietic stem cell transplant (HSCT). However, interviewed experts report that approximately 90% of the MDS patient population is ineligible for HSCT owing to advanced age, difficulty finding a donor, or most commonly, poor health status. Current treatment options for MDS are very limited and frequently ineffective. They include lenalidomide (Revlimid) and erythropoiesis-stimulating agents (ESAs) for lower-risk patients, and the hypomethylating agents azacitidine (Vidaza) and decitabine (Dacogen) for higher-risk patients. Nearly half of patients using these approved therapies are refractory or become unresponsive, and there are no approved second-line therapies for MDS.

Questions Answered in This Report:

- MDS is typically a disorder of the elderly. What is the size of the U.S. and EU5 (France, Germany, Italy, Spain, and the United Kingdom) diagnosed prevalent and incident MDS patient population, and how will it change over the next ten years? What are the key MDS subpopulations (e.g., age, morphology, IPSS-R status)?

- The pathology of MDS is not well understood and the disease is heterogeneous. How are clinicians currently managing the treatment of MDS patients? What are the key drivers for prescribing in MDS? What are current barriers and challenges in the diagnosis and management of MDS?

- Resistance to hypomethylating agents (HMAs) remains an issue for higher-risk patients, while resistance to lenalidomide and ESAs remains an issue for lower-risk patients. Which treatments in clinical development are likely to address the needs of specific subsets of MDS patients,
such as HMA-refractory patients and lower-risk patients with a significantly reduced quality of life? What are key opinion leader (KOL) perceptions regarding the MDS pipeline? What percentage of lower-risk and higher-risk populations will be served by new treatment options by 2024 in the United States and Europe?

- Many unmet needs remain in MDS, spanning multiple areas. What are the new avenues of research and understanding of MDS pathophysiology? Which emerging therapies with new mechanisms of action are in clinical development? Which emerging therapies are poised to address unmet needs in the MDS market and see uptake in the next ten years? What are the opportunities for new product development? How can drug companies optimize drug development and capitalize on commercial opportunities in the MDS space?

Scope:

Market covered: United States, France, Germany, Italy, Spain, and the United Kingdom.

Primary research: Eight country-specific interviews with thought-leading hematologists.

Epidemiology: Diagnosed incident cases and diagnosed prevalent cases of MDS by country and by age. Diagnosed prevalent cases by morphology and by prognostic risk.

Emerging therapies: Phase II: 26; Phase III/PR: 5; detailed market analysis is provided for 8 agents.

Report Details

- Pub Date: November 2015
- Author(s): ["James T. Heeres, Ph.D.
   Jing Wu, M.S., M.B.A.
   Alison Isherwood, Ph.D., M.Res, M.Sc."]