Myelodysplastic syndrome

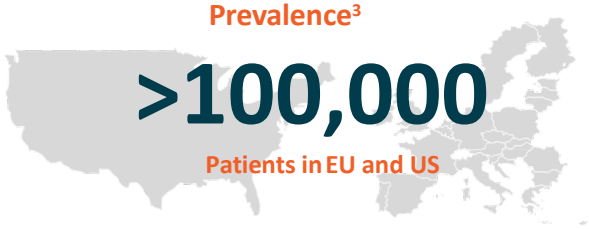
Group of clonal bone marrow neoplasms, generally affecting elderly patients, characterized by ineffective hematopoiesis which invariably leads to anaemia and other cytopenias.

Drug candidate
SLN124

Causes[1,2]

The exact causes of MDS are frequently unknown, but more often than not patients present with genetic alterations in the hematopoietic lineage. Mutations in some genes (e.g SF3B1, TP53) have been associated with MDS.

Prevalence[3]



>100,000

Patients in EU and US

Symptoms and Complications[2]

- > Chronic Fatigue
- > Shortness of breath
- > Bone problems
- > Easy bleeding (when thrombocytopenia is present)
- > Susceptibility to infections

Treatments[4]

Treatment encompasses drugs such as erythropoiesis stimulating agents and transfusions or bone marrow transplantation in severe cases. Transfusional iron overload is typically managed with badly tolerated chelators.

Advantages

SLN124 offers a safer effective and tolerable alternative for the treatment of anaemia and iron overload in this generally older patient population.

[1] https://www.mds-foundation.org/what-is-mds/
[2] Diagnosis and treatment of primary myelodysplastic syndromes in adults: recommendations from the European LeukemiaNet. Malcovati et al. Blood, 2013; 122(17): 2943–2964
[3] Incidence and Burden of the Myelodysplastic Syndromes. Cogle. Curr Hematol Malig Rep, 2015; 10(3):272-81
[4] First-line Therapeutic Strategies for Myelodysplastic Syndromes. Valeria Santini.Clinical Lymphoma, Myeloma & Leukemia,2017; 17S:S31-S36