Novartis sabatolimab receives orphan drug designation from the European Commission for myelodysplastic syndromes

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Novartis announced today that the European Commission (EC) has granted orphan drug designation to sabatolimab (MBG453) for the treatment of myelodysplastic syndromes (MDS), based on clinical data showing a high rate of responses in patients with high-risk MDS who were treated with sabatolimab in combination with hypomethylating agents (HMAs). The decision follows a positive opinion from the Committee for Orphan Medicinal Products (COMP) of the European Medicines Agency (EMA)[1].

- MDS are a group of rare and often underdiagnosed blood cancers characterized by two major components, a dysfunctional immune system and leukemic stem cell proliferation, which limits the ability of current treatment options to achieve durable responses[2-4].
- Patients with MDS face the possibility of poor outcomes with a limited duration of response and a median overall survival rate of less than two years despite treatment with the current standard of care[5,6].
- Sabatolimab is an investigational, potential first-in-class immuno-myeloid therapy that binds to TIM-3, a novel target expressed on immune and leukemic cells, but not on normal hematopoietic stem cells. It is in development for the treatment of higher-risk MDS and acute myeloid leukemia (AML). Targeting TIM-3 reawakens the immune system to selectively attack leukemic stem cells, the source of MDS/AML, and has the potential to safely deliver a durable response[7,8].

Orphan drug designation is reserved for medicines that treat, prevent or diagnose a life-threatening or chronically debilitating rare disease with a prevalence in the EU of below 5 in 10,000 and with either no currently approved method of diagnosis, prevention or treatment or with significant benefit to those affected by the disease[9]. At the time of the EMA's evaluation of the marketing authorization application for sabatolimab, the COMP will determine whether the orphan designation can be maintained based on an analysis of available data[1].

The EC decision follows the US Food and Drug Administration's fast track designation for sabatolimab in May 2021, for the treatment of adult patients with MDS defined with an IPSS-R risk category of high- or very high-risk in combination with HMAs[1].

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9. European Medicines Agency. Orphan Designation Overview. Accessed August 2021. Available at: https://www.ema.europa.eu/en/human-regulatory/overview/orphan-designati…

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