EUROPEAN HEMATOLOGY ASSOCIATION

EHA2021 VIRTUAL

S156

ELTROMBOPAG FOR MYELODYSPLASTIC SYNDROMES OR CHRONIC MYELOMONOCYTIC LEUKEMIA WITH NO EXCESS BLASTS AND THROMBOCYTOPENIA: A FRENCH MULTICENTER RETROSPECTIVE REAL-LIFE STUDY.

Topic: 10. Myelodysplastic syndromes - Clinical

Keywords: Myelodysplasia, Thrombocytopenia, Thrombopoietin (TPO)

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Background:

Anemia is generally the predominant cytopenia in Low-Risk Myelodysplastic syndromes (LR-MDS) but thrombocytopenia may predominate in 10 to 15% of these patients. Options to improve thrombocytopenia are limited but recently, two thrombopoietin receptor agonists, romiplostim and eltrombopag (ELT) have been evaluated in clinical trials and yield 30 to 50% platelet responses in relatively selected LR-MDS patients. Only two clinical trials, have been performed in LR-MDS with ELT monotherapy and one in CMML.

Aims:

The main objective of this study was to assess the efficacy and safety profile of ELT in thrombocytopenia related to MDS and CMML with no excess blasts (EB), in a “real life” context.

Methods:

We collected data from MDS and CMML patients treated by ELT between January 2011 and January 2019 and included in the 'Groupe Francophone des Myélodysplasies' (GFM) registry of MDS patients. Inclusion criteria were (1) MDS or chronic myelo-monocytic leukemia with leucocytes < 13 G/L (according to WHO 2016 classification)(2) less than 5% bone marrow blasts (3) treatment with ELT outside of a clinical trial (4) platelet count ≤ 50.10^9/L at the onset of ELT. The primary endpoint was platelet response (HI-P), according to International Working Group (IWG 2006) response criteria. We also reported other hematological responseswhen available, safety and tolerability, bleeding and thrombotic events. This study has been approved by institutional review board, in accordance with the French data protection authority (MR004, Commission Nationale de l'Informatique et des Libertés, CNIL)

Results:

Platelet response occurred in 38 (76%) MDS patients and 9 (82%) CMML. Median (IQR) duration of response was 13 (4-24) months and 6 (4-11) months in MDS or CMML patients respectively. Three MDS and 3 in CMML were still responders 6 to 23 months after ELT discontinuation. Although 34% MDS and 46% CMML were anticoagulated or anti aggregated, only 10% patients experienced ≥ grade 3 bleeding events (6% in responders vs 21% in non-responders). Thrombotic events were observed in 6 (12%) MDS patients, who all but one had a medical history of arterial or venous thrombosis. Progression to AML occurred in 4 (7%) patients.

Summary/Conclusion:

We reported for the first-time real-life data about ELT in LR-MDS and CMML patients with thrombocytopenia. We confirmed that ELT is effective with a sustained effect on platelet counts reduction in the platelet transfusion burden and in severe bleeding. The effect of ELT was often prolonged after drug discontinuation. The tolerance profile was favorable in this population with no excess of blast, but previous history of thrombosis remains a major risk factor for thrombotic events on treatment.

Abstract Book Citations: Authors, Title, HemaSphere, 2021;5:(S2):pages. Abstract Book, DOI:
http://dx.doi.org/10.1097/HS9.0000000000000566