Abstract: P1628

Title: PROLONGED RESPONSE AFTER TPO-RA DISCONTINUATION IN PRIMARY ITP: LONG TERM FOLLOW-UP OF THE STOPAGO STUDY, A PROSPECTIVE MULTICENTER STUDY

Abstract Type: Poster Presentation

Topic: Platelet disorders

Background:

Thrombopoietin receptor agonists (TPO-RAs) are effective treatments for immune thrombocytopenia (ITP). Because of their mechanism of action, TPO-RAs have long been considered as a supportive therapy. Recently, several studies have suggested that TPO-RAs may induce prolonged remissions, but their study population included heterogenous patients in whom spontaneous remission may occur regardless of the initial treatment. In this setting, the French prospective multicenter STOPAGO study showed a sustained response rate of 50% after TPO-RAs discontinuation among selected patients with chronic ITP who achieved a stable complete response for one year on TPO-RAs. Most relapses occurred within four weeks after TPO-RAs discontinuation. No major bleeding events were reported, and TPO-RAs rechallenge resulted in complete response in 90% of these patients.

Aims:

The objective of this work is to update the follow-up of patients enrolled in the STOPAGO study with long-term monitoring.

Methods:

The STOPAGO study (#NCT03119974) was an open prospective, multicenter, interventional study involving 20 centers from the French ITP reference center network. Patients were adults with a persisting or chronic ITP who achieved a stable complete response (platelet count > 100 × 109/L for > 2 months) on TPO-RAs (eltrombopag or romiplostim) for more than 3 months. After enrolment, TPO-RAs were gradually tapered and discontinued according to a standardized procedure within 10 weeks. Sustained response off treatment (SROT) was defined as platelet count ≥30 × 109/L and no bleeding. Sustained complete response off treatment (SCROT) was defined as a platelet count ≥100 × 109/L and no bleeding without ITP-specific medications. Updated long-term follow-up data after the last inclusion were collected by the investigators of the participating centers.

Results:

Between September 2017 and February 2020, 48 patients were enrolled in the STOPAGO study. SROT and SCROT were achieved in 27/48 (56%) and 15/48 (31%) patients at 6 months, and 25/48 (52%) and 14/48 (29%) patients at 12 months, respectively. The 25 patients who achieved SROT at 12 months were followed for a median of an additional 5 years (ranging from 3.3 to 6.3 years). Of these, all but 2 patients received eltrombopag for a median of 2.1 [1.1-4.1] years prior discontinuation. SROT and SCROT were achieved in 22/46 (48%) and 17/46% (37%) patients at 4 years, respectively (2 patients lost to follow-up after 3 years). Only 2 (8%) patients relapsed during extended follow-up. No severe bleeding events were reported at the time of relapse. The first patient relapsed 5 years after eltrombopag discontinuation. Complete response was achieved within 5 weeks after eltrombopag rechallenge. The second patient relapsed 2 years after eltrombopag discontinuation. This relapse was associated with an influenza virus infection. Eltrombopag rechallenge and 3 other treatment lines were ineffective, leading to the recent introduction of fostamatinib. One pregnancy (without relapse or neonatal thrombocytopenia), one SARS-CoV-2 infection, one small-cell B lymphoma and one scleroderma without ITP relapse were reported during follow-up.

Summary/Conclusion:

Our results show that almost 50% of patients with chronic ITP who achieved a stable complete response with TPO-RAs maintained a sustained response during long-term follow-up after TPO-RAs discontinuation. These results confirm that relapses are predominantly early in the first weeks after discontinuation, while late relapses are rare (2 of 25 patients). These findings support a discontinuation strategy of TPO-RAs in patients with chronic ITP in stable complete remission.

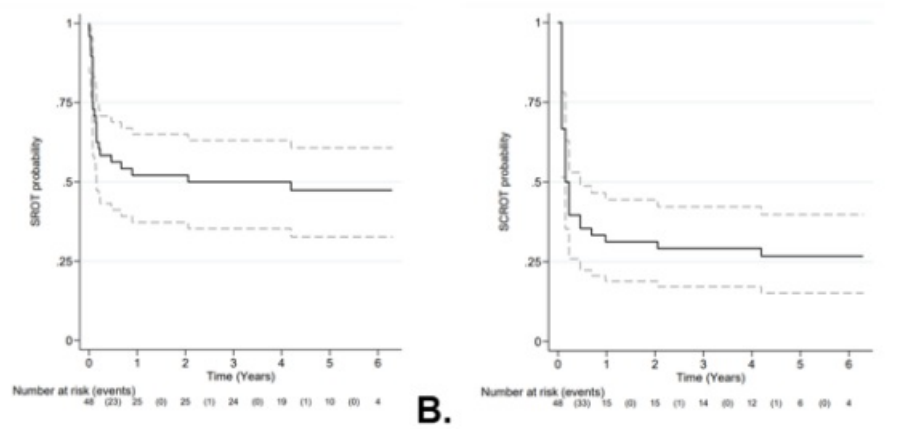

Figure 1. Relapse-free probability of SROT and SCROT after TPO-RA discontinuation. Kaplan-Meier curves for relapse-free probability of SROT (A) and SCROT (B) after TPO-RA discontinuation (W0).

Keywords: Immune thrombocytopenia (ITP), TPO