ELSEVIER

Blood 142 (2023) 1209–1210

The 65th ASH Annual Meeting Abstracts

POSTER ABSTRACTS

311.DISORDERS OF PLATELET NUMBER OR FUNCTION: CLINICAL AND EPIDEMIOLOGICAL

Evaluation of the Efficacy and Safety during Treatment with TPO-Ras in 40 SLE-ITP and APS-ITP Patients

Marques Cindy[1], *Mickael Roussotte*[2], *Guillaume Moulis*[3], *Bernard Bonnotte, MD PhD*[4], *Jean-Francois Viallard, MDPhD*[5], *Nathalie Costedoat-Chalumeau*[6], *Alban Deroux*[7], *Pascal Sève*[2], *Bertrand Godeau*[8], *Matthieu Mahevas, MD PhD*[8], *Marc Michel*[9]

[1] Department of Internal Medicine, French Referral Centre for Adult Immune Cytopenia, Henri Mondor Hospital, AP-HP, UPEC University, Créteil, Créteil, France
[2] Department of Internal Medicine, Hospices Civils de Lyon, Lyon, Lyon, France
[3] Toulouse University hospital, Toulouse, France
[4] Faculty of Medicine - University Hospital of Dijon, Dijon, FRA
[5] Service Médecine Interne, Haut-Leveque Hospital, Pessac, France
[6] Service de médecine interne, APHP, Hôpital Cochin, Paris, France
[7] Service De MéDecine Interne, CHU Grenoble-Alpes, La Tronche, FRA
[8] Internal medicine department, Henri Mondor Hospital, APHP, Créteil, France
[9] Department of Internal Medicine, National Reference Center for Immune Cytopenias, Henri-Mondor University Hospital, Assistance Publique-Hôpitaux de Paris, Université Paris-Est Créteil, Creteil, France

Objectives: Thrombopoietin receptor agonists (TPO-RAs) have emerged over the last decade as a therapeutic option in primary immune thrombocytopenia (ITP). TPO-RAs have demonstrated their efficacy in more than 70% of cases and are taking an increasingly important place in second line therapy. However, to date, no prospective cohort has been able to question the efficacy and safety of TPO-RAs in systemic lupus erythematosus (SLE-ITP) and antiphospholipid syndrome (APS-ITP) patients, particularly regarding venous and arterial thrombosis risks in this population.
Methods: This multicenter retrospective cohort study was conducted from 2012 to 2023 in seven hospitals belonging to the French national network for adult ITP. We assessed the efficacy and the tolerance of TPO-RAs on a large retrospective cohort with a long follow-up of patients with ITP associated with 1) SLE or 2) primary or secondary APS or 3) with antinuclear antibodies (ANA) and anti-dsDNA or anti-Sm antibodies or 4) with ANA and at least one antiphospholipid antibody (APA) without thrombotic events (TEs). We studied the efficacy according the international criteria of response (Rodeghiero *et al*, Blood 2009) and the incidence of TEs, the risk factors for the occurrence of thrombosis, the management of TPO-RAs and their combination with antiplatelet agents or anticoagulants.
Results: We included 40 ITP patients treated with TPO-RAs; 28 SLE-ITP, four APS-ITP, four with both SLE and APS, and four with significant ANA and APA positivity without definite SLE or APS. Five patients were triple positive for APA. The median duration of follow up was 46 months (1-132). The overall response rate was 84.2% for patients treated with romiplostim and 71% for those treated with eltrombopag after failure of standard treatments such as corticosteroids, intravenous immunoglobulins, rituximab and immunosuppressive agents. Eight TEs occurred in seven (17.5%) patients at a median of 119 days (IQR=171.5) after eltrombopag (n=4) or romiplostim (n=4) initiation with a median exposure duration of 37 days (13-119) for romiplostim and 255 days (30-807) for eltrombopag. Two venous TEs were observed including one cerebral vein thrombosis and one pulmonary embolism. We observed six arterial thrombotic events, such as myocardial infarctions and ischemic strokes including one fatal catastrophic APS. Four of these patients had a history of APS, and most had thrombotic risk factors and positive APA. TPO-RAs were continued after TEs in two patients for a total duration of 20 and 53 months in association with an antiplatelet agent for one patient and a combination of an antiplatelet agent and an anticoagulant for the second one without a new TE thereafter. TPO-RAs were transiently stopped and then reintroduced due to ITP flare in two patients. A new episode of myocardial infarction occurred in one of them despite association with antiplatelet agents requiring TPO-RAs definitive arrest.
Conclusion: We confirmed the effectiveness of TPO-RAs in over 70% of SLE-ITP and APS-ITP patients who failed to respond to corticosteroids, intravenous immunoglobulins, rituximab and/or standard immunosuppressive agents. Nevertheless, these findings highlight the need to carefully consider the potential benefits and thrombotic risks before initiating TPO-RAs, especially in elderly patients with thrombotic risk factors and positive APA or a confirmed APS. Further studies are needed

to evaluate more precisely the TEs risk factors of these agents in this specific population, in order to better select SLE and APS-ITP patients who could benefit from them.

Disclosures Moulis: *Novartis:* Honoraria, Research Funding; *Sanofi:* Honoraria, Research Funding; *Amgen:* Honoraria, Research Funding; *Argenx:* Honoraria; *Grifols:* Honoraria, Research Funding. **Viallard:** *EUSAPHARMA:* Consultancy. **Godeau:** *Novartis:* Honoraria; *Amgen:* Honoraria; *Grifols:* Honoraria; *Sobi:* Honoraria. **Mahevas:** *Amgen:* Honoraria; *Novartis:* Honoraria; *Sanofi:* Research Funding. **Michel:** *Alexion:* Consultancy; *Sobi:* Consultancy; *Sanofi:* Consultancy; *argenx:* Honoraria; *UCB:* Honoraria; *Novartis:* Consultancy.

https://doi.org/10.1182/blood-2023-187461