



The 65th ASH Annual Meeting Abstracts

POSTER ABSTRACTS

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

Eltrombopag Plus Rhtpo for Corticosteroid-Resistant or Relapsed ITP: A Prospective Observed StudyHaixia Fu, MD¹, Qiusha Huang², Yun He^{3,4}, Chen-Cong Wang³, Kaiyan Liu³, Xiaohui Zhang³¹ Peking University People's Hospital, Peking University Institute of Hematology, Beijing, Beijing, China² Peking University People's Hospital, Beijing, China³ Peking University People's Hospital, Peking University Institute of Hematology, Beijing, China⁴ Peking University People's Hospital, Beijing, China

During the COVID-19 pandemic, the classical subsequent treatment regimen for immune thrombocytopenia (ITP) of immunosuppressants and/or steroids might increase patients' susceptibility of virus infections. To minimize ITP patients' risk during the COVID-19 global crisis and to improve treatment efficacy, non-immunosuppressive therapies, mainly including thrombopoietin mimetics, such as eltrombopag or recombinant human thrombopoietin (rhTPO), may be better for them. Eltrombopag, a small molecule agonist of thrombopoietin receptor (TPO-RA), was recommended as the subsequent treatment for ITP patients, which also already showed robust efficacy. However, inefficacy, loss of response and fluctuation of platelet counts during treatment were the serious problems in eltrombopag treatment for corticosteroid-resistant or relapsed ITP. RhTPO is a full-length glycosylated-TPO produced by Chinese hamster ovary cells, which showed its effectiveness in ITP in a variety of studies. For the difference of rh-TPO and eltrombopag, it had distinct molecular structures, treatment mechanisms, and pharmacokinetic characteristics and thus different response patterns and side effect profile. Previous studies have confirmed the safety and effectiveness of switching between rh-TPO and eltrombopag for ITP patients who had no response to or experienced adverse events with their first TPO-RA. However, no study has focused on the safe and effectiveness of the combination therapy of eltrombopag and rhTPO. The investigators hypothesized that the combination of these two agents could be a promising option for ITP treatment.

Methods

This is a prospective observed study to investigate the efficacy and safety of eltrombopag plus rhTPO as treatment for corticosteroid-resistant or relapsed ITP during the COVID-19 pandemic.

Participation Criteria

The inclusion criteria were as follows: Clinically confirmed corticosteroid-resistant or relapsed primary ITP; Platelet count less than $30 \times 10^9/L$ on two occasions or platelets above $30 \times 10^9/L$ combined with bleeding manifestation (WHO bleeding scale 2 or above); ≥ 18 years.

Exclusion Criteria: with secondary ITP such as drug-related thrombocytopenia, viral infection-induced thrombocytopenia, or a known diagnosis of another autoimmune disease; with other possible causes of thrombocytopenia, such as leukaemia, myelodysplastic syndrome, and aplastic anaemia; impaired renal function as indicated by a serum creatinine level > 2.0 mg/dL; inadequate liver function as indicated by a total bilirubin level > 2.0 mg/dL and/or an aspartate aminotransferase or alanine aminotransferase level $> 3 \times$ upper limit of normal; with a New York Heart Classification III or IV heart disease; with a history of severe psychiatric disorder or are unable to comply with study and follow-up procedures; pregnant or lactating women, or plan to become pregnant or impregnated within 12 months of receiving study drug; previous splenectomy; with previous or concomitant malignant disease; expected survival of < 2 years; not willing to participate in the study.

Procedure

For the patients with the combination therapy of eltrombopag and rhTPO, they received eltrombopag 25-75 mg oral daily and rh-TPO 300U/kg subcutaneous injection once daily for 7 consecutive days, followed by a tapering dose in maintenance therapy to maintain the platelet count between $50-150 \times 10^9/L$.

Results

From September 2nd, 2020, to July 1st, 2023, a total of 29 patients with corticosteroid-resistant or relapsed ITP were enrolled to receive eltrombopag plus rhTPO treatment, which included 11 male and 18 female patients. The median age were 55 (23-91) years old. The baseline platelet count was $7 (1-17) \times 10^9/L$. Most patients (69.0%) were with severe ITP (platelet counts $\leq 10 \times 10^9/L$ or bleeding score ≥ 5). Response was defined as the achievement of platelet counts $\geq 30 \times 10^9/L$ and ≥ 2 times the baseline

platelet count at least once with treatment. The response rates at 1 week (early response) and 1 month (initial response) were 37.9%(11/29) and 62.1%(18/29), respectively. After 6 weeks' treatment, 82.8% (25/29) of patients achieved response, and the improvement of bleeding symptoms were observed in most patients (89.7%). No thromboembolic events occurred during treatment.

Conclusion

Elrtoombopag and rhTPO was a safe and effective therapy for corticosteroid-resistant or relapsed ITP, especially for severe ITP.

Disclosures No relevant conflicts of interest to declare.

<https://doi.org/10.1182/blood-2023-186682>