



The 65th ASH Annual Meeting Abstracts

POSTER ABSTRACTS

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

Real-World Evidence of Avatrombopag for the Treatment of Immune Thrombocytopenia Intolerant or Ineffective to Eltrombopag/HetrombopagHong Tian¹, Yun Li¹, Xin Lv¹, Lu Zhou², Depei Wu, MD³, Ziqiang Yu⁴, Jie Yin, MD PhD¹¹The First Affiliated Hospital of Soochow University, Suzhou, China²Affiliated Hospital of Nantong University, Nantong, China³The First Affiliated Hospital of Soochow University, SUZHOU, China⁴National Clinical Research Center for Hematologic Diseases, Jiangsu Institute of Hematology, First Affiliated Hospital of Soochow University, Suzhou, China**Abstract****Objectives:**

Thrombopoietin receptor agonists (TPO-RA) have dramatically changed the treatment landscape for immune thrombocytopenia (ITP). Although avatrombopag has been confirmed to achieve platelet response for chronic ITP in clinical trials, and it has no hepatotoxicity unlike eltrombopag or hetrombopag, there is still a lack of real-world research on the application of avatrombopag in Chinese ITP patients. Here, we aimed to evaluate the effect and safety of avatrombopag in ITP treatment in a real-world clinical practice.

Methods:

In this retrospective analysis, we enrolled consecutive cohorts of 50 adult ITP patients who received avatrombopag in two centers from January 2021 to March 2023. The median duration of follow-up of these patients was 16.5 months (range from 3.8 to 25.4 months).

Results:

The median age of patients was 44 years when they received the treatment of avatrombopag, and 42% of them were males. Most patients (n=45) had a persistent (3-12 months) or chronic course (more than 12 months) of ITP. The patients in our study were heavily treated, who received a median of 4 kinds of ITP treatment prior to avatrombopag. The baseline TPO levels was 88.1 (110.2±84.5) pg/ml. Based on the reasons for choosing avatrombopag, these patients were divided into eltrombopag/hetrombopag intolerance group (IG, n=14) and eltrombopag/hetrombopag unresponsive group (UG, n=36). The reasons for choosing avatrombopag in IG included active hepatitis B (n=4), hepatic insufficiency before using TPO-RA (n=6), autoimmune hepatitis (n=1), hepatic lesion after using eltrombopag (n=2), and myelofibrosis after using eltrombopag (n=1). Compared with UG, more patients had a history of liver disease (active Hepatitis B and autoimmune liver diseases, P=0.003). The median platelet count before avatrombopag was $9 \times 10^9/L$. The median duration of avatrombopag treatment was 12 weeks and the median weekly dose of avatrombopag was 140 mg. Among the total cohort, platelet response (platelet count $\geq 30 \times 10^9/L$) was achieved in 44/50 (88%) and complete platelet response (platelet count $\geq 100 \times 10^9/L$) was seen in 35/50 patients (70%) after using avatrombopag. The median time of platelets rising above $30 \times 10^9/L$ was 7 days, while it was 8 days when platelet count exceeded $50 \times 10^9/L$. Nearly half patients (47%) were able to discontinue more than 1 concomitant ITP medication after avatrombopag treatment. Besides, most patients (74%) in our study tapered off or withdrew steroids. There were no significant differences in baseline platelet count, 30-day response rate, the maximum effective dose of avatrombopag and duration of avatrombopag between IG and UG.

So far, 19 patients achieved platelet response were still taking avatrombopag, and 58% of them maintained platelet count above $100 \times 10^9/L$. Meanwhile, 31 patients discontinued avatrombopag. The reasons for withdrawal of drug included adverse events (n=2), sustained remission in ITP (n=8), no/lose response to avatrombopag (n=10), and inability to afford the cost consistently (n=11).

Avatrombopag was well tolerated in most patients, even those suffered from hepatic insufficiency and/or myelofibrosis after other TPO-RA. However, two cases had thrombosis and two patients reported mild fatigue.

Conclusion:

In the ITP population, avatrombopag was effective and safe, especially for those intolerant or ineffective to eltrombopag/hetrombopag. However, most patients with ITP were not able to afford the long-term usage of avatrombopag in China.

Disclosures No relevant conflicts of interest to declare.

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