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POSTER ABSTRACTS

508.BONE MARROW FAILURE: ACQUIRED

Avatrombopag Is Effective in First-Line Treatment of Severe Aplastic Anemia: Early Outcomes of a Prospective Study

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Acquired severe aplastic anemia (SAA) is a life-threatening bone marrow failure disease and its pathogenesis involves T cell-mediated autoimmunity. Antithymocyte globulin (ATG) and cyclosporine based immunosuppressive therapy (IST) is effective for patients not eligible for hematopoietic stem cell transplant, and the response rate is 60-70%. Complete hematological response (CR) predicts better survival of patients received IST treatment, but only a few patients achieve CR within 6 months (5% at 3 months and 12-17% at 6 months). Thrombopoietin receptor agonist (TPO-RA) eltrombopag improves the overall response (OR) rate and CR rate of SAA patients when combined with IST, but its hepatotoxicity is still worrying.

Avatrombopag is another small-molecule TPO-RA without hepatic toxicity. We previously found that SAA patients received avatrombopag and IST as first-line treatment showed both higher CR rate and OR rate at 3 and 6 months. To validate our findings, a prospective, single-arm study of avatrombopag and IST for newly diagnosed SAA was carried out (NCT05720234). There were 25 patients enrolled in the study by June 31, 2023, including 21 SAA patients and 4 very severe aplastic anemia patients. Patients were diagnosed as AA within 6 months and received ATG, cyclosporine and avatrombopag as first-line treatment. Avatrombopag was administered at the first day of ATG initiation at dose of 60mg per day for patients weight $\geq$ 50kg and 40mg per day for patients weight $<$ 50kg. ATG was given for 5 consecutive days. Cyclosporine was given orally at dose of 3.5-5mg/kg.d.

There were 15 males and 10 males, at a median age of 37 years old. Eighteen patients achieved the end-point of 3 months and 10 patients achieved end-point of 6 months. There were 15 patients achieved hematologic response at 3 months (83.3%), including 5 complete responders (27.8%) and 10 partial responders. The overall response rate at 6 months was 80%, and CR rate was 60%. One patient evolved to hemolytic paroxysmal nocturnal hemoglobinuria at 3 months, with normal count of platelet and white blood cell.

In conclusion, the addition of avatrombopag to IST as first-line treatment showed earlier hematologic response and higher complete response rate for patients with SAA.

Disclosures No relevant conflicts of interest to declare.

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