Effectiveness and safety of eltrombopag in the first-line therapy of severe aplastic anemia in children

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Abstract

Background: Eltrombopag (E-PAG) is being investigated for the treatment of aplastic anemia (AA) by stimulating hematopoietic stem cell (HSC) proliferation. Objective: To evaluate the effectiveness and safety of E-PAG in first-line therapy for pediatric AA. Methods: The present retrospective study reviewed the pediatric patients with newly diagnosed AA in immunosuppressive therapy (IST) therapy with E-PAG at the single center from March to September in 2017. All patients were followed up for >2 years. Results: A total of 14 patients (8 males), aged 86 months, were enrolled in this study. E-PAG was administered with a median time to initiation of 19.5 days after IST, and the median course of treatment was 253 days. The rate of complete response and overall response at 6 months were 64.3% (9/14 case) and 78.6% (11/14 cases) respectively. The survival rate was 100%, and no relapses occurred in responders. E-PAG was well-tolerated; however, the most common adverse events included indirect bilirubin elevation, jaundice, and transient liver-enzyme elevation. By the end of follow-up, bone marrow chromosomes were normal, and no abnormal myelodysplastic syndromes (MDS) -related clones appeared. Conclusions: The addition of E-PAG to IST was associated with the markedly increased complete response with respect to hematology in pediatric patients with SAA than in a historical cohort without the unacceptable side effects.

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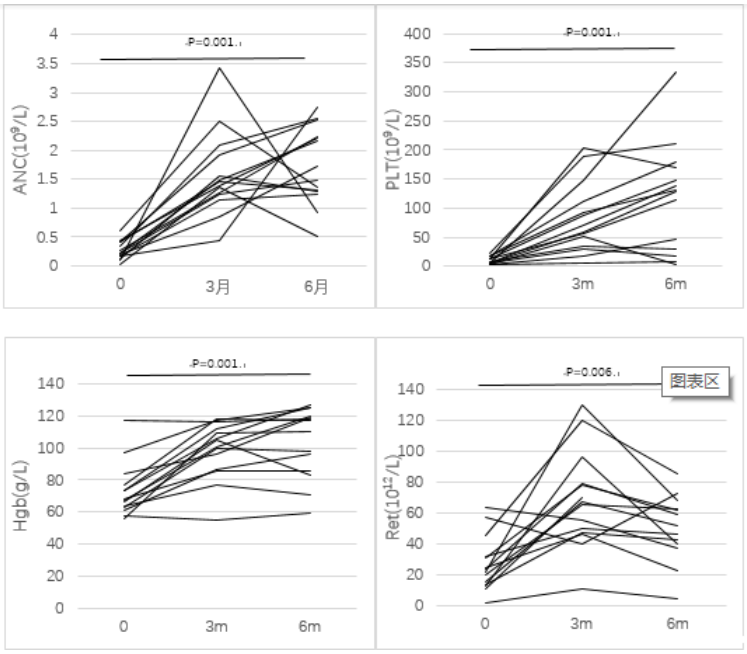

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