Abstract: PB3159

Title: THE EFFICACY AND SAFETY OF AVATROMBOPAG IN PEDIATRIC PATIENTS WITH THROMBOCYTOPENIA AFTER ALLOGENEIC HEMATOPOIETIC STEM-CELL TRANSPLANTATION: CASE REPORTS

Abstract Type: Publication Only

Topic: Stem cell transplantation - Clinical

Background:

Thrombocytopenia following allogeneic hematopoietic stem cell transplantation (allo-HSCT) is a common and serious complication that leads to an increased risk of bleeding and poor prognosis. Traditional strategies consist of platelet transfusion, glucocorticoid therapy, intravenous human immunoglobulin, and recombinant human thrombopoietin (rhTPO) injection, but the effects of these treatments are not satisfactory and the treatment continues to be challenged. Thus, improved prevention and treatment strategies for post-HSCT thrombocytopenia are urgently required. In recent studies, thrombopoietin receptor agonists (TPO-RA) for treating post-HSCT thrombocytopenia indicated efficacy and safety. Avatrombopag is a new TPO-RA, which is effective and safe on increasing platelet counts in adult patients with thrombocytopenia after allo-HSCT. However, there was few relevant study in children. Herein, we retrospectively analyzed the efficacy and safety of avatrombopag in pediatric patients with thrombocytopenia after allo-HSCT.

Aims:

This study aims to determine the efficacy of avatrombopag, which is a novel thrombopoietin receptor agonist, on thrombocytopenia after allo-HSCT in children patients.

Methods:

Four patients with thrombocytopenia after allo-HSCT underwent avatrombopag treatment were retrospectively studied. Of these patients, two patients had delayed platelet engraftment (DPE) ,one patient had secondary failure of platelet recovery (SFPR), and another patient had immune thrombocytopenia. The efficacy and safety of the treatment were assessed.

Results:

Four children (two boys and two girls, respectively) with a median age of 7.9 (range: 1.6– 13.8) years were included. The platelet count of all patients reached the complete response (the complete response was defined as the platelet ≥50×109/L for at least 7 consecutive days without transfusion) standard after drug withdrawal. Alanine aminotransferase (ALT) increased in one patient, and no patients stopped treatment due to adverse reactions or drug intolerance.

Summary/Conclusion:

Avatrombopag is effective on increasing platelet counts in pediatric patients with thrombocytopenia after allo-HSCT, with a superior safety profile. It is an efficient and safe drug for treating post-HSCT thrombocytopenia in children.

Keywords: Children, Allogeneic stem cell transplant, Thrombocytopenia