

components of red cell membranes. It is characterized by anemia, jaundice, splenomegaly and cholelithiasis. The clinical manifestations vary widely, ranging from nearly asymptomatic to transfusion-dependent or severe life-threatening anemia. It is difficult to identify atypical cases with classical approaches. The known HS gene mutations are SPTA1 gene, SPTB gene, ANK1 gene, SLC4A1 gene and EPB42 gene. In this report, the next-generation sequencing (NGS) was used to analyze our patients with HS and we identified mutations responsible for HS.

**Methodology:** Patients who were diagnosed with hereditary spherocytosis with osmotic fragility testing between 2007–2019; ten were further tested for molecular background. Diagnosed in our center were analyzed retrospectively. Either NGS or ANK1 Sanger testing were used.

**Results:** The 10 cases of HS comprised 8 males and 2 females. The age of patients ranged from 5 months to 17 years. Hemolytic anemia, jaundice and splenomegaly were the most common findings in our cases. Gallstones were detected in four patients (40%). The family history was positive in 5 (50%) patients. Splenectomy and cholecystectomy was performed in two cases and three cases, respectively. The results confirmed ANK1 gene mutation in 50%; SPTB gene mutation in 20%, EPB42 gene mutation in 10%; SPTA1 gene mutation in 10%. The clinical features of the patients are summarized in the Table 1. Table 1. Patient Age Sex Age of diagnosis Family history Splenomegaly Gallstone Splenectomy/Cholecystectomy Mutated gene

1	1	Female	1 year	Yes	+	-	-	-	SPTB
2	10	Female	10 years	Yes	+	-	+/-	-	ANK1
3	12	Female	7 years	Yes	+	+	+/+	-	ANK1
4	12	Female	2 years	Yes	-	+	-/+	-	ANK1
5	10	Male	6 years	No	-	+	-/-	-	ABCG8
6	2	Female	5 months	No	+	-	-/-	-	ANK1
7	13	Female	15 years	Yes	+	-	-/-	-	ANK1
8	8	Female	7 years	No	+	-	-/+	-	EBP42
9	2.5	Male	2 years	No	+	-	-/-	-	SPTB
10	19	Female	17 years	No	+	-	-/-	-	SPTA1

**Conclusion:** Consistent with the literature, the most common gene mutated was ANK1. Collectively, our results suggest that mutation analyses will complement other conventional tests for accurate diagnosis of HS, especially in those who are under transfusion programme and are followed with a diagnosis of unspecified hemolytic anemia.

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## STEM CELL TRANSPLANTATION

### OP 26

#### Eltrombopag for thrombocytopenia following pediatric allogeneic hematopoietic stem cell transplantation



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**Objective:** Failure of platelet recovery is a complication occurring after allogeneic hematopoietic stem cell transplantation (HSCT). Poor graft function, relapse, viral infections, drug toxicity, immune processes may lead to decreased platelets production. Treatment options are limited for thrombocytopenia caused by poor platelet production. While the

use of Eltrombopag (ELT) was retrospectively investigated in adult patients, data regarding the potential benefit of these agent for pediatric posttransplant thrombocytopenia are lacking. We report three pediatric patients who received ELT for thrombocytopenia occurring after HSCT.

**Case report:** The median patients' age at HSCT was 13.3 years (10–18). All patients had HSCT from a sibling donor with the bone marrow stem cell source. All patients were treated with a myeloablative conditioning regimen. Patients engrafted at a median time of 19 days (10–24) for neutrophils and 49 days (44–49) for platelets. Bone marrow aspirates showed a decrease number of megakaryocytes, and all patients had been ineffectively treated with high-dose intravenous gamma globulin and with steroids before ELT initiation.

**Methodology:** ELT was started at a median time after HSCT of 57 days (42–90), the starting dose being 25 mg/day, and the maximum administered dose was 75 mg/day. ELT was continued for a median period of 64 days (28–286). All patients reached sustained platelets count >50,000/ $\mu$ L after a median time from starting ELT of 197 days (87–210). The median platelet count at last evaluation was 115,000/ $\mu$ L (range 66,000–125,000/ $\mu$ L). ELT was well tolerated, and no patient have developed important side effect.

**Results:** Our cases became transfusion independent after a median time from starting ELT of 197 days. In the pediatric post-HSCT setting, only few previously published case reports described the successful use of ELT as a treatment for thrombocytopenia. Li et al. reported three children transplanted for nonmalignant disease treated for both primary and secondary failure of platelet engraftment. Treatment was effective in two patients, but not in one patient transplanted for Gaucher disease. In Masettis' study, the 60-day cumulative incidence of platelet recovery >50,000/ $\mu$ L after ELT treatment was 75%. Similarly, Tanaka et al. described 12 adults treated for primary and secondary post-HSCT thrombocytopenia who reached platelet count >50,000/ $\mu$ L in 60% and 71% of cases respectively.

**Conclusion:** Our study supports the safety and efficacy of ELT for treatment of prolonged thrombocytopenia after allogeneic HSCT in children. Future prospective studies are needed to confirm these findings.

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