



CLINICAL OUTCOMES IN PEDIATRIC PATIENTS WITH SEVERE APLASTIC ANEMIA TREATED WITH THAI GUIDELINE

NACHAPOL JATUTEN, BUNCHOO PONGTANAKUL

Division of Hematology and Oncology, Department of Pediatrics, Faculty of Medicine Siriraj Hospital, Mahidol university

Introduction

Severe aplastic anemia is a rare serious condition observed in Thailand, Thai guidelines recommended treatment include matched sibling hematopoietic stem cell transplantation and immunosuppressive therapy in combination with eltrombopag. However, there are no reports regarding the treatment outcomes of using eltrombopag combined with immunosuppressive therapy specifically in pediatric patients in Thailand.

Objectives

To investigate the clinical outcomes of pediatric patients with severe aplastic anemia who received treatment according to aplastic anemia Thai guideline.

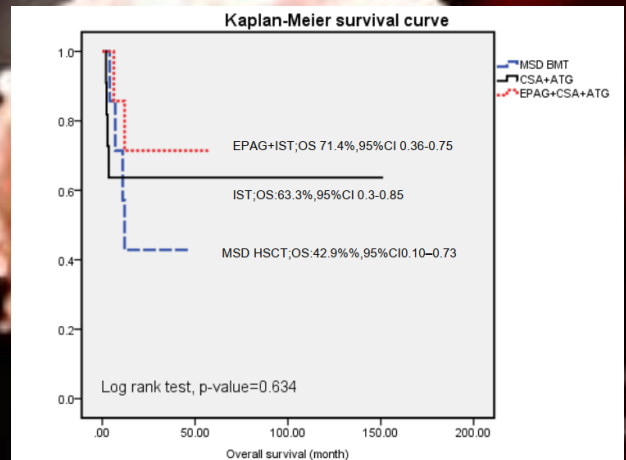
Methods

This study is a retrospective study of pediatric patients with severe aplastic anemia who received treatment at Siriraj Hospital from January 1, 2013, to December 31, 2023

Results

- At three months, the IST + EPAG group exhibited a statistically significant partial response in 4 patients (57.1%) compared to the IST group ($p = 0.015$).
- By six months, the IST + EPAG group continued to show a significantly better response ($p = 0.036$).
- Overall survival rates were better in the IST+EPAG group at 71.4% (95% CI: 0.36-0.75) compared to the IST group at 63.3% (95% CI: 0.30-0.85).
- The overall survival rate for MSD HSCT was 42.9% (95% CI: 0.10-0.73)

Characteristics	IST(n=13)	IST+EPAG(n=7)	P value
Time to initial treatment (day) (mean±SD)	48.5 ± 28.7	126.4 ± 78.1	0.004
Time course of IST therapy (month) (mean±SD)	16.1 ± 11.8	16.1±6.3	0.237
Time course of EPAG (month) (mean±SD)	-	7.8 ± 4.9	-
Max dose of EPAG ,median (IQR)	-	125 (100-150)	-
3 month response			
CR (%)	0	0	
PR (%)	1 (7.7 %)	4 (57.1 %)	0.015
NR (%)	12 (92.3 %)	3 (42.9 %)	
6 month response			
CR (%)	0	0	
PR (%)	1 (7.7 %)	4 (57.1 %)	0.036
NR (%)	12 (92.3 %)	3 (42.9 %)	
12 month response			
CR (%)	1 (100 %)	1 (33.3 %)	
PR (%)	0	2 (66.7 %)	0.248
NR (%)	0	0	
Relapse (%)	1 (7.7 %)	0	0.333
Second treatment			
● Haploidentical HSCT (%)	0	1 (14.3%)	-
● IST (%)	6 (46.2%)	0	-
● IST+EPAG (%)	1 (7.7%)	0	-
● Androgen (%)	0	1 (14.3%)	-
Time to response,month (mean±SD)	6.7±1.9	4.5±1.8	0.085
Time to transfusion free,month (mean±SD)	6.4±2.1	4.5±1.7	0.162
Death (%)	4 (30.8 %)	2 (28.6%)	0.732
Loss to follow up (%)	2 (15.4 %)	0	0.179
Duration of FU (months), (mean±SD)	54.5±15.7	23.7±17.1	0.180



Conclusions

The addition of Eltrombopag to immunosuppressive therapy significantly improved partial response (PR) at three and six months compared to immunosuppressive therapy alone, reduced the need for a second immunosuppressive therapy course, and enhanced overall survival (OS) without intolerable adverse events.