

Safety and efficacy of romiplostim in children with acquired aplastic anemia who are naïve to immunosuppressive therapy

Authors

Mohammadreza Bordbar,¹ Alireza Faghihi,² Omid Reza Zekavat,¹ Mahdi Shahriari¹ and Shayan Bordbar³

¹Hematology Research Center; ²Pediatric Department, Shiraz Medical School and ³Student Research Committee, Shiraz Medical School, Shiraz University of Medical Sciences, Shiraz, Iran

Correspondence:

M. BORDBAR - mbordbar53@gmail.com; bordbarm@sums.ac.ir

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Table S1- Definition of hematologic response

<ul style="list-style-type: none">• Platelet Response:<ul style="list-style-type: none">○ Increase of $\geq 20 \times 10^9/L$ above baseline; OR○ Increase to $\geq 10 \times 10^9/L$ with a $\geq 100\%$ increase from baseline; AND○ No platelet transfusions for 8 weeks in patients who had received transfusions in the 8 weeks prior to romiplostim initiation
<ul style="list-style-type: none">• Erythrocyte Response:<ul style="list-style-type: none">○ Increase in hemoglobin to ≥ 9 g/dL without RBC transfusion in patients with pre-treatment hemoglobin < 9 g/dL; OR○ $\geq 50\%$ reduction in cumulative RBC transfusion volume over 8 weeks in patients who had received transfusions in the 8 weeks prior to romiplostim initiation
<ul style="list-style-type: none">• Neutrophil Response:<ul style="list-style-type: none">○ $\geq 100\%$ increase in patients with baseline neutrophils $< 0.5 \times 10^9/L$; OR○ Increase of $\geq 0.5 \times 10^9/L$ above baseline in patients with baseline neutrophils of $0.5-1 \times 10^9/L$.
<ul style="list-style-type: none">• Trilineage Response:<ul style="list-style-type: none">○ Achievement of concurrent platelet, erythrocyte, and neutrophil responses
<ul style="list-style-type: none">• Complete hematologic response (CHR)<ul style="list-style-type: none">○ Platelet $\geq 150 \times 10^9/L$; AND○ Hb ≥ 2 SD above the mean for age; AND○ Neutrophil $\geq 1.5 \times 10^9/L$; AND○ Transfusion independence in the past 8 weeks
<ul style="list-style-type: none">• Partial response (PR): Achievement of unilineage or bilineage hematologic response
<ul style="list-style-type: none">• Overall response (OR): The sum of responders with partial and trilineage responses

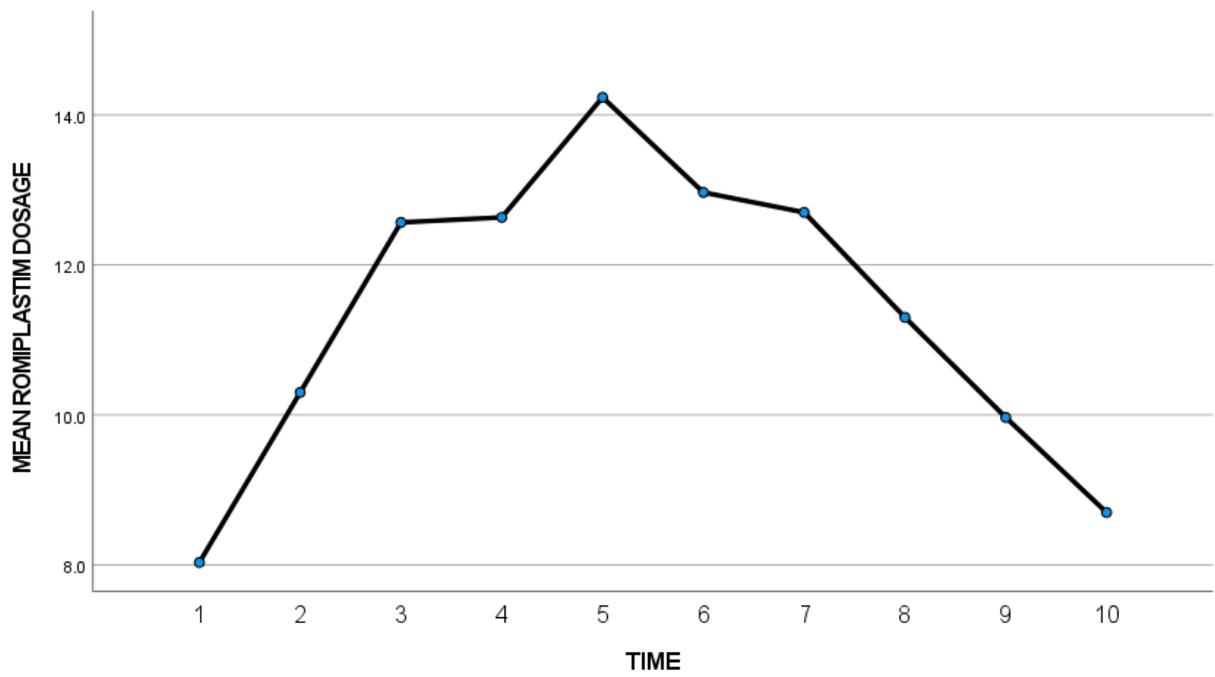
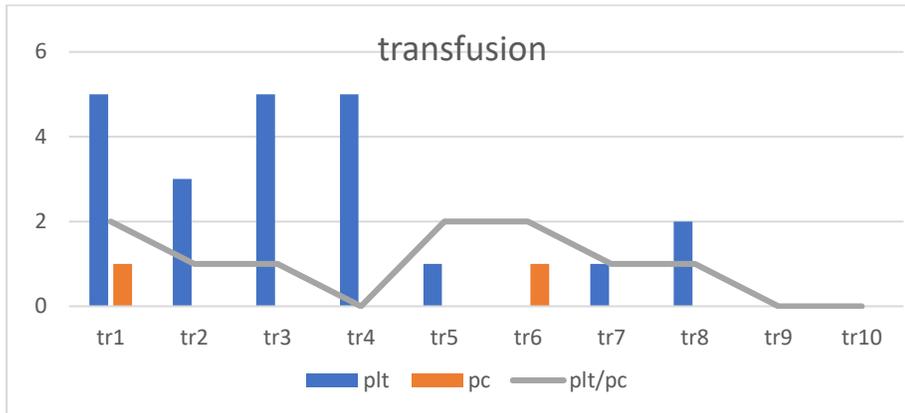


Figure S1: The trend of changes in romiplostim dosages during the 10-month follow-up



PLT: platelet; PC: packed red blood cells

Figure S2- Transfusion requirements in the 10 months' treatment with romiplostim