

# Real-world safety and efficacy of rADAMTS13 prophylaxis in congenital thrombotic thrombocytopenic purpura: experience from Polish patients previously treated with fresh frozen plasma

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Supplementary Table 1. Laboratory parameters during 12 months of fresh frozen plasma therapy and 12 months following transition to rADAMTS13 prophylaxis

Parameter	N <sup>^</sup>	FFP	N	rADAMTS13	p
<b>All patients</b>					
Hemoglobin (g/dL)	17	13.0 (1.5)	23	13.8 (1.3)	0.02*
Platelets ( $\times 10^3/\mu\text{L}$ )	17	170.4 (78.3)	23	218.2 (64.2)	0.0003*
LDH (mg/dL)	17	335.5 (193.2)	23	210.0 (56.6)	0.0008 <sup>#</sup>
Bilirubin (mg/dL)	17	0.7 (0.4)	22 <sup>^</sup>	0.5 (0.4)	0.0001 <sup>#</sup>
Creatinine (mg/dL)	17	0.8 (0.3)	23	0.7 (0.3)	0.06 <sup>#</sup>
<b>Patients aged over 18 years</b>					
Hemoglobin (g/dL)	11	13.8 (1.2)	16	14.3 (1.2)	0.18*
Platelets ( $\times 10^3/\mu\text{L}$ )	11	153.8 (86.9)	16	207.9 (71.6)	0.007*
LDH (mg/dL)	11	287.1 (159.7)	16	203.3 (60.2)	0.054 <sup>#</sup>
Bilirubin (mg/dL)	11	0.8 (0.4)	15 <sup>^</sup>	0.6 (0.4)	0.008 <sup>#</sup>
Creatinine (mg/dL), mean (SD)	11	1.0 (0.3)	16	0.9 (0.3)	0.16 <sup>#</sup>
<b>patients aged 18 years or younger</b>					
Hemoglobin (g/dL), mean (SD)	6	11.5 (0.5)	7	12.7 (1.0)	0.04*
Platelets ( $\times 10^3/\mu\text{L}$ ), mean (SD)	6	200.8 (52.6)	7	241.7 (37.0)	0.01*
LDH (mg/dL), mean (SD)	6	424.3 (232.1)	7	225.3 (47.9)	0.03 <sup>#</sup>
Bilirubin (mg/dL), mean (SD)	6	0.7 (0.3)	7	0.4 (0.3)	0.03 <sup>#</sup>

Creatinine (mg/dL), mean (SD)	6	0.6 (0.3)	7	0.5 (0.1)	0.50 <sup>#</sup>
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Data are presented as mean (SD).

Abbreviations: FFP – fresh frozen plasma; LDH – lactate dehydrogenase

\*Paired t-test; <sup>#</sup>Wilcoxon matched-pairs signed rank test; <sup>^</sup>A reduced number attributable to incomplete or missing data

Supplementary Table 2. Hospitalizations during the last 12 months of fresh frozen plasma therapy and 12 months following transition to rADAMTS13 prophylaxis in all patients

Parameter	FFP	rADAMTS13	p
<b>All patients</b>			
Number of hospitalizations	17.2 (12.6)	0.7 (1.2)	<0.0001
Days of hospitalization	24.8 (16.1)	0.8 (1.6)	<0.0001
Number of exacerbation-related hospitalizations	0.7 (1.5)	0.0 (0.2)	0.049
Days of exacerbation-related hospitalizations	2.6 (6.0)	0.1 (0.4)	0.094
<b>&gt;18 years old</b>			
Number of hospitalizations	19.8 (14.6)	0.9 (1.3)	<0.0001
Days of hospitalization	24.7 (18.6)	1.1 (1.8)	<0.0001
Number of exacerbation-related hospitalizations	0	0.1 (0.3)	0.99
Days of exacerbation-related hospitalizations	0	0.1 (0.5)	0.99
<b>≤18 years old</b>			
Number of hospitalizations	12.9 (7.1)	0	<0.0001
Days of hospitalization	25.0 (11.9)	0	<0.0001
Number of exacerbation-related hospitalizations	1.7 (2.2)	0	0.07

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Days of exacerbation-related hospitalizations	6.7 (8.4)	0	<0.0001
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Data are presented as mean (SD).