Long-term follow-up of patients with moderate aplastic anemia and pure red cell aplasia treated with daclizumab

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Online Supplementary Table S1. Response criteria.

Criteria for Complete Response: Moderate Aplastic Anemia or Pure Red Cell Aplasia

Subjects will be considered a complete responder if their blood counts return to normal on at least a minimum of 2 serial measurements at least one month apart.

Criteria for Partial Response (Moderate Aplastic Anemia)

Peripheral blood parameters (improvement in one or more of the listed parameter (a,b,c) will be recorded as a response

(*see also Statistical Consideration*)

a) ANC
If baseline ANC below 500/mm^3, increase in ANC $\geq$300/mm^3;
If baseline ANC above 500/mm^3, any increase in ANC $\geq$500/mm^3 of blood

b) Platelets
If baseline platelet count $\leq$70$\times$10^9/L but >50$\times$10^9/L, increase in platelet count $\geq$30$\times$10^9/L
If baseline platelet count $\leq$50$\times$10^9/L, any increase in platelet count ~20$\times$10^9/L of blood.

c) Hemoglobin
Any increase in Hb by 1.5 g/dL of blood in transfusion-independent patients and in absolute reticulocyte count to $\geq$60$\times$10^9/L of blood in transfusion-dependent patients.
Transfusion requirements (in previously transfusion-dependent patients)

a) Transfusion-independence (no transfusions for >8 weeks),

b) Significant decrease in the transfusion requirement as defined by decrease in transfusion number over time >50% for RBC, platelets or both (>8 weeks from the last transfusion). Change in the transfusion requirements will be determined either at the 4- or 12- week time point after completion of the treatment cycle.

Criteria for Partial Response (Pure Red Cell Aplasia and Diamond Blackfan anemia)

Hemoglobin: Any increase in Hbg by 1.5 g/dL of blood and/or in absolute reticulocyte count > 50,000/mm^3

Transfusion requirements

a) Transfusion independence (>8 weeks from the last transfusion),

b) Significant decrease in the transfusion requirement as defined by decrease in transfusion number over time by > 50% for RBC (>8 weeks from the last transfusion). Change in the transfusion requirements will be determined either at the 1- or 3-month time point after completion of the treatment cycle.

Online Supplementary Table S2. Patients' characteristics.

Variable	N	MAA Patients Mean 95% CI	N	PRCA Patients Mean 95% CI
Duration of disease (years)	45	2.77 (1.48, 4.05)	27	1.89 (1.05, 2.72)
Transfusion Dependent (%)	28	62.2 (47.5, 77.0)	25	92.6 (82.0, 100)
Age (years)	45	37.3 (31.8, 42.9)	27	44.9 (36.5, 53.3)
Sex				
Male (%)	21	46.7 (31.5, 61.8)	13	48.1 (28.0, 68.3)
Female (%)	24	53.3 (38.2, 68.5)	14	51.9 (31.7, 72.0)
Race				
Asian (%)	7	15.6 (4.5, 26.6)	0	0.0 (----, ----)
Black (%)	2	4.4 (0.0, 10.7)	3	11.1 (0.0, 23.8)
Hispanic (%)	8	17.8 (6.2, 29.4)	3	11.1 (0.0, 23.8)
White (%)	28	62.2 (47.5, 77.0)	21	77.8 (61.0, 94.5)
Platelet count	45	33.5 (27.7, 39.3)	27	272.6 (219.3, 325.8)
Absolute neutrophil count	45	3.48 (0.0, 7.12)	27	3.04 (2.09, 3.99)
Reticulocyte count	45	64.9 (56.6, 73.3)	27	11.8 (4.7, 19.0)
Response at 3 months (%)	45	42.2 (27.2, 57.2)	27	37.0 (17.6, 56.5)

Online Supplementary Table S3. Univariate logistic regression analysis for response at three months.

Variable	MAA Patients Coeff. β	SE (β)	P value $\beta=0$	PRCA Patients Coeff. β	SE (β)	P value $\beta=0$
Duration (years)	-0.065	0.081	0.421	-0.165	0.572	0.505
Transf. dep.	-1.974	0.688	0.004	14.161	624.194	0.982
Age (years)	0.021	0.017	0.218	-0.010	0.019	0.603
Sex: male	-0.318	0.608	0.600	-0.523	0.808	0.517
female	-----	-----	-----	-----	-----	-----
Race						
Asian	-0.916	0.918	0.318	NA	NA	NA
Black	3.693	1.464	>0.99	-0.208	1.305	0.874
Hispanic	-1.099	0.900	0.222	-0.208	1.305	0.874
White	-----	-----	-----	-----	-----	-----
Platelet	0.010	0.016	0.541	-0.002	0.003	0.594
Absolute neutrophil	-0.027	0.043	0.532	0.073	0.166	0.659
Reticulocyte	-0.0004	0.011	0.972	0.011	0.022	0.613

A positive value for the coefficient (β) would suggest that the corresponding variable was positively associated with the probability of response at three months. The P values were based on t-tests for testing the null hypothesis that the corresponding covariate did not affect the probability of response at three months. For categorical variables, "—" represents the baseline category, and "NA" implies that the category is not available.