

Table 2 Transplant characteristics and outcome

Pt No	Age at HSCT (years)	Donor type/ TYMP mutation carrier status/ Graft source	Number of TNC/CD34	Conditioning	Chimerism	dUrd;+days p-T dThd;+days p-T normal <0.05 mmol/L	dUrd; +years p-T dThd; +years p-T normal <0.05 mmol/L	GVHD and organ toxicity	Long term outcome
1	10	MFD/ Heterozygote/ peripheral blood stem cells	TNC- no data CD34-10.5 10 ⁶ /kg	Bu/Flu/Cy/ATG Partial T-cell depletion*	100% donor	0.65; +14 d 0.39; +14 d	0.72; +3 yr 0.0; +3 yr	AGVHD skin stage 2-3	15 years post HSCT: Alive and seemingly asymptomatic: gained weight, free of TPN and normal EMG
2	16	MSD/ Heterozygote/ Bone marrow	TNC- 8.5x10 ⁸ /kg CD34- 7.2x10 ⁶ /kg	Bu/Flu/ATG [†]	100% donor	0.4; +15 d 0.0; +15 d	1; +7.5 yr 0.2; +7.5 yr	line sepsis with disseminated disease and right atrium infected thrombus. Abnormal liver function tests	Died 8 years post HSCT: from MNGIE complications
3	27	MFD/ Unknown/ Bone marrow	TNC- 4.2x10 ⁸ /kg CD34- 4.8x10 ⁶ /kg	Treo/Flu/ATG ^{††}	100% donor on day 12	28.1; +12 d 44.6; +12 d	No data	septic shock and multiorgan failure on day +3 post HSCT	Died on day +12 post HSCT
4	20	MFD/ Not a carrier/ Bone marrow	TNC- 6.3x10 ⁸ /kg CD34-4x10 ⁶ /kg	Treo/Flu/ATG ^{††}	100% donor	0.1; +30 d 0.1; +30 d	No data	No GVHD or organ toxicity	4 years post HSCT: Alive with severe GI complications and chronic pain
5	10	MSD/ Heterozygote/ Bone marrow	TNC-5x10 ⁸ /kg CD34-3x10 ⁶ /kg	Treo/Flu/ATG ^{††}	Secondary engraftment failure	1.3; +23 d 0.7; +23 d	10; +1.5 yr 7.9; +1.5 yr	No GVHD or organ toxicity	4 years post HSCT: Alive with severe GI complications and chronic pain

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6	8	MSD/ Heterozygote/ Bone marrow	TNC- 13.9x10 ⁸ /kg CD34- 12.4x10 ⁶ /kg	Treo/Flu/ATG††	Second engraftment failure	No data	9.1; +1.2 yr 5.2; +1.2 yr	No GVHD or organ toxicity	4 years post HSCT: Alive with severe GI complications and chronic pain
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Abbreviations: Pt, patient; TNC, total nucleated cells; dUrd, deoxyuridine; p-T, post hematopoietic stem cell transplantation; dThd, thymidine; GVHD, graft versus host disease; MFD, matched family donor; Bu, busulfan; Flu, fludarabine; Cy, cyclophosphamide; ATG, antithymocyte globulin; AGVHD, acute graft versus host disease; TPN, total parenteral nutrition; EMG, electromyography; MSD, matched sibling donor; Treo, treosulfan; GI, gastrointestinal

* busulfan (1mg/kg/dose, 4 times/day, 16 doses with correction dose according to area under the curve (AUC), fludarabine (40 mg/m²/day, 5 doses), cyclophosphamide (50 mg/kg/day, 4 doses) and anti-thymoglobulin (ATG) fresenius (5 mg/kg/day, 5 doses) with partial T-cell depletion¹⁸

† busulfan (1 mg/kg/dose, 4 times/day, 16 doses with correction dose according to AUC) , fludarabine (40 mg/m²/day, 5 doses) and ATG fresenius (5 mg/kg/day, 5 doses)

†† treosulfan (14 gr/m²/day, 3 doses), fludarabine (40 mg/m²/day, 5 doses) and ATG Fresenius (5 mg/kg/day, 5 doses)