

Supplementary Table 1: Individual Transplant Characteristics and Outcomes Post transplant

Diagnosis	Age at HCT (years)	HCT (First or Second)	Donor	HLA match	Busulfan dosing (hours)	Busulfan AUC (mg/L × h)	Fludarabine dose	Serotherapy (Alemtuzumab or rATG, dose)	Graft source	GVHD prophylaxis	Donor chimerism (engraftment)	Mixed chimerism (Y/N)	Donor chimerism (1 year)	Post HCT Complications	Outcome Last follow up
WAS	1.1	First	URD	10/10	q12	62	180 mg/m2	ALTMB, 0.5mg/kg	BM	CSA/MMF	99% WB	N	99% WB	Grade 1 acute GVHD, Norovirus, C.diff infection	A & W, 51 months, off IS
WAS	6.0	First	URD	10/10	q12	63	180 mg/m2	ALTMB, 0.5mg/kg	BM	CSA/MMF/MV	100% WB	N	100% WB	CMV viremia	A & W, 29 months, off IS
WAS	1.3	First	URD	9/10	q12	63	180 mg/m2	ALTMB, 0.5mg/kg	BM	CSA/MMF	100% WB	N	100% WB	Grade 3 acute GVHD, CMV, EBV viremia, TMA, BO, GBS, ITP	Died (day+ 562) due to GVHD, BO
Primary HLH	3.6	Second	URD	10/10	q12	62	180 mg/m2	ALTMB, 0.5mg/kg	BM	CSA/MMF	100% WB	N	100% WB	None	A & W, 35 months, off IS
WAS	0.9	First	URD	10/10	q12	62	180 mg/m2	ALTMB, 0.5mg/kg	BM	CSA/MMF	100% WB	N	98% WB	VZV infection	A & W, 49 months, off IS
IFNGR1 Def.	1.1	First	URD	10/10	q12	62	180 mg/m2	ALTMB, 0.5mg/kg	BM	CSA/MMF	100% WB	N	100% WB	Norovirus, CMV viremia, Grade 2 acute GVHD, BCGosis	A & W, 13 months, off IS
Primary HLH	2.3	Second	MSD	10/10	q12	62	180 mg/m2	ALTMB, 0.5mg/kg	BM	CSA/MMF	100% WB	Y	100% Myeloid 45% T-cell	CMV viremia, C. diff infection	A & W, 41 months, off IS, on IVIG
WAS	5.8	First	MSD	10/10	q12	63	180 mg/m2	ALTMB, 0.5mg/kg	BM	CSA/MMF	100% WB	N	99% WB	CMV viremia	A & W, 28 months, off IS
CD40 Ligand Def.	0.8	First	MSD	10/10	q12	63	180 mg/m2	ALTMB, 0.5mg/kg	BM	CSA/MMF	100% WB	Y	97% Myeloid 79% T-cell	None	A & W, 39 months, off IS
WAS	1.4	First	URD	10/10	q12	62	180 mg/m2	ALTMB, 0.5mg/kg	BM	CSA/MMF	100% WB	N	100% WB	Norovirus infection	A & W, 25 months, off IS
IPEX	0.5	First	URD	10/10	q12	62	180 mg/m2	ALTMB, 0.5mg/kg	BM	Pred/Tacro	99% WB	N	99% WB	Autoimmune hemolytic anemia	A & W, 33 months, off IS
CD40 Ligand Def.	0.8	First	MSD	10/10	q12	62	180 mg/m2	ALTMB, 0.5mg/kg	BM	CSA/MMF	100% WB	Y	100% Myeloid 93% T-cell	CMV/EBV viremia	A & W, 31 months, off IS
CD40 Ligand Def.	12.8	First	URD	10/10	q12	62	180 mg/m2	ALTMB, 0.5mg/kg	BM	CSA/MMF	100% WB	N	NA	Adenovirus encephalitis, progressive neurodegeneration	Died (day+ 51) due to progressive neurodegeneration, adenovirus encephalitis
Primary HLH	1.9	Second	URD	9/10	q12	62	180 mg/m2	ALTMB, 0.5mg/kg	BM	CSA/MMF/ABT	100% WB	N	NA	DAH, TMA, CMV/EBV viremia	Died (day+ 52) due to DAH
WAS	0.7	First	URD	10/10	q12	63	180 mg/m2	ALTMB, 0.5mg/kg	BM	CSA/MMF	100% WB	N	100% WB	None	A & W, 18 months, off IS
WAS	0.6	First	URD	10/10	q12	60	180 mg/m2	ALTMB, 0.5mg/kg	BM	CSA/MMF	100% WB	N	100% WB	Mild chronic skin GVHD, GBS, Autoimmune hemolytic anemia	A & W, 26 months, off IS
CD40 Ligand Def.	16.4	First	URD	9/10	q12	63	180 mg/m2	ALTMB, 0.5mg/kg	BM	CSA/MMF/MV	100% WB	N	NA	IPS, CMV viremia	Died (day+ 56) due to IPS
Primary HLH	1.9	Second	URD	10/10	q12	63	180 mg/m2	ALTMB, 0.5mg/kg	BM	CSA/MMF	99% WB	Y	95% WB	Grade 1 acute GVHD	A & W, 25 months, off IS
IL-10Rb Def.	0.5	First	URD	10/10	q12	61	180 mg/m2	ALTMB, 0.5mg/kg	CD34+ selected PBSC	None	100% WB	Y	33% Myeloid 80% T-cell	Norovirus infection	A & W, 24 months, off IS
XLP	2.0	First	UCB	8/10	q12	60	180 mg/m2	ALTMB, 0.5mg/kg	Cord	CSA/MMF	35% WB	Y	NA	EBV viremia	Secondary graft failure (day+48)
WAS	0.3	First	MSD	10/10	q12	63	180 mg/m2	ALTMB, 0.5mg/kg	BM	CSA/MMF	91% WB	Y	92% Myeloid 90% T-cell	Grade 1 acute GVHD	A & W, 18 months, off IS
LAD type III	0.8	First	MMRD	8/10	q12	61	180 mg/m2	ALTMB, 0.5mg/kg	CD34+ selected PBSC	None	97% WB	Y	100% Myeloid 92% T-cell	Adenoviremia	A & W, 18 months, off IS, on IVIG
XLP	5.1	Second	MUD	9/10	q12	60	180 mg/m2	ALTMB, 0.5mg/kg	BM	CSA/MMF/ABT	NA	NA	NA	None	Primary graft failure (day+34)
Primary HLH	15.4	Second	MSD	10/10	q12	60	180 mg/m2	ALTMB, 1.0 mg/kg	PBSC	CSA/MMF	100% WB	NA	NA	DAH, CMV infection	Died (day+ 63) due to DAH
Primary HLH	19.8	Second	MSD	10/10	q12	62	180 mg/m2	ALTMB, 1.0mg/kg	PBSC	CSA/MMF	98% WB	N	100% WB	moderate chronic GVHD (myositis)	A & W, 17 months, off IS, on IVIG

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Diagnosis	Age at HCT (years)	HCT (First or Second)	Donor	HLA match	Busulfan dosing (hours)	Busulfan AUC (mg/L x h)	Fludarabine dose	Serotherapy (Alemtuzumab or rATG, dose)	Graft source	GVHD prophylaxis	Donor chimerism (engraftment)	Mixed chimerism (Y/N)	Donor chimerism (1 year)	Post HCT Complications	Outcome Last follow up
X-SCID	3.0	First	MUD	10/10	q24	57	180 mg/m2	ALTMB, 0.5mg/kg	BM	CSA/MMF	100% WB	Y	33% Myeloid 94% T-cell	None	A & W, 27 months, off IS
WAS	15.0	First	MUD	10/10	q6	64	150 mg/m2	ALTMB, 0.5mg/kg	BM	CSA/MMF	100% WB	Y	45% Myeloid 92% T-cell	Adenoviremia	A & W, 35 months, off IS
CD40 Ligand Def.	3.5	First	MSD	10/10	q24	60	160 mg/2	rATG, 7.5mg/kg	BM	CSA/MMF	100% Myeloid 5% T-cell	Y	100% Myeloid 85% T-cell	None	A & W, 32 months, off IS
IPEX	17.0	First	MUD	10/10	q24	63	160 mg/m2	rATG, 7.5mg/kg	BM	CSA/MMF	100% Myeloid 20% T-cell	Y	100% Myeloid 76% T-cell	None	A & W, 48 months, off IS
Primary HLH	2.3	Second	MSD	10/10	q24	63	160 mg/m2	ALTMB, 0.3mg/kg	BM	CSA/MMF	100% Myeloid. 73% T-cell	Y	100% Myeloid 100% T-cell	SOS, grade 1 acute GVHD	A & W, 44 months, off IS
WAS	0.8	First	MUD	10/10	q24	61	160 mg/2	ALTMB, 0.5mg/kg	BM	CSA/MMF	100% Myeloid 51% T-cell	Y	100% Myeloid 62% T-cell	Grade 1 acute GVHD	A & W, 44 months, off IS
SCN	1.4	First	MUD	10/10	q24	63	160 mg/m2	ALTMB, 0.5mg/kg	BM	CSA/MMF	100% Myeloid 8% T-cell	Y	99% Myeloid 53% T-cell	AIHA, pancytopenia, secondary graft failure	Secondary graft failure (12 months post HCT)
WAS/XLT	2.3	First	MUD	10/10	q24	73	160 mg/m2	ALTMB, 0.5mg/kg	BM	CSA/MMF	100% Myeloid 61% T-cell	Y	100% Myeloid 95% T-cell	Grade 1 acute GVHD	A & W, 28 months, off IS
XLP	9.0	First	MUD	10/10	q24	65	160 mg/2	None	BM	CSA/MMF	100% Myeloid 15% T-cell	Y	100% Myeloid 89% T-cell	None	A & W, 51 months, off IS
VEOIBD	11.0	First	MUD	10/10	q24	72	160 mg/m2	None	BM	CSA/MMF/MV	100% Myeloid 100% T-cell	Y	100% Myeloid 87% T-cell	None	A & W, 24 months, off IS
CID	14.0	First	MMRD	5/10	q24	69	160 mg/m2	rATG, 7.5mg/kg	BM	CSA/MMF	100% Myeloid 16% T-cell	Y	100% Myeloid 66% T-cell	None	A & W, 21 months, off IS
CD40 Ligand Def.	2.0	First	MUD	10/10	q24	68	160 mg/2	rATG, 7.5mg/kg	BM	CSA/MMF	100% Myeloid 17% T-cell	Y	100% Myeloid 59% T-cell	Grade 1 acute GVHD	A & W, 14 months, off IS
CD40 Ligand Def.	0.8	First	MUD	10/10	q24	65	160 mg/m2	rATG, 7.5mg/kg	BM	CSA/MMF	97% Myeloid 27% T-cell	Y	97% Myeloid 46% T-cell	None	A & W, 18 months, on IS, on IVIG
MSN1	2.5	First	MUD	10/10	q24	67	160 mg/m2	rATG, 7.5mg/kg	BM	CSA/MMF	100% Myeloid 92% T-cell	Y	100% Myeloid 100% T-cell	Grade 2 acute GVHD	A & W, 14 months, off IS, on IVIG
CID	18.0	First	MSD	10/10	q24	74	160 mg/2	rATG, 7.5mg/kg	BM	CSA/MMF	100% Myeloid 26% T-cell	Y	100% Myeloid 100% T-cell	Grade 2 acute GVHD	A & W, 14 months, on IS
CTLA4 def.	13.0	First	MSD	10/10	q24	69	160 mg/m2	ALTMB, 0.5mg/kg	BM	CSA/MMF	100% Myeloid 21% T-cell	Y	100% Myeloid 22% T-cell	None	A & W, 14 months, on IS

WAS – Wiskott-Aldrich Syndrome, XLT- X-linked thrombocytopenia, HLH- Hemophagocytic Lymphohistiocytosis, XLP- X-linked lymphoproliferative disorder, IFNGR1 def. – Interferon gamma receptor type 1 deficiency, X-SCID – X-linked Severe Combined Deficiency, IPEX- Immunodysregulation polyendocrinopathy enteropathy X-linked syndrome, VEOIBD – Very Early Onset Inflammatory Bowel Disease, MSN1 – X-linked primary immunodeficiency associated with hemizygous mutations in the moesin gene, CID – Combined Immune Deficiency, LAD type III – Leukocyte adhesion deficiency type III, HCT- Hematopoietic cell transplant, MSD – matched sibling donor, MUD - matched unrelated donor, MMUD – mismatched unrelated donor, MMRD - mismatched unrelated donor, UCB – Umbilical Cord Blood, ALTMB – alemtuzumab, rATG – Rabbit ATG, BM – Bone marrow, PBSC – Peripheral blood stem cells, CSA – Cyclosporine, MMF – mycophenolate, MV – maraviroc, ABT – abatacept, GVHD – Graft versus host disease, DAH – Diffuse alveolar hemorrhage, SOS – Sinusoidal obstruction syndrome, IPS – Idiopathic pneumonia syndrome, BO – Bronchiolitis obliterans, GBS – Guillaine-Barre syndrome, ITP – Immune thrombocytopenic purpura, WB – whole blood, A & W – alive and well, IS – immune suppression, IVIG – intravenous immunoglobulin, Y=yes, N=no.