Supplementary Material

Supplementary Figures

Suppl. Figure 1 Engraftment and graft failure.
A. Cumulative incidence recovery of neutrophil counts > 500 cells/µl and primary graft failure (PGF) as competing risk event. The cumulative incidence of neutrophil engraftment was 86% (95% CI: 78-93) at day +28. Eighty-one patients achieved primary neutrophil in a median of 17 days. **B**. Cumulative incidence of secondary graft failure was 15% (7-23) at 36 months after transplant.

Suppl. Figure 2
Patients transplantation outcome overview. 84 pediatric patients with LAD underwent alloHSCT. The state of engraftment and patient's outcome at their last follow-up are given. PGF, primary graft failure; SGF, secondary GF; F/U, follow-up; d, day

Supplementary Tables

Suppl. Table 1 Univariate analysis of overall survival (OS) for the entire cohort.

Suppl. Table 2 Univariate analysis EFS for the entire cohort.

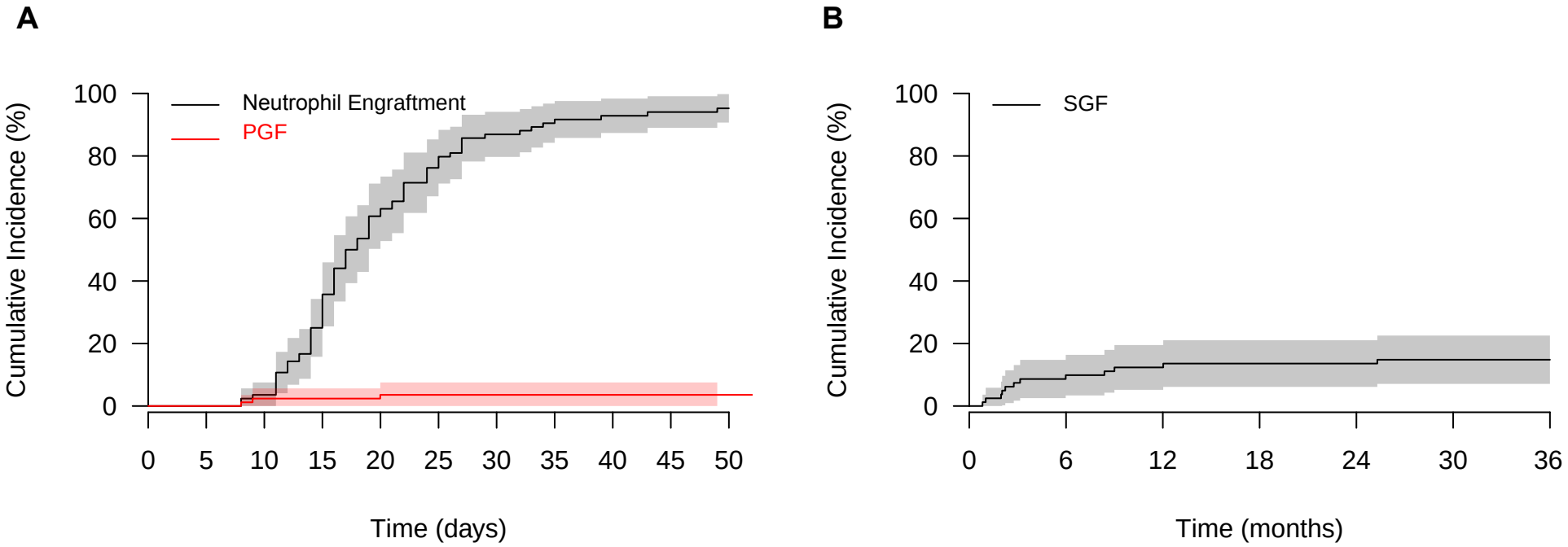
Suppl. Table 3 Univariate analysis cumulative incidence of aGVHD II-IV for the entire cohort.

Suppl. Table 4 Univariate analysis graft failure (GF) for the entire cohort.

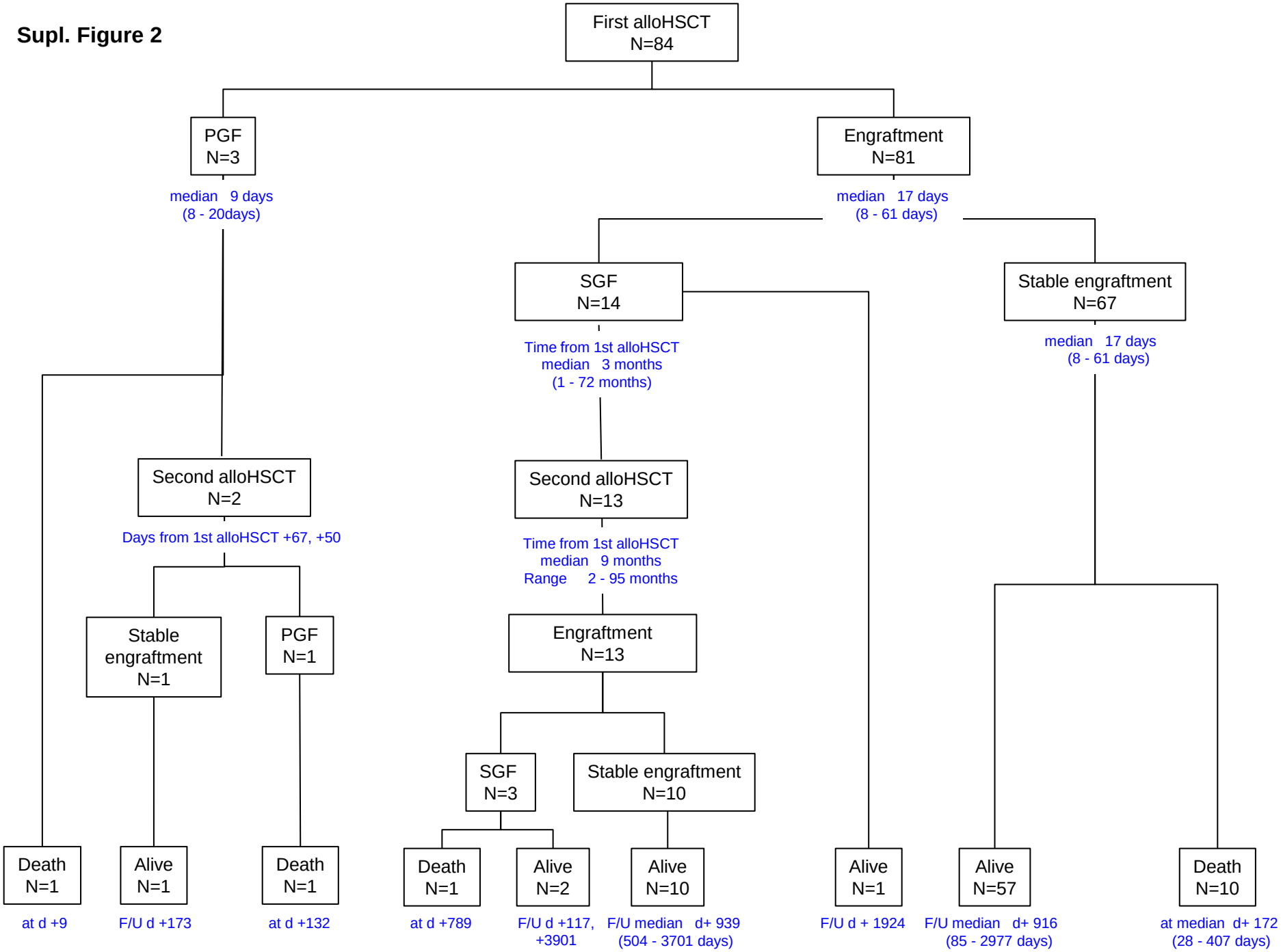
Suppl. Table 5 Patients' and transplant's characteristics stratified by age group.

Suppl. Table 6 Outcomes stratified by age at transplant.

Supl. Figure 1



Supl. Figure 2



Supplement Table 1: Univariate analysis OS for the entire cohort

	ev/N	Estimate at 36 months (95% CI)	P
Sex			0.913
Female	6/40	84% (73-96)	
Male	7/43	81% (67-94)	
LAD type			0.771
LAD type I	10/69	84% (75-93)	
LAD type III	2/11	75% (44-100)	
AlloHSCT year			0.369
2007-2012	8/41	78% (64-92)	
2013-2017	5/43	88% (78-98)	
Age at alloHSCT			0.272
< 13 months	5/42	88% (78-98)	
≥ 13 months	8/42	75% (59-91)	
Lansky/Karnofsky Performance			0.043
Score 100-90	6/44	84% (72-96)	
Score <90	7/19	61% (38-84)	
AlloHSCT source			0.336
BM	5/44	87% (76-98)	
CB	4/16	71% (46-95)	
PB	4/20	80% (62-98)	
Donor			0.001
MSD	2/32	93% (85-100)	0.918¥
MUD 10/10 + MFD	2/28	93% (84-100)	
MUD 9/10	3/5	60% (29-100)*	
MMD (CB + MMFD)	4/17	70% (49-100)	
CMV recipient-donor			0.003
-/-	1/19	95% (85-100)	
-/+	3/9	64% (24-100)	
+/-	4/10	50% (13-87)	
+/+	1/32	97% (90-100)	
CMV recipient-donor			<0.001
Matched	2/51	96% (90-100)	
Mismatched	7/19	56% (35-89)	
Conditioning regimen			0.098
Non-MA	4/17	73% (50-100)	
MA	9/64	85% (75-94)	
Conditioning drugs			0.292
Busulfan based	8/42	80% (67-92)	
Treosulfan based	2/26	92% (82-100)	
Other	3/15	76% (51-100)	
Serotherapy			0.120
No	1/21	95% (86-100)	
ATG	11/51	74% (60-88)	

	ev/N	Estimate at 36 months (95% CI)	P
Alemtuzumab	1/11	91% (74-100)	
GVHD Prophylaxis			
CSA	2/20	89% (75-100)	
CSA+MMF	3/15	80% (60-100)	0.638
CSA+MTX	3/27	89% (76-100)	
CSA+corticosteroids	3/8	57% (20-94)	
Other	1/8	88% (65-100)	

AlloHSCT, allogeneic hematopoietic stem cell transplantation; ATG, anti-thymocyte globulin; BM, bone marrow; CB, cord blood; CI, confidence interval; CMV, cytomegalovirus; CSA, cyclosporine A; ev, event; HLA, human leucocyte antigen; LAD, leukocyte adhesion deficiency; MA, myeloablative; MFD, matched family donor; MMF, mycophenolate mofetil; MMD mismatch donor; MSD, match sibling donor; MUD, match unrelated donor; MTX, methotrexate; PB, peripheral blood; y, year
* reached estimate before 36 months.
¥Subgroup comparison

Supplement Table 2: Univariate analysis EFS for the entire cohort

	ev/N	Estimate at 36 months (95% CI)	P
Sex			0.137
Female	14/39	66% (51-81)	
Male	20/39	48% (32-64)	
LAD type			0.934
LAD type I	28/66	58% (46-70)	
LAD type III	4/9	56% (23-88)	
Age at alloHSCT			0.004
< 13 months	12/39	72% (57-86)	
≥ 13 months	23/40	42% (26-57)	
Lansky/Karnofsky Performance			0.323
Score 100-90	18/42	57% (41-72)	
Score <90	11/18	39% (16-61)	
AlloHSCT source			0.054
BM	14/44	68% (54-82)	
CB	10/15	38% (12-63)	
PB	10/19	47% (25-70)	
Donor			0.003
MSD	5/29	82% (68-98)	0.011¥
MUD 10/10 + MFD	14/28	50% (35-72)	
MUD 9/10	4/5	20% (35-100)*	
MMD	10/15	40% (22-74)	
CMV recipient-donor			0.004
-/-	9/19	58% (36-80)	
-/+	6/9	33% (13-84)*	
+/-	7/10	30% (2-58)	
+/+	5/29	82% (68-96)	
CMV recipient-donor			<0.001
Matched	12/48	72 (60-86)	
Mismatched	13/19	32 (16-61)	
Conditioning regimen			0.414
Non-MA	8/16	48% (23-74)	
MA	27/62	58% (46-70)	
Conditioning drugs			0.812
Busulfan based	17/40	57% (42-73)	
Treosulfan based	11/23	57% (36-77)	
Other	7/15	51% (25-78)	
Serotherapy			0.008
No	4/20	80% (62-98)	
ATG	28/48	42% (28-57)	
Alemtuzumab	3/10	70% (42-98)	
GVHD Prophylaxis			
CSA	7/19	68% (46-89)	
CSA+MMF	5/14	64% (39-89)	0.986

CSA+MTX	9/27	66% (48-84)
CSA+corticosteroids	6/8	25% (0-55)
Other	6/8	25% (8-83)*

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¥Subgroup comparison.

Supplement Table 3: Univariate analysis cumulative incidence of aGVHD II-IV for the entire cohort.

	ev/N	Estimate at 100 d (95% CI)	P
Sex			0.332
Female	8/39	18% (6-30)	
Male	11/39	28% (14-42)	
LAD type			0.798
LAD type I	16/66	24% (14-35)	
LAD type III	2/9	11% (0-32)	
Age at alloHSCT			0.008
< 13 months	5/39	10% (1-20)	
≥ 13 months	15/40	38% (22-53)	
Lansky/Karnofsky Performance			0.888
Score 100-90	11/42	26% (13-40)	
Score <90	5/18	28% (7-48)	
AlloHSCT source			0.14
BM	7/44	16% (5-27)	
CB	5/15	27% (4-49)	
PB	7/19	37% (15-59)	
Donor			0.071
MSD	3/29	10% (0-21)	0.087¥
MUD 10/10 + MFD	8/28	25% (9-41)	
MUD 9/10	3/5	60% (17-100)	
MMD (CB + MMFD)	4/15	27% (4-49)	
CMV recipient-donor			0.110
-/-	3/19	11% (0-24)	
-/+	4/9	44% (12-77)	
+/-	4/10	40% (10-70)	
+/+	4/29	14% (1-26)	
CMV recipient-donor			0.018
Matched	7/48	13% (3-22)	
Mismatched	8/19	42% (20-64)	
Conditioning regimen			0.066
Non-MA	7/16	44% (20-68)	
MA	13/62	19% (10-29)	
Conditioning drugs			0.333
Busulfan based	9/40	23% (10-35)	
Treosulfan based	6/24	21% (2-33)	
Other	5/14	38% (16-68)	
Serotherapy			0.434
No	3/20	10% (0-23)	
ATG	14/48	29% (16-42)	
Alemtuzumab	3/10	30% (2-58)	
GVHD Prophylaxis			
CSA	4/19	22% (3-41)	
CSA+MMF	2/14	14% (0-33)	0.884
CSA+MTX	5/27	19% (4-33)	
CSA+corticosteroids	4/8	50% (15-85)	
Other	5/8	50% (15-85)	

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* reached estimate before 36 months.
¥Subgroup comparison.

Supplement Table 4: Univariate analysis graft failure (GF) for the entire cohort.

	ev/N	Estimate at 36 months (95% CI)	P
Sex			0.759
Female	8/40	17% (4-30)	
Male	9/43	22% (9-36)	
LAD type			0.321
LAD type I	13/69	18% (8-27)	
LAD type III	3/11	30% (2-58)	
Age at alloHSCT			0.074
< 13 months	6/42	13% (2-24)	
≥ 13 months	11/42	26% (12-40)	
Lansky/Karnofsky Performance			0.396
Score 100-90	7/44	16% (5-27)	
Score <90	5/19	29% (7-50)	
alloHSCT source			0.320
BM	6/44	16% (4-28)	
CB	5/16	26% (4-48)	
PB	4/20	20% (2-38)	
Donor			0.105
MSD	4/32	14% (0-30)	0.651¥
MUD 10/10 + MFD	5/28	18% (4-32)	
MUD 9/10	1/5	20 (0-55)*	
MMD (CB + MMFD)	7/17	36% (13-60)	
CMV recipient-donor			0.818
-/-	5/19	21% (3-39)	
-/+	1/9	11% (0-32)*	
+/-	2/10	20% (0-45)	
+/+	4/32	18% (1-34)	
CMV recipient-donor			0.958
Matched	9/51	18% (0 - 32)	
Mismatched	3/19	16% (0 -26)	
Conditioning regimen			0.225
Non-MA	1/17	6% (0-17)	
MA	15/64	20% (11-30)	
Conditioning drugs			0.295
Busulfan based	8/42	20% (8-33)	
Treosulfan based	8/26	25% (8-43)	
Other	1/15	7% (0-19)	
GVHD Prophylaxis			
CSA	3/20	13% (0-30)	
CSA+MMF	4/15	27% (4-49)	0.441
CSA+MTX	3/27	14% (0-29)	
CSA+corticosteroids	3/8	27% (0-59)	
Other	1/8	12% (0-35)	

AlloHSCT, allogeneic hematopoietic stem cell transplantation; BM, bone marrow; CB, cord blood; CI, confidence interval; CMV, cytomegalovirus; CSA, cyclosporine A; ev, event; HLA, human leucocyte antigen; LAD, leukocyte adhesion deficiencies; MA, myeloablative; MFD, matched family donor; MMD mismatch donor; MSD, match sibling donor; MTX, methotrexate; MUD, match unrelated donor; PB, peripheral blood; y, year
* reached estimate before 36 months.
¥Subgroup comparison

Supplemental Table 5. Patient's and transplant's characteristics stratified by age group

	Stratified by Median of Age		
	< 13 months	≥ 13 months	P
N	42	42	
Age at Diagnose, (median [IQR]) (months)	1.92 [0.72 - 3.09]	11.64 [4.77 - 19.44]	<0.001
Range	0 -9.72	0.24 – 130.56	
Time between diagnosis and alloHSCT, median [IQR] (months)	3.00 [1.82 - 5.08]	17.10 [8.96 - 50.29]	<0.001
Range	0.55 – 11.50	0.60 – 353.35	
Sex, N (%)			0.512
Female	22 (52)	18 (44)	
Male	20 (48)	23 (56)	
No data	0	1	
LAD type, N (%)			0.520
LAD type I	35 (83)	34 (81)	
LAD type III	4 (10)	7 (17)	
Other	3 (7)	1 (2)	
Lansky/Karnofsky Performance, N (%)			0.033
Score 100	8 (28)	14 (41)	
Score 90	11 (38)	11 (32)	
Score 80	9 (31)	7 (21)	
Score 70	1 (3)	0	
Score 60	0	2 (6)	
No data/not evaluated	13	8	
AlloHSCT source, N (%)			0.371
BM	23 (59)	21 (51)	
CB	9 (23)	7 (17)	
PB	7 (18)	13 (32)	
No data	3	1	
Donor, N (%)			0.138
MSD	15 (37)	17 (41)	
MUD 10/10 + MFD	17 (41)	11 (27)	
MUD 9/10	0	5 (12)	
MMD (CB + MMFD)	9 (22)	8 (20)	
No data	1	1	
CMV recipient-Donor, N (%)			0.281
-/-	13 (36)	6 (18)	
-/+	3 (8)	6 (18)	
+/-	4 (11)	6 (18)	
+/+	16 (44)	16 (47)	
No data	6	8	
Conditioning regimen, N (%)			0.592
Non-MA	7 (18)	10 (24)	
MA	32 (82)	32 (67)	
No data	3	0	
Conditioning drugs, N (%)			1
Busulfan based	21 (51)	21 (50)	
Bu+Cy	8 (20)	9 (21)	
Bu+Flu	10 (24)	11 (26)	
Bu+Flu+Cy	0	1 (2.4)	
Bu+Flu+TT	3 (7)	0 (0.0)	
Treosulfan based	13 (32)	13 (31)	
Treo+Cy	1 (2)	0 (0)	

	Stratified by Median of Age		
	< 13 months	≥ 13 months	P
Treo+Flu	6 (15)	8 (19)	
Treo+Flu+TT	6 (15)	4 (10)	
Treo+Mel+Flu	0 (0)	1 (2)	
Other	7 (17)	8 (19)	
Mel+Flu	6 (15)	7 (17)	
Mel+Flu+TT	1 (2)	1 (2)	
No data	1	0	
Serotherapy, N (%)			0.159
No	13 (32)	8 (19)	
Yes	28 (68)	34 (81)	
ATG	21 (51)	30 (71)	
Alemtuzumab	7 (17)	4 (10)	
No data	1	0	
GVHD Prophylaxis, N (%)			0.477
CSA	10 (26)	10 (24)	
CSA+corticosteroids	4 (11)	4 (10)	
CSA+MMF	10 (26)	5 (12)	
CSA+MTX	11 (29)	16 (38)	
No	0 (0)	2 (5)	
Other	3 (8)	5 (12)	
No data	4	0	

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Supplemental Table 6. Outcomes stratified by age at transplantation

	< 13 months	≥ 13 months	P
Neutrophil engraftment			0.991
28d- cumulative incidence, % (95% CI)	83 (72-95)	88 (78-99)	
At day 100, N (%)	41 (98)	40 (95)	
Median time to engraftment, days	18	17	
Range time to engraftment, days	11 - 43	8 - 61	
Primary graft failure			0.560
28d- cumulative incidence, % (95% CI)	2 (0 -7)	5 (0-11)	
Secondary graft failure			0.079
3y-estimate, % (95% CI)	10 (1-19)	20 (8-32)	
Range time to SGF, months	3 – 43	1 – 72	
Overall survival			0.272
3y-OS estimate, % (95% CI)	88 (79-98)	75 (59-91)	
Event-Free survival			
3y-EFS estimate, % (95% CI)	72 (57-86)	42 (26-57)	0.004
Acute GVHVD			
100d-cumulative incidence grade II-IV, % (95% CI)	10 (1-20)	38 (23-53)	0.008
100d-cumulative incidence grade III-IV, % (95% CI)	3 (0-8)	23 (10-35)	0.007
Chronic GVHD			
3y-cumulative incidence, % (95% CI)	3 (0-8)	22 (9-36)	0.011
Yes; N	1	8	
Limited/extensive/not specified			
No, N	36	28	
Not applicable (OS < 100 days), N	1	4	
No data, N	4	2	

CI, confidence interval; d, day; EFS, event-free-survival; GVHD, graft-versus-host disease; LAD, leukocyte adhesion deficiencies, OS, overall survival; PGF, primary graft failure; SGF, secondary graft failure; y year.
P values determined using log-rank test or Gray Test as appropriate.