

## Supplementary Online Content

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**eTable 1. Detailed baseline, demographic, patient-related and transplant-related characteristics of infants receiving first allogeneic hematopoietic cell transplant**

Characteristics of patients	Malignant N (%)			Non-Malignant N (%)			All N (%)
	2000-2004	2005-2009	2010-2014	2000-2004	2005-2009	2010-2014	
<b>Time period</b>							
Number of patients	199	161	112	577	699	750	2498
Number of centers	88	77	61	118	121	102	181
<b>Age</b>							
Median (range), months	9(<1 - 12)	9(2 - 12)	9(<1 - 12)	6(1 - 12)	7(<1 - 12)	6(<1 - 12)	7(<1 - 12)
≤ 6 months	26 (13)	23 (14)	12 (11)	255 (44)	294 (42)	363 (48)	973 (39)
6-12 months	173 (87)	138 (86)	100 (89)	322 (56)	405 (58)	387 (52)	1525 (61)
<b>Sex</b>							
Male	99 (50)	86 (53)	55 (49)	340 (59)	437 (63)	477 (64)	1494 (60)
Female	100 (50)	75 (47)	57 (51)	237 (41)	262 (37)	273 (36)	1004 (40)
<b>Race/Ethnicity</b>							
White	138 (69)	120 (75)	77 (69)	384 (67)	472 (68)	439 (59)	1630 (65)
African-American	9 (5)	11 (7)	5 (4)	29 (5)	52 (7)	75 (10)	181 (7)
Asian	8 (4)	7 (4)	10 (9)	27 (5)	44 (6)	34 (5)	130 (5)
Pacific Islander	0	2 (1)	0	0	1 (<1)	2 (<1)	5 (<1)
Native American	2 (1)	0	2 (2)	3 (<1)	13 (2)	13 (2)	33 (1)
Other <sup>a</sup>	21 (11)	9 (6)	0	54 (9)	64 (9)	0	148 (6)
Not reported	21 (11)	12 (7)	18 (16)	80 (14)	53 (8)	187 (25)	371 (15)
<b>Transplant region</b>							
US	124 (62)	114 (71)	75 (67)	297 (51)	437 (63)	492 (66)	1539 (62)
Canada	11 (6)	11 (7)	3 (3)	22 (4)	32 (5)	31 (4)	110 (4)
Europe	34 (17)	9 (6)	9 (8)	120 (21)	54 (8)	27 (4)	253 (10)

<sup>a</sup> Other races/ethnicities: Hispanic unspecified (n=47), Mexican SW U.S. Hispanic (n=10), South or Central American Hispanic (n=1), other race not specified (n=90)

Characteristics of patients	Malignant N (%)			Non-Malignant N (%)			All N (%)
	2000-2004	2005-2009	2010-2014	2000-2004	2005-2009	2010-2014	
<b>Time period</b>							
Asia	6 (3)	3 (2)	3 (3)	6 (1)	8 (1)	4 (<1)	30 (1)
Australia/New Zealand	9 (5)	12 (7)	8 (7)	19 (3)	28 (4)	37 (5)	113 (5)
Mideast/Africa	6 (3)	10 (6)	9 (8)	78 (14)	123 (18)	149 (20)	375 (15)
Central/South America	9 (5)	2 (1)	5 (4)	35 (6)	17 (2)	10 (1)	78 (3)
<b>Ethnic groups</b>							
White, non-Hispanic	132 (66)	100 (62)	61 (54)	363 (63)	406 (58)	344 (46)	1406 (56)
African-American, non-Hispanic	9 (5)	11 (7)	5 (4)	29 (5)	51 (7)	67 (9)	172 (7)
Asian, non-Hispanic	8 (4)	7 (4)	9 (8)	27 (5)	40 (6)	34 (5)	125 (5)
Pacific islander, non-Hispanic	0	2 (1)	0	0	1 (<1)	2 (<1)	5 (<1)
Native American, non-Hispanic	1 (<1)	0	2 (2)	3 (<1)	12 (2)	12 (2)	30 (1)
Hispanic, white	6 (3)	20 (12)	16 (14)	21 (4)	66 (9)	95 (13)	224 (9)
Hispanic, African-American	0	0	0	0	1 (<1)	8 (1)	9 (<1)
Hispanic, Asian	0	0	1 (<1)	0	4 (<1)	0	5 (<1)
Hispanic, Native American	1 (<1)	0	0	0	1 (<1)	1 (<1)	3 (<1)
Hispanic, race not reported	14 (7)	3 (2)	0	34 (6)	11 (2)	0	62 (2)
Other	7 (4)	6 (4)	0	20 (3)	53 (8)	0	86 (3)
Not reported	21 (11)	12 (7)	18 (16)	80 (14)	53 (8)	187 (25)	371 (15)
<b>Malignant disease</b>							
Acute myelogenous leukemia	73 (37)	55 (34)	37 (33)	N/A	N/A	N/A	165 (35)
Acute lymphoblastic leukemia and other acute leukemia	81 (41)	58 (36)	43 (38)	N/A	N/A	N/A	182 (39)
MDS/MPD	40 (20)	35 (22)	31 (28)	N/A	N/A	N/A	106 (22)
Other leukemia, CML, other malignancy	5 (3)	13 (8)	1 (1)	N/A	N/A	N/A	19 (4)
<b>Non-malignant disease</b>							
Inherited bone marrow failure	N/A	N/A	N/A	21 (4)	12 (2)	22 (3)	55 (3)
Hemoglobinopathies	N/A	N/A	N/A	5 (<1)	14 (2)	6 (<1)	25 (1)
Severe combined immunodeficiency (SCID)	N/A	N/A	N/A	221 (38)	290 (41)	323 (43)	834 (41)

Characteristics of patients	Malignant N (%)			Non-Malignant N (%)			All N (%)
	2000-2004	2005-2009	2010-2014	2000-2004	2005-2009	2010-2014	
<b>Time period</b>							
Non-SCID	N/A	N/A	N/A	103 (18)	126 (18)	149 (20)	378 (19)
Osteopetrosis	N/A	N/A	N/A	62 (11)	45 (6)	36 (5)	143 (7)
Mucopolysaccharidosis/other storage disorder	N/A	N/A	N/A	81 (14)	77 (11)	54 (7)	212 (10)
Histiocytic disorder	N/A	N/A	N/A	82 (14)	132 (19)	146 (19)	360 (18)
Other non-malignant	N/A	N/A	N/A	2 (<1)	3 (<1)	14 (2)	19 (<1)
<b>Disease status<sup>b</sup> for subset<sup>c</sup></b>							
Early disease	107 (54)	82 (51)	58 (52)	N/A	N/A	N/A	247 (10)
Intermediate disease	25 (13)	18 (11)	16 (14)	N/A	N/A	N/A	59 (2)
Advanced disease	25 (13)	13 (8)	8 (7)	N/A	N/A	N/A	46 (2)
Not reported	11 (6)	8 (5)	3 (3)	N/A	N/A	N/A	22 (<1)
N/A, MDS/MPD	26 (13)	27 (17)	26 (23)	N/A	N/A	N/A	79 (3)
Unclassified, other malignant disease	5 (3)	13 (8)	1 (<1)	N/A	N/A	N/A	19 (<1)
<b>Donor type</b>							
UCB (HLA 6/6)	10 (5)	13 (8)	6 (5)	18 (3)	66 (9)	71 (9)	184 (7)
UCB (HLA 5/6)	9 (5)	37 (23)	23 (21)	50 (9)	96 (14)	137 (18)	352 (14)
UCB (HLA ≤ 4/6)	12 (6)	11 (7)	4 (4)	25 (4)	54 (8)	39 (5)	145 (6)
UCB (HLA match not reported)	33 (17)	13 (8)	7 (6)	102 (18)	89 (13)	36 (5)	280 (11)
HLA-identical sibling	64 (32)	44 (27)	29 (26)	136 (24)	138 (20)	170 (23)	581 (23)
Other relative <sup>d</sup>	12 (6)	10 (6)	7 (6)	126 (22)	119 (17)	104 (14)	378 (15)
8/8 HLA-matched unrelated	17 (9)	22 (14)	20 (18)	36 (6)	76 (11)	135 (18)	306 (12)
7/8 HLA partially matched unrelated	15 (8)	6 (4)	8 (7)	24 (4)	31 (4)	38 (5)	122 (5)
≤ 6/8 HLA mis-matched unrelated	3 (2)	1 (<1)	0	12 (2)	7 (1)	3 (<1)	26 (1)

<sup>b</sup> Disease status was defined as early, acute leukemia (AL) in first complete remission (CR), myelodysplastic syndrome (MDS) without excess of blasts; as intermediate, AL in second or greater CR; and advanced, AL not in remission and MDS with excess blasts.

<sup>c</sup> Subset includes: AML/ALL/other acute leukemia/MDS

<sup>d</sup> Other relative includes haploidentical donors

Characteristics of patients	Malignant N (%)			Non-Malignant N (%)			All N (%)
	2000-2004	2005-2009	2010-2014	2000-2004	2005-2009	2010-2014	
<b>Time period</b>							
Unrelated, HLA-match not reported	24 (12)	4 (2)	8 (7)	44 (8)	22 (3)	17 (2)	119 (5)
Not reported	0	0	0	4 (<1)	1 (<1)	0	5 (<1)
<b>Graft source</b>							
Bone marrow	106 (53)	62 (39)	59 (53)	269 (47)	309 (44)	407 (54)	1212 (49)
Peripheral blood	29 (15)	25 (16)	13 (12)	113 (20)	85 (12)	60 (8)	325 (13)
Umbilical cord blood	64 (32)	74 (46)	40 (36)	195 (34)	305 (44)	283 (38)	961 (38)
<b>Donor–recipient match, sex</b>							
Male–Male	47 (24)	51 (32)	33 (29)	139 (24)	216 (31)	228 (30)	714 (29)
Female–Male	41 (21)	35 (22)	21 (19)	160 (28)	216 (31)	248 (33)	721 (29)
Male–Female	47 (24)	36 (22)	29 (26)	92 (16)	125 (18)	126 (17)	455 (18)
Female–Female	45 (23)	37 (23)	28 (25)	122 (21)	135 (19)	147 (20)	514 (21)
Not reported	19 (10)	2 (1)	1 (<1)	64 (11)	7 (1)	1 (<1)	94 (4)
<b>ATG and Alemtuzumab use</b>							
ATG alone	75 (38)	66 (41)	50 (45)	280 (49)	368 (53)	345 (46)	1184 (47)
Alemtuzumab alone	1 (<1)	4 (2)	0	6 (1)	34 (5)	4 (<1)	49 (2)
No ATG or Alemtuzumab	123 (62)	91 (57)	62 (55)	291 (50)	297 (42)	401 (53)	1265 (51)
<b>Conditioning regimen intensity</b>							
MAC	163 (82)	153 (95)	107 (96)	431 (75)	492 (70)	454 (61)	1800 (72)
RIC/NMA	2 (1)	2 (1)	5 (4)	76 (13)	164 (23)	190 (25)	439 (18)
No conditioning	0	0	0	70 (12)	43 (6)	106 (14)	219 (9)
Not reported	34 (17)	6 (4)	0	0	0	0	40 (2)
<b>TBI used in conditioning regimen</b>							
No	144 (72)	120 (75)	92 (82)	553 (96)	684 (98)	730 (97)	2323 (93)
Yes	55 (28)	41 (25)	20 (18)	24 (4)	15 (2)	20 (3)	175 (7)

Characteristics of patients	Malignant N (%)			Non-Malignant N (%)			All N (%)
	2000-2004	2005-2009	2010-2014	2000-2004	2005-2009	2010-2014	
<b>Time period</b>							
<b>TBI and conditioning intensity</b>							
MAC + TBI	55 (28)	41 (25)	20 (18)	24 (4)	15 (2)	20 (3)	175 (7)
MAC without TBI	108 (54)	112 (70)	87 (78)	407 (71)	477 (68)	434 (58)	1625 (65)
RIC without TBI	2 (1)	2 (1)	5 (4)	76 (13)	164 (23)	190 (25)	439 (18)
Not reported or no conditioning	34 (17)	6 (4)	0	70 (12)	43 (6)	106 (14)	259 (10)
<b>GVHD prophylaxis</b>							
Ex vivo T-cell depletion	1 (<1)	3 (2)	1 (<1)	36 (6)	31 (4)	35 (5)	107 (4)
CNI regimens <sup>e</sup>	144 (72)	145 (90)	105 (94)	373 (65)	550 (79)	616 (82)	1933 (77)
Ex vivo T-cell depletion + CNI	8 (4)	3 (2)	1 (<1)	35 (6)	24 (3)	21 (3)	92 (4)
Others	5 (3)	2 (1)	5 (6)	13 (2)	15 (2)	14 (2)	54 (2)
Not reported	41 (21)	8 (5)	0	120 (21)	79 (11)	64 (9)	312 (12)
<b>Median follow-up, range (months)</b>	122(3-173)	74(3-125)	35(6-63)	119(3-193)	73(3-127)	34(3-76)	60(3-193)

Abbreviations: ATG, anti-thymocyte globulin; CNI, calcineurin inhibitor; HLA, human leukocyte antigen; MAC, myeloablative conditioning; MDS/MPD, myelodysplastic syndrome/myeloproliferative disorder; N/A, not applicable; RIC/NMA, reduced-intensity/nonmyeloablative conditioning; SCID, severe combined immunodeficiency; TBI, total body irradiation; UCB, umbilical cord blood

<sup>e</sup> CNI regimens: ALL CNI regimens including MMF or MTX

**eTable 2. Baseline demographics of infants who received allogeneic transplants reported to the CIBMTR on Comprehensive Report Form between 2008-2014**

<b>Characteristics of patients</b>	<b>N (%)</b>
Number of patients	641
Number of centers	92
<b>Age</b>	
Median (range), months	6 (< 1 - 12)
≤ 6 months	294 (46)
6-12 months	347 (54)
<b>Sex</b>	
Male	393 (61)
Female	248 (39)
<b>Race</b>	
White	452 (71)
African-American	68 (11)
Asian	38 (6)
Pacific Islander	4 (<1)
Native American	17 (3)
Not reported	62 (10)
<b>Ethnic groups</b>	
Caucasian, non-Hispanic	354 (55)
African-American, non-Hispanic	61 (10)
Asian, non-Hispanic	36 (6)
Pacific Islander, non-Hispanic	4 (<1)
Native American, non-Hispanic	16 (2)
Hispanic, White	98 (15)
Hispanic, African-American	7 (1)
Hispanic, Asian	2 (<1)

<b>Characteristics of patients</b>	<b>N (%)</b>
Hispanic, Native American	1 (<1)
Not reported	62 (10)
<b>Malignant disease</b>	
Acute myelogenous leukemia	31 (36)
Acute lymphoblastic leukemia	22 (26)
Other leukemia	2 (2)
Myelodysplastic syndrome/myeloproliferative disorder	26 (31)
Other acute leukemia	4 (5)
<b>Non-malignant disease</b>	
Acquired/Inherited bone marrow failure	10 (2)
Hemoglobinopathies	7 (1)
SCID	274 (49)
Non-SCID	85 (15)
Osteopetrosis	32 (6)
Mucopolysaccharidosis and other storage disorder	57 (10)
Histiocytic disorder	85 (15)
Others	6 (1)
<b>Disease status<sup>a</sup> for AML/ALL/other acute leukemia/MDS</b>	
Early disease	42 (7)
Intermediate disease	11 (2)
Advanced disease	7 (1)
Not reported	3 (<1)
N/A, MDS	20 (3)
Unclassified, other malignant disease	2 (<1)
N/A, non-malignant disease	556 (87)

<sup>a</sup> Disease status was defined as early, acute leukemia (AL) in first complete remission (CR), myelodysplastic syndrome (MDS) without excess of blasts; as intermediate, AL in second or greater CR; and advanced, AL not in remission and MDS with excess blasts.

<b>Characteristics of patients</b>	<b>N (%)</b>
<b>Hematopoietic cell transplant comorbidity index</b>	
0	459 (72)
1	65 (10)
2	11 (2)
3+	55 (9)
Not reported	50 (8)
<b>History of mechanical ventilation</b>	
No	473 (74)
Yes	114 (18)
<b>Donor type</b>	
UCB (HLA 6/6)	101 (16)
UCB (HLA 5/6)	199 (31)
UCB (HLA $\leq$ 4/6)	68 (11)
UCB (HLA match unknown)	23 (4)
HLA-identical sibling	70 (11)
HLA-matched/mismatched other relative	66 (10)
HLA-matched unrelated (HLA 8/8 URD)	85 (13)
HLA partially matched unrelated (HLA 7/8 URD)	21 (3)
HLA mismatched unrelated (HLA 6/8 URD)	3 (<1)
Unrelated, HLA match unknown	5 (<1)
<b>Graft source</b>	
Bone marrow	201 (31)
Peripheral blood	49 (8)
Umbilical cord blood	391 (61)
<b>Donor–recipient match, sex</b>	
Male–Male	189 (29)
Female–Male	203 (32)
Male–Female	115 (18)

<b>Characteristics of patients</b>	<b>N (%)</b>
Female–Female	133 (21)
Not reported	1 (<1)
<b>ATG and Campath use</b>	
ATG alone	366 (57)
Alemtuzumab alone	3 (<1)
No ATG or alemtuzumab	272 (42)
<b>Conditioning regimen intensity</b>	
MAC	447 (70)
RIC/NMA	138 (22)
No conditioning	56 (9)
<b>GVHD prophylaxis</b>	
Ex vivo T-cell depletion	30 (5)
CNI (all CNI regimens including MMF, MTX etc.)	564 (88)
Ex vivo T-cell depletion + CNI	20 (3)
Others	9 (1)
Not reported	18 (3)
<b>Year of transplant</b>	
2005-2009	244 (38)
2010-2014	397 (62)
Median follow-up of survivors, range, months	47 (6 - 97)

Abbreviations: ALL, acute lymphoblastic leukemia; AML, acute myelogenous leukemia; ATG, anti-thymocyte globulin; CML, chronic myelogenous leukemia; CNI, calcineurin inhibitor; HCT-CI, hematopoietic cell transplant comorbidity index; HLA, human leukocyte antigen; MAC, myeloablative conditioning; MDS/MPD, myelodysplastic syndrome/myeloproliferative disorder; MMF, mycophenolate mofetil; MTX, methotrexate; N/A, not applicable; RIC/NMA, reduced-intensity/nonmyeloablative conditioning; SCID, severe combined immunodeficiency; TBI, total body irradiation; UCB, umbilical cord blood; URD, unrelated donor

**eTable 3. Univariable analysis of outcomes by year of transplant group in Comprehensive Report Form patients between 2008-2014**

<b>Outcomes</b>	<b>N Eval</b>	<b>Prob (95% CI)</b>
Idiopathic pneumonia syndrome (IPS)	639	
100-day		5 (4-7)%
1-year		7 (5-9)%
Sinusoidal obstruction syndrome (SOS)	639	
100-day		16 (13-19)%
1-year		16 (13-19)%
Neutrophil engraftment	635	
100-day		87 (84-90)%
Platelet engraftment	626	
100-day		82 (79-85)%
Grade 2-4 acute graft-versus-host disease (GVHD)	640	
100-day		21 (18-24)%
Grade 3-4 acute GVHD	640	
100-day		8 (6-10)%
Chronic GVHD	632	
1-year		17 (14-20)%
3-year		19 (16-22)%
Overall survival for malignant disease	85	
1-year		67 (57-77)%
3-year		65 (54-74)%
Overall survival for non-malignant disease	556	
1-year		78 (74-81)%
3-year		73 (69-76)%

**eTable 4. Multivariable analysis of overall survival among malignant cohort**

	HR	95% CI Lower Limit	95% CI Upper Limit	P value	Overall P value	N
<b>Main effect: Year of transplant group</b>					.57	
2000-2004	1					199
2005-2009	0.84	0.61	1.17	.30		161
2010-2014	0.90	0.63	1.30	.58		112
<b>Age</b>					.06	
< 6 months	1					61
> 6 months	0.69	0.47	1.01	.06		411
<b>Malignant disease type</b>					.0012	
Acute myelogenous leukemia (AML)	1					165
Acute lymphoblastic leukemia (ALL) and other acute leukemias	1.45	1.06	1.98	.02		182
Myelodysplastic syndrome/myeloproliferative disorder (MDS/MPD)	0.79	0.52	1.19	.26		106
Other leukemias, chronic myelogenous leukemia (CML), and other malignancies	0.32	0.10	1.04	.06		19
<b>Contrast</b>						
2005-2009 vs 2010-2014	0.93	0.63	1.38	.72		
ALL and other acute leukemias vs MDS/MPD	1.84	1.24	2.73	.002		
ALL and other acute leukemias vs Other leukemias, CML, and other malignancies	4.49	1.41	14.31	.01		
MDS/MPD vs Other leukemias, CML, and other malignancies	2.44	0.74	8.01	.14		

**eTable 5. Multivariable analysis of overall survival among non-malignant cohort, including donor type**

	HR	95% CI Lower Limit	95% CI Upper Limit	P value	Overall P value	N
<b>Main effect: Year of transplant group</b>					.006	577
2000-2004	1					699
2005-2009	0.77	0.63	0.93	.007		750
2010-2014	0.74	0.60	0.91	.004		
<b>Age</b>					.91	
< 6 months	1					912
> 6 months	0.99	0.84	1.17	.91		1114
<b>Donor Type</b>					< .0001	
HLA-identical sibling	1					444
UCB (HLA 6/6)	1.38	0.95	2.01	.09		155
UCB (HLA ≤ 5/6)	2.03	1.55	2.65	< .0001		401
HLA-matched/mismatched other relative	1.66	1.25	2.21	.0004		349
HLA-matched unrelated (HLA 8/8 URD)	1.26	0.91	1.76	0.16		247
HLA-mismatched unrelated (HLA ≤ 7/8 URD)	1.55	1.06	2.27	0.02		115
Not reported	1.79	1.35	2.38	< .0001		315
<b>Non-malignant disease type (≤ 4 months)</b>					< 0.0001	
SCID	1					834
Non-SCID	0.60	0.41	0.86	.006		378
Osteopetrosis	2.07	1.48	2.89	< .0001		143
Mucopolysaccharidosis	0.99	0.69	1.42	.95		212
Histiocytic disorder	1.47	1.11	1.95	.007		360
Others	1.68	1.08	2.61	.02		99

	HR	95% CI Lower Limit	95% CI Upper Limit	P value	Overall P value	N
<b>Non-malignant disease type (&gt; 4 months)</b>					< .0001	
SCID	1					147
Non-SCID	0.82	0.56	1.22	.33		43
Osteopetrosis	2.05	1.28	3.27	.003		52
Mucopolysaccharidosis	2.04	1.44	2.89	< .0001		42
Histiocytic disorder	1.30	0.90	1.87	.16		84
Others	0.51	0.21	1.27	.15		24
<b>Contrast</b>						
2005-2009 vs. 2010-2014	1.04	0.85	1.27	.71		
UCB (HLA 6/6) vs. UCB (HLA ≤ 5/6)	0.68	0.48	0.96	.03		
UCB (HLA 6/6) vs. HLA-matched/mismatched other relative	0.83	0.57	1.20	.32		
UCB (HLA 6/6) vs. HLA (8/8 URD)	1.09	0.73	1.63	.66		
UCB (HLA 6/6) vs. HLA (≤ 7/8 URD)	0.89	0.57	1.38	.61		
UCB (HLA 6/6) vs. Not reported	0.77	0.54	1.11	.16		
UCB (HLA ≤ 5/6) vs. HLA-matched/mismatched other relative	1.22	0.94	1.58	.13		
UCB (HLA ≤ 5/6) vs. HLA (8/8 URD)	1.61	1.19	2.16	.002		
UCB (HLA ≤ 5/6) vs. HLA (≤ 7/8 URD)	1.31	0.92	1.86	.13		
UCB (HLA ≤ 5/6) vs. Not reported	1.13	0.89	1.45	.31		
HLA-matched/mismatched other relative vs. HLA (8/8 URD)	1.32	0.95	1.82	.10		
HLA-matched/mismatched other relative vs. HLA (≤ 7/8 URD)	1.07	0.74	1.56	.71		
HLA-matched/mismatched other relative vs. Not reported	0.93	0.71	1.22	.60		
HLA (8/8 URD) vs. HLA (≤ 7/8 URD)	0.81	0.55	1.22	.32		
HLA (8/8 URD) vs. Not reported	0.71	0.51	0.97	.03		
HLA (≤ 7/8 URD) vs. Not reported	0.87	0.60	1.24	.44		
Non-SCID vs. Osteopetrosis (< 5 months)	0.29	0.19	0.44	< .0001		

	HR	95% CI Lower Limit	95% CI Upper Limit	P value	Overall P value	N
Non-SCID vs. Mucopolysaccharidosis(< 5 months)	0.60	0.38	0.95	.03		
Non-SCID vs. Histiocytic disorder (< 5 months)	0.41	0.27	0.60	< .0001		
Non-SCID vs. Others (< 5 months)	0.35	0.21	0.59	< .0001		
Osteopetrosis vs. Mucopolysaccharidosis(< 5 months)	2.10	1.37	3.21	.0007		
Osteopetrosis vs. Histiocytic disorder (< 5 months)	1.40	0.98	2.02	.07		
Osteopetrosis vs. Others (< 5 months)	1.23	0.75	2.02	.41		
Mucopolysaccharidosis vs. Histiocytic disorder (< 5 months)	0.67	0.46	0.98	.04		
Mucopolysaccharidosis vs. Others (< 5 months)	0.58	0.35	0.98	.04		
Histiocytic disorder vs. Others (< 5 months)	0.88	0.56	1.38	.57		
Non-SCID vs. Osteopetrosis (> 5 months)	0.40	0.24	0.68	.0008		
Non-SCID vs. Mucopolysaccharidosis(> 5 months)	0.40	0.27	0.62	< .0001		
Non-SCID vs. Histiocytic disorder (> 5 months)	0.63	0.41	0.98	.04		
Non-SCID vs. Others (> 5 months)	1.60	0.63	4.08	.32		
Osteopetrosis vs. Mucopolysaccharidosis(> 5 months)	1.00	0.61	1.66	.99		
Osteopetrosis vs. Histiocytic disorder (> 5 months)	1.58	0.94	2.63	.08		
Osteopetrosis vs. Others (> 5 months)	3.98	1.50	10.52	.005		
Mucopolysaccharidosis vs. Histiocytic disorder (> 5 months)	1.57	1.06	2.33	.03		
Mucopolysaccharidosis vs. Others (> 5 months)	3.96	1.58	9.94	.003		
Histiocytic disorder vs. Others (> 5 months)	2.53	1.00	6.37	.05		

Abbreviations: HLA, human leukocyte antigen; N/A, not applicable; SCID, severe combined immunodeficiency; UCB, umbilical cord blood; URD, unrelated donor

**eTable 6. Multivariable analysis of overall survival among non-malignant cohort, including graft source**

	HR	95% CI Lower Limit	95% CI Upper Limit	P value	Overall P value	N
<b>Main effect: Year of transplant group</b>					.004	577
2000-2004	1					699
2005-2009	0.76	0.63	0.92	.006		750
2010-2014	0.74	0.60	0.90	.003		
<b>Age</b>					.97	
< 6 months	1					912
> 6 months	1.00	0.85	1.17	.97		1114
<b>Graft Source</b>					< .0001	
Bone Marrow	1					985
Peripheral blood	1.50	1.17	1.91	.001		258
Umbilical cord blood	1.59	1.33	1.90	< .0001		783
<b>Non-malignant disease type (≤ 4 months)</b>					< .0001	
Severe combined immunodeficiency (SCID)	1					834
Non-SCID	0.58	0.40	0.84	.004		378
Osteopetrosis	2.03	1.46	2.84	< .0001		143
Mucopolysaccharidosis	0.97	0.67	1.39	.86		212
Histiocytic disorder	1.48	1.12	1.95	.006		360
Others	1.67	1.08	2.59	.02		99
<b>Non-malignant disease type (&gt; 4 months)</b>					< .0001	
SCID	1					147
Non-SCID	0.81	0.55	1.19	.28		43
Osteopetrosis	2.02	1.26	3.22	.003		52

	HR	95% CI Lower Limit	95% CI Upper Limit	P value	Overall P value	N
Mucopolysaccharidosis	2.00	1.41	2.83	.0001		42
Histiocytic disorder	1.30	0.91	1.87	.15		84
Others	0.51	0.21	1.27	.15		24
<b>Contrast</b>						
2005-2009 vs. 2010-2014	1.03	0.85	1.26	.75		
Peripheral blood vs. Umbilical cord blood	0.94	0.74	1.20	.63		
Non-SCID vs. Osteopetrosis (< 5 months)	0.29	0.19	0.44	< .0001		
Non-SCID vs. Mucopolysaccharidosis (< 5 months)	0.60	0.38	0.95	.03		
Non-SCID vs. Histiocytic disorder (< 5 months)	0.40	0.27	0.58	< .0001		
Non-SCID vs. Others (< 5 months)	0.35	0.21	0.59	< .0001		
Osteopetrosis vs. Mucopolysaccharidosis (< 5 months)	2.10	1.37	3.22	.0007		
Osteopetrosis vs. Histiocytic disorder (< 5 months)	1.38	0.96	1.97	.08		
Osteopetrosis vs. Others (< 5 months)	1.22	0.74	2.0	.43		
Mucopolysaccharidosis vs. Histiocytic disorder (< 5 months)	0.66	0.45	0.96	.03		
Mucopolysaccharidosis vs. Others (< 5 months)	0.58	0.35	0.97	.04		
Histiocytic disorder vs. Others (< 5 months)	0.89	0.56	1.39	.60		
Non-SCID vs. Osteopetrosis (> 5 months)	0.40	0.24	0.68	.0007		
Non-SCID vs. Mucopolysaccharidosis (> 5 months)	0.40	0.26	0.62	< .0001		
Non-SCID vs. Histiocytic disorder (> 5 months)	0.62	0.40	0.95	.03		
Non-SCID vs. Others (> 5 months)	1.57	0.62	4.00	.34		
Osteopetrosis vs. Mucopolysaccharidosis (> 5 months)	1.01	0.61	1.67	.97		
Osteopetrosis vs. Histiocytic disorder (> 5 months)	1.55	0.93	2.58	.09		
Osteopetrosis vs. Others (> 5 months)	3.92	1.48	10.37	.006		
Mucopolysaccharidosis vs. Histiocytic disorder (> 5 months)	1.53	1.03	2.28	.04		
Mucopolysaccharidosis vs. Others (> 5 months)	3.89	1.55	9.76	.004		
Histiocytic disorder vs. Others (> 5 months)	2.54	1.01	6.39	.05		

**eTable 7. Multivariable analysis of transplant-related mortality among malignant cohort**

	HR	95% CI Lower Limit	95% CI Upper Limit	P value	Overall P value	N
<b>Main effect: year of transplant group</b>					.15	
2000-2004	1					176
2005-2009	0.75	0.47	1.18	.21		157
2010-2014	0.59	0.33	1.05	.07		112
<b>Age</b>					.26	
< 6 months	1					55
> 6 months	0.72	0.41	1.28	.26		389
<b>Malignant disease type</b>					.0005	
Acute myelogenous leukemia (AML)	1					158
Acute lymphoblastic leukemia (ALL) and other acute leukemias	3.08	1.77	5.34	.0001		166
Myelodysplastic syndrome/myeloproliferative disorder (MDS/MPD)	2.29	1.24	4.26	.008		103
Other leukemias, CML, and other malignancies	0.87	0.20	3.79	.85		18
<b>Contrast</b>						
2005-2009 vs 2010-2014	1.26	0.68	2.33	.46		
ALL and other acute leukemias vs MDS/MPD	1.34	0.83	2.17	.23		
ALL and other acute leukemias vs Other leukemias, CML, and other malignancies	3.56	0.85	14.86	.08		
MDS/MPD vs Other leukemias, CML, and other malignancies	2.65	0.62	11.37	.19		

Abbreviations: ALL, acute lymphoblastic leukemia; AML, acute myelogenous leukemia; CML, chronic myelogenous leukemia; MDS/MPD, myelodysplastic syndrome/myeloproliferative disorder

**eTable 8. Multivariable analysis of relapse among malignant cohort**

	HR	95% CI Lower Limit	95% CI Upper Limit	P value	Overall P value	N
<b>Main effect: Year of transplant group</b>					.001	
2000-2004	1					173
2005-2009	1.32	0.83	2.10	.25		157
2010-2014	2.34	1.47	3.75	.0004		112
<b>Age</b>					.66	
< 6 months	1					56
> 6 months	0.89	0.51	1.53	.66		389
<b>Malignant disease type</b>					.006	
Acute myelogenous leukemia (AML)	1					158
Acute lymphoblastic leukemia (ALL) and other acute leukemias	0.76	0.49	1.18	.22		166
Myelodysplastic syndrome/myeloproliferative disorder (MDS/MPD)	0.36	0.20	0.67	.001		103
Other leukemias, CML, and other malignancies	0.30	0.07	1.26	.10		18
<b>TBI</b>					.003	
No	1					356
Yes	1.90	1.24	2.92	.003		116
<b>Contrast</b>						
2005-2009 vs 2010-2014	0.56	0.36	0.89	.01		
ALL and other acute leukemias vs MDS/MPD	2.10	1.10	4.01	.03		
ALL and other acute leukemias vs Other leukemias, CML, and other malignancies	2.50	0.59	10.57	.21		
MDS/MPD vs Other leukemias, CML, and other malignancies	1.19	0.27	5.36	.82		

Abbreviations: ALL, acute lymphoblastic leukemia; AML, acute myelogenous leukemia; CML, chronic myelogenous leukemia; MDS/MPD, myelodysplastic syndrome/myeloproliferative disorder

**eTable 9. Multivariable analysis of disease-free survival among malignant cohort**

	HR	95% CI Lower Limit	95% CI Upper Limit	P value	Overall P value	N
<b>Main effect: Year of transplant group</b>					.38	
2000-2004	1					176
2005-2009	0.98	0.71	1.36	.93		157
2010-2014	1.24	0.88	1.75	.22		112
<b>Age</b>					.26	
< 6 months	1					56
> 6 months	0.80	0.54	1.18	.26		389
<b>Malignant disease type</b>					.001	
Acute myelogenous leukemia (AML)	1					158
Acute lymphoblastic leukemia (ALL) and other acute leukemias	1.51	1.10	2.06	.01		166
Myelodysplastic syndrome/myeloproliferative disorder (MDS/MPD)	0.83	0.55	1.23	.35		103
Other leukemias, CML, and other malignancies	0.45	0.16	1.23	.12		18
<b>Contrast</b>						
2005-2009 vs 2010-2014	0.79	0.55	1.14	.21		
ALL and other acute leukemias vs MDS/MPD	1.82	1.25	2.66	.002		
ALL and other acute leukemias vs Other leukemias, CML, and other malignancies	3.38	1.23	9.30	.02		
MDS/MPD vs Other leukemias, CML, and other malignancies	1.85	0.65	5.25	.25		

Abbreviations: ALL, acute lymphoblastic leukemia; AML, acute myelogenous leukemia; CML, chronic myelogenous leukemia; MDS/MPD, myelodysplastic syndrome/myeloproliferative disorder

**eTable 10. Causes of death**

<b>Cause of death, n (%)</b>	<b>Malignant disease</b>	<b>Non-malignant disease</b>
Primary disease	86 (43)	64 (10)
New malignancy	1 (1)	2 (<1)
Graft-versus-host disease	10 (5)	34 (5)
Idiopathic pneumonia syndrome	10 (5)	41 (7)
Infection	18 (9)	104 (17)
Organ failure	33 (17)	156 (25)
Others	33 (17)	206 (33)
Not reported	8 (4)	14 (2)
Total	199	621

**eTable 11. Breakdown of primary disease as cause of death**

<b>Cause of death: primary disease, n (%)</b>	<b>Malignant disease</b>	<b>Non-malignant disease</b>
Acute myelogenous leukemia	42 (49)	0
Acute lymphoblastic leukemia	30 (35)	0
Other leukemia	1 (1)	0
Myelodysplastic syndrome/ myeloproliferative disorder	8 (9)	0
Other acute leukemia	5 (6)	0
Severe combined immunodeficiency (SCID)	0	16 (25)
Non-SCID	0	6 (9)
Osteopetrosis	0	9 (14)
Mucopolysaccharidosis and other storage disorders	0	18 (28)
Histiocytic disorder	0	14 (22)
Others	0	1 (2)
Total	86	64

**eTable 12. Breakdown of organ failure as cause of death**

<b>Cause of death: organ failure, n (%)</b>	<b>Malignant disease</b>	<b>Non-malignant disease</b>
Organ failure	1 (3)	5 (3)
Liver failure	2 (6)	9 (6)
Veno-occlusive disease (SOS)	5 (15)	26 (17)
Cardiac failure	2 (6)	17 (11)
Pulmonary failure	10 (30)	52 (33)
Central nervous system (CNS) failure	0	3 (2)
Renal failure	0	2 (1)
Multiple organ failure	12 (36)	36 (23)
Other organ failure	0	4 (3)
Lymphoproliferative disorder	1 (3)	2 (1)
Total	33	156

**eTable 13. Breakdown of “other” as cause of death**

<b>Cause of death: other, n (%)</b>	<b>Malignant disease</b>	<b>Non-malignant disease</b>
Graft rejection or failure	1 (3)	11 (5)
Hemorrhage	0	1 (<1)
Pulmonary	3 (9)	16 (8)
Intracranial	1 (3)	7 (3)
Gastrointestinal	1 (3)	3 (1)
Other hemorrhage	0	3 (1)
Accidental death	1 (3)	0
Other vascular	0	2 (1)
Prior malignancy	2 (6)	0
Other transplant-related cause	11 (33)	52 (25)
Other cause	13 (39)	111 (54)
<b>Total</b>	<b>33</b>	<b>206</b>

**eTable 14 Cause of death in malignant vs. non-malignant split by year of transplant**

Time period	Malignant cohort N (%)			Non-malignant cohort N (%)			All N (%)
	2000-2004	2005-2009	2010-2014	2000-2004	2005-2009	2010-2014	All years
Number of total patients	199	161	112	577	699	750	2498
Number of deaths	93	62	44	227	207	187	820
Cause of death							
Primary disease	34 (37)	30 (48)	22 (50)	26 (11)	16 (8)	22 (12)	150 (18)
New malignancy	1 (1)	0	0	1 (<1)	0	1 (<1)	3 (<1)
GVHD	7 (8)	2 (3)	1 (2)	21 (9)	6 (3)	7 (4)	44 (5)
IPS	5 (5)	2 (3)	3 (7)	17 (7)	14 (7)	10 (5)	51 (6)
Infection	9 (10)	6 (10)	3 (7)	46 (20)	35 (17)	23 (12)	122 (15)
Organ failure	12 (13)	16 (26)	5 (11)	50 (22)	72 (35)	34 (18)	189 (23)
Other cause	18 (19)	5 (8)	10 (23)	56 (25)	61 (29)	89 (48)	239 (29)
Not reported	7 (8)	1 (2)	0	10 (4)	3 (1)	1 (<1)	22 (3)

Abbreviations: GVHD, graft-versus-host-disease; IPS, idiopathic pneumonia syndrome

**eTable 15. Multivariable analysis sinusoidal obstruction syndrome in non-malignant cohort (including donor of type)**

	HR	CI Lower Limit	CI Upper Limit	P value	Overall P value	N
<b>Main effect: Year of transplant group</b>					.38	
2000-2004	1					357
2005-2009	1.09	0.69	1.72	.70		475
2010-2014	1.36	0.87	2.12	.18		353
<b>Age</b>					.27	
< 6 months	1					561
> 6 months	1.19	0.87	1.63	.27		624
<b>Conditioning regimen intensity</b>					< .0001	
MAC	1					845
RIC/NMA	0.11	0.04	0.28	< .0001		242
Not reported	0.05	0.01	0.46	.008		98
<b>Donor Type</b>					.10	
HLA-identical sibling	1					190
UCB (HLA 6/6)	1.54	0.72	3.31	.26		130
UCB (HLA ≤ 5/6)	0.81	0.43	1.53	.52		363
HLA-matched/mismatched other relative	1.56	0.97	2.50	.06		167
HLA-matched unrelated (HLA 8/8 URD)	1.09	0.47	2.50	.85		157
HLA-mismatched unrelated (HLA ≤ 7/8 URD)	1.08	0.42	2.79	.87		78
Not reported	1.37	0.61	3.10	.45		100
<b>Non-malignant disease type</b>					.0006	
Immune deficiencies: SCID	1					531

	HR	CI Lower Limit	CI Upper Limit	P value	Overall P value	N
Immune deficiencies: Non-SCID	0.98	0.60	1.60	.94		196
Inherited disorder of metabolism: osteopetrosis	2.60	1.50	4.52	.001		79
Inherited disorder of metabolism: mucopolysaccharidosis	0.69	0.35	1.36	.28		138
Histiocytic disorder	1.66	0.95	2.88	.07		188
Others	1.57	0.79	3.14	.20		53
<b>Contrast</b>						
2005-2009 vs 2010-2014	0.80	0.52	1.25	.33		
RIC/NMA vs Not reported	1.97	0.22	18.06	.55		
UCB (HLA 6/6) vs UCB (HLA ≤ 5/6)	1.90	1.13	3.19	.01		
UCB (HLA 6/6) vs HLA-matched/mismatched other relative	0.99	0.50	1.96	.98		
UCB (HLA 6/6) vs HLA-matched unrelated (HLA 8/8 URD)	1.42	0.75	2.69	.28		
UCB (HLA 6/6) vs HLA-mismatched unrelated (HLA ≤ 7/8 URD)	1.43	0.63	3.22	.39		
UCB (HLA 6/6) vs Not reported	1.13	0.55	2.32	.74		
UCB (HLA ≤ 5/6) vs HLA-matched/mismatched other relative	0.52	0.28	0.95	.03		
UCB (HLA ≤ 5/6) vs HLA-matched unrelated (HLA 8/8 URD)	0.75	0.40	1.41	.37		
UCB (HLA ≤ 5/6) vs HLA-mismatched unrelated (HLA ≤ 7/8 URD)	0.75	0.35	1.63	.47		
UCB (HLA ≤ 5/6) vs Not reported	0.59	0.31	1.13	.11		
HLA-matched/mismatched other relative vs HLA- matched unrelated (HLA 8/8 URD)	1.44	0.67	3.10	.36		
HLA-matched/mismatched other relative vs HLA- mismatched unrelated (HLA ≤ 7/8 URD)	1.44	0.62	3.36	.39		
HLA-matched/mismatched other relative vs Not reported	1.14	0.51	2.52	.75		
HLA-matched unrelated (HLA 8/8 URD) vs HLA- mismatched unrelated (HLA ≤ 7/8 URD)	1.00	0.43	2.37	.99		

	<b>HR</b>	<b>CI Lower Limit</b>	<b>CI Upper Limit</b>	<b>P value</b>	<b>Overall P value</b>	<b>N</b>
HLA-matched unrelated (HLA 8/8 URD) vs Not reported	0.79	0.37	1.70	.55		
HLA-mismatched unrelated (HLA $\leq$ 7/8 URD) vs Not reported	0.79	0.37	1.70	.55		
Immune deficiencies: Non-SCID vs Inherited disorder of metabolism: osteopetrosis	0.38	0.22	0.65	.0005		
Immune deficiencies: Non-SCID vs Inherited disorder of metabolism: mucopolysaccharidosis	1.42	0.69	2.92	.34		
Immune deficiencies: Non-SCID vs Histiocytic disorder	0.59	0.34	1.02	.06		
Immune deficiencies: Non-SCID vs Other	0.62	0.29	1.35	.23		
Inherited disorder of metabolism: osteopetrosis vs Inherited disorder of metabolism: mucopolysaccharidosis	3.78	1.82	7.81	.0003		
Inherited disorder of metabolism: osteopetrosis vs Histiocytic disorder	1.57	0.90	2.75	.12		
Inherited disorder of metabolism: osteopetrosis vs Other	1.66	0.83	3.32	.15		
Inherited disorder of metabolism: mucopolysaccharidosis vs Histiocytic disorder	0.42	0.22	0.78	.006		
Inherited disorder of metabolism: mucopolysaccharidosis vs Other	0.44	0.19	1.03	.06		
Histiocytic disorder vs Other	1.06	0.51	2.18	.88		

Abbreviations: HLA, human leukocyte antigen; MAC, myeloablative conditioning; NMA, nonmyeloablative conditioning; RIC, reduced-intensity conditioning SCID, severe combined immunodeficiency; UCB, umbilical cord blood; URD, unrelated donor

**eTable 16. Multivariable analysis sinusoidal obstruction syndrome in non-malignant cohort (including graft of source)**

	HR	CI Lower Limit	CI Upper Limit	P value	Overall P value	N
<b>Main effect: Year of transplant group</b>					.41	
2000-2004	1					356
2005-2009	1.14	0.74	1.74	.56		475
2010-2014	1.34	0.87	2.04	.18		353
<b>Age</b>					.25	
< 6 months	1					560
> 6 months	1.20	0.88	1.65	.25		624
<b>Conditioning regimen intensity</b>					< .0001	
MAC	1					844
RIC/NMA	0.11	0.04	0.27	< .0001		242
Not reported	0.06	0.007	0.45	.007		98
<b>Graft Source</b>					.22	
Bone Marrow	1					469
Peripheral blood	1.45	0.83	2.54	.20		135
Umbilical cord blood	0.91	0.62	1.35	.65		580
<b>Non-malignant disease type</b>					.005	
Immune deficiencies: SCID	1					530
Immune deficiencies: Non-SCID	1.01	0.63	1.63	.95		196
Inherited disorder of metabolism: osteopetrosis	2.45	1.36	4.40	.003		79
Inherited disorder of metabolism: mucopolysaccharidosis	0.71	0.35	1.44	.34		138
Histiocytic disorder	1.66	0.96	2.86	.07		188

	HR	CI Lower Limit	CI Upper Limit	P value	Overall P value	N
Others	1.55	0.76	3.14	.23		53
<b>Contrast</b>						
2005-2009 vs 2010-2014	0.85	0.55	1.32	.47		
RIC/NMA vs Not reported	1.83	0.20	16.49	.59		
Peripheral blood vs. Umbilical cord blood	1.59	0.94	2.66	.08		
Immune deficiencies: Non-SCID vs Inherited disorder of metabolism: osteopetrosis	0.41	0.24	0.73	.002		
Immune deficiencies: Non-SCID vs Inherited disorder of metabolism: mucopolysaccharidosis	1.43	0.69	2.98	.33		
Immune deficiencies: Non-SCID vs Histiocytic disorder	0.61	0.36	1.03	.07		
Immune deficiencies: Non-SCID vs Other	0.66	0.30	1.41	.28		
Inherited disorder of metabolism: osteopetrosis vs Inherited disorder of metabolism: mucopolysaccharidosis	3.46	1.61	7.44	.001		
Inherited disorder of metabolism: osteopetrosis vs Histiocytic disorder	1.48	0.86	2.53	.15		
Inherited disorder of metabolism: osteopetrosis vs Other	1.58	0.80	3.13	.19		
Inherited disorder of metabolism: mucopolysaccharidosis vs Histiocytic disorder	0.43	0.23	0.80	.008		
Inherited disorder of metabolism: mucopolysaccharidosis vs Other	0.46	0.19	1.11	.08		
Histiocytic disorder vs Other	1.07	0.53	2.16	.85		

Abbreviations: MAC, myeloablative conditioning; NMA, nonmyeloablative conditioning; RIC, reduced-intensity conditioning SCID, severe combined immunodeficiency;

**eTable 17. Multivariable analysis of idiopathic pneumonia syndrome in malignant cohort**

	HR	CI Lower Limit	CI Upper Limit	P value	Overall P value	N
<b>Main effect: Year of transplant group</b>					.85	
2000-2004	1					101
2005-2009	1.06	0.46	2.47	.89		103
2010-2014	0.74	0.20	2.68	.64		44
<b>Age</b>					.03	
< 6 months	1					27
> 6 months	0.36	0.14	0.92	.03		221
<b>Contrast</b>						
2005-2009 vs 2010-2014	1.45	0.40	5.19	.57		

**eTable 18. Multivariable analysis of idiopathic pneumonia syndrome in non-malignant cohort**

	HR	CI Lower Limit	CI Upper Limit	P value	Overall P value	N
<b>Main effect: Year of transplant group</b>					.59	
2000-2004	1					348
2005-2009	0.84	0.53	1.32	.44		471
2010-2014	0.77	0.46	1.30	.33		353
<b>Age</b>					.30	
< 6 months	1					554
> 6 months	1.23	0.83	1.84	.30		618
<b>Contrast</b>						
2005-2009 vs 2010-2014	1.08	0.66	1.78	.77		