UNA NUOVA STORIA:
il **TAGRAXOFUSP**
e la **BPDCN**

BOLOGNA - 2 MAGGIO 2023
STARHOTELS EXCELSIOR

INDICAZIONI PER TRAPIANTO AUTOLOGO E ALLOGENICO

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FONDAZIONE IRCCS CA' GRANDA
OSPEDALE MAGGIORE POLICLINICO
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CLINICAL AND LABORATORY INVESTIGATIONS

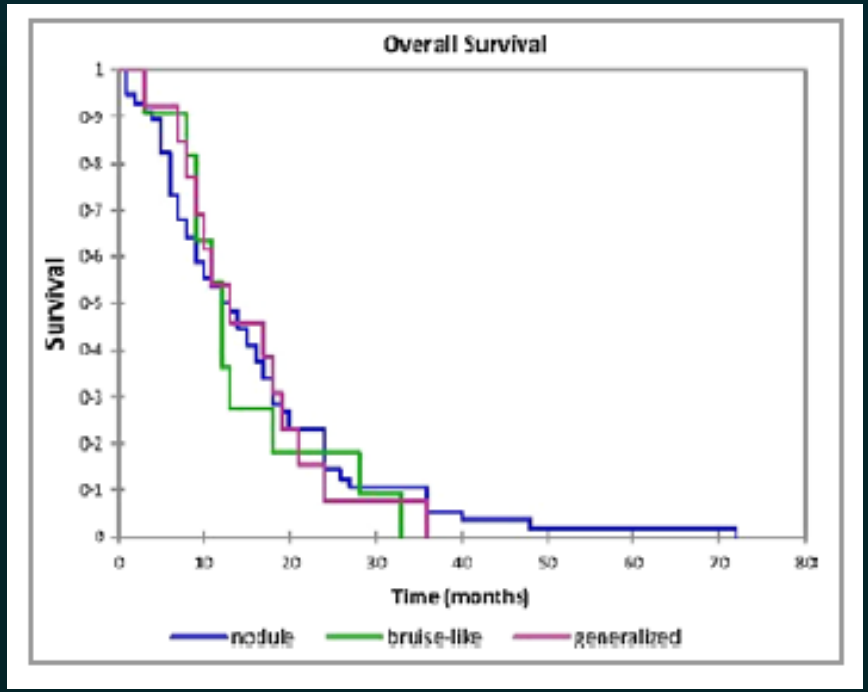
BJD
British Journal of Dermatology

Blastic plasmacytoid dendritic cell neoplasm: clinical features in 90 patients

F. Julia,[1] T. Petrella,[2,3] M. Beylot-Barry,[3,4] M. Bagot,[3,5] D. Lipsker,[3,6] L. Machet,[3,7] P. Joly,[3,8] O. Dereure,[3,9] M. Wetterwald,[3,10] M. d'Incan,[3,11] F. Grange,[3,12] J. Cornillon,[13] G. Tertian,[14] E. Maubec,[3,15] P. Saiag,[3,16] S. Barete,[3,17] I. Templier,[3,18] F. Aubin[19] and S. Dalle[1,3]

1995-2012

Number of patients	90
Sex ratio	2·2 (62 M/28 F)
Mean age (years)	67·2
Age range (years)	8–103
Clinical features, n (%)	
Generalized lesions	13 (14)
Nodular lesions	66 (73)
Bruise-like lesions	11 (12)
Initial staging, n (%)	
Negative	28 (31)
Positive	55 (61)
Unknown	7 (8)



Julia F et al . Br J Derm 2013

CLINICAL TRIALS AND OBSERVATIONS

Stem cell transplantation can provide durable disease control in blastic plasmacytoid dendritic cell neoplasm: a retrospective study from the European Group for Blood and Marrow Transplantation

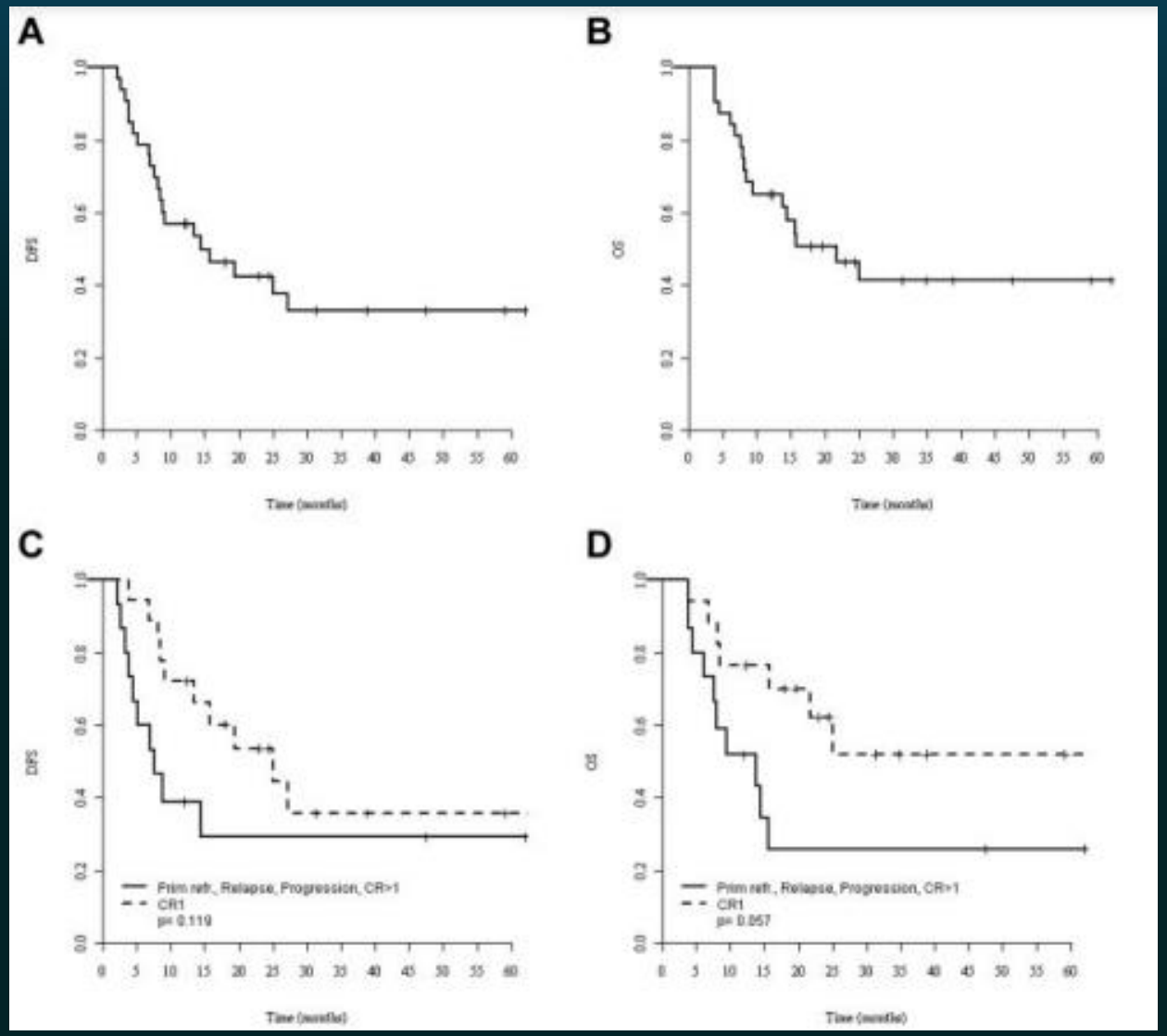
*Damien Roos-Weil,[1] *Sascha Dietrich,[2,3] Ariane Boumendil,[3] Emmanuelle Polge,[4] Dominique Bron,[5] Enric Carreras,[6] Arturo Iriondo Atienza,[7] William Arcese,[8] Dietrich W. Beelen,[9] Jan J. Cornelissen,[10] Nicolaus Kröger,[11] Giuseppe Milone,[12] Giuseppe Rossi,[13] Fabrice Jardin,[14] Christina Peters,[15] Vanderson Rocha,[16] Anna Sureda,[17] Mohamad Mohty,[18] and Peter Dreger,[2,3] on behalf of the European Group for Blood and Marrow Transplantation Lymphoma, Pediatric Diseases, and Acute Leukemia Working Parties

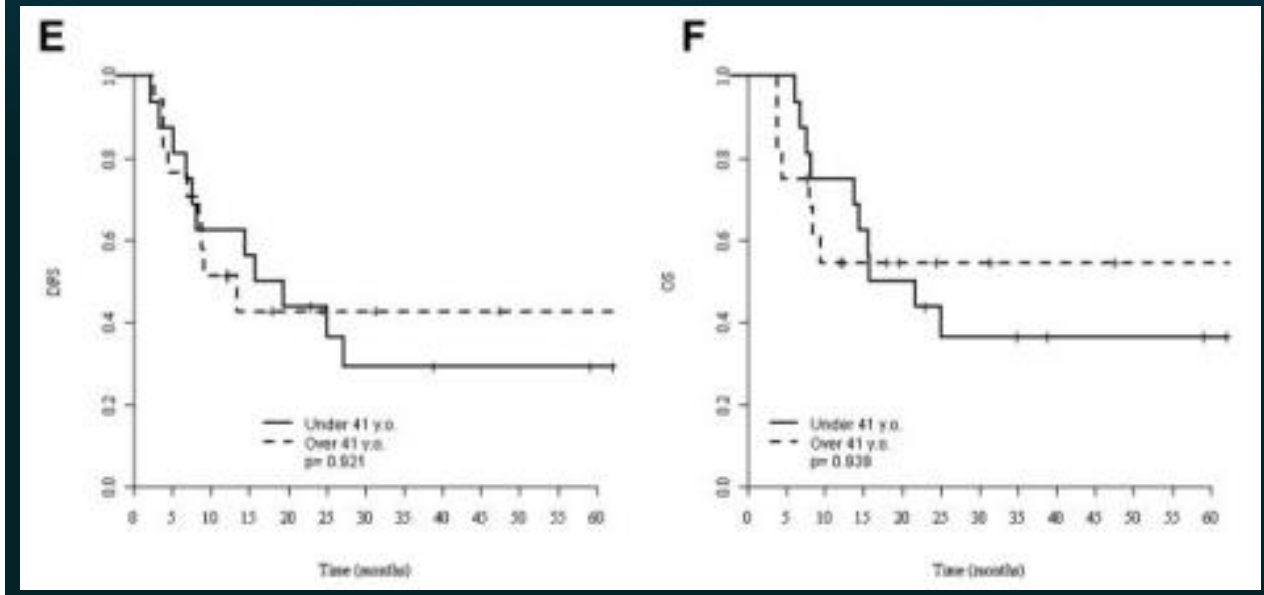
2000-2009

Characteristic	Whole population (N = 34)	MAC (n =25)	RIC (n = 9)	P*
Age at allo-SCT, y				
Median	41	36	62	.005
Range	10-70	10-64	15-70	
Sex, n (%)				
Female	13 (38)	12 (48)	1 (11)	.10
Male	21 (62)	13 (52)	8 (89)	
Clinical presentation at diagnosis, n (%)				
Skin involvement	26 (79)	21 (84)	5 (56)	.81
Lymph nodes	13 (38)	9 (36)	4 (44)	
Blood involvement	18 (53)	15 (60)	3 (33)	
BM infiltration	29 (85)	23 (92)	6 (67)	
No. of prior therapies, range	1-4	1-4	1-4	.50
Type of first-line treatment, n (%)				
AML or ALL-type	27 (82)	21 (84)	6 (67)	.35
NHL-type	7 (8)	4 (16)	3 (33)	
Time from diagnosis to allo-SCT, mo				
Median	6	6	8	.58
Range	3-63	3-63	5-16	
Status at allo-SCT, n (%)				
CR1	19 (56)	15 (60)	4 (44)	.46
> CR1 or refractory	15 (44)	10 (40)	5 (66)	
Donor type, n (%)				
Related	11 (32)	7 (28)	4 (44)	.43
Unrelated	23 (68)	18 (72)	5 (66)	
Stem cell source, n (%)				
BM	19 (56)	16 (64)	3 (33)	.18
PBSCs	9 (26)	6 (24)	3 (33)	
Cord blood	6 (18)	3 (12)	3 (33)	
T-cell depletion, n (%)				
No T-cell depletion	23 (68)	19 (76)	4 (44)	.11
Antithymocyte globulin	9 (26)	4 (16)	5 (66)	
Alemtuzumab	2 (6)	2 (8)	0 (0)	

Roos-Weil et al. Blood 2013

Outcome according to disease status and age (EBMT)



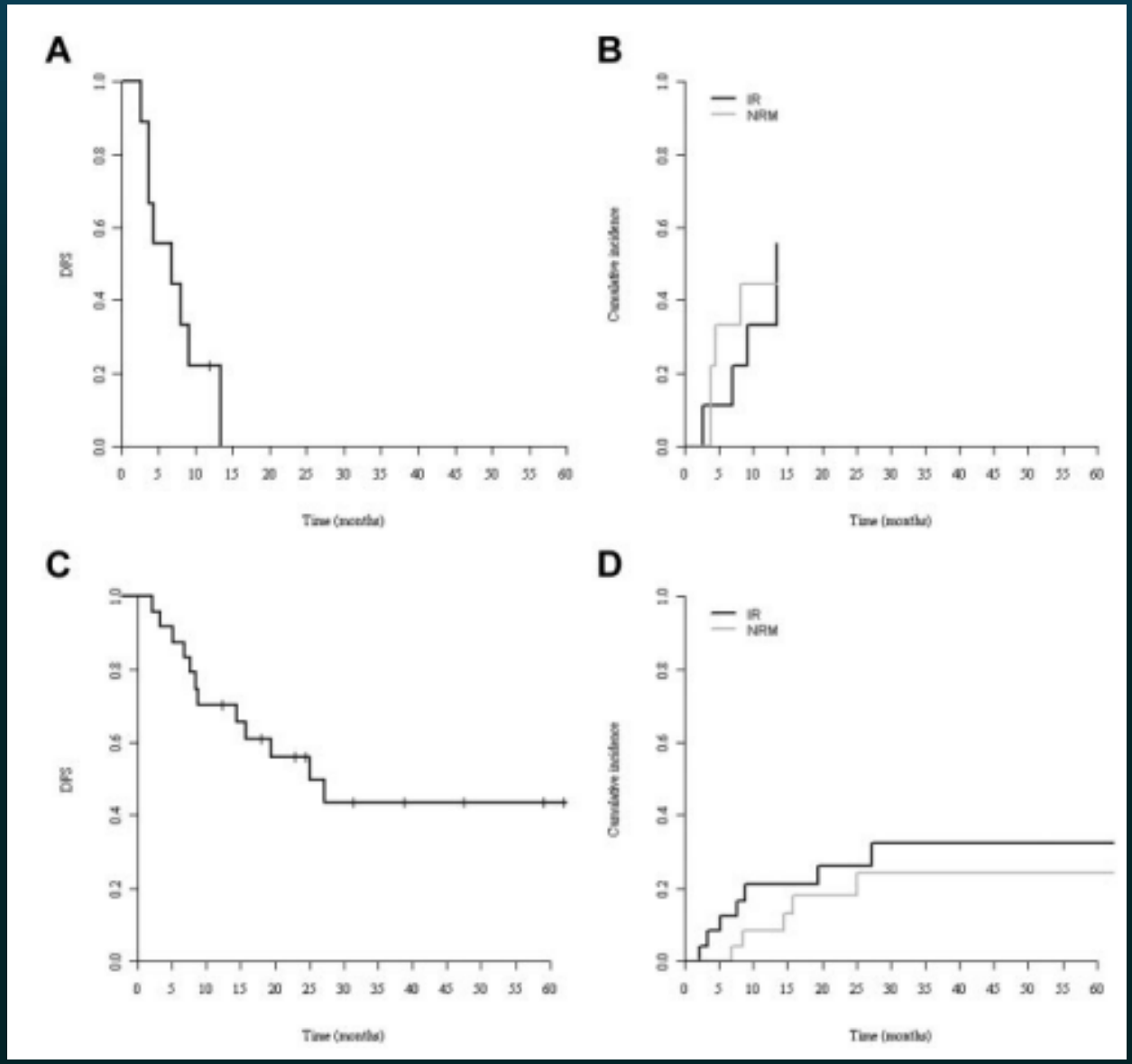


Roos-Weil et al. Blood 2013

Outcome according to conditioning intensity (EBMT)

RIC

MAC



Roos-Weil et al. Blood 2013

UVA for outcome in allo-HCT recipients (EBMT)

	NRM			Relapse			DFS			OS		
Variable	**RR**	**95% CI**	***P***	**RR**	**95% CI**	***P***	**RR**	**95% CI**	***P***	**RR**	**95% CI**	***P***
Age at allo-SCT, y												
< 41	1	0.27-3.23	.90	1	0.27-2.67	.77	1	0.39-2.33	.92	1	0.37-2.55	.95
≥ 41	0.93			0.84			0.96			0.97		
Status at allo-SCT												
CR1	1	0.16-2.44	.49	1	1.02-9.99	.05	1	0.82-4.86	.13	1	0.94-6.75	.06
> CR1 or refractory	0.63			3.19			2			2.53		
Donor type												
Related	1	0.33-7.72	.55	1	0.34-4.52	.74	1	0.56-4.25	.40	1	0.61-5.77	.27
Unrelated	1.61			1.24			1.53			1.88		
Conditioning regimens												
RIC	1	0.09-1.16	.08	1	0.17-1.85	.35	1	0.07-0.58	.003	1	0.07-0.63	.005
MAC	0.32			0.57			0.21			0.22		
Stem cell source												
BM	1		.70	1		.80	1		.93	1		.89
PBSCs	0.77	0.20-2.99		1.24	0.32-4.82		1.09	0.40-2.98		1.26	0.43-3.72	
Cord blood	1.33	0.24-7.42		0.46	0.05-4.06		0.86	0.21-3.44		0.99	0.19-5.13	
Chronic GVHD												
Absence	1		.59	1		.54	1		.46	1		.83
Presence	1.49	0.35-6.43		1.61	0.35-7.41		1.55	0.54-4.44		1.12	0.40-3.16	

Roos-Weil et al. Blood 2013

CLINICAL TRIALS AND OBSERVATIONS

Long-term survival following autologous and allogeneic stem cell transplantation for blastic plasmacytoid dendritic cell neoplasm

Tomohiro Aoki,[1,2] Ritsuro Suzuki,[3] Yachiyo Kuwatsuka,[4] Shinichi Kako,[5] Katsuya Fujimoto,[6] Jun Taguchi,[7] Tadakazu Kondo,[8] Kinya Ohata,[9] Toshiro Ito,[10] Yoshimasa Kamoda,[11] Takahiro Fukuda,[12] Tatsuo Ichinohe,[13] Kengo Takeuchi,[14] Koji Izutsu,[15] and Junji Suzumiya[16]

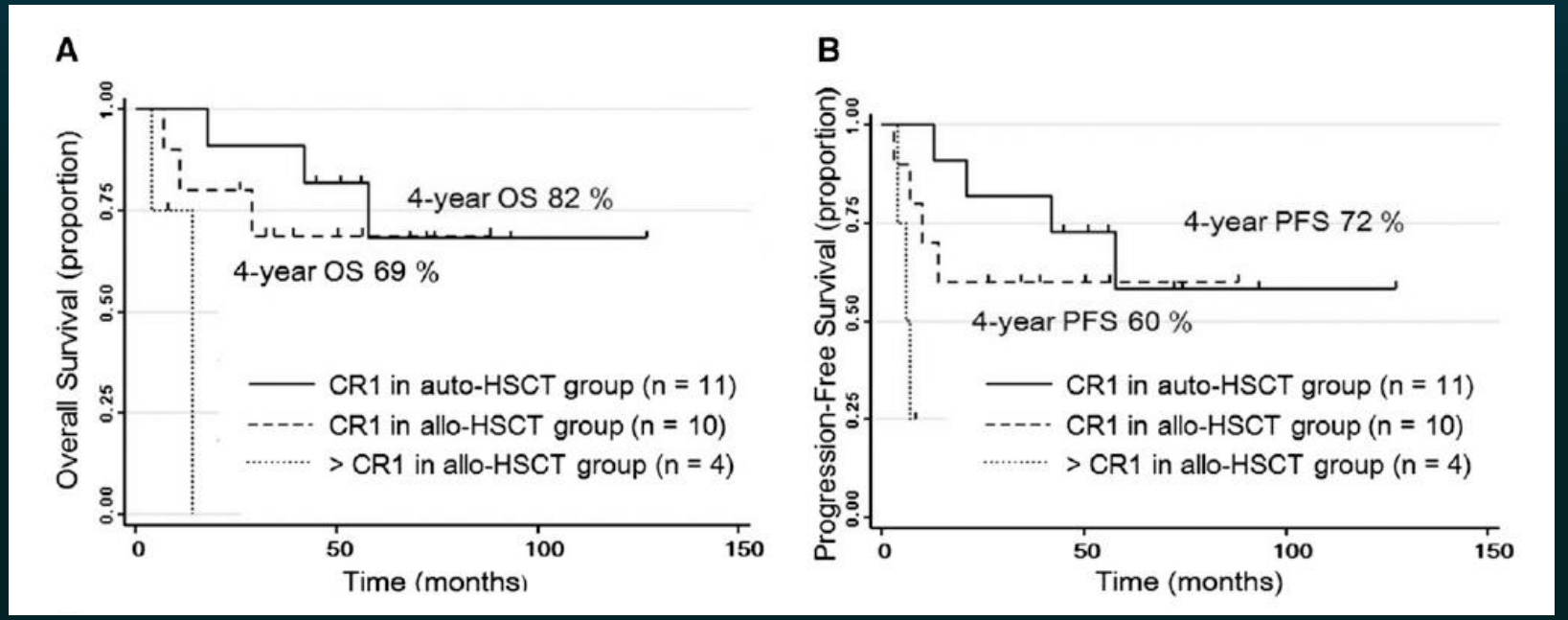
2002-2014

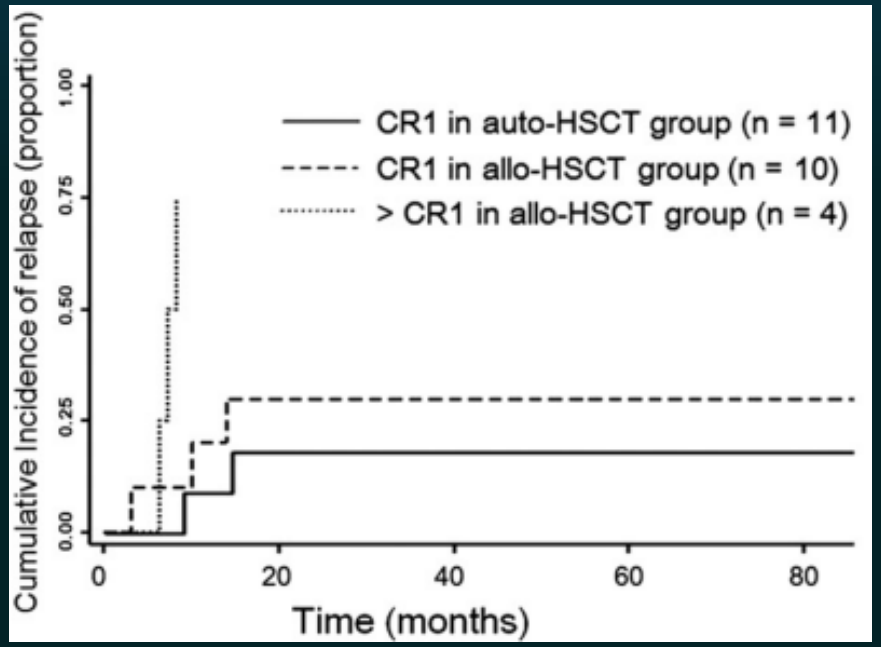
Characteristic	All No.	All %	Auto-HSCT No.	Auto-HSCT %	Allo-HSCT No.	Allo-HSCT %	*P* value
Patient number	25		11		14		
Age at HSCT (years)							
Median	58		57		58		.891
Range	17-67		19-67		17-64		
>60 years	9	36	3	27	6	43	.335
Sex, Male	20	80	9	82	11	79	.622
PS, ≥2 at HSCT	2	8	1	9	1	7	.697
Clinical presentation at diagnosis							
Skin	22	88	10	91	12	86	.593
Lymph nodes	12	48	4	36	8	57	.265
Peripheral blood	7	28	2	18	5	36	.496
Bone marrow	17	68	8	73	9	64	.496
Induction treatment							
NHL-like	11	44	7	64	4	29	.089
ALL-like	10	40	4	36	6	43	.534
AML-like	4	16	0	0	4	29	.079
Disease status at HSCT							
CR1	21	84	11	100	10	71	.079
CR2	2	8	0	0	2	14	
Refractory	2	8	0	0	2	14	
Time from diagnosis to HSCT, months							
Median	6		6		5		.496
Range	2-22		2-7		2-22		

Characteristic	All No.	All %	Auto-HSCT No.	Auto-HSCT %	Allo-HSCT No.	Allo-HSCT %
Donor type						
Related					7	50
Unrelated					7	50
Stem cell source						
Peripheral blood					5	36
Bone marrow					8	57
Cord blood					1	7
Myeloablative conditioning					8	57
TBI+CY					4	29
TBI+CY+CA					1	7
Other TBI based					1	7
BU based					2	14
Reduced-intensity conditioning					6	43
Flu+Bu+TBI					2	14
Flu+Mel+TBI					1	7
Flu+Bu					1	7
Flu+Mel+TBI					1	7
Other Flu based					1	7

Aoki T et al. Blood 2015

Outcome according to type of transplant and disease status





Aoki T et al. Blood 2015

bjh research paper

Haematopoietic cell transplantation for blastic plasmacytoid dendritic cell neoplasm: a North American multicentre collaborative study

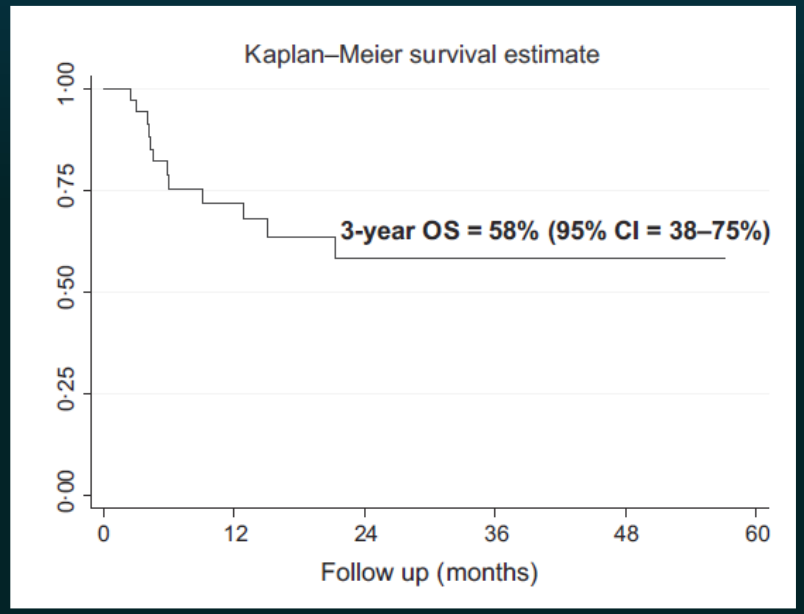
2000-2017

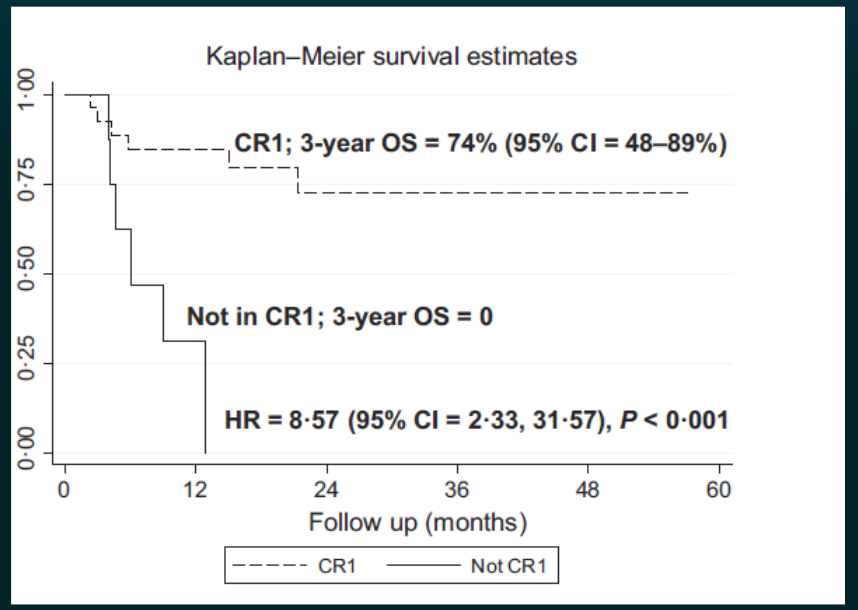
Characteristics	Allo-HCT (N = 37)	Auto-HCT (N = 8)
Patient median age (range), years	50 (14–74)	67 (45–72)
Donor median age (range), years	38 (13–70)	–
Patient gender, N (%)		
Male	29 (78%)	5 (63%)
Female	8 (22%)	3 (37%)
Donor gender, N (%)		
Male	26 (70%)	–
Female	7 (19%)	
UCB	4 (11%)	
Organ involvement at diagnosis, N (%)		
BM+skin	15 (41%)	7 (88%)
Skin	8 (22%)	–
BM	7 (19%)	1 (13%)
BM+CNS	3 (8%)	–
BM+skin+CNS	3 (8%)	–
BM+ lymph node	1 (3%)	
Disease status at time HCT, N (%)		
CR1	28 (76%)	5 (63%)
CR2	4 (11%)	1 (13%)
CR3	1 (2%)	1 (13%)
PIF	4 (11%)	1 (13%)
Pre-HCT therapy, N (%)		
Hyper-CVAD-based	25 (68%)	4 (50%)
Others*	12 (32%)	4 (50%)¶

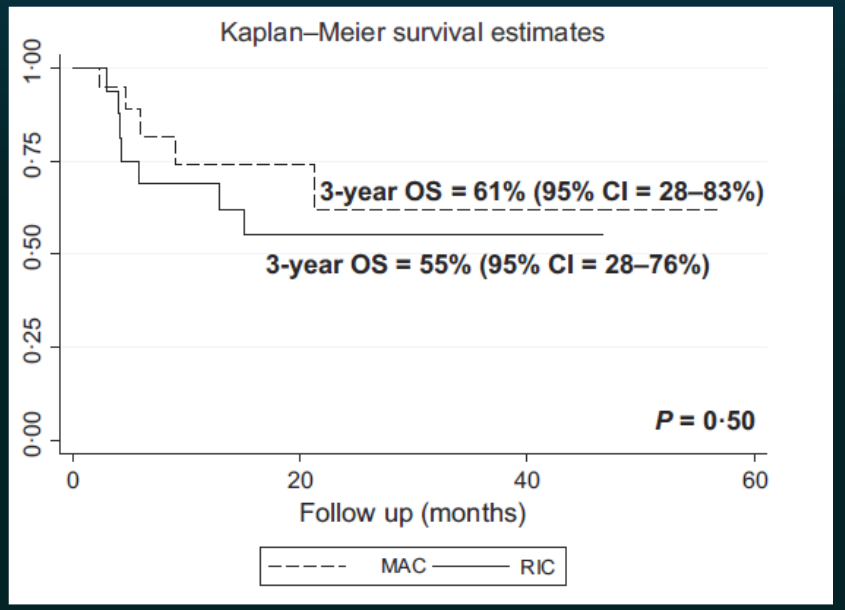
Characteristics	Allo-HCT (N = 37)	Auto-HCT (N = 8)
Cell source, N (%)		
PBSC	25 (68%)	8 (100%)
BM	8 (22%)	–
UCB	4 (11%)	–
Donor source, N (%)		
MRD	16 (43%)	–
MUD	12 (32%)	
UCB	4 (11%)	
Haploidentical	3 (8%)	
MMUD	2 (5%)	
Conditioning regimen intenstity, N (%)		BEAM (n = 8, 100%)
MAC	20 (54%)	
RIC	17 (46%)	
GVHD prophylaxis		
MMF-based†	13 (35%)	–
MTX-based	13 (35%)	
Sirolimus-based	7 (19%)	
Others‡	4 (11%)	
Median (range) CD34 cell dose/kg recipient body weight (N = 32)§	4·92 (1·14–37·86)	5·62 (0·88–8·53)
HCT-comorbidity index at time of HCT		
0	15 (41%)	1 (13%)
1	6 (16%)	1 (13%)
2	10 (27%)	1 (13%)
3	6 (16%)	2 (25%)
≥4	–	3 (38%)

Kharfan-Dabaja MA et al. BJH 2017

Outcome in allo-HCT recipients







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UVA and MVA for PFS and OS in allo-HCT

Variables	PFS		OS	
	HR (95% CI)	*P*-value	HR (95% CI)	*P*-value
Recipient age				
<55 years	–	0·47	–	0·63
≥55 years	0·65 (0·20, 2·13)		0·75 (0·22, 2·49)	
CD34 cell dose (continuous)	0·92 (0·77, 1·11)	0·39	0·87 (0·69, 1·10)	0·24
Regimen intensity				
MAC	–	0·72	–	0·50
RIC	1·23 (0·41, 3·66)		1·48 (0·47, 4·70)	
Remission status at allo-HCT				
CR1	–	**<0·001**	–	**<0·001**
Not in CR1	**7·56 (2·19, 26·07)**		**8·57 (2·33, 31·57)**	
Cell source				
PBSC	–	0·85	–	0·97
BM	0·86 (0·18, 4·05)		0·97 (0·20, 4·66)	
Acute GVHD				
0–1	–	0·81	–	0·66
2–4	1·13 (0·37, 3·49)		1·30 (0·41, 4·12)	
Chronic GVHD				
None	–		–	–
Mild	0·93 (0·12, 7·30)	0·93	0·99 (0·12, 8·10)	0·99
Moderate/severe	2·50 (0·75, 8·36)	0·14	2·84 (0·82, 9·80)	0·09

Variables	PFS		OS	
	HR (95% CI)	*P*-value	HR (95% CI)	*P*-value
Recipient age				
<55	–	0·94	–	0·90
≥55	0·93 (0·13, 6·43)		1·14 (0·14, 9·32)	
CD34 cell dose (continuous)	0·97 (0·83, 1·14)	0·72	0·94 (0·70, 1·25)	0·65
Regimen intensity				
MAC	–	0·90	–	0·99
RIC	0·88 (0·13, 5·88)		1·01 (0·13, 7·90)	
Remission status at allo-HCT				
CR1	–	**0·01**	–	**0·01**
Not in CR1	**6·40 (1·45, 28·23)**		**7·72 (1·60, 37·32)**	
Cell source				
PBSC	–	0·81	–	0·86
BM	0·81 (0·14, 4·76)		0·84 (0·11, 6·23)	

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Allogeneic Hematopoietic Cell Transplantation Is an Effective Treatment for Blastic Plasmacytoid Dendritic Cell Neoplasm in First Complete Remission: Systematic Review and Meta-analysis

Table 1 Patient-, Disease-, and Transplant-Related Characteristics of Eligible Studies

Study (Year)	No. of Subjects	Data Source	Age (Years), Median (Range)	Gender	Remission Status at Time of HCT	Cell Source	Conditioning Regimen	Survival Outcomes (All Patients)	Survival Outcomes (Patients in CR1)
Roos-Weil (2013)[13,a]	34	European Society for Blood and Marrow Transplantation (2000-2009)	41 (10-70)	M = 21 F = 13	CR1 = 19 > CR1 = 15	PBSC = 9 BM = 19 UCB = 6	MAC = 25 RIC = 9	DFS = 33% OS = 41% (3 year)	DFS = 45% OS = 60% (3 year)
Aoki (2015)[14]	14	Japanese Transplant Registry Unified Management Program (2002-2014)	58 (17-64)	M = 11 F = 3	CR1 = 10 > CR1 = 4	PBSC = 5 BM = 8 UCB = 1	MAC = 8 RIC = 6	PFS = 48% OS = 53% (4 year)	PFS = 60% OS = 69% (4 year)
Kharfan-Dabaja (2017)[7]	37	North America (2000-2017)	50 (14-74)	M = 29 F = 8	CR1 = 28 > CR1 = 9	PBSC = 25 BM = 8 UCB = 4	MAC = 20 RIC (n = 17)	PFS = 55% OS = 58% (3 year)	PFS = 69% OS = 74% (3 year)
Lecleric (2017)[15,a]	43	French Society of Bone Marrow Transplantation and Cell Therapy (2003-2014)	57 (20-72)	M = 29 F = 14	CR1 = 34 > CR1 = 9	PBSC = 30 BM = 7 UCB = 6	MAC = 14 RIC = 29	DFS = 45% OS = 52% (2 year)	NR

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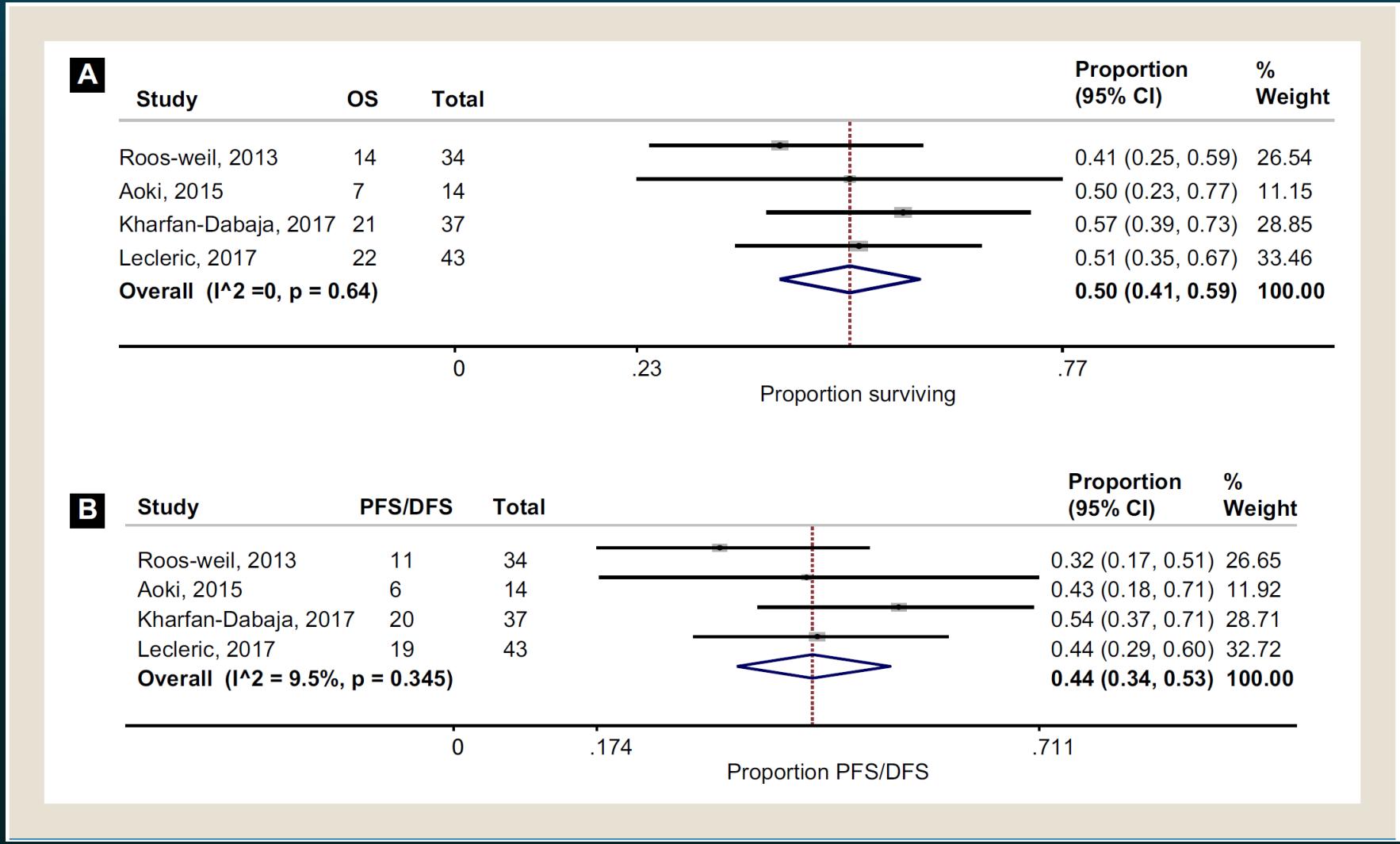
Forest Plot of pooled OS (A) and PFS/DFS (B) rates



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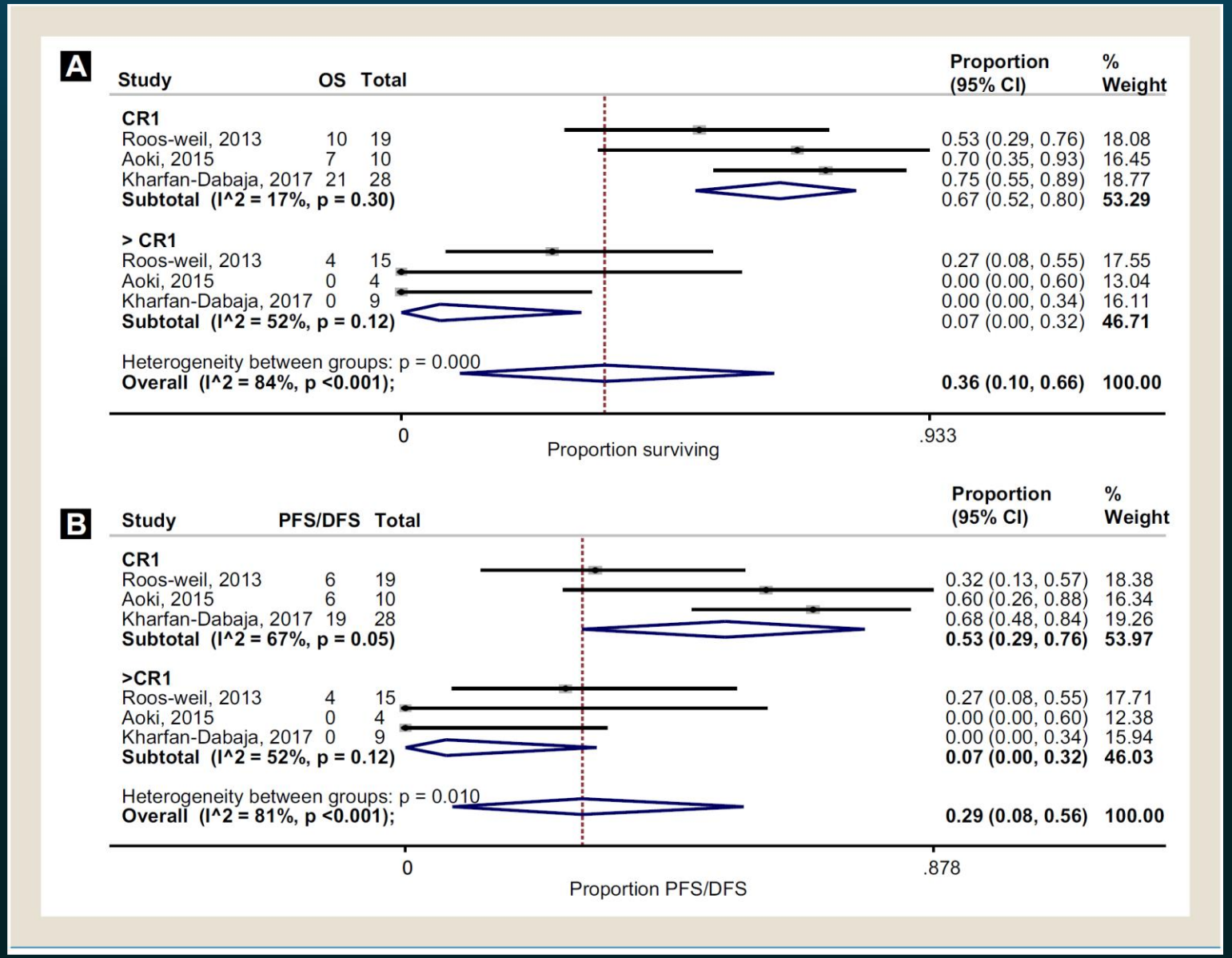
Forest Plot of pooled OS (A) and PFS/DFS (B) rates by CR status



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Allogeneic hematopoietic cell transplantation for patients with blastic plasmacytoid dendritic cell neoplasm (BPDCN)

Qaiser Bashir[1], Denái R. Milton[2], Uday R. Popat[1], Partow Kebriaei[1], Chitra Hosing[1], Issa F. Khouri[1], Katayoun Rezvani[1], Yago Nieto[1], Betul Oran[1], Samer A. Srour[1], Neeraj Y. Saini[1], Amanda L. Olson[1], Sairah Ahmed[1,3], Gheath Al-Atrash[1], Gabriela Rondon[1], Marina Y. Konopleva[4], Richard E. Champlin[1], Elizabeth J. Shpall[1], Muzaffar H. Qazilbash[1] and Naveen Pemmaraju[4]✉

Variable	All patients ($N = 17$)
Age at allo-HCT (years)	
Median (Range)	39 (18–67)
Age at allo-HCT, *n* (%)	
<60 years	13 (76)
≥60 years	4 (24)
Gender, *n* (%)	
Female	3 (18)
Male	14 (82)
Year of allo-HCT, *n* (%)	
≤2010	1 (6)
2011–2015	5 (29)
>2015	11 (65)
History of prior auto-HCT	
Yes[a]	2 (12)
No	15 (88)
Prior hematologic malignancy, *n* (%)	
No	13 (76)
Yes	4 (24)
Lymphoblastic lymphoma	1 (6)
Multiple myeloma	1 (6)
Mycoses fungoides	1 (6)
Myelofibrosis	1 (6)
Organs involved, *n* (%)	
Skin	11 (65)
Bone marrow	13 (76)
Skin + Bone marrow	10 (59)
Other organs involved	10 (59)

Variable	All patients ($N = 17$)
Absent	8 (47)
Present	9 (53)
Complex karyotype[b], *n* (%)	
Absent	14 (82)
Present	3 (18)
TET2 mutation, *n* (%)	
Absent	10 (67)
Present	5 (33)
Unknown	2
ASXL1 mutation, *n* (%)	
Absent	14 (93)
Present	1 (7)
Induction therapy, *n* (%)	
Tagraxofusp only	3 (18)
HYPER-CVAD	8 (47)
HYPER-CVAD + Tagraxofusp	3 (18)
Other	3 (18)
Months from diagnosis to allo-HCT	
Median	6.7
Range	(3.9–42.4)
Disease status at allo-HCT, *n* (%)	
CR1	10 (59)
CR2	4 (24)
PR	2 (12)
SD	1 (6)

Variable	All patients ($N = 17$)
Conditioning regimen, *n* (%)	
Fludarabine + Busulfan	9 (53)
Fludarabine + Melphalan	6 (35)
Other	2 (12)
Myeloablative	13 (76)
Non-myeloablative	4 (24)
Donor type, *n* (%)	
Matched-related	5 (29)
Matched unrelated	5 (29)
Haploidentical	4 (24)
Cord blood	3 (18)
GVHD prophylaxis, *n* (%)	
Tacrolimus + PTCy	4 (24)
Tacrolimus + MMF + PTCy	6 (35)
Tacrolimus + MMF	1 (6)
Tacrolimus + Methotrexate	6 (35)

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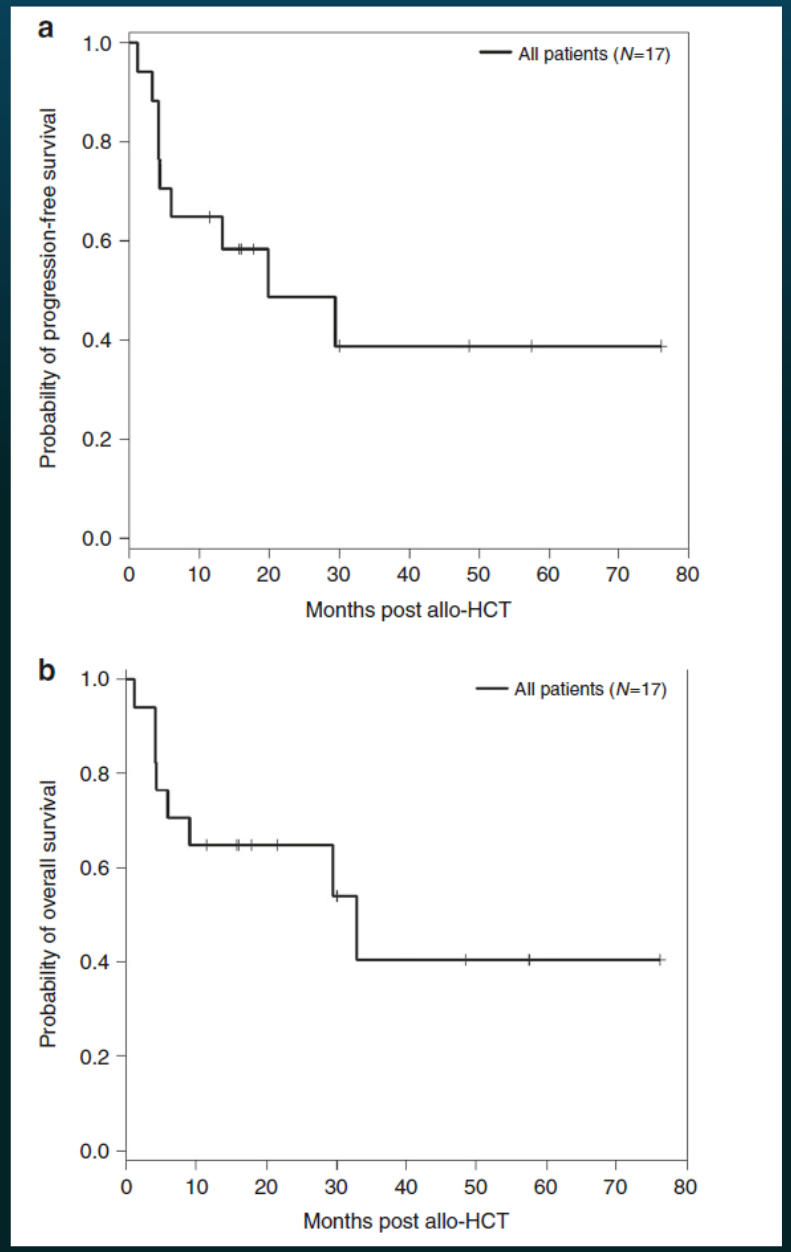
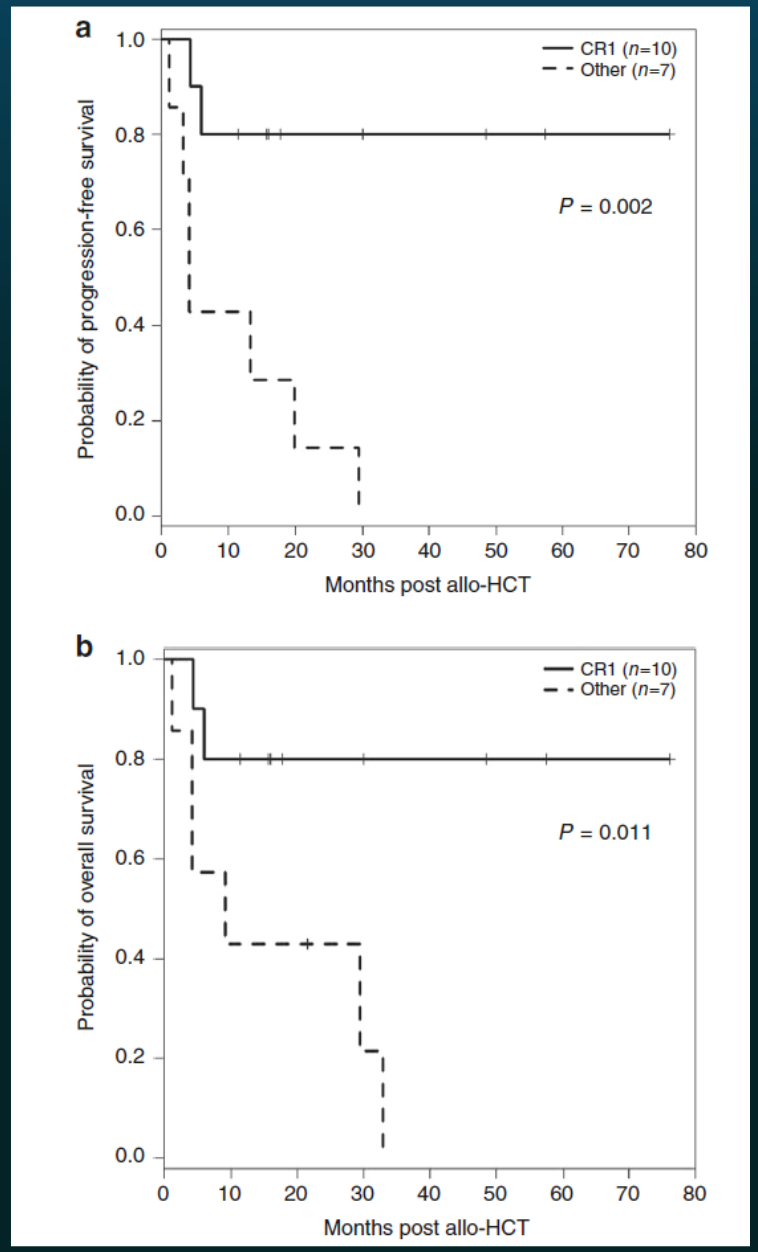
Oucome post allo-HCT





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UVA for PFS and OS

Variable	PFS		OS	
	Median (95% CI) months	*p* value	Median (95% CI) months	*p* value
Age at allo-HCT				
<60 years	19.9 (4.1, NE)	0.99	32.9 (4.3, NE)	0.63
≥60 years	29.5 (1.2, 29.5)		29.5 (1.2, 29.5)	
Gender				
Male	13.3 (4.1, NE)	0.39	29.5 (4.1, NE)	0.37
Female	NE (19.9, NE)		NE (32.9, NE)	
Prior hematologic malignancy				
No	NE (5.9, NE)	0.015	NE (9.1, NE)	<0.001
Yes	4.1 (1.2, 29.5)		4.1 (1.2, 29.5)	
Cytogenetic abnormalities present				
No	NE (4.1, NE)	0.08	NE (4.1, NE)	0.18
Yes	13.3 (1.2, 29.5)		29.5 (1.2, NE)	
Complex karyotype				
No	29.5 (4.1, NE)	0.63	32.9 (4.3, NE)	0.76
Yes	13.3 (1.1, NE)		NE (1.1, NE)	
TET2				
No	13.3 (1.1, NE)	0.49	NE (1.1, NE)	0.68
Yes	29.5 (19.9, NE)		32.9 (29.5, NE)	
ASXL1				
No	29.5 (4.1, NE)	0.51	32.9 (5.9, NE)	0.57
Yes	NE (NE, NE)		NE (NE, NE)	
Induction therapy				
Tagraxofusp only	NE (19.9, NE)	0.34	32.9 (NE, NE)	0.63
Hyper-CVAD	NE (4.1, NE)		NE (4.1, NE)	
Hyper-CVAD + Tagraxofusp	29.5 (1.2, 29.5)		29.5 (1.2, 29.5)	
Other	8.7 (3.3, NE)		NE (4.1, NE)	
Disease status at allo-HCT				
CR1	NE (4.3, NE)	0.002	NE (4.3, NE)	0.011
Other	4.1 (1.2, 19.9)	0.014	9.1 (1.2, 32.9)	0.11
CR1/CR2	NE (4.3, NE)		NE (4.3, NE)	
Other	3.3 (1.2, 19.9)		9.1 (1.2, 32.9)	
Conditioning regimen				
Myeloablative	19.9 (4.1, NE)	0.16	29.5 (4.3, NE)	0.25
Non-myeloablative	NE (4.1, NE)		NE (4.1, NE)	
Donor type				
MRD/MUD	19.9 (1.2, NE)	0.44	NE (1.2, NE)	0.12
Haploidentical/CBT	5.9 (4.1, NE)		5.9 (4.1, NE)	
Donor type				
MRD	NE (3.3, NE)	0.50	NE (9.1, NE)	0.15
MUD/Haploidentical/CBT	19.9 (4.1, NE)		29.5 (4.1, NE)	

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STEM CELL TRANSPLANTATION

Retrospective analysis of hematopoietic cell transplantation for blastic plasmacytoid dendritic cell neoplasm: conditioning intensity matters

Peter-Martin Bruch[1,2], Sascha Dietrich[1,2,3]✉, Herve Finel[3], Ariane Boumendil[3], Hildegard Greinix[4], Thomas Heinicke[5], Wolfgang Bethge[6], Dietrich Beelen[7], Christoph Schmid[8], Hans Martin[9], Luca Castagna[10], Christof Scheid[11], Kerstin Schäfer-Eckart[12], Jörg Bittenbring[13], Jürgen Finke[14], Henrik Sengeloev[15], Mael Heiblig[16], Jan Cornelissen[17], Patrice Chevallier[18], Mohamad Mohty[19], Stephen Robinson[20], Silvia Montoto[21] and Peter Dreger[2]

	All ($n = 162$)	autoHCT ($n = 16$)	MAC ($n = 79$)	RIC ($n = 66$)	MAC vs. RIC *p*-value
Age (median, [range])	57.2 [19.7;73.3]	62.5 [22.0;72.1]	**47.1 [19.7;71.5]**	**61.6 [22.2;73.3]**	**<0.001**
Sex					
Female	52 (32.1%)	4 (25.0%)	32 (40.5%)	16 (24.2%)	0.058
Male	110 (67.9%)	12 (75.0%)	47 (59.5%)	50 (75.8%)	
Months from diagnosis to transplantation (median, [range])	6.20 [2.59;102]	7.87 [3.67;21.4]	5.84 [2.62;102]	6.36 [2.59;19.3]	0.339
Karnofsky Index					
≥90%	124 (76.5%)	11 (68.8%)	64 (81.0%)	48 (72.7%)	0.324
<90%	38 (23.5%)	5 (31.2%)	15 (19.0%)	18 (27.3%)	
Disease status					
First complete remission	108 (78.3%)	8 (61.5%)	57 (81.4%)	42 (77.8%)	0.782
Other	30 (21.7%)	5 (38.5%)	13 (18.6%)	12 (22.2%)	
Year of transplant (median, [range])	2013 [2009;2017]	2012 [2010;2017]	2014 [2009;2017]	2013 [2010;2017]	0.580
Donor					
Autologous	16 (9.88%)	16 (100%)			
Haploidentical	14 (8.64%)		**8 (10.1%)**	**6 (9.09%)**	**0.038**
Matched sibling	37 (22.8%)		**26 (32.9%)**	**10 (15.2%)**	
Unrelated	95 (58.6%)		**45 (57.0%)**	**50 (75.8%)**	
Graft source					
Bone marrow	24 (14.8%)		16 (20.3%)	8 (12.1%)	0.277
Peripheral blood	138 (85.2%)	16 (100%)	63 (79.7%)	58 (87.9%)	
In-vivo T-cell depletion					
None	76 (46.9%)	16 (100%)	38 (48.1%)	21 (31.8%)	0.069
ATG and/or Campath	86 (53.1%)		41 (51.9%)	45 (68.2%)	
ATG	73 (45.1%)		38 (48.1%)	35 (53.0%)	
Campath	16 (9.88%)		5 (6.33%)	11 (16.7%)	
Total body irradiation					
No	95 (59.0%)	15 (93.8%)	**31 (39.2%)**	**48 (73.8%)**	**<0.001**
Yes	66 (41.0%)	1 (6.25%)	**48 (60.8%)**	**17 (26.2%)**	
Chronic GVHD					
Limited or extensive GVHD	34 (23.3%)		20 (25.3%)	14 (21.2%)	0.701
No GVHD reported	112 (76.7%)		59 (74.7%)	52 (78.8%)	

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UVA and MVA for prognostic factors

	PFS				OS			
	***p*-value**	**HR**	**95% CI**		***p*-value**	**HR**	**95% CI**	
a								
Sex Male (vs female)	0.583	1.2	0.7	2.1	0.511	1.2	0.7	2.4
HCT type AlloHCT (vs autoHCT)	0.616	0.8	0.3	2.0	0.583	0.7	0.3	2.1
Disease status Non-CR1 (vs CR1)	**0.006**	**2.4**	**1.3**	**4.4**	**0.005**	**2.6**	**1.3**	**5.1**
Age (per 10 years)	0.516	0.8	0.5	1.4	0.6	0.9	0.5	1.5
Karnofsky Index <90% (vs 90–100%)	0.107	1.6	0.9	2.8	0.086	1.7	0.9	3.2
b								
NIS study Yes (vs No)	0.549	0.8	0.4	1.7	0.758	1.1	0.5	2.6
Karnofsky Index <90% (vs 90–100%)	0.292	1.4	0.7	2.8	0.212	1.6	0.8	3.3
Sex Male (vs female)	0.383	1.4	0.7	2.8	0.684	1.2	0.5	2.6
HCT type alloHCT (vs autoHCT)	0.496	1.5	0.5	5.0	0.565	1.5	0.4	5.9
Disease status <CR1 (vs CR1)	**0.003**	**2.7**	**1.4**	**5.2**	**0.002**	**3.0**	**1.5**	**6.0**
Age (per 10 years)	0.882	1.0	0.8	1.3	0.622	0.9	0.7	1.2

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MVA according to conditioning

	p-value	HR	95% CI		*p*-value	HR	95% CI	
	PFS				OS			
Conditioning								
RIC without TBI (vs MAC with TBI)	**0.001**	**7.7**	**2.4**	**25.0**	**0.020**	**4.4**	**1.3**	**15.2**
RIC TBI (vs MAC with TBI)	**0.000**	**15.2**	**3.8**	**61.2**	**0.000**	**14.9**	**3.3**	**66.9**
MAC without TBI (vs MAC with TBI)	**0.003**	**5.2**	**1.8**	**15.3**	**0.002**	**6.2**	**1.9**	**20.4**
Age (per 10 years)	**0.007**	**0.7**	**0.5**	**0.9**	**0.016**	**0.7**	**0.5**	**0.9**
Sex (male vs female)	0.447	1.3	0.6	2.8	0.606	1.2	0.5	2.9
Karnofsky Index (<90% vs 90–100%)	0.176	1.6	0.8	3.2	0.194	1.7	0.8	3.6
Donor								
Haplo (vs Matched sibling)	0.337	0.6	0.2	1.9	0.726	1.3	0.3	5.1
Unrelated (vs Matched sibling)	0.138	0.6	0.3	1.2	0.602	1.3	0.5	3.6
Disease status (non-CR1 vs CR1)	**0.000**	**4.3**	**1.9**	**9.5**	**0.001**	**4.3**	**1.9**	**10.0**
In-vivo T-cell depletion (no vs yes)	0.082	2.2	0.9	5.4	0.137	2.3	0.8	6.8
Study type (NIS vs EBMT register)	0.337	0.6	0.2	1.9	0.726	1.3	0.3	5.1
Chronic GVHD (Limited or extensive vs no chronic GVHD reported)	0.138	0.6	0.3	1.2	0.602	1.3	0.5	3.6
	Relapse				NRM			
Conditioning								
RIC without TBI (vs MAC with TBI)	**0.023**	**8.0**	**1.8**	**36.1**	0.340	1.9	0.6	5.4
RIC TBI (vs MAC with TBI)	**0.064**	**7.5**	**1.2**	**44.9**	**0.030**	**5.8**	**1.5**	**22.0**
MAC without TBI (vs MAC with TBI)	0.140	3.5	0.9	14.6	0.120	3.0	0.9	9.4
Age (per 10 years)	0.400	0.8	0.5	1.2	0.052	0.7	0.5	1.0
Sex (male vs female)	0.730	1.3	0.4	3.6	0.690	0.8	0.3	2.2
Karnofsky Index (<90% vs 90–100%)	0.310	1.7	0.7	3.8	0.630	1.3	0.6	2.8
Donor								
Haplo (vs Matched sibling)	0.820	0.8	0.2	3.3	0.900	0.9	0.2	4.1
Unrelated (vs Matched sibling)	0.160	0.4	0.2	1.2	0.970	1.0	0.4	2.5
Disease status non-CR1 (vs CR1)	**0.031**	**3.9**	**1.4**	**10.8**	0.710	1.3	0.4	3.6
In-vivo T-cell depletion no (vs yes)	0.650	1.3	0.5	3.2	0.370	1.8	0.6	5.2
Study type (NIS vs EBMT register)	0.380	0.5	0.1	1.9	0.460	1.5	0.6	3.5
Chronic GVHD (Limited or extensive vs no chronic GVHD reported)	0.890	1.1	0.4	2.9	0.610	1.3	0.6	2.7

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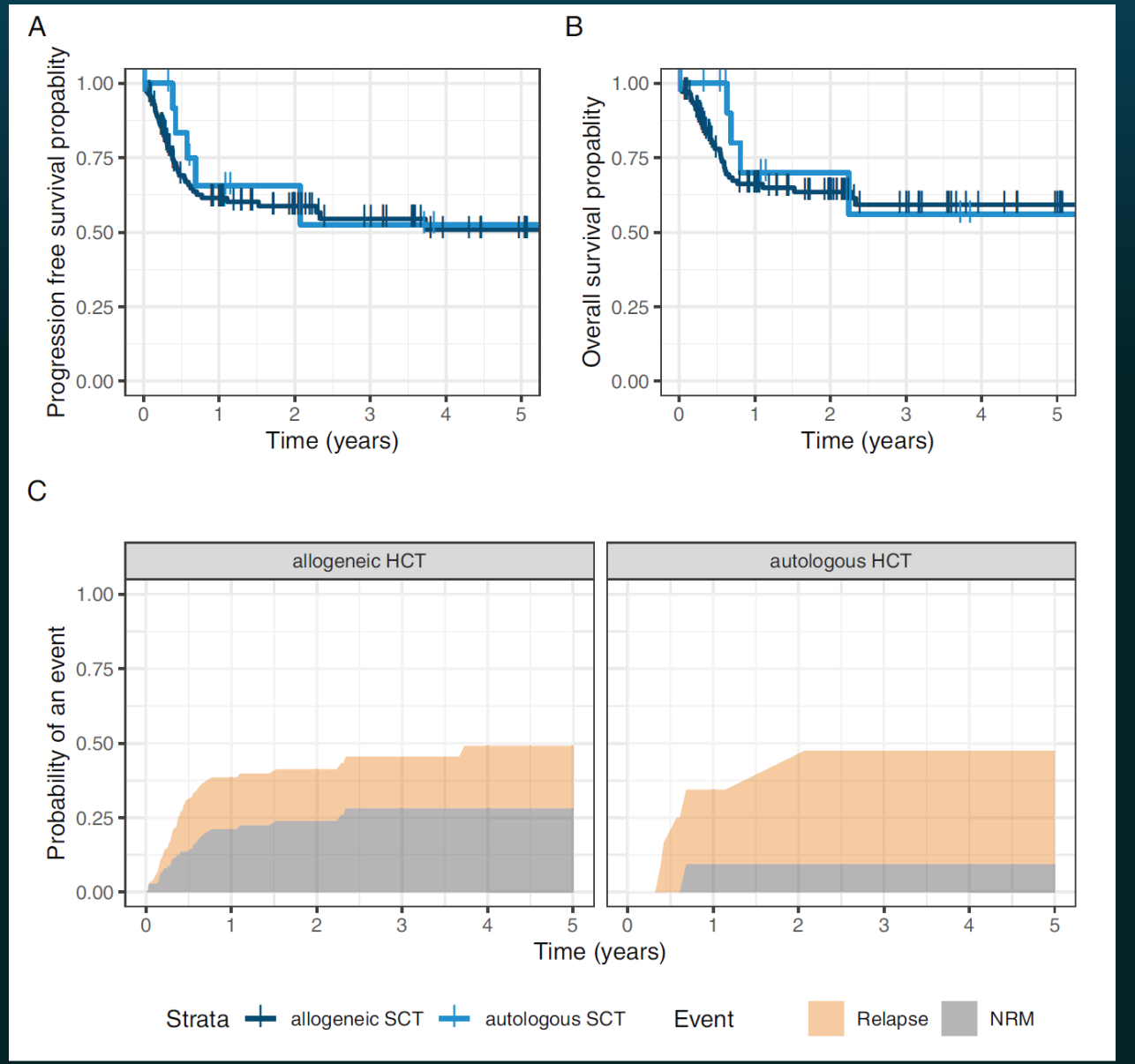
Oucome of allo- and auto-HCT



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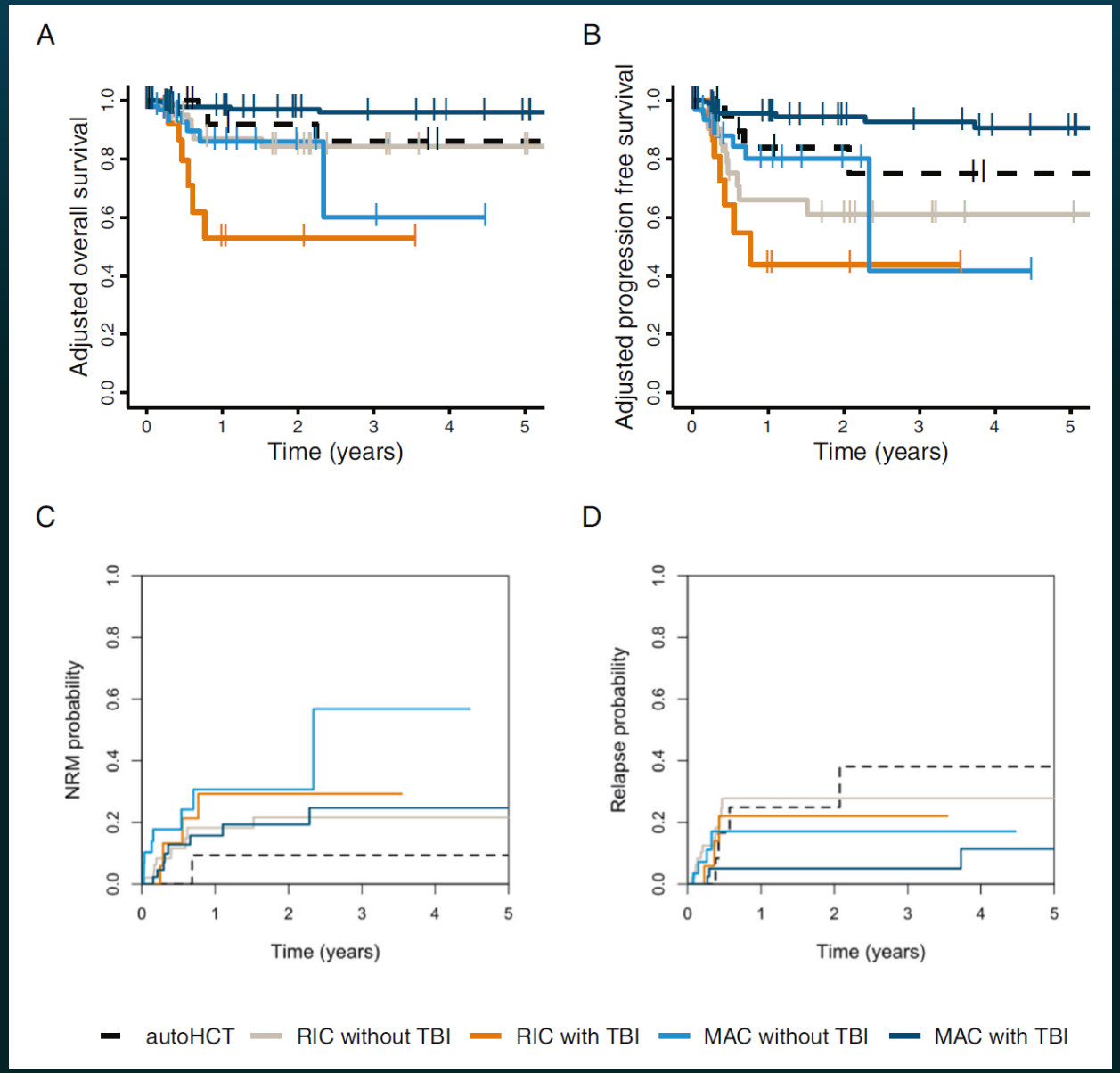
Oucome of allo-HCT by conditioning



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Special Report

North American Blastic Plasmacytoid Dendritic Cell Neoplasm Consortium: position on standards of care and areas of need

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BPDCN: overview of outcomes from therapy approaches

Author and study period	Type of study	Therapy details*	Response rates [(N)/%]†	OS dynamics	SCT-specific outcomes
Roos-Weil et al[33] (EBMT analysis; 2000-2009)	Retrospective (only patients receiving transplant included)	**Nontargeted** AML/ALL-type = 27 NHL-type = 7	N/A		Allo-SCT in CR1 = 19 Allo-SCT ≥ CR1 = 15 3 y OS = 43%
Laribi et al[121] 2001-2017	Retrospective	**Nontargeted** AML-type = 53 ALL-type = 96 NHL-type = 150	AML-type Rx f/b SCT = 14/16 (88%) ALL-type Rx f/b SCT = 31/33 (94%) NHL-type Rx f/b SCT = 12/12 (100%)	Median OS = 18 mo	**5 years OS** AML-type therapy f/b allo-SCT = 59.5% ALL-type therapy f/b allo-SCT = 47.3% NHL-type therapy f/b allo-SCT = 71.1% No allo-SCT = 9.6% for leukemia-type therapy, 8.3% for lymphoma-type therapy
Yun et al[72] 2001-2019	Retrospective	**Mixed** AML-like = 1 ALL-type = 11 NHL-like = 10 SL-401 = 12	N/A 11 (100) 9 (90) 9 (75)		mOS with SCT = NR mOS without SCT ≈ 3 y
Pagano et al[10] 2005-2011	Retrospective	**Nontargeted** AML-type = 26 ALL-type = 15	7/16 (44) 10/15 (67)	Median OS = 8.7 mo After AML-type Rx = 7.1 mo After ALL-type Rx = 12.3 mo	**SCT = 6** mOS with SCT = 22.7 mo mOS without SCT= 7.1 mo
Aoki et al[31] (JSHCT analysis; 2002-2015)	Retrospective (only patients receiving transplant included)	**Nontargeted** AML-type =4 ALL-type = 10 NHL-type = 11	N/A	4 y OS = 65% After AML-type Rx = 67% After ALL-type Rx = 70% After NHL-type Rx = 62%	**4 y OS** After auto-SCT = 82% After allo-SCT = 69%
Taylor et al[24] 2000-2017	Retrospective	**Nontargeted** AML-type = 9 ALL-type = 35 Others = 10	N/A	2 y OS = 49%	**SCT = 25; (allo = 20, auto = 5)** 2 y OS after SCT = 60%
Pemmaraju et al[30] 1999-2020	Retrospective	**Mixed** ALL-type = 35 SL-401 = 37 Others = 28	80% 59% 43%	**Median OS** After ALL-type Rx= 28.3 mo After SL-401 = 13.7 mo After other Rx = 22.8 mo	**% responders proceeding to allo-SCT in CR1** 15/28 (54%) 13/22 (59%) 4/12 (33%) SCT specific survival data N/A
Pemmaraju et al[71] 2014-2019	Prospective clinical trial (long-term follow-up, 34 mo)	**Targeted** SL-401 (F/L) = 65§	75%	2 y OS = 40%	**SCT = 19‡** 2 y OS after SCT = 66% 2 y OS without SCT ≈ 30%

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HSCT in BPDCN: North-American position

- The treatment goal for all patients with BPDCN should be HSCT in CR1 unless contraindicated
- Long-term management following successfull HSCT is being investigated using tagraxofusp maintenance monotherapy, with a focus on long-term remission and survival (NCT04317781)
- Future studies with combination therapeutics, CNS-directed therapy and maintenance strategies should attempt to elucidate the ability to achieve cure without HSCT
- Autologous HSCT has been used in several series of selected patients, including those who are older/unfit for allo-HSCT, or those with skin limited disease
- Currently, we recommend all fit/younger patients for consideration of allo-HSCT in CR1. We did not reach consensus on the role and timing of auto-HSCT and await additional clinical research

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- **Indication for alloSCT in BPDCN is independent of the stage of disease at diagnosis**, as hematological organs involvement with extremely aggressive clinical course occurs in almost all relapsing patients.

- **Patients <70 years should undergo human leukocyte antigen (HLA)-typing at the time of diagnosis**. Exceptionally, patients >70 with good performance status and no major comorbidities may also be referred for possible evaluation in experienced BMT centers.

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- In younger and fit patients who achieve CR following AL-like induction chemotherapy treatment, **alloSCT is recommended within 3 months if a donor is identified**, to minimize the risk of early on-treatment relapse

- **As far as the type of alternative donor** (in patients lacking HLA-identical sibling donor) is concerned, **anyone would be a reasonable choice**

- **No data are available concerning specific conditioning regimens** (total body irradiation [TBI]-based vs. chemotherapy [CT]-based) and the stem cell source (BM versus PB versus cord blood)

- **For patients with CT sensitive extra-hematological disease, in first CR** but **with** age-related and/or comorbidity-related **high risk of transplant-related mortality**, as well as for patients lacking HLA-suitable hemopoietic cell donor, **autoSCT may represent another consolidation strategy**

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- To minimize the risk of collecting and cryopreserving neoplastic cells, **mobilization and collection of PBSC should be accomplished as soon as a PET-documented CR is achieved** and possibly with a documented measurable residual disease (MRD) negative flow cytometry test both in BM and blood/apheresis.

- Due to the well-known skin localization of the disease and its not unfrequent CNS involvement, **myeloablative TBI-based or chemotherapy-based conditioning should be preferred**.

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THANK YOU!

Questions?