

# Unrelated alternative donor transplantation for severe acquired aplastic anemia: a study from the French Society of Bone Marrow Transplantation and Cell Therapies and the EBMT Severe Aplastic Anemia Working Party

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## **Online Supplementary Appendix**

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**Supplemental Table 1: Univariate analyses of confounding factors to select those used to adjust the multivariate analyses**

	Univariate analysis		
	4-yr OS	[95%CI]	p
<b>Transplantation period</b>			
2000-05 (n = 46)	52%	[39-69]	
2006-12 (n = 93)	74%	[66-84]	0.018
<b>Donor age</b>			
< 35 years (n = 61)	68%	[55-83]	
≥ 35 years (n = 65)	62%	[51-75]	0.269
<b>CMV serostatus</b>			
D-/R- (n = 53)	71%	[59-87]	
Other (n = 83)	61%	[53-73]	0.125
<b>Graft source</b>			
BM (n = 118)	67%	[58-76]	
PBSC (n = 21)	60%	[42-86]	0.573
<b>GVHD prophylaxis</b>			
CSA + MTX (n = 95)	67%	[57-78]	
Other (n = 45)	64%	[51-80]	0.558
<b>TBI-based conditioning</b>			
No (n = 75)	66%	[56-78]	
Yes (n = 64)	66%	[54-79]	0.964
<b>In vivo T-cell depletion</b>			
No (n = 27)	55%	[39-68]	
Yes (n = 112)	67%	[67-89]	0.145

95%CI = 95% confidence interval; BM = bone marrow; CSA = cyclosporine A; D-/R- = seronegative donor and recipient; GVHD = graft versus host disease; MTX = methotrexate; OS = overall survival; PBSC = peripheral blood stem cell; TBI = total body irradiation

**Supplemental Table 2: Impact on overall survival of the presence of selected risk factors: age > 30 years; time from diagnosis to Allo-HSCT > 12 months; presence of an HLA mismatch**

	Multivariate analysis		
	HR	[95%CI]	p
<b>Study population</b>			
0 risk factor (n = 35)	1		
1 risk factor (n = 59)	1.41	[0.53-3.74]	0.488
2 risks factors (n = 41)	3.61	[1.36-9.55]	0.010
3 risks factors (n = 4)	11.90	[2.65-53.35]	0.001
<b>Validation cohort</b>			
0 risk factor (n = 87)	1		
1 risk factor (n = 145)	1.87	[0.988-3.57]	0.055
2 risks factors (n = 55)	2.49	[1.177-5.26]	0.017
3 risks factors (n = 9)	10.19	[3.87-26.89]	<0.001

4-y = 4-year; HR = Hazard ratio; 95%CI = 95% confidence interval

**Supplemental Table 3: baseline characteristics of patients in the validation cohort for the SAAWP of EBMT**

	<b>Control cohort (N = 296)</b>	
	<b>N</b>	<b>%</b>
<b>Age at Allo-HSCT</b>		
≤ 30 years	233	79%
> 30 years	63	21%
<b>Time from diagnosis to Allo-HSCT (months)</b>		
≤ 12 months	121	41%
> 12 months	175	59%
<b>Unrelated donor</b>		
10/10 MUD	252	85%
9/10 MUD	44	15%
<b>Predictive score</b>		
0-1	232	78%
≥ 2	64	22%
<b>Conditioning</b>		
RIC	176	71%
MAC	71	29%
missing data	49	
<b>Use of TBI</b>	82	28%
missing data	1	
<b>In vivo T-cell depletion</b>	176	85%
missing data	89	
<b>Graft source</b>		
BM	184	62%
PBSC	112	38%

BM = bone marrow; MUD = matched unrelated donor; PBSC = peripheral blood stem cell; TBI = total body irradiation

**Supplemental Table 4: Review of major series involving UD Allo-HSCT: number of patients transplanted from a UD; Allo-HSCT period; OS; significant age cut-off for better OS; delay before Allo-HSCT; and impact of HLA matching**

Series	N	Allo-HSCT period	OS	Impact of age	Impact of time from diagnosis to Allo-HSCT	Impact of HLA matching
Deeg <i>et al.</i> <sup>16</sup>	50	1994-1999	58%	20 years	1 year and 3 years	No
Kojima <i>et al.</i> <sup>17</sup>	154	1993-2000	56%	20 years	1 year and 3 years	Yes
Bacigalupo <i>et al.</i> <sup>3</sup>	87	1998-2004 2005-2008	68% 83%	13 years	2 years	No
Marsh <i>et al.</i> <sup>5</sup>	29	1999-2009	83%	*	*	N/A
Maury <i>et al.</i> <sup>4</sup>	37 52	1989-1999 2000-2004	29% 50%	17 years	1 year	Yes
Viollier <i>et al.</i> <sup>18</sup>	35 62	1990-1997 1998-2005	32% 57%	*	*	Yes
Devillier <i>et al.</i>	46 93	2000-2005 2006-2012	52% 74%	30 years	1 year	Yes

\*Age and interval to Allo-HSCT were not specifically assessed for UD Allo-HSCTs.

N/A = Not available