

**Sangue Cordonale:
Nuove prospettive
della Raccolta**

**CONVEGNO
NAZIONALE
ADISCO OdV**

**ALESSANDRIA
Salone di Rappresentanza AOU AL
Via Venezia, 16**

in collaborazione con

Il TCSE da SCO: risultati consolidati, limiti e possibilità di sviluppi futuri

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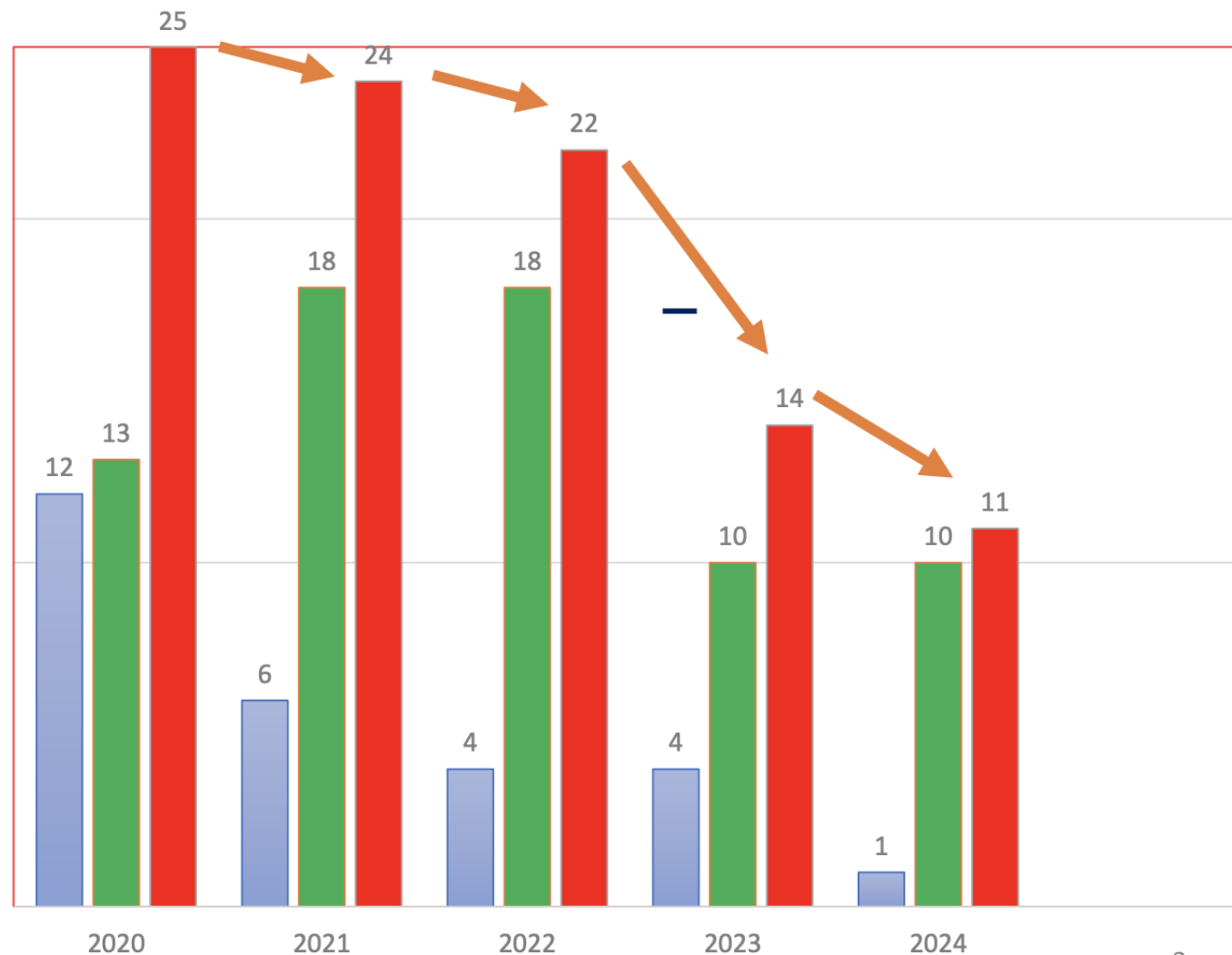
Cordone: trapianti in Italia 2020-2024

■ Ricevente <18 anni ■ Ricevente > 18 anni ■ Totali

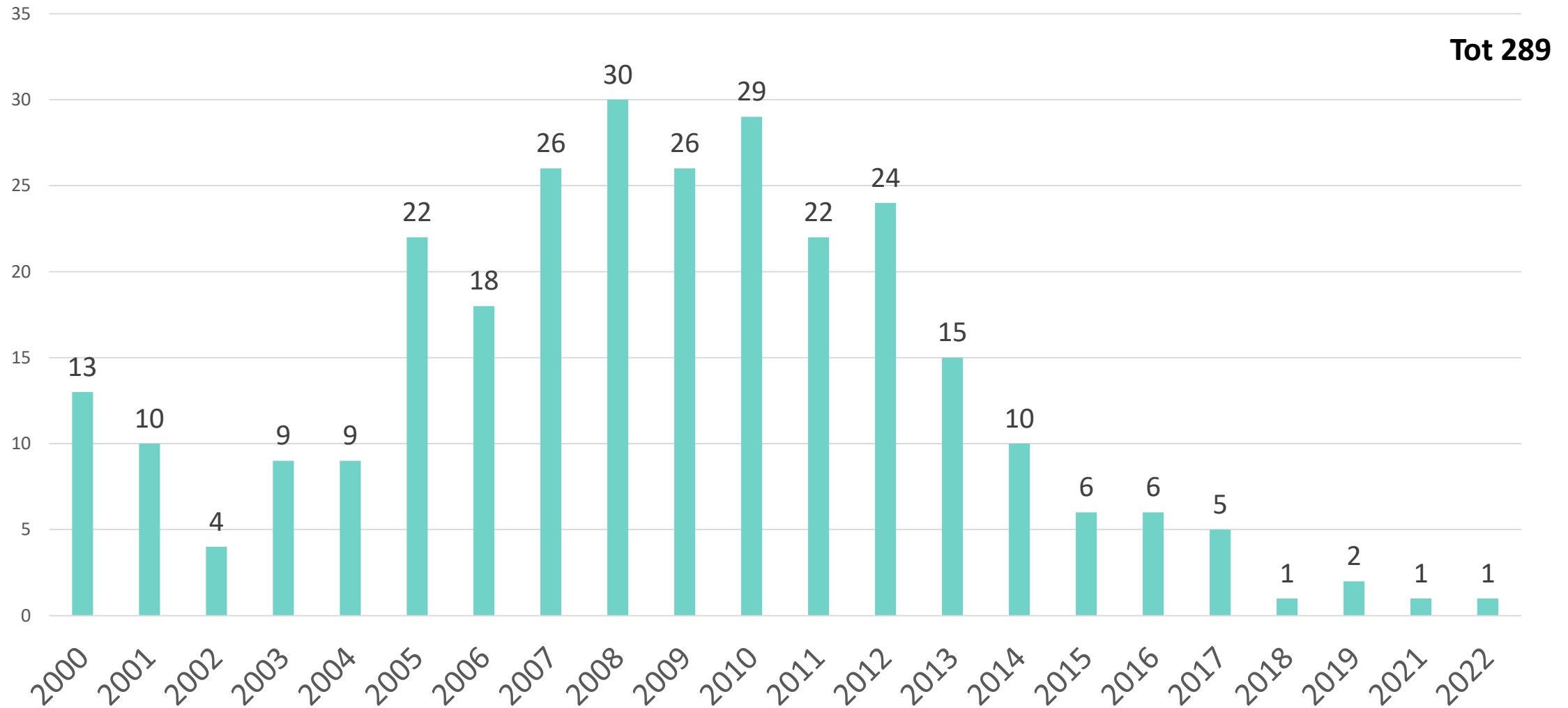
Anno	Ricevente < 18 anni	Ricevente ≥ 18 anni	Totali
2020	12	13	25
2021	6	18	24
2022	4	18	22
2023	4	10	14
2024	1	10	11
Totali	27	69	96

- 44%

Il ricorso al SCO come fonte cellulare per il trapianto è andato calando negli ultimi anni sia nella popolazione adulta che in quella pediatrica



TCSE CB nella popolazione pediatrica affetta da leucemia acuta 2000-2025



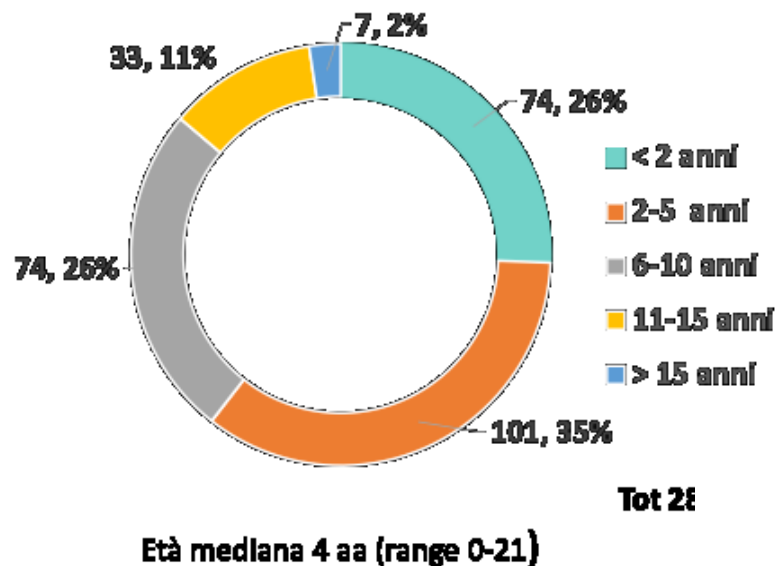
Registro AIEOP TCSE

Ottobre 2025

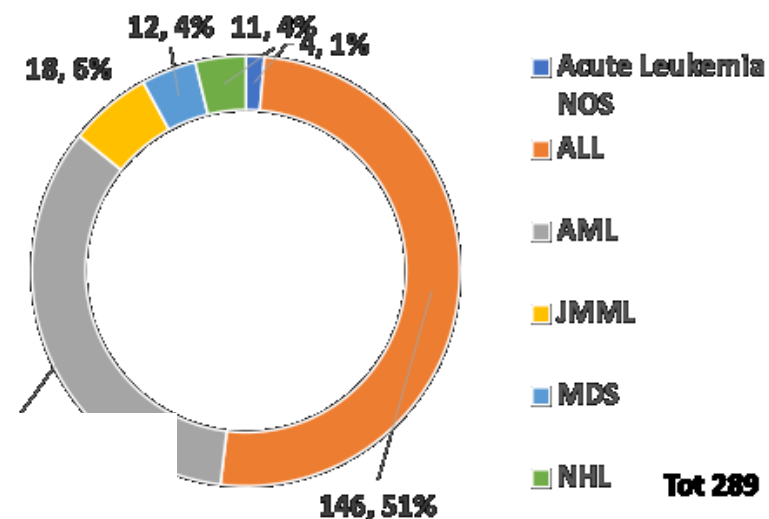
La disponibilità del registro AIEOP-TCSE ha consentito di raccogliere i dati relativi a 289 procedure eseguite fra il 2000-2025

TCSE CB nella popolazione pediatrica

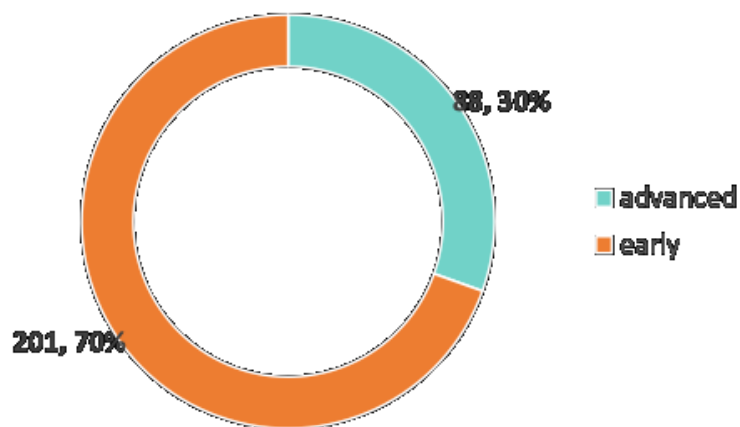
Età al trapianto



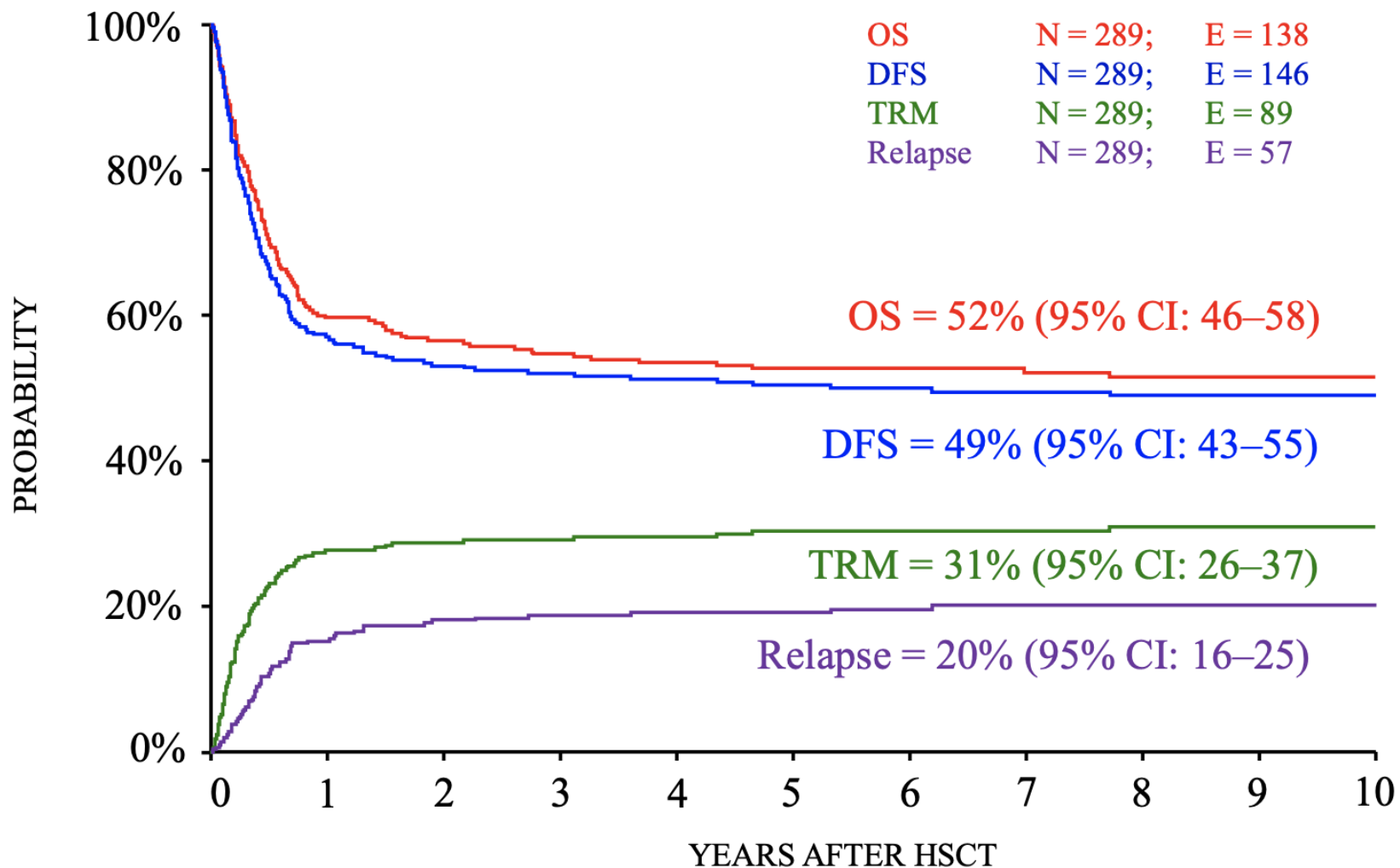
Patologia



Fase al TCSE*



*advanced= no CR al HSCT e/o 2^ HSCT
early= tutte le altre situazioni



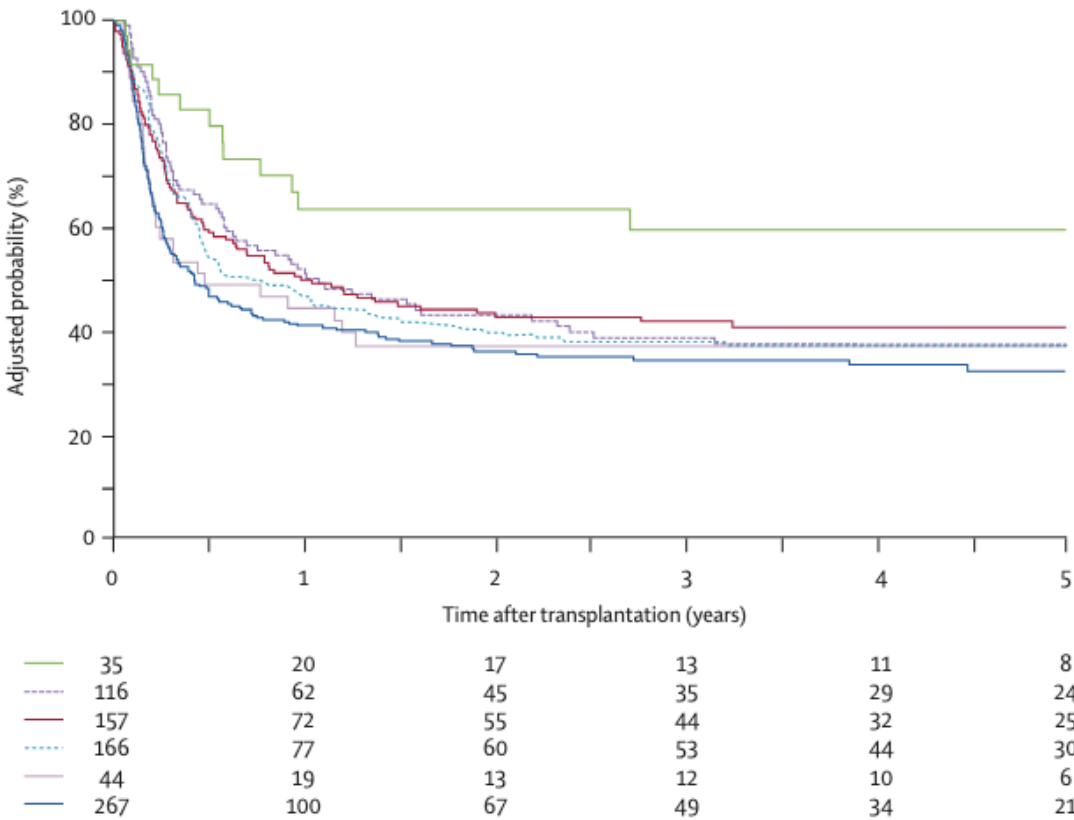
Outcomes of transplantation of unrelated donor umbilical cord blood and bone marrow in children with acute leukaemia: a comparison study

Mary Eapen, Pablo Rubinstein, Mei-Jie Zhang, Cladd Stevens, Joanne Kurtzberg, Andromachi Scaradavou, Fausto R Loberiza, Richard E Champlin, John P Klein, Mary M Horowitz, John E Wagner

Leucemia acuta < 16 anni

503 CB versus 282 BM

EFS, RI e TRM
sovrapponibili
(se CB TNC > 2 x 10⁷ /kg e
HLA-matched)



	Matched bone marrow (n=116)	Mismatched bone marrow (n=166)	Matched umbilical cord blood (n=35)	One-antigen mismatched umbilical cord blood (low cell dose) (n=44)	One-antigen mismatched umbilical cord blood (high cell dose) (n=157)	Two-antigen mismatched umbilical cord blood (any cell dose) (n=267)
Male/female	73 (63%)/43 (37%)	98 (59%)/68 (41%)	22 (63%)/13 (37%)	26 (59%)/18 (41%)	76 (48%)/81 (52%)	160 (60%)/107 (40%)
Age at transplant (years)						
≤1	3 (3%)	5 (3%)	3 (9%)	2 (5%)	22 (14%)	11 (4%)
>1-5	28 (24%)	44 (27%)	14 (40%)	5 (11%)	66 (42%)	73 (27%)
>5-10	46 (40%)	56 (34%)	12 (34%)	19 (43%)	53 (34%)	89 (33%)
>10-≤16	39 (34%)	61 (37%)	6 (17%)	18 (41%)	16 (10%)	94 (35%)
Race						
White	102 (88%)	116 (70%)	30 (86%)	27 (61%)	111 (71%)	139 (52%)
Non-white	14 (12%)	50 (30%)	5 (14%)	17 (39%)	46 (29%)	128 (48%)
Disease						
AML	36 (31%)	60 (36%)	16 (46%)	8 (18%)	69 (44%)	101 (38%)
ALL	80 (69%)	106 (64%)	19 (54%)	36 (82%)	88 (56%)	166 (62%)
Disease status						
First CR	20 (17%)	34 (20%)	6 (17%)	7 (16%)	44 (28%)	49 (18%)
≥ Second CR	78 (67%)	103 (62%)	20 (57%)	25 (57%)	71 (45%)	135 (51%)
Relapse	18 (16%)	29 (17%)	9 (26%)	12 (27%)	42 (27%)	83 (31%)
Median time from diagnosis to transplant* (range)†	23 (3-119)	20 (3-151)	13 (3-109)	16 (3-74)	10 (2-139)	15 (2-146)
Year of transplant						
1995-98	66 (57%)	97 (58%)	17 (49%)	11 (25%)	93 (59%)	158 (59%)
1999-2003	50 (43%)	69 (42%)	18 (51%)	33 (75%)	64 (41%)	109 (41%)
Conditioning regimen						
TBI	98 (84%)	153 (92%)	24 (69%)	36 (82%)	106 (68%)	208 (78%)
Non-TBI	18 (16%)	13 (8%)	11 (31%)	6 (14%)	48 (30%)	54 (20%)
Unknown	2 (4%)	3 (2%)	5 (2%)
GVHD prophylaxis						
Cyclosporine	89 (77%)	137 (83%)	33 (94%)	39 (89%)	136 (87%)	231 (87%)
Tacrolimus	25 (22%)	28 (17%)	2 (6%)	3 (7%)	15 (10%)	22 (8%)
Other agents	2 (2%)	1 (1%)	2 (1%)	9 (3%)
Unknown	2 (4%)	4 (3%)	5 (2%)
Donor-recipient sex match						
M-M	53 (46%)	60 (36%)	15 (43%)	12 (27%)	39 (25%)	71 (27%)
M-F	23 (20%)	39 (23%)	6 (17%)	6 (14%)	35 (22%)	54 (20%)
F-M	20 (17%)	38 (23%)	6 (17%)	13 (30%)	36 (23%)	85 (32%)
F-F	20 (17%)	29 (17%)	7 (20%)	12 (27%)	46 (29%)	50 (19%)
Unknown	1 (3%)	1 (2%)	1 (1%)	7 (3%)
Total nucleated cell dose						
Median (range)‡	4.2 (<1.0-8.0)	3.5(<1.0-9.0)	0.45 (0.10-2.0)	0.22 (0.10-0.30)	0.69 (0.30-3.5)	0.48(0.01-3.2)
Follow-up of survivors						
Median time* (range)	60 (8-123)	59 (11-121)	45 (3-124)	56 (12-120)	40 (3-121)	44 (3-119)

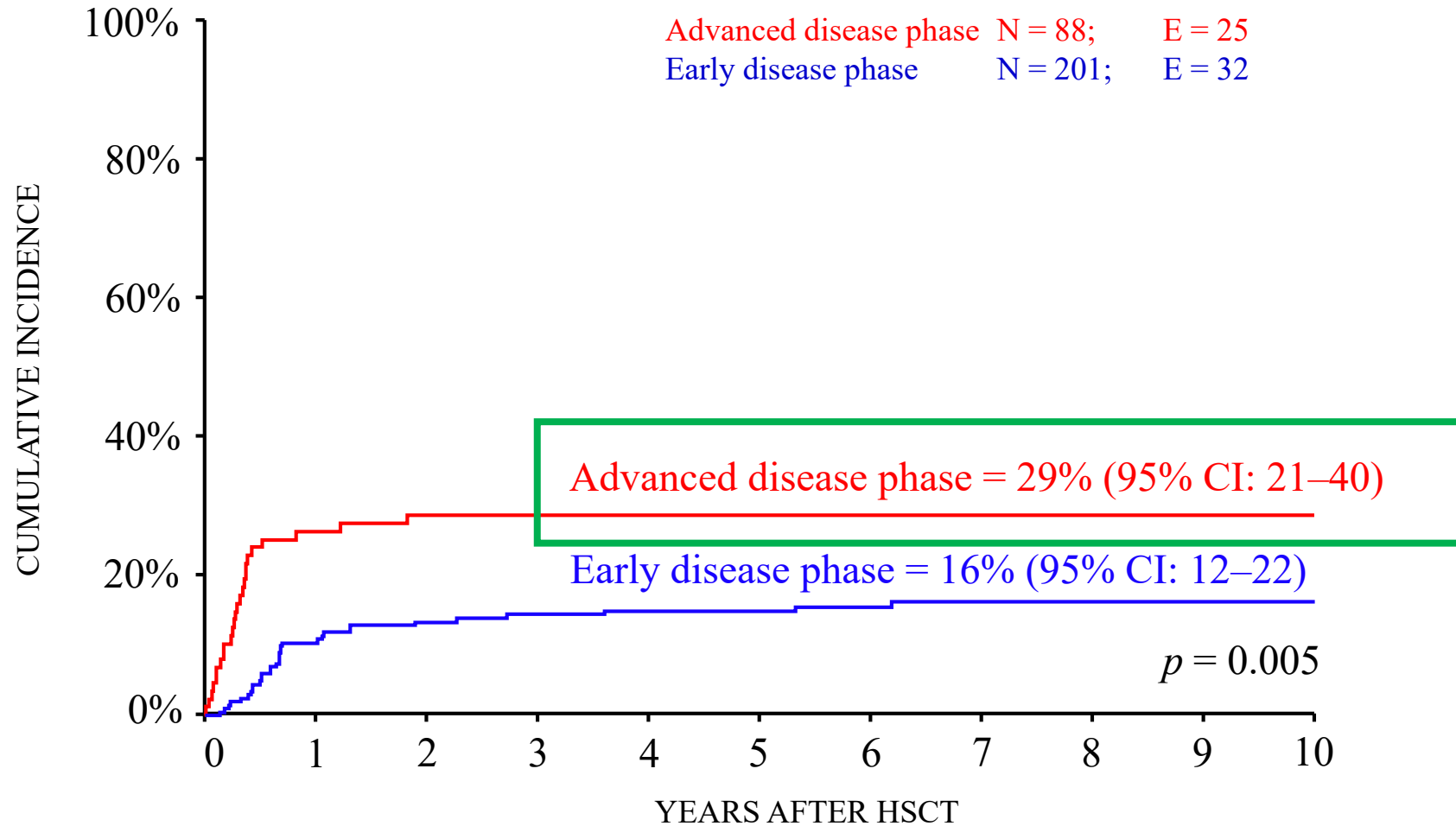
Data are number (%) unless specified otherwise. 91 transplant teams provided patients for this study, 45 of which contributed with one to five patients, 28 with six to ten patients, and 18 teams with more than ten patients. 28 teams reported bone-marrow and cord-blood grafts and contributed about 50% of the patients, 25 reported only bone-marrow grafts and 38 only cord-blood grafts. CR=continuous complete remission, TBI=total body irradiation, M=male, F=female. *Data are expressed in months. †70-80% of transplants took place within 3 years from diagnosis. ‡x10⁷/kg.

Table 1: Characteristics of study patients

Il trapianto CB è in grado di produrre gli stessi effetti del trapianto da midollo osseo o da cellule staminali ematopoietiche periferiche nei pazienti affetti da emopatie maligne

Risultati del TCSE CB nella popolazione pediatrica

Incidenza di recidiva

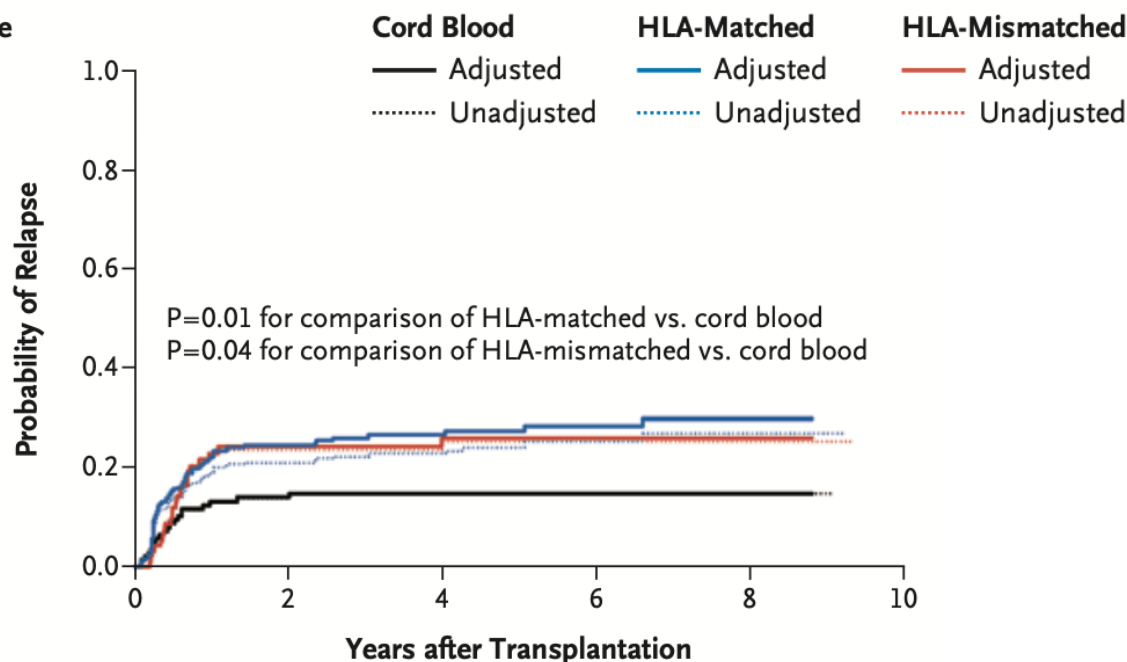


Il trapianto CB si è dimostrato in grado di controllare malattie con un rischio di recidiva particolarmente elevato

Risultati del TCSE CB nella popolazione adulta

Cord-Blood Transplantation in Patients with Minimal Residual Disease

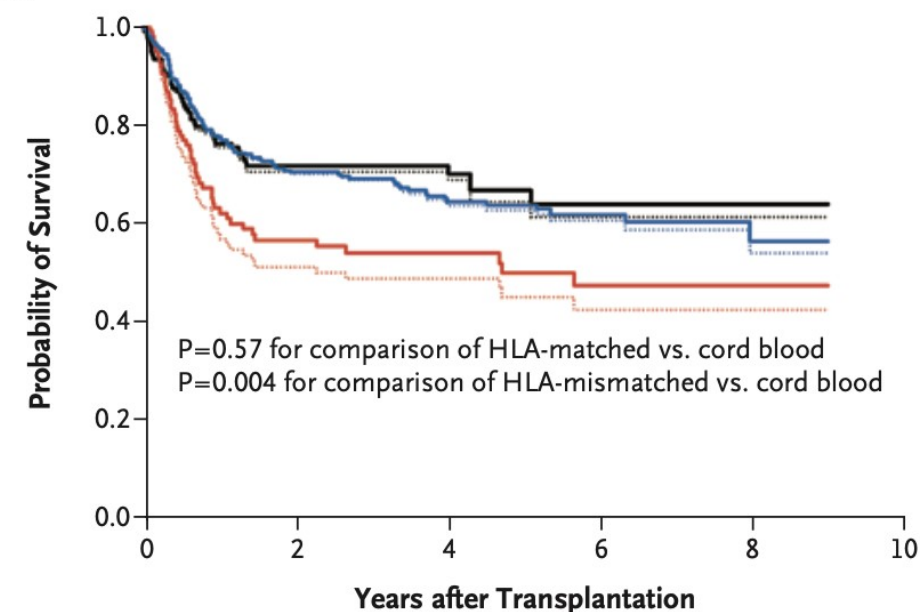
B Relapse



No. at Risk

Cord blood	140	74	39	13	4
HLA-matched	344	161	87	35	11
HLA-mismatched	98	40	29	15	6

Survival



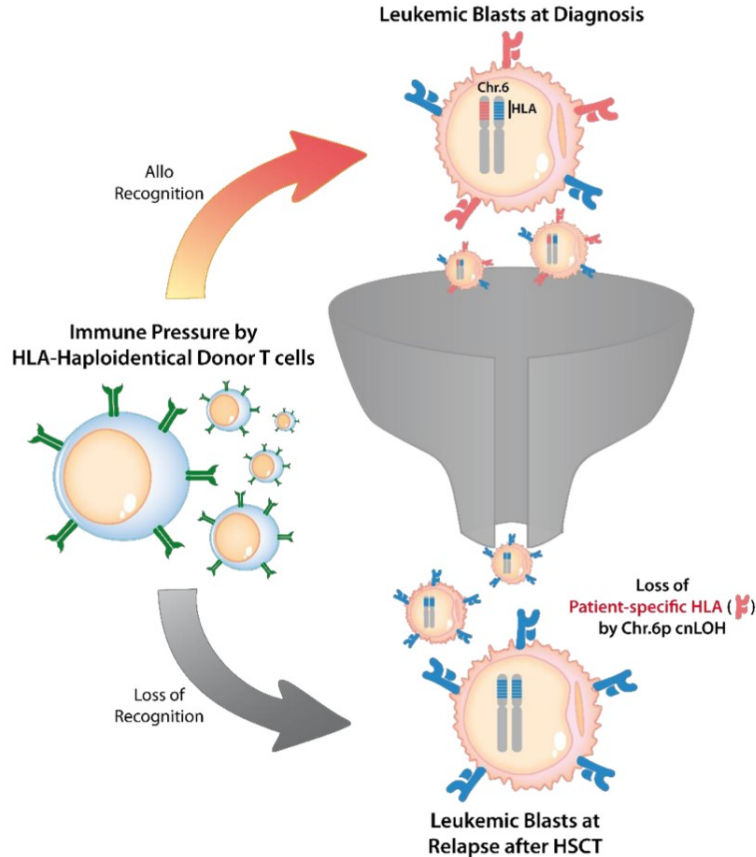
No. at Risk

Cord blood	140	73	36	13	4
HLA-matched	344	170	95	39	11
HLA-mismatched	98	39	28	16	6

Il trapianto CB si è dimostrato in grado di controllare malattie con un rischio di recidiva particolarmente elevato anche nella popolazione adulta

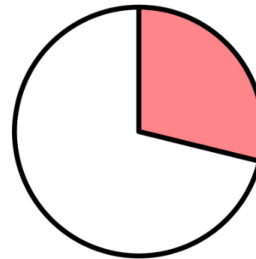
Milano et al New Engl J Med 2016

Meccanismi biologici della ridotta incidenza di recidive post TCSE CB



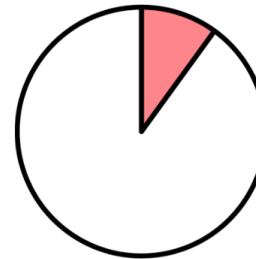
HLALOSS study: 27 centres (20 European, 5 USA, 2 Japan)
More than 500 post-transplantation relapses analyzed by NGS

Haplo
5-6 HLA mm
n=222



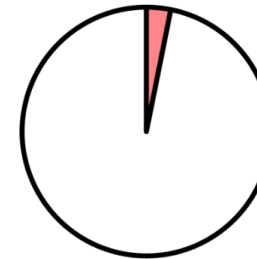
HLA loss
n= (29%)

MMUD
3-4 HLA mm
n=110



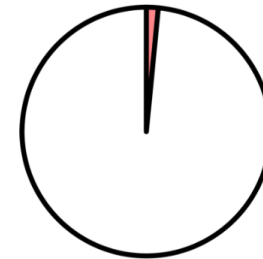
HLA loss
n=11 (10%)

MUD
1-2 HLA mm
n=127



HLA loss
n=4 (3%)

Cord Blood
4-6 HLA mm
n=65



HLA loss
n=1 (1%)



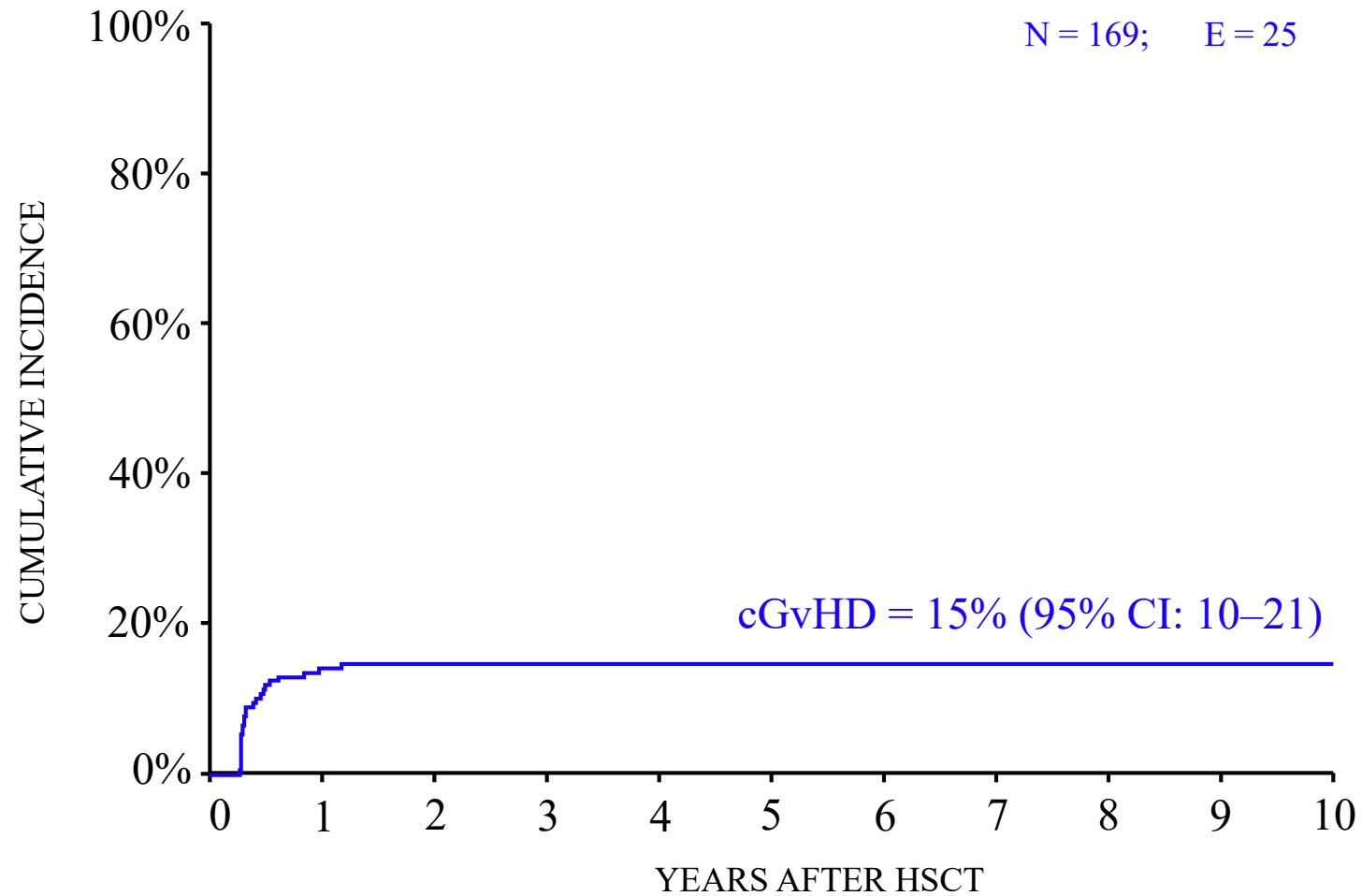
■ HLA Loss
□ Classical

Vago et al, under submission

Vago et al NEJM 2009
Vago et al Transpl Cell Therapy 2017

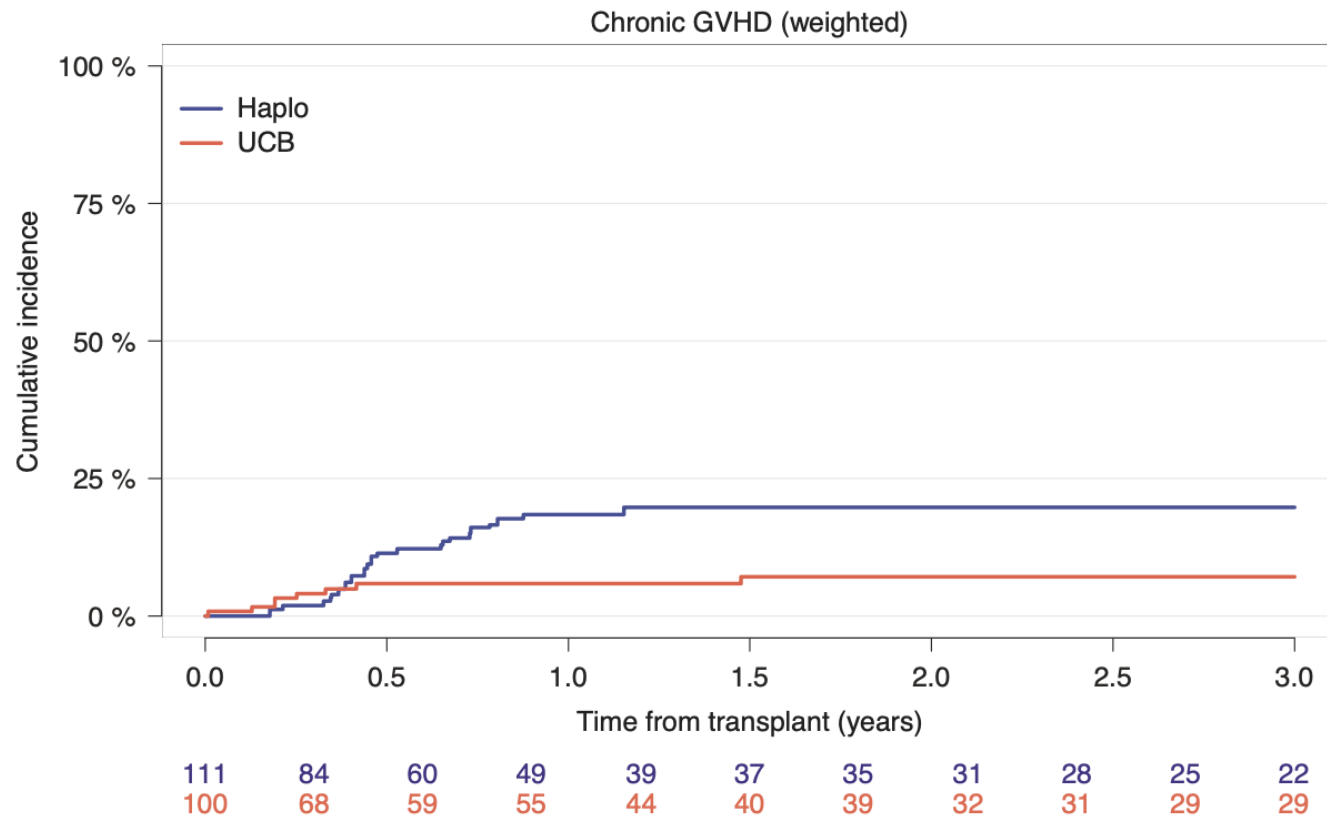
Risultati del TCSE CB nella popolazione pediatrica

cGVHD



Il trapianto CB è associato ad una incidenza di gvHD cronica ridotta, complicanza particolarmente temibile in pediatria

Single unrelated umbilical cord blood *versus* unmanipulated haploidentical HCT using PTCy in pediatric AML: a retrospective study on behalf of the EBMT PDWP and CTIWP

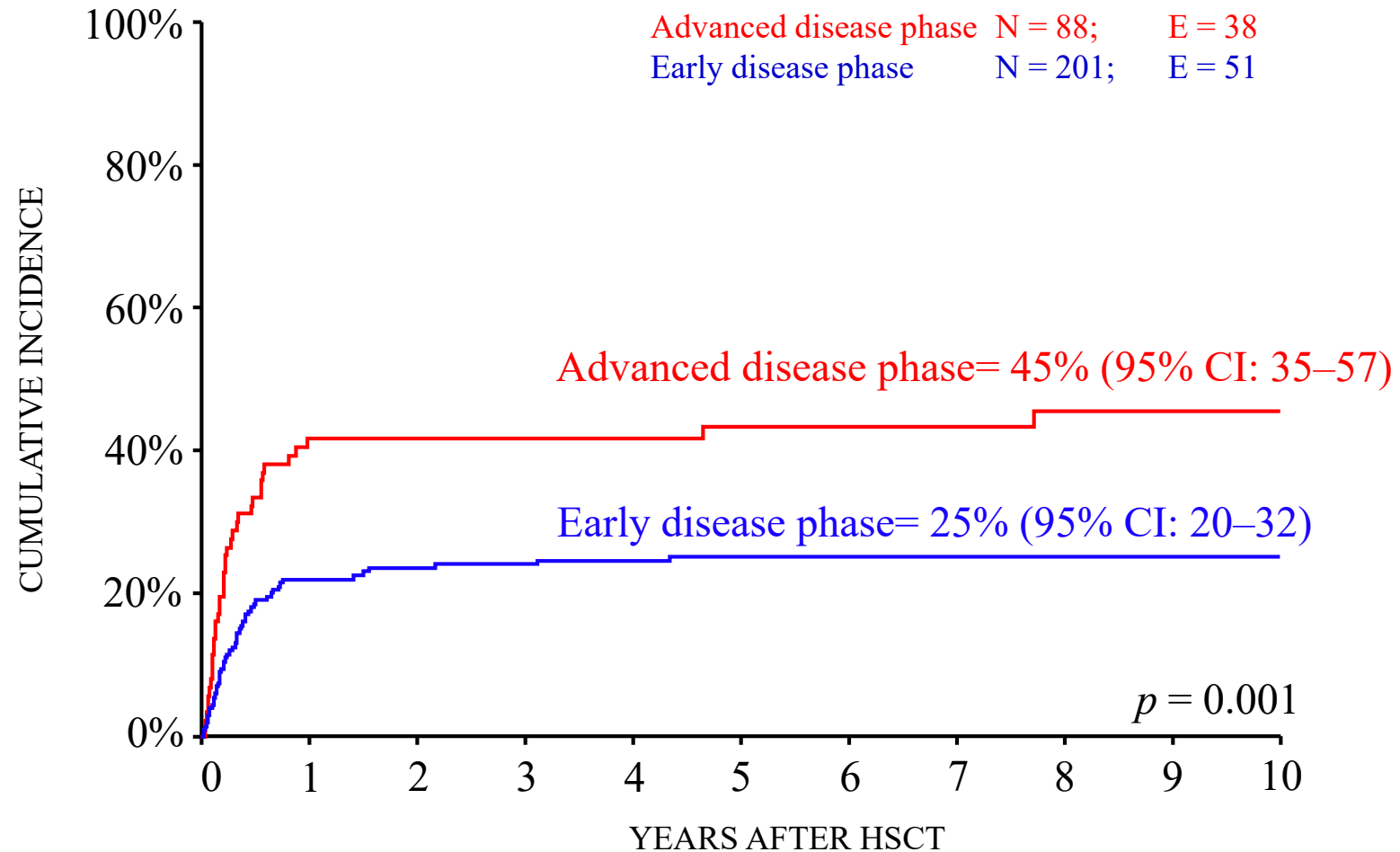


Di Majo et al Bone Marrow Transpl 2025

Il trapianto CB è associato ad una incidenza di gvHD cronica significativamente ridotta rispetto al trapianto aploidentico

Risultati del TCSE CB nella popolazione pediatrica

Mortalità legata al trapianto – TRM

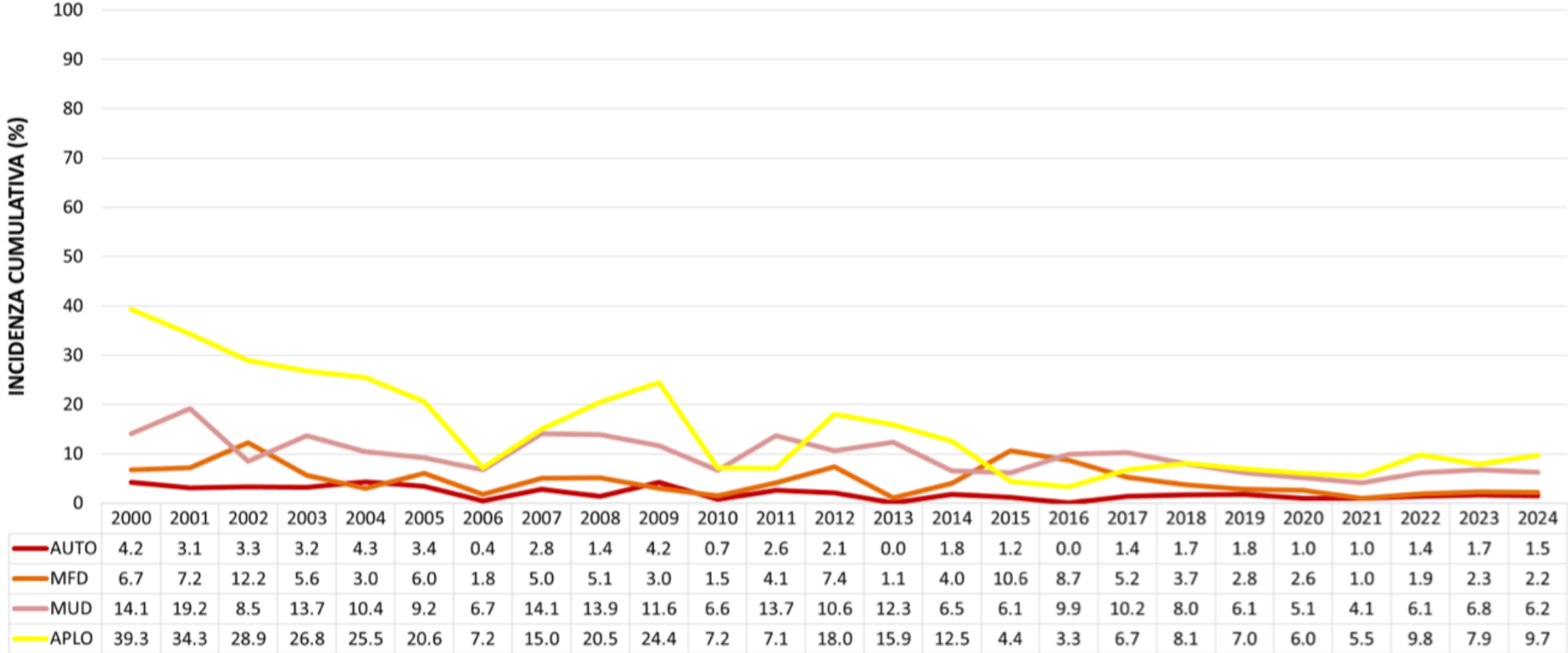


Registro AIEOP TCSE

Ottobre 2025

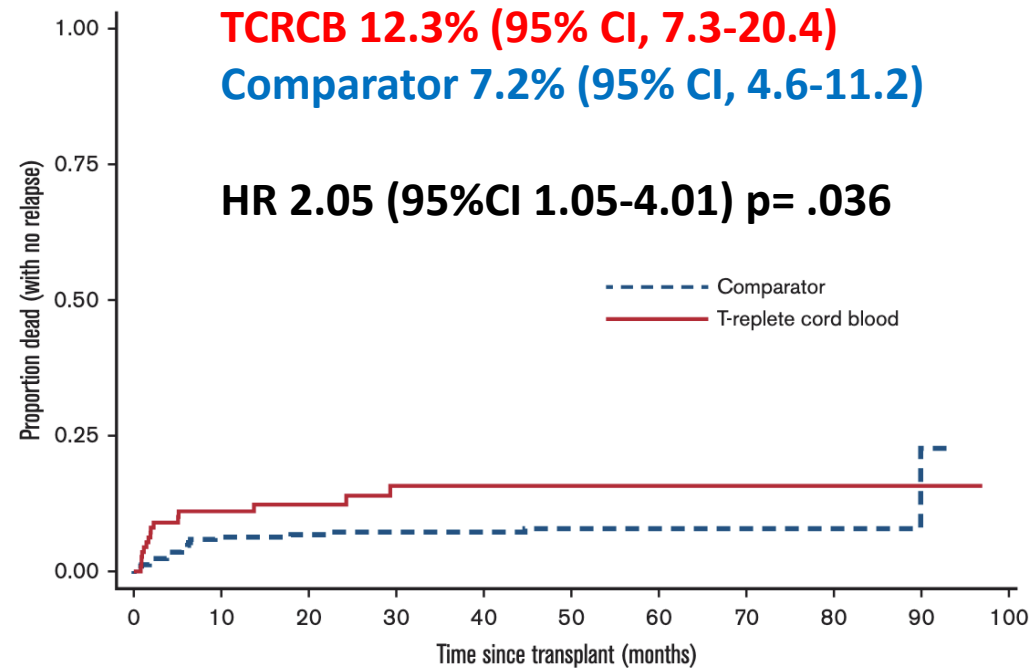
Il trapianto CB risulta tuttora gravato da un rischio di tossicità maggiore rispetto alle altre tipologie di trapianto

Mortalità trapianto-correlata a 100 giorni dal trapianto per anno e tipo di trapianto



T-cell replete cord transplants give superior outcomes in high-risk and relapsed/refractory pediatric myeloid malignancy

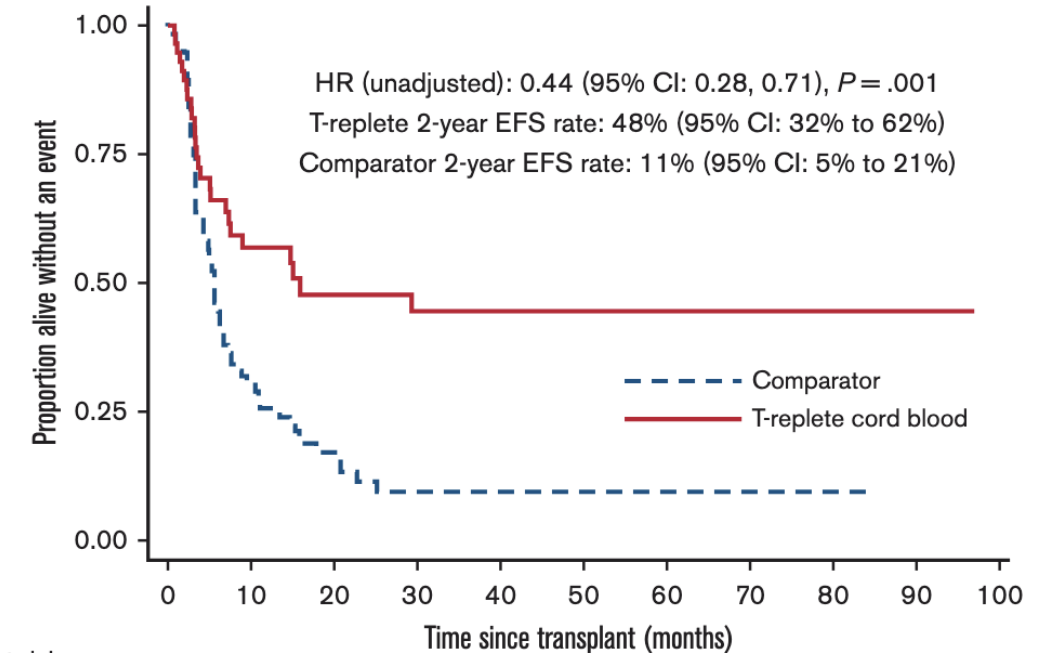
TRM



TRCB (112 patients) or other cell source (255 patients)
 Pediatric AML/MDS
 CBU HLA 8/8 matched – NO ATG!!!!

A

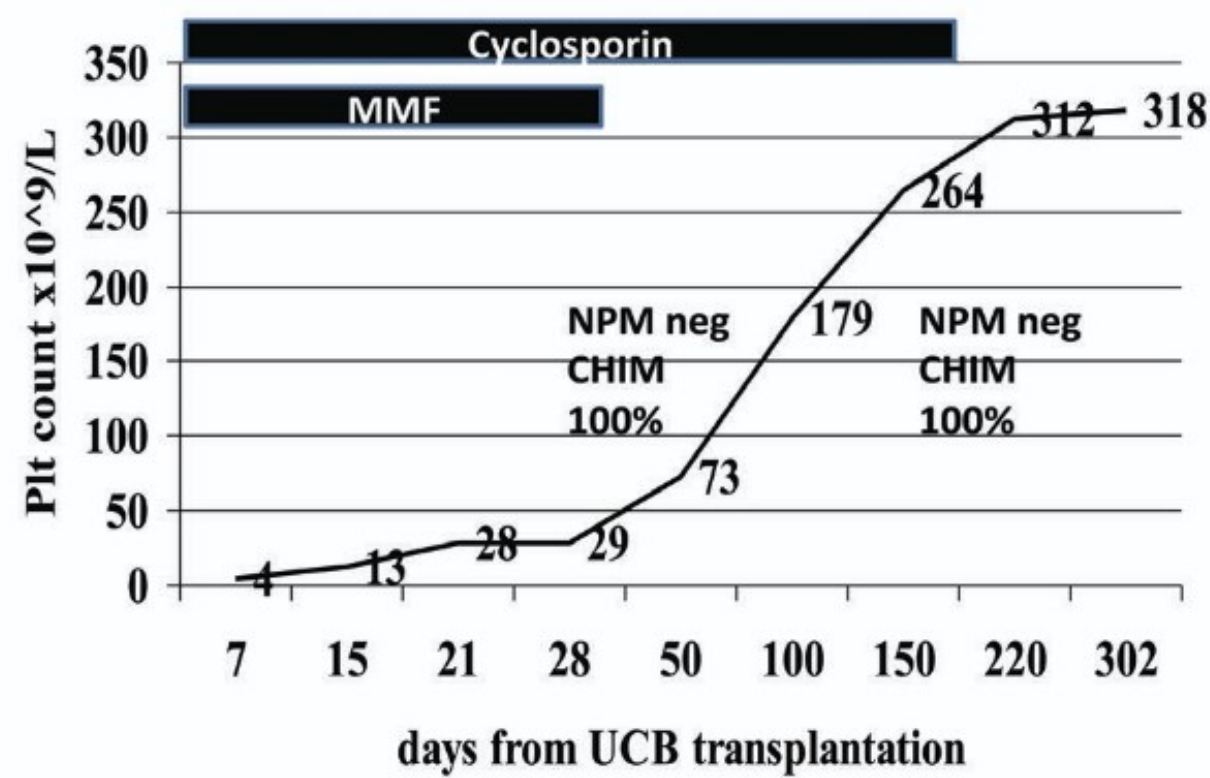
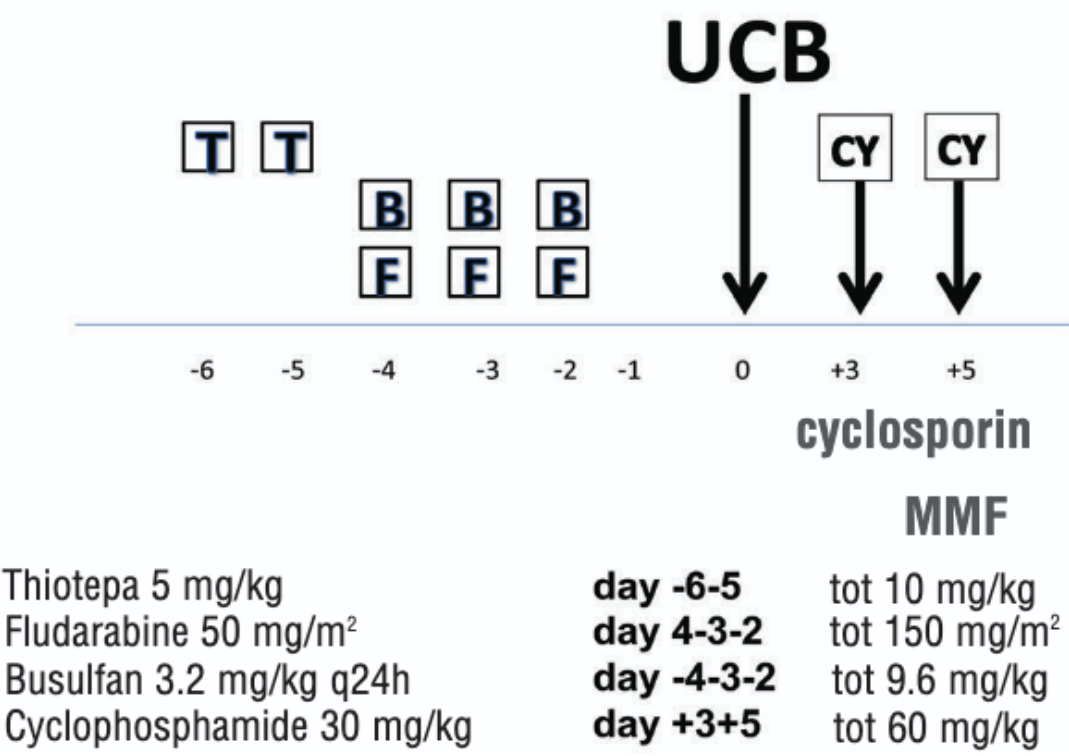
GFRF for MRD Positive



Number at risk

Comparator	60	18	9	5	5	5	4	3	1	0
T-replete cord blood	57	23	15	14	10	5	2	1	1	1

Unrelated cord blood transplantation and post-transplant cyclophosphamide



Bacigalupo et al Haematologica 2019

Take Home Messages

- *Il TCSE CB è in calo in tutti i contesti*
- *Probabilmente la concezione del TCSE CB come alternativa universale al TCSE aploidentico deve essere superata*
- *Il TCSE CB potrebbe offrire alcuni vantaggi in tutte le situazioni in cui sia necessario massimizzare l'effetto anti-tumorale della procedura (i.e. 2^a TCSE, non remissione al momento del trapianto, MRD positività etc...) senza incrementare il rischio di GvHD*

...un sentito ringraziamento



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