Comprehensive Cancer Center
Tübingen-Stuttgart

Post ASH 2025 Orlando

Stammzelltransplantation/Zelluläre Therapie

Prof. Dr. W. Bethge

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Themen

Stammzelltransplantation:

1. #171: Kröger: Randomisierte Studie MUD oder Haplo Post Cy
2. #506: Robin: Haplo SZT in Myelofibrosis – Phase 2 Studie
3. #936: Jimenez: ACCESS Trial Mismatched MUD Post Cy

Zelluläre Therapie:

1. #570: Wang: CD19/CD22 bispecific CAR-T cells for R/R LBCL
2. #1041: Chiesa: Universal CAR-T targeting CD7 for T-ALL
3. #815: Roddie: CD19 CAR-T for SLE

#171 Matched unrelated vs haploidentical donor for SZT with identical GvHD prophylaxis

American Society of Hematology
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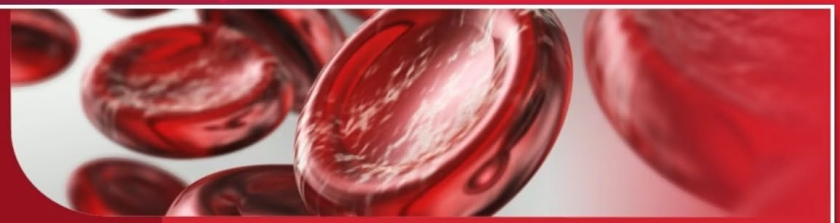
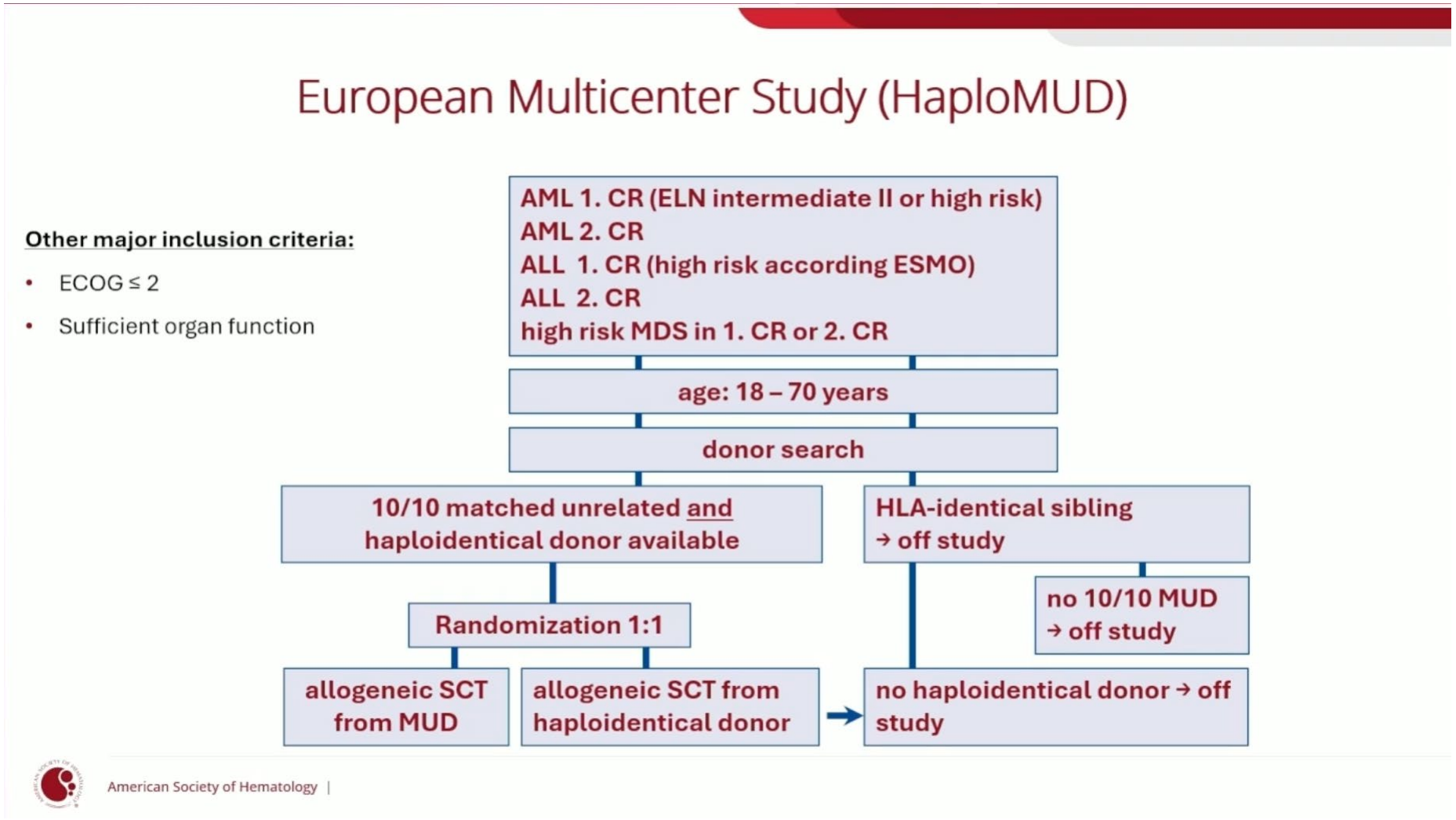


Matched unrelated vs. Haploidentical donor for allogeneic stem cell transplantation in patients with acute leukemia with identical GvHD prophylaxis – a randomized prospective european trial

Nicolaus Kröger, Jaime Sanz, Matthias Stelljes, Rodrigo Martino, Alessandro Rambaldi, Mi Kwon, Fermin Sanchez-Guijo, Marketa Markova, Sergey Bondarenko, Matthias Eder, Michael Heuser, Carlos Solano, Francesca Bonifazi, Philipp Wohlfarth, Radwan Massoud, Francis Ayuk, Johannes Clausen, Goda Choi, Hildegard Greinix, Patrizia Chiusolo, Francesca Patriarca, Nico Gagelmann, Alessandro Busca, Gesine Bug, Thomas Schroeder, Wolfgang Bethge, Annemarie Glöckner, Dieter Hoelzer, Selia Zeyß, Hannes Buchner, Christine Wolschke

67th ASH® Annual Meeting and Exposition

#171 Matched unrelated vs haploidentical donor for SZT with identical GvHD prophylaxis



#171 Matched unrelated vs haploidentical donor for SZT with identical GvHD prophylaxis

HaploMUD Study

Conditioning in both arms:

≤ 50 years:

Busulfan (12.8 mg/kg) w/o Thiotepa (10mg/kg) and fludarabine (160 mg/m^2) or TBI (12 Gy) / fludarabine (160 mg/m^2)

> 50 years (or Sorror score ≥ 3):

Busulfan (9.6 mg/kg) w/o Thiotepa (5mg/kg) and fludarabine (160 mg/m^2) or TBI (8 Gy) / fludarabine (160 mg/m^2)

GVHD prophylaxis in both arms:

Cyclophosphamide	50 mg/kg on day +3 and +4
Tacrolimus	from day +5 until day +120
MMF	from day +5 until day +35

Stem Cell Source:

PBSC

Statistics

- To detect a difference of 10% in relapse incidence at 2 years, 220 patients per arm are needed.
- A planned interim and futility analysis was performed after 171 pts were included and 158 pts were randomized to MUD (n=78) or HaploHCT (n= 80)

Countries n=7 centers n= 22

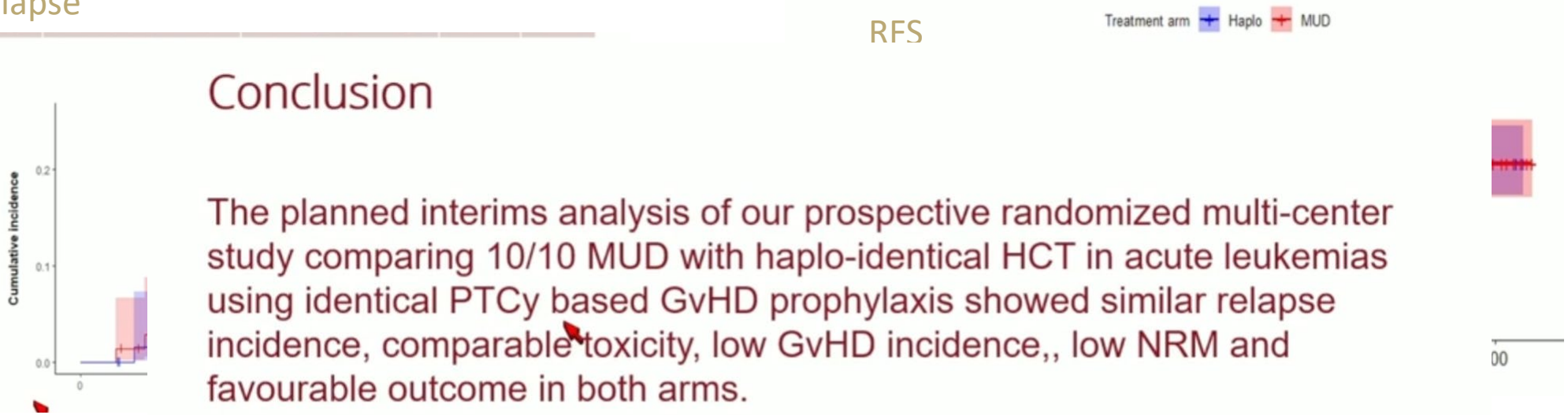
Results

	MUD	Haplo
Primary graft failure	0 (0%)	3 (5%)
Engraftment		
ANC > 1.0x10e9/L days(range)	18 (13-49)	19 (13-36)
platelets > 20x10e9/L days(range)	16 (1-76)	23 (6-87)
Infectious complications	40%	31%
SAE grade 3 or higher	40%	36%
Acute GvHD		
Grade I-IV	25(36%)	30 (42%)
Grade III/IV	3 (4%)	5 (7%)
Chronic GvHD	15 (22%)	12(17%)
Mild	9 (13%)	5 (7%)
Moderate	5 (7%)	5 (7%)
Severe	1(1%)	2 (3%)

#171 Matched unrelated vs haploidentical donor for SZT with identical GvHD prophylaxis

Relapse

RFS

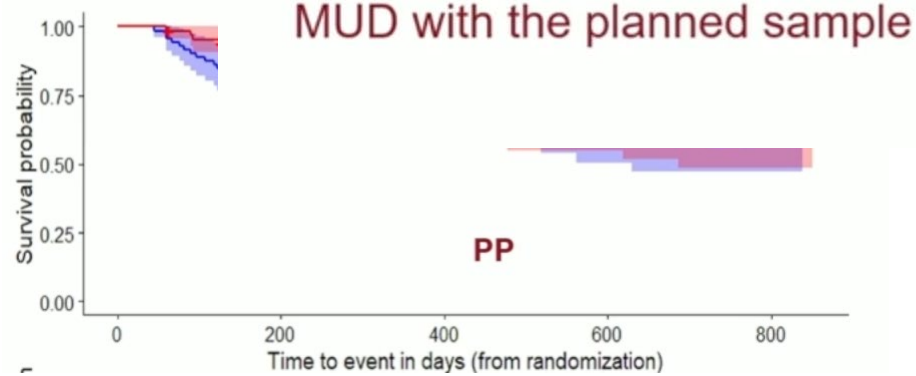


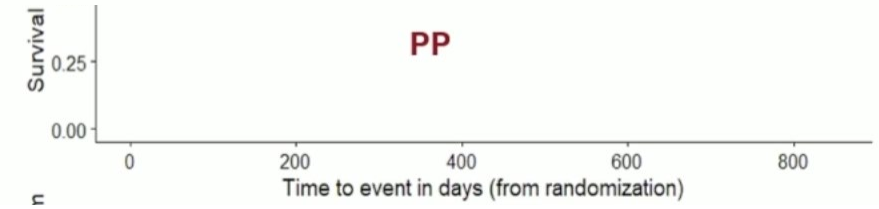
Conclusion

The planned interims analysis of our prospective randomized multi-center study comparing 10/10 MUD with haplo-identical HCT in acute leukemias using identical PTCy based GvHD prophylaxis showed similar relapse incidence, comparable toxicity, low GvHD incidence,, low NRM and favourable outcome in both arms.

The DSMB recommend stopping recruitment because it is unlikely to show a 10% superiority regarding endpoint of relapse incidence by HaploHCT to MUD with the planned sample size.

GFRS





#506 Haploidentical SZT for Myelofibrosis

Haplo-identical transplantation in patients with myelofibrosis

A phase two prospective multicentric study

Marie Robin[1], Michael Loschi[2], Marie-Thérèse Rubio[3], Etienne Daguindau[4], Felipe Suarez[5], Anouk Walter-Petrich[6], Alban Villate[7], Ibrahim Yakoub-Agha[8], Martin Carre[9], Sylvain Thepot[10], Mathilde Ruggiu[1], Patrice Chevallier[11], Anne Huynh[12], Pascal Turlure[13], Raynier Devillier[14], Bruno Cassinat[15], Tony Marchand[16], Clémence Mediavilla[17] and Sylvie Chevret[6]

1- Hematology Department, Saint-Louis Hospital, AP-HP, Paris Cité University, Paris; 2- Hematology Department, UHC Nice; 3- Hematology Department, CHRU Nancy; 4- Hematology Department, UHC Besançon; 5- Hematology Department, UHC Necker, AP-HP, Paris; 6- Biostatistics and Medical Informatics Department, Saint-Louis Hospital, Paris Cité University, AP-HP, Paris; 7- Hematology Department, Tours UHC; 8- Hematology Department, Lille RUHC; 9- Hematology Department, Grenoble UHC; 10- Hematology Department, Angers UHC; 11- Hematology Department, Nantes UHC; 12- Hematology Department, Toulouse UCIT; 13- Hematology Department, UHC Limoges; 14-Institut Paoli Calmette, Hematology Department, Marseille; 15- Cell Biology Laboratory, Saint-Louis Hospital, AP-HP, Paris Cité University, Paris; 16- Hematology Department, UHC Rennes; 17- Hematology Department, UHC Bordeaux

On behalf of the FIM FRANCE INTERGROUPE SYNDROMES MYÉLOPROLIFÉRATIFS — SFGM-TC Société Francophone de GREFFE DE MOELLE et de Thérapie Cellulaire

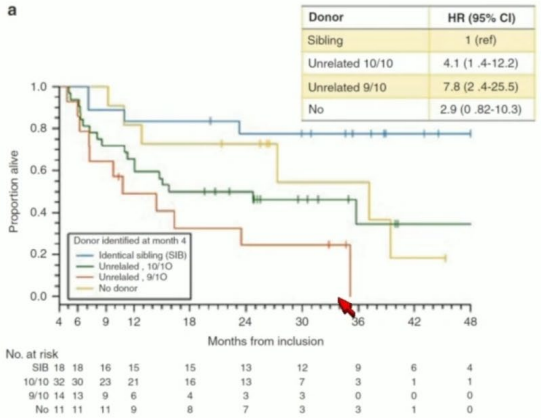
Marie Robin, Associate Professor, Saint-Louis University Hospital, Paris, France — Hôpital Saint-Louis AP-HP — Université Paris Cité

67th ASH® Annual Meeting and Exposition

Rational of the study (2019)

- High mortality after transplantation from HLA-mismatched unrelated donor

- FLU 90 mg/m2
- MEL 140 mg/m2
- +/- ATG
- CSA
- MMF



JAK ALLO prospective trial, NCT01795677

Bone Marrow Transplant. 2021 Aug;56(8):1888-1899.

FIM — SFGM-TC — Hôpital Saint-Louis AP-HP — Université Paris Cité

#506 Haploidentical SZT for Myelofibrosis

Objectives

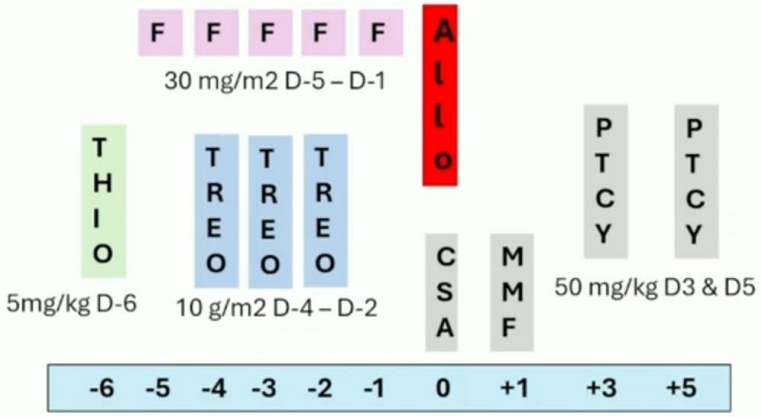
- **Primary**: **To assess the event-free survival 12 months after haplo-identical transplantation.**
- **Secondary**
  - Incidence of acute GVHD grade 2/4 and 3/4 at 100 days
  - Engraftment at 100 days & Rejection incidence at 12 months
  - Incidence of chronic GVHD at 12 months
  - Non-relapse mortality at 12 months
  - Overall survival at 12 months
  - Relapse/progression incidence at 12 months
  - Time to neutrophil and platelet engraftment at 60 days
  - Infection incidence at 100 days and at 12 months
  - Cytokine profile during transplantation
  - Impact of genetic alterations on outcome
  - Amendment : assessment at 24 months

Inclusion criteria

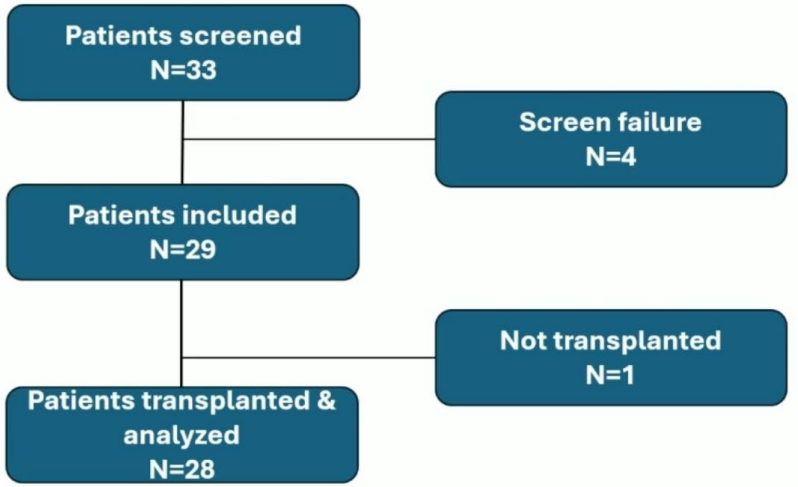
- **Diagnosis:** Primary myelofibrosis or myelofibrosis secondary to essential thrombocythemia or polycythemia vera proven by marrow biopsy
- **Disease criteria,** at least two of the following criteria:
  - Constitutional symptoms: weight loss > 10% in one year, fever (without infection), recurrent muscle, bone or join pains, extreme fatigue
  - Anemia with hemoglobin < 10 gr/dL or red blood cell transfusion
  - Thrombocytopenia < 100 G/L
  - Peripheral blast > 1%
  - White blood cell count > 25 G/L (before a cytoreductive treatment)
  - Karyotype: +8, -7/7q-, i(17q), -5, 5q-, 12p-, inv(3), 11q23
  - *ASXL1* or *TP53* mutation
- **Patient criteria**
  - Age ≤ 70 years
  - Performance status according to ECOG at 0, 1 or 2
- **Donor criteria**: No HLA-matched donor

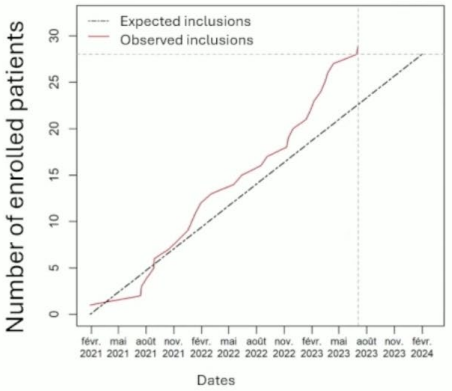
#506 Haploidentical SZT for Myelofibrosis

Conditioning regimen and GVHD prophylaxis



Enrollment





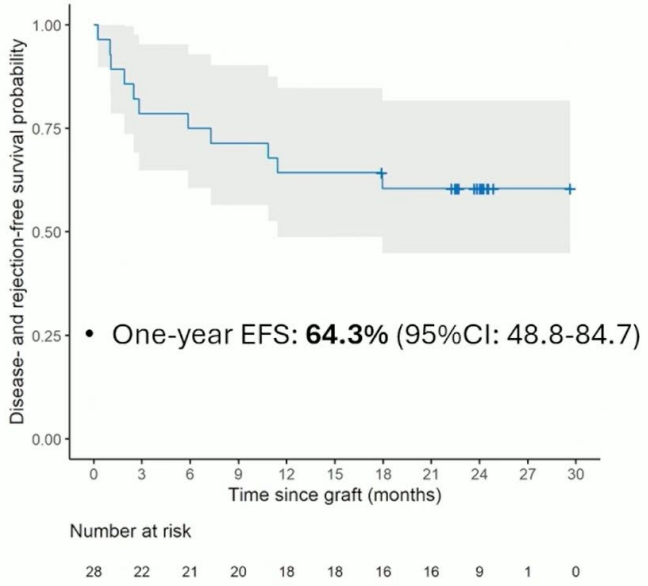
#506 Haploidentical SZT for Myelofibrosis

Patient characteristics

Characteristics	Values
Median age (years, IQR)	65 (58-67)
Patient sex male at birth	19 (68%)
HCT-comorbidity index < 3	22 (79%)
Disease risk	
• General symptoms	11 (39%)
• Anemia < 10 g/dl	21 (75%)
• Platelet count < 100 G/L	13 (46%)
• Peripheral blast > 1%	13 (46%)
• WBC > 25 G/L	7 (25%)
Molecular risk	
• Complex karyotype	3/16 (19%)
• *JAK2, CALR, MPL* mutated, triple neg	19 (61%) / 6 (21%) / 1 (4%) / 2 (7%)
• *ASXL1, TET2, EZH2, RUNX1* mutated	19 (68%) / 7 (25%) / 6 (21%) / 5 (18%)
DIPSS low, int-1, int-2, high	0 / 12 (43%) / 8 (29%) / 8 (29%)

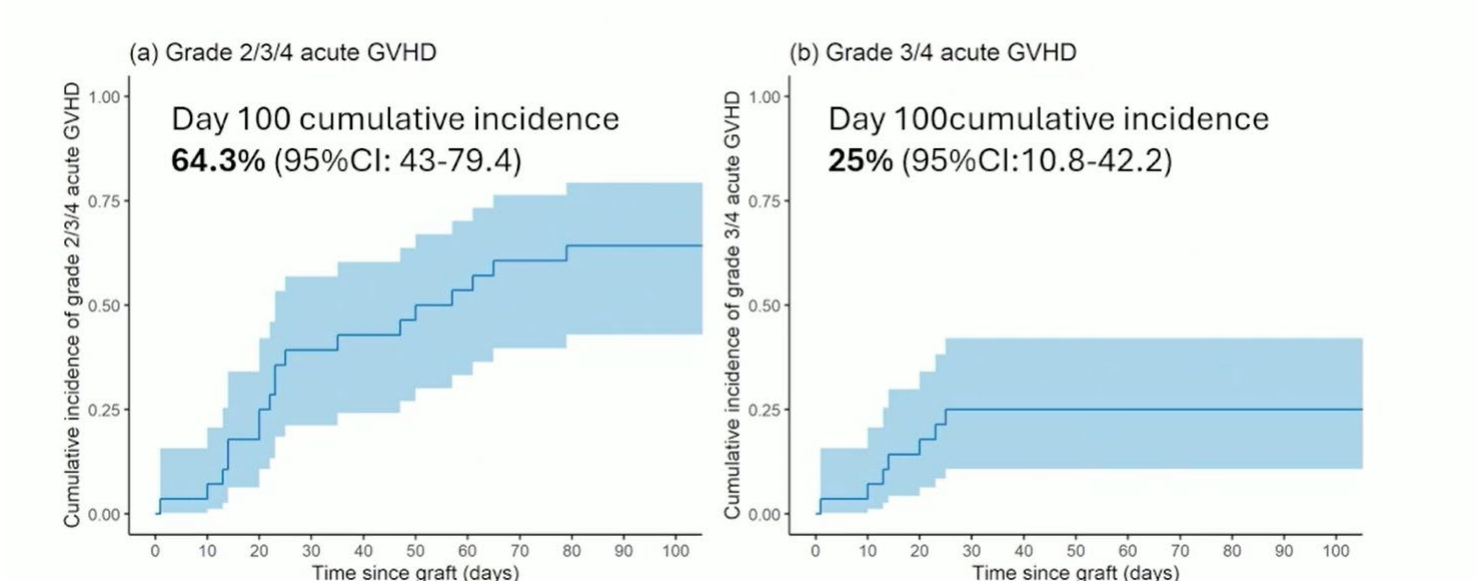
Primary end point: Event-free survival

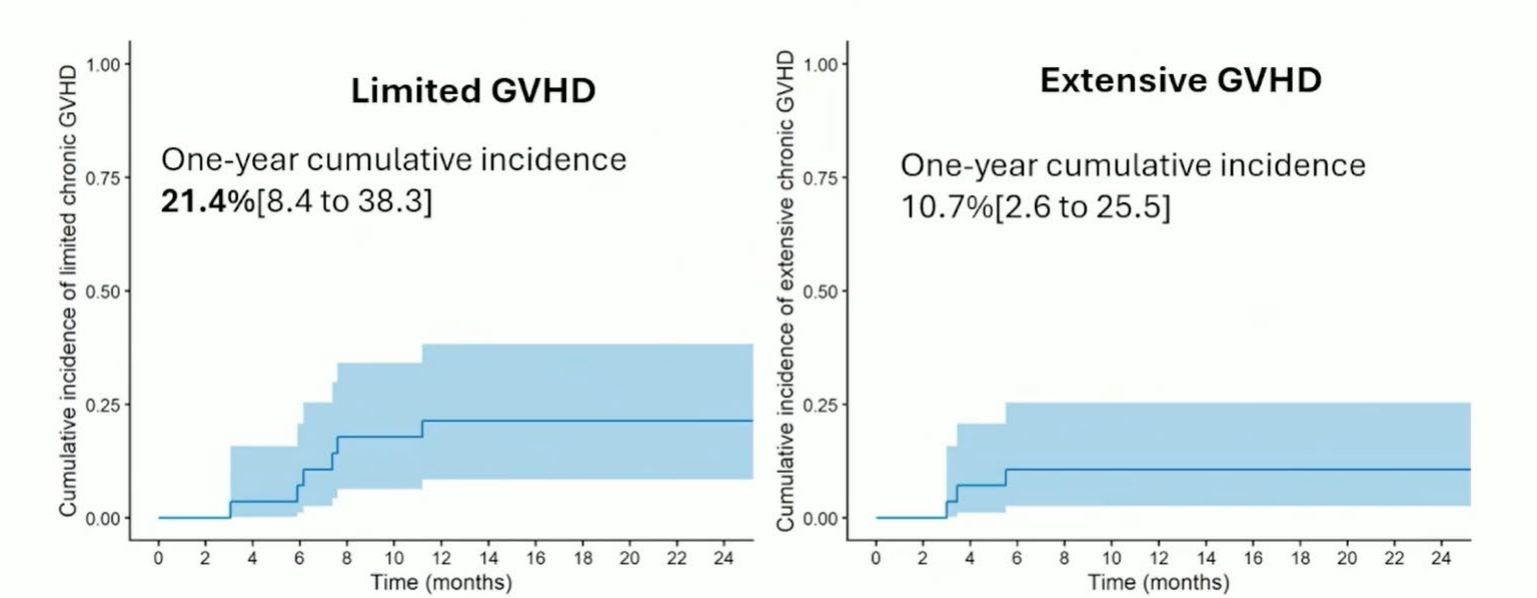
Reminder:
EFS > 55% to prove that haplo-identical transplantation is better than the historical 9/10 UD cohort



- One-year EFS: **64.3%** (95%CI: 48.8-84.7)

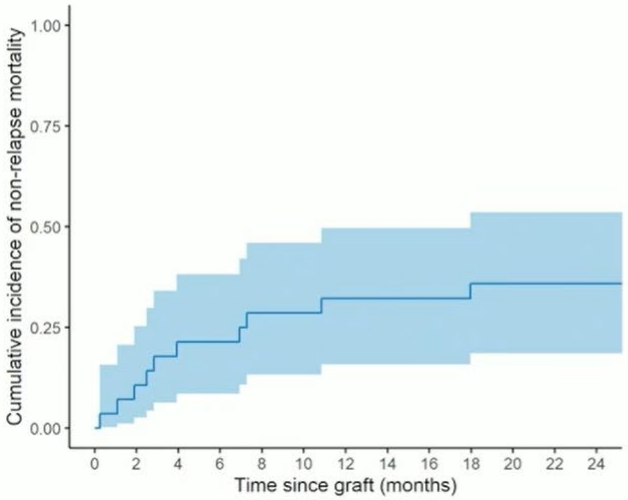
#506 Haploidentical SZT for Myelofibrosis





#506 Haploidentical SZT for Myelofibrosis

One-year cumulative incidence of NRM: **32.1%** (95%CI: 15.8 – 49.7)



Conclusion

- **The primary objective of the trial was achieved**
- Haplo-identical transplantation using **treosulfan, thiotepa and fludarabine + PTCY, CSA, MMF** appears better than results expected with mismatched unrelated donor (historical cohort, FLU MEL, CSA, MMF, ATG)
- This platform may also be used for **mismatched unrelated donors** or matched **unrelated donors**
- The only cause of death is **NRM,** including one non-engraftment and one rejection
- The incidence of acute **GVHD** is relatively high: the challenge may be to improve GVHD prevention without worsening the engraftment, role of ruxolitinib? Role of thiotepa?
- The incidence of **infections** is also high
- More analyses with the **24-month** endpoint will be available soon, as well as the **cytokine profile**

#936: Jimenez: ACCESS Trial Mismatched MUD Post Cy

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A RESEARCH COLLABORATION BETWEEN THE MEDICAL COLLEGE OF WISCONSIN AND NMDP

Mismatching of unrelated donors beyond a single HLA-locus does not adversely impact outcomes at one year following transplantation: Results from t sponsored ACCESS Study

Monzr M. Al Malki, Stephanie Bo-Subait, Brent Logan, Sarah Smith, E
Jeffery Auletta, Stephen Spellman, Craig Malmberg, Medhat Askar, R
Modi, Farhad Khimani, Mahasweta Gooptu, Mehdi Hamadani, Marti
Meade, Uttam Rao, Jordan Milner, Ramzi Abboud, Katarzyna Jamiesc
William Hogan, Ran Reshef, Satyajit Kosuri, Rachel Cook, Karen Balle
Muna Qayed, Sung Choi, Larisa Broglie, Bronwen Shaw, Steven Devir

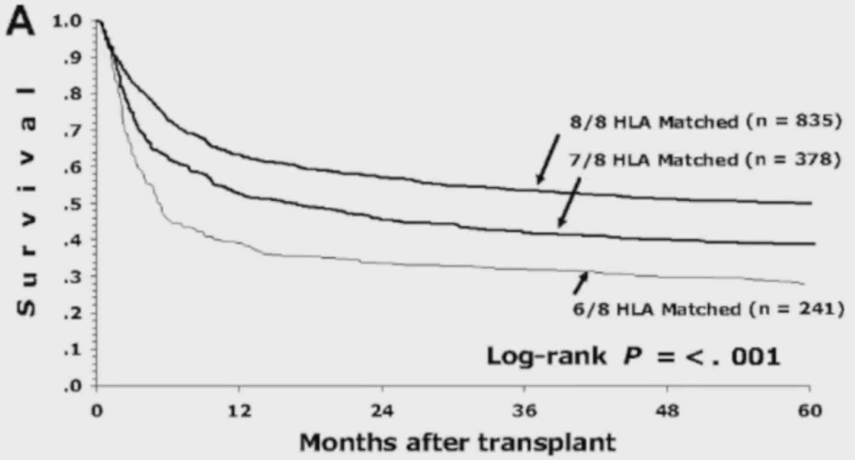
Study Sponsored by:

nmdp
FIND CURES. SAVE LIVES.

NCT04904588

Background:

*Historically, unrelated donor HLA mismatching associated with clinically important and statistically significantly worse survival using standard calcineurin-based GVHD prophylaxis**



Overall Survival	One year	Five year
Match level		
8/8	63%	50%
7/8	**52%**	**39%**
6/8	**39%**	**28%**

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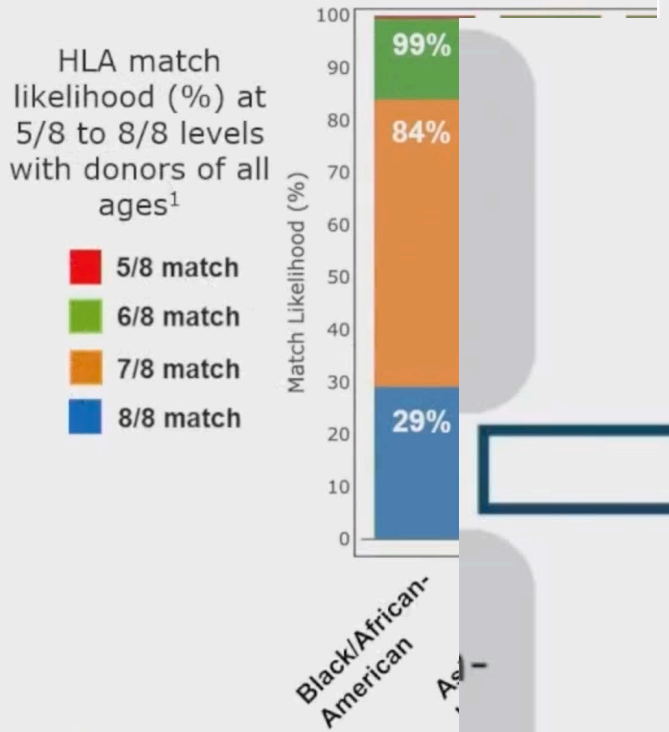
*Lee et al. Blood. 2007 Dec 15;110(13):4576-83

#936: Jimenez: ACCESS Trial Mismatched MUD Post Cy

Background

HLA match likelihood (%) at 5/8 to 8/8 levels with donors of all ages[1]



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Matching Requirement Impacts

Current analysis focused on all adult recipients of <7/8 grafts enrolled on the study across both conditioning strata, with descriptive comparison to patients who received 7/8 MMUD PBSC grafts.

for 70 adults

45 (n=75 MAC
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5

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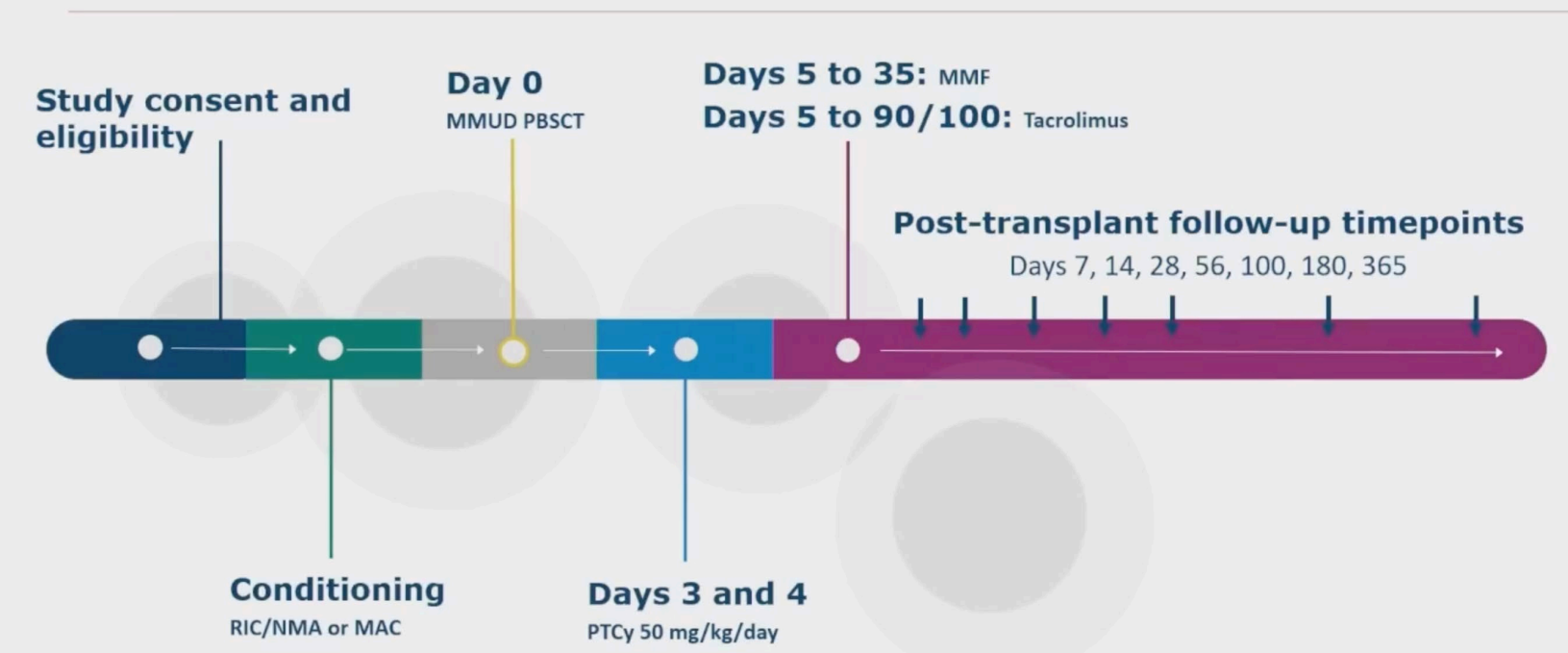
to 193, **see**

of PTCy-based GVHD
al malignancies.

#936: Jimenez: ACCESS Trial Mismatched MUD Post Cy

Key Inclusion Criteria	Key Exclusion Criteria
Hematological malignancy requiring HCT	Availability of a suitable HLA-matched related or 8/8 high resolution matched URD
HCT-CI: 0-4	Presence of donor-specific HLA antibodies to any mismatched allele/antigen with **mean fluorescence intensity > 3000**
PBSC donor product	Prior allogeneic HSC transplant
Patient age > 18 years	Primary myelofibrosis
KPS of ≥ 60%	Concurrent enrollment on other interventional GVHD clinical trial
Available MMUD (4/8-7/8 match at HLA-A, -B, -C, and -DRB1) **≤ 35 years old**	
Estimated creatinine clearance > 60 mL/min	



#936: Jimenez: ACCESS Trial Mismatched MUD Post Cy

Patient Demographics

Characteristic	7/8 n (%)	<7/8 n (%)
No. of patients	183	85
No. of centers	30	21
Age at HCT		
Median (min-max)	**63.3** (20.4-78.9)	**57.4** (24.3-77.9)
Sex		
Male	95 (51.9)	42 (49.4)
Female	88 (48.1)	43 (50.6)
Cryopreservation		
Cryopreserved	117 (60.6)	58 (77.3)
Fresh	76 (39.4)	27 (22.7)
COST score	Median (range)	Median (range)
NHW	30 (4-44)	26.5 (11-44)
Diverse	23 (0-40)	19 (4-41)

Characteristic	7/8 n (%)	<7/8 n (%)
Patient Race/ethnicity		
Non-Hispanic White (NHW)	98 (53.6)	30 (35.3)
Hispanic	45 (24.6)	21 (24.7)
Black/African-American	15 (8.2)	20 (23.5)
Asian or Pacific Islander	15 (8.2)	7 (8.2)
Native American	2 (1.1)	1 (1.2)
Mult/unknown	8 (4.3)	6 (7.1)
SVI	Median (range)	Median (range)
NHW	0.46 (0.01-0.95)	0.38 (0.01-0.86)
Diverse	0.78 (0.06-0.97)	0.80 (0.02-0.99)

Patient Demographics

Characteristic	7/8 n (%)	<7/8 n (%)
Conditioning Intensity		
MAC	52 (28.4)	23 (27.1)
RIC	114 (62.3)	47 (55.3)
NMA	17 (9.3)	15 (17.6)
Conditioning regimen detail		
Bu/Flu MAC	19 (10.4)	18 (21.2)
Flu/TBI MAC	33 (18.0)	5 (5.9)
Bu/Flu RIC	29 (15.8)	9 (10.6)
Flu/Mel RIC	83 (45.4)	37 (43.5)
Flu/Cy/TBI NMA	17 (9.3)	15 (17.6)
Other	2 (1.1)	1 (1.2)

Characteristic	7/8 n (%)	<7/8 n (%)
Primary diagnosis		
ALL	40 (21.9)	9 (10.6)
AML	82 (44.8)	47 (55.3)
MDS	39 (21.3)	13 (15.3)
Lymphoma	11 (6.0)	12 (14.1)
Other Leukemia	3 (1.6)	3 (3.5)
CLL	1 (0.5)	1 (1.2)
CML	7 (3.8)	0 (0.0)
KPS		
80-100	169 (92.3)	75 (88.2)
<80	14 (7.7)	10 (11.8)
HCT-CI		
0-2	124 (67.8)	53 (62.4)
≥3	59 (32.2)	32 (37.6)

#936: Jimenez: ACCESS Trial Mismatched MUD Post Cy

Donor Characteristics

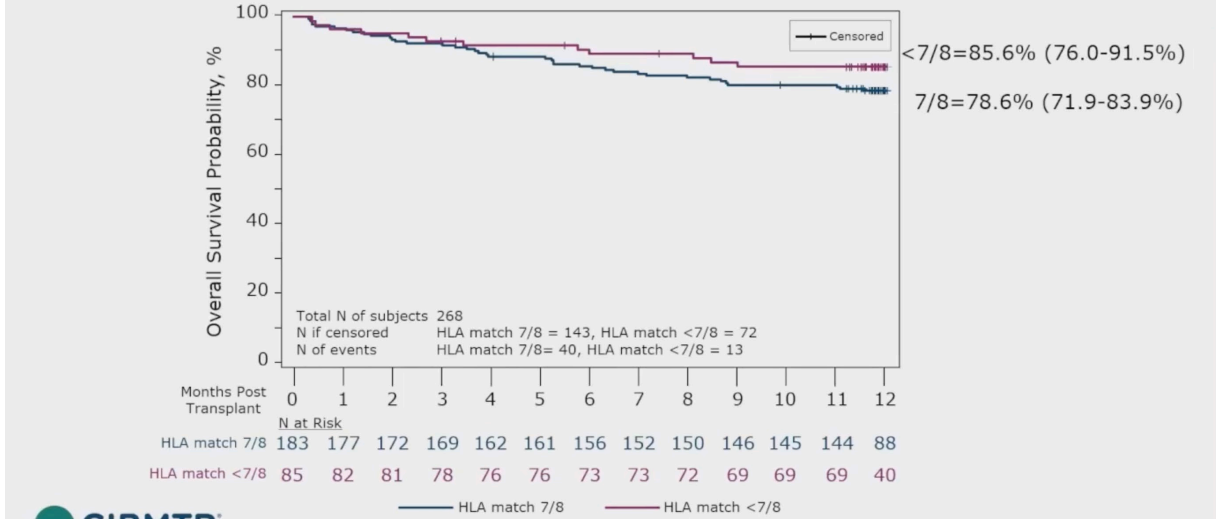
Characteristic	7/8 n (%)	<7/8 n (%)
Donor Age		
Median (min-max)	25.8 (18.7-35.7)	25.0 (18.3-34.8)
18-24	79 (43.2)	41 (48.2)
25-29	67 (36.6)	32 (37.6)
30-35	37 (20.2)	12 (14.1)
Donor Sex		
Male	86 (47.0)	37 (43.5)
Female	97 (53.0)	48 (56.5)
Cell Dose median(range)		
Fresh	5.04 (3.13-12.8)	5.50 (2.47-18.7)
Cryopreserved	5.99 (2.30-22.4)	5.52 (2.47-18.7)

Characteristic	7/8 n (%)	<7/8 n (%)
HLA match level		
7/8	183 (100)	
6/8	0 (0.0)	70 (82.4)
5/8	0 (0.0)	12 (14.1)
4/8	0 (0.0)	3 (3.5)

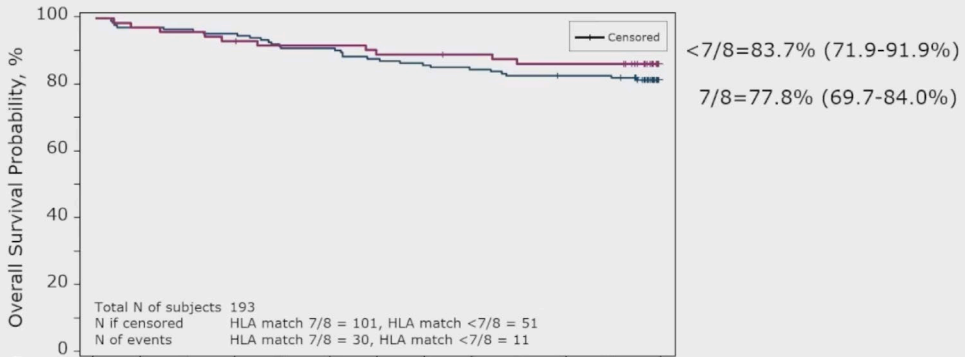
	MAC n (%)	RIC n (%)
HLA match level by conditioning intensity		
7/8	52 (69.3)	131 (67.9)
6/8	19 (25.3)	51 (26.4)
5/8	3 (4.0)	9 (4.7)
4/8	1 (1.3)	2 (1.0)

#936: Jimenez: ACCESS Trial Mismatched MUD Post Cy

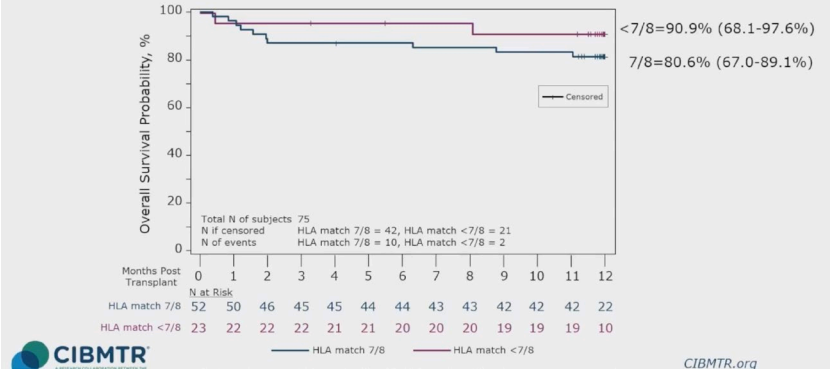
Results – Primary Endpoint



Results – Primary Endpoint, RIC



Results – Primary Endpoint, MAC



#936: Jimenez: ACCESS Trial Mismatched MUD Post Cy

Results – Secondary Endpoints

Clinical Endpoint#	7/8 Match (n=183)	<7/8 Match (n=85)
	1yr est. (95% CI)	**1yr est. (95% CI)**
GVHD-free, relapse free survival (GRFS)	**51%** (43.5-58.2%)	**53%** (41.2-63.6%)
Acute GVHD grade II-IV	**39%** (31.7-45.8%)	**34%** (24.3-44.4%)
Acute GVHD grade III-IV	**8%** (4.8-12.8%)	**7%** (2.9-14.0%)
NIH moderate/severe chronic GVHD	**11%** (7.1-16.4%)	**8%** (3.1-15.1%)
Primary graft failure by Day 28	**3%** (0.8-7.6%)	**7%** (1.8-15.7%)
Non-relapse mortality (NRM)	**14**% (9.2-19.1%)	**8**% (3.7-15.6%)
Relapse	**17%** (12.0-22.9%)	**23%** (14.1-32.8%)

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Conclusions

- Among adult recipients of <7/8 MMUD PBSC grafts with PTCy-based GVHD prophylaxis enrolled on the ACCESS study, 1-year OS exceeded 80% and was comparable to 7/8 recipients.
- Relapse, NRM, and GVHD rates were similarly favorable and consistent with outcomes reported in 7/8 recipients.
- Ongoing (OPTIMIZE; NCT06001385 and ACCELERATE; NCT06859424) and future studies will target improvements in acute and chronic GVHD, infection and relapse rates.
- These findings support extending suitable MMUD match considerations to include all match levels in the context of PTCy, potentially enabling near-universal donor access regardless of ancestry, while allowing for optimization of other non-HLA donor factors.

#570: Wang: CD19/CD22 bispecific CAR-T cells for R/R LBCL

American Society of Hematology
Helping hematologists conquer blood diseases worldwide

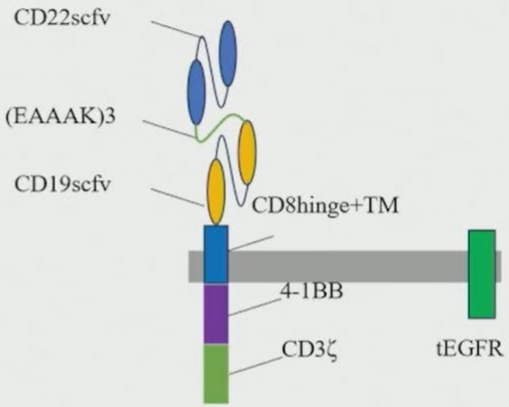
CD19/CD22 bispecific CAR-T cell
relapsed/refractory large b-cell lymphon
single-arm, single-center, phase 2

Xin-di Liu[1#], He-nan Wang[1#], Yuan-zheng Liang[1#], Jin Ye[1], Na Yao[1], Jing Yang[1],
Beijing Tongren Hospital, CMU

CD19/CD22 Bispecific CAR-T Cell Therapy for Relapsed/Refractory Large B-cell Lymphoma: a Prospective, Single-arm, Single-center, Phase 2 Clinical Trial

ClinicalTrials.gov Identifier:
NCT06081478

Beijing Tongren Hospital Protocol Record CAR-T001/TR, CD19/CD22 Bispecific CAR-T Cell Therapy for Relapsed/Refractory B-cell Lymphoma or Acute Lymphoblastic Leukemia, is registered and will be posted on the ClinicalTrials.gov public website.



CD22 scFv	(EAAAK)3	CD19 scFv	CD8 hinge	CD8 TM	4-1BB	CD3ζ	T2A	tEGFR

CAR2219 consisted of a single-chain variable fragment (scFv) derived from the anti-CD22 monoclonal antibody fused to a second scFv derived from the anti-CD19 monoclonal antibody, and the cytoplasmic signaling domains of human 4-1BB and CD3ζ, and parallel Truncated Epidermal Growth Factor Receptor (tEGFR) expression was used to detect the expression efficiency of CAR.

#570: Wang: CD19/CD22 bispecific CAR-T cells for R/R LBCL

Patients' characteristics

Lines of therapy, n (%)	
<=2	14 (45)
>2	17 (55)
The efficacy of bridging therapy, n (%)*	
CR	1 (3.4)
PR	9 (31)
SD	18 (62.1)
PD	1 (3.4)
No bridging therapy	2
Prognostic marker per central laboratory, n (%)	
DEL/DHL	12 (39)
P53 (+)	13 (42)
more than two extranodal sites invasion, n (%)	17 (55)
LDH > ULN, n (%)	19 (61)
Lymphoma present in bone marrow, n (%)	4 (13)

With a median follow-up of 8.1 months after CAR-T cells infusion, the best ORR was 100%, comprising a CRR of 67.7% (21/31) and a partial response (PR) rate of 32.3% (10/31)

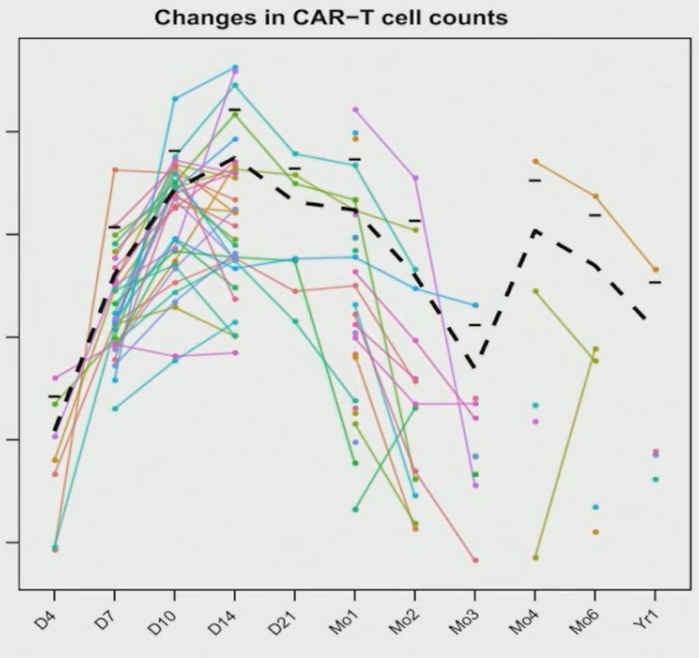
Figure 2

Efficacy at 4-weeks after infusion:
ORR=93.5%
CRR=61.3%

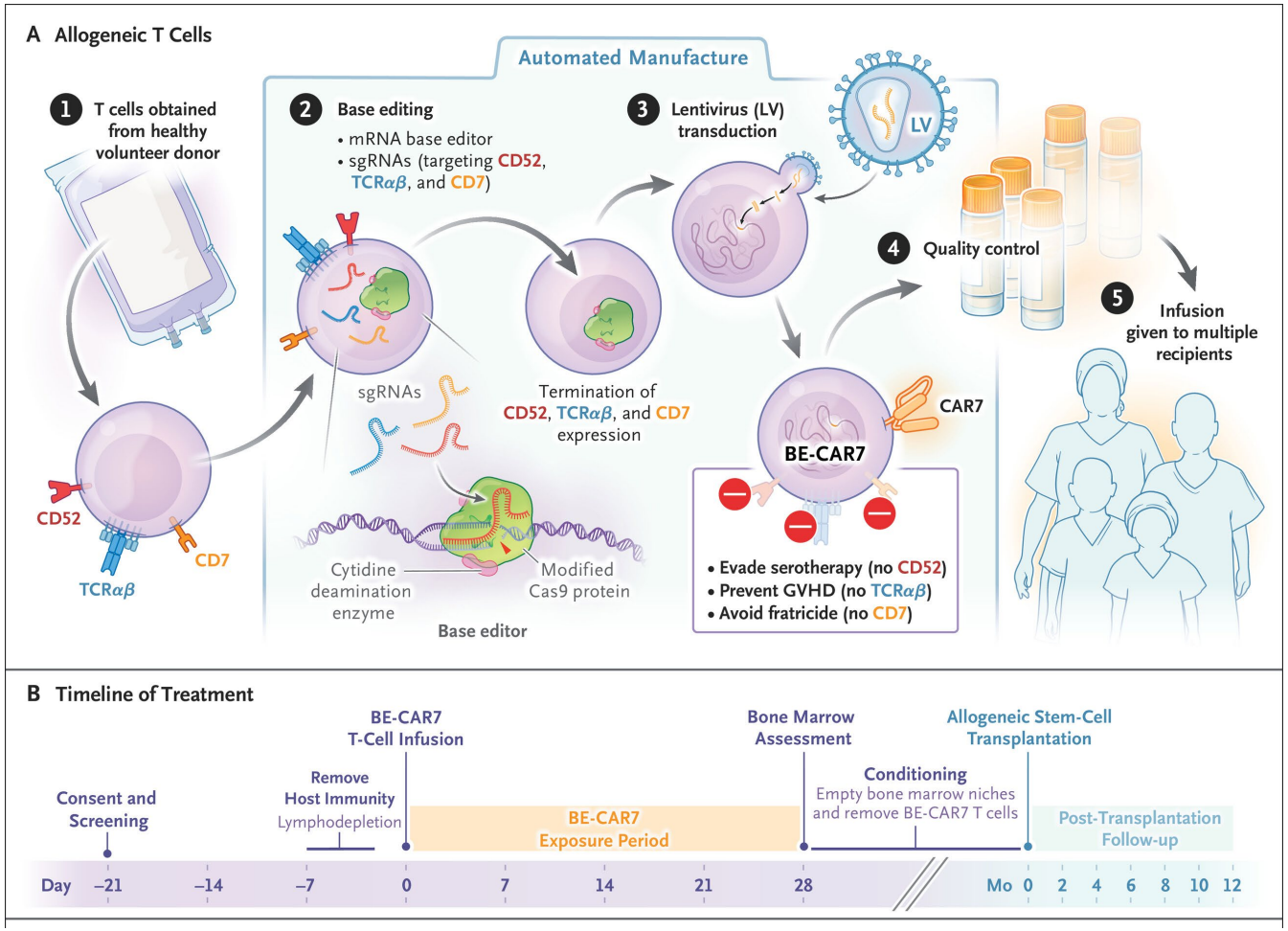
#570: Wang: CD19/CD22 bispecific CAR-T cells for R/R LBCL

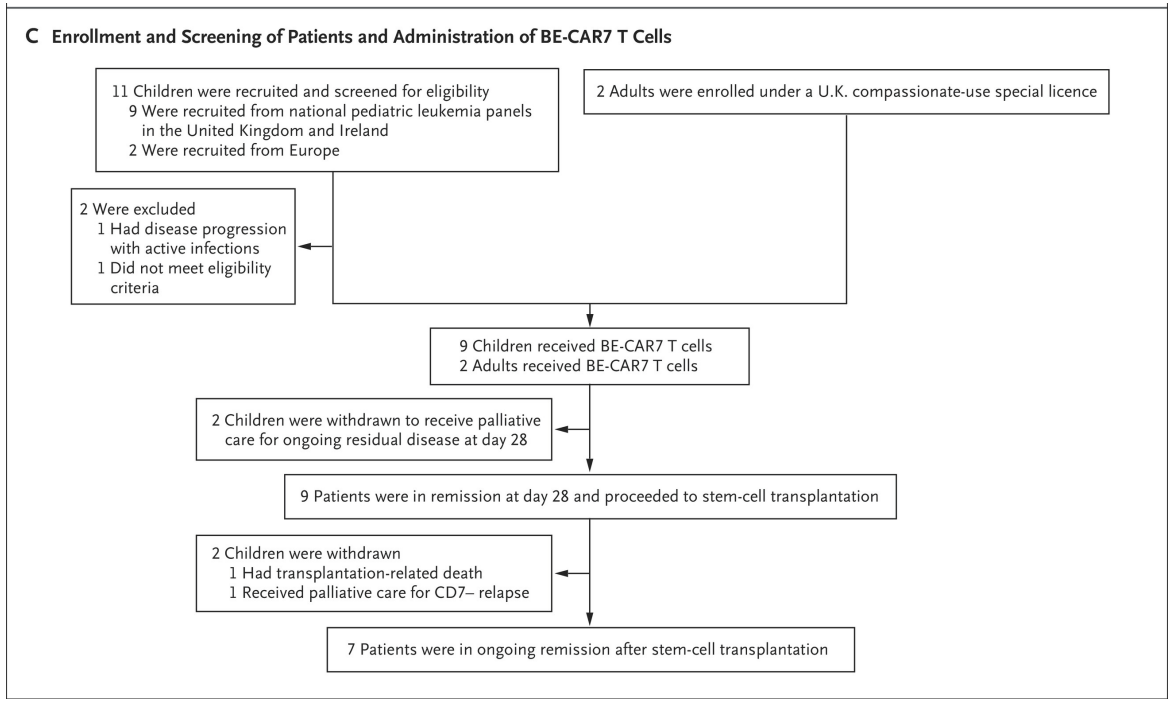
CAR2219 was well tolerated for most patients, and no AE-related deaths were reported. 74% patients suffered from grade 1-2 CRS, and only one patient had grade 3 ICANS.

Adverse events	All patients(N=31)	
	any grade	grade ⩾3
Neutropenia	29(93%)	28(90%)
Thrombocytopenia	24(77%)	14(45%)
Anemia	22(71%)	10(32%)
Cytokine release syndrome	23(74%)	0
Immune effector cell-associated neurotoxicity syndrome	2(6%)	1(3%)

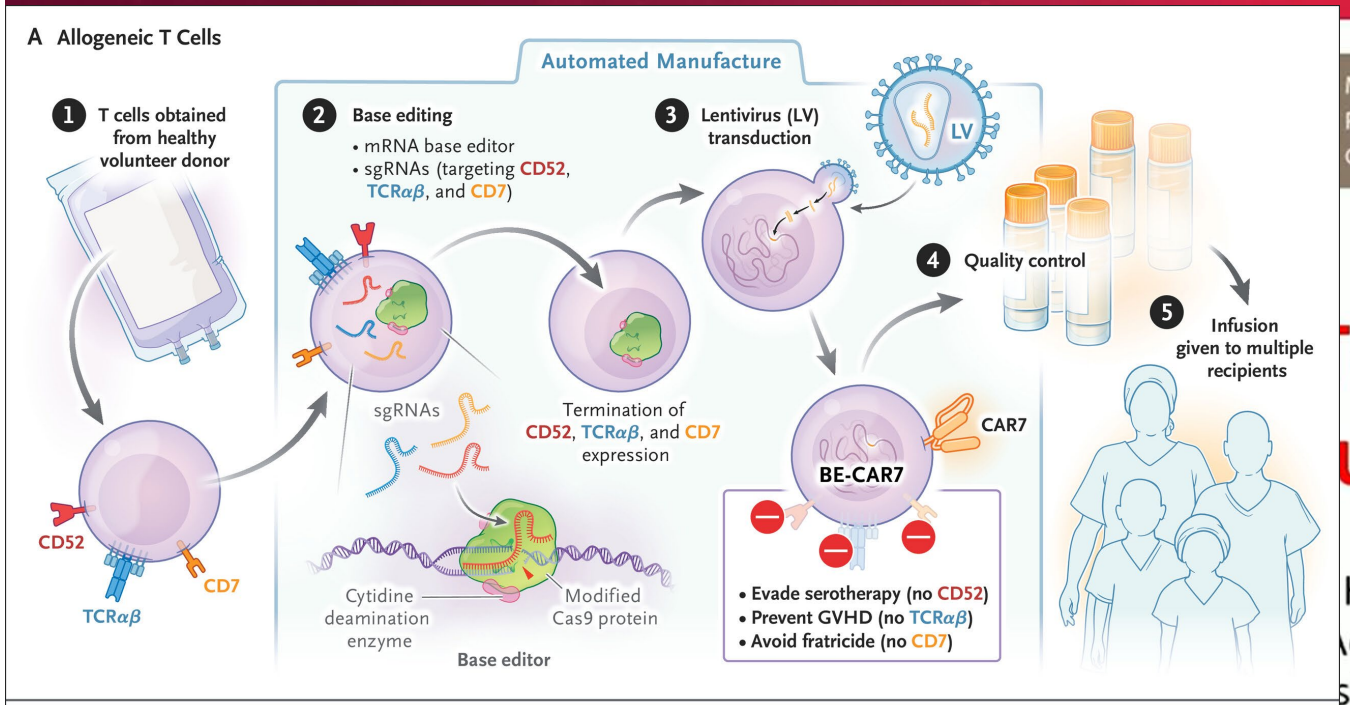


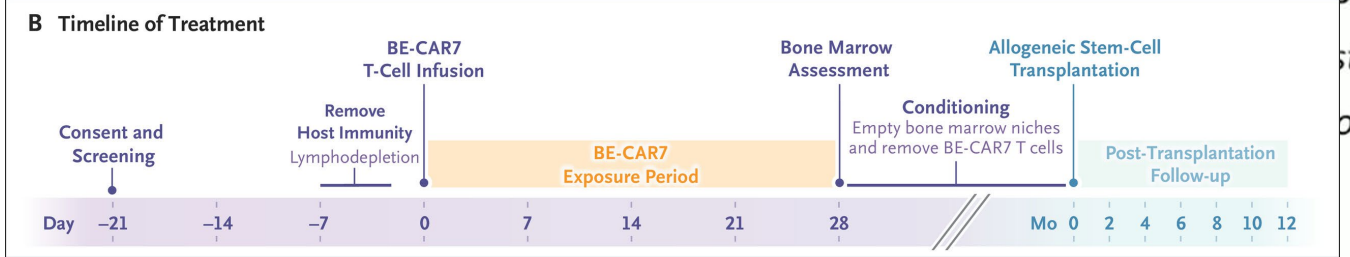
#1041: Chiesa: Universal CAR-T targeting CD7 for T-ALL

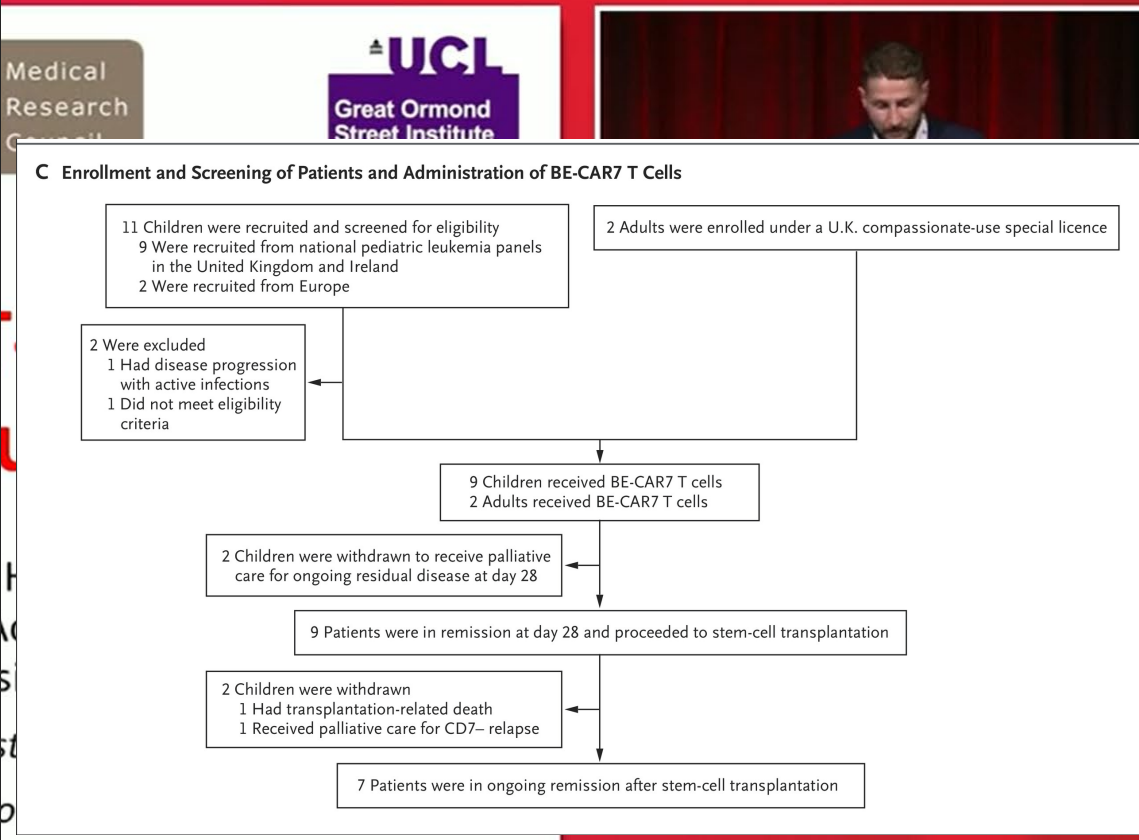




#1041: Chiesa: Universal CAR-T targeting CD7 for T-ALL







Medical Research

UCL Great Ormond Street Institute

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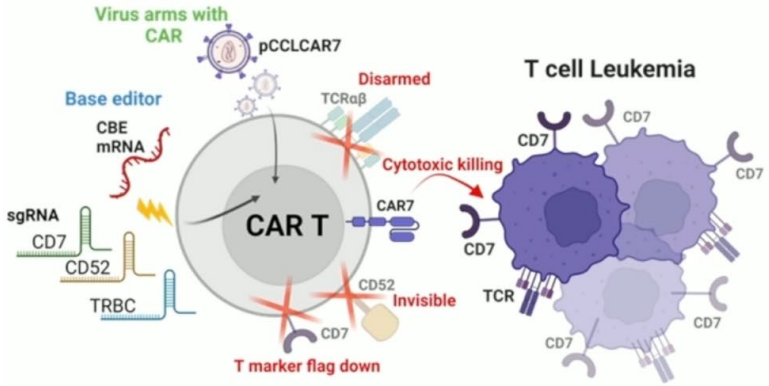
NEJM Published December 8, 2025; DOI: 10.1056/NEJMoa2505478

#1041: Chiesa: Universal CAR-T targeting CD7 for T-ALL

NHS Great Ormond Street Hospital for Children NHS Foundation Trust

Allogeneic CD7 CART cells



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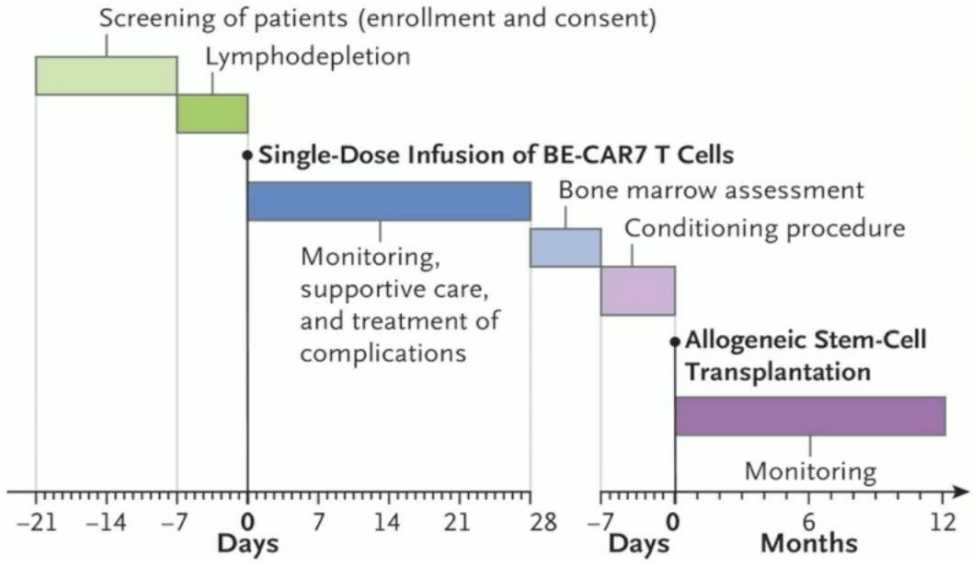
TvT CAR7 Study



- Phase 1 study;
- Feasibility, safety and efficacy of BE-CD7 CART-cells in children & adults with r/r T-ALL;
- Eligibility: r/r CD7 +ve T-ALL, ahead of allo-SCT;
- >99% expression CD7 on blasts;
- N=11 children recruited, n= 9 dosed;
- N= 2 adults (compassionate);

#1041: Chiesa: Universal CAR-T targeting CD7 for T-ALL

NHS Great Ormond Street Hospital for Children NHS Foundation Trust

BE CAR7 Study

Patients and SCT characteristics

Pt	P001	P002	P003	P004	P005	P007	P008	P009	P010	Sp1	Sp2
Diagnosis	T-ALL	MPAL	T-ALL	T-ALL	ETP	ETP	N-ETP	T-ALL	T-ALL	T-ALL	T-ALL
Previous SCT	Y	Y	N	N	N	N	N	N	N	N	Y
Pre-LD (LAIP) %	9.0	0.5	86.9	53.0	3.0	0.2	15.6	0.5	0.02	63.1	8.4
SCT Donor	10/10 MUD	10/10 MUD	N/A	N/A	9/10 MMUD	9/10 UCB	8/10 UCB	10/10 UCB	Haplo	10/10 MUD	10/10 MUD
Conditioning	F160* C120	F120* C120	N/A	N/A	E60	F150* T42000*	E60	E60	E60 Rtx200*	C120	F150* M140*
ATG	Y	Y	N/A	N/A	Y	N	Y	N	Y	N	Y
Radiotherapy	2 Gy	2 Gy	N/A	N/A	12 Gy	4 Gy	12 Gy	8 Gy	12 Gy	13 Gy	N/A

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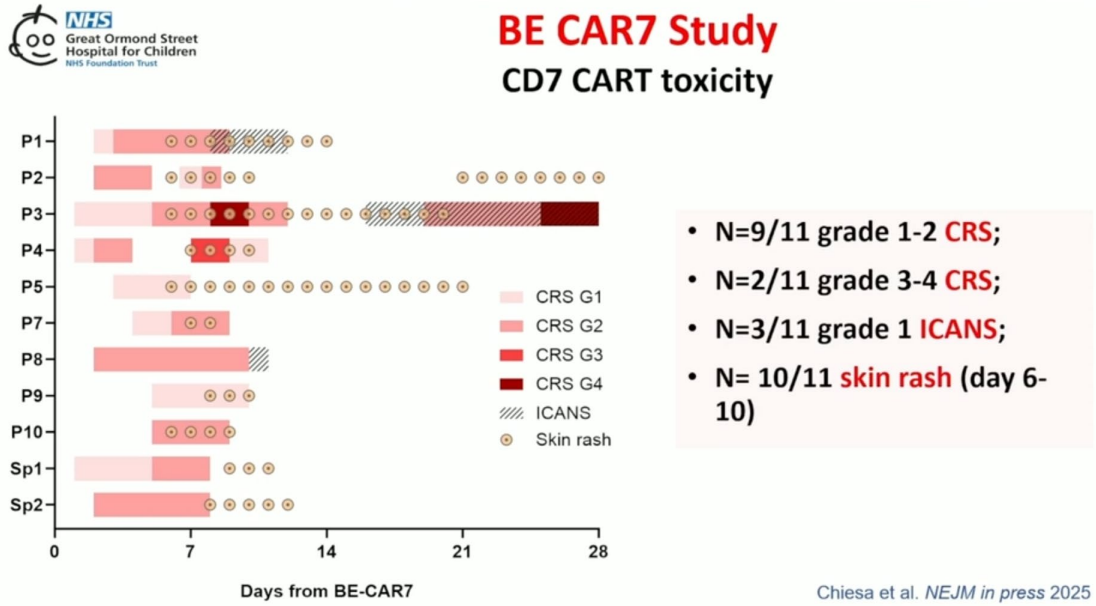
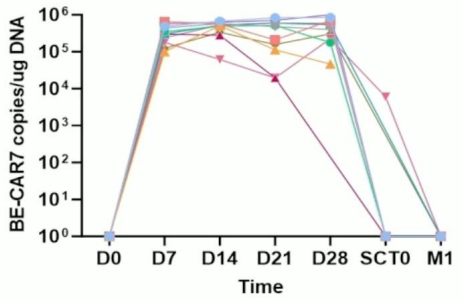


#1041: Chiesa: Universal CAR-T targeting CD7 for T-ALL

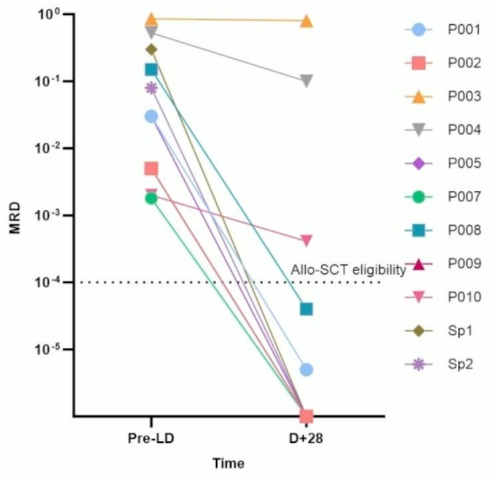
BE CAR7 Study

CART expansion and disease response

BE-CAR7 tracking by vector copies



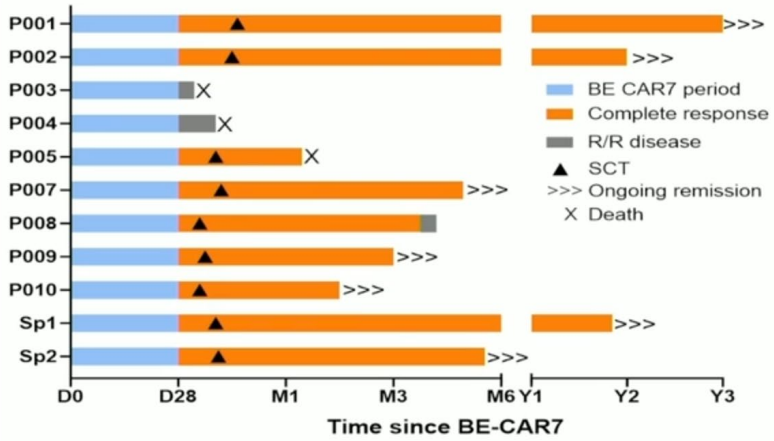
MRD response after CD7 CART



Chiesa et al. *NEJM in press* 2025

BE CAR7 Study

outcomes



- 82% deep remission → SCT;
- N= 2 refractory (1 CD7 –ve);
- N=1 CD7 –ve relapse post BMT;
- N=1 TRM;
- 64% alive & CR post SCT.

Chiesa et al. *NEJM in press* 2025

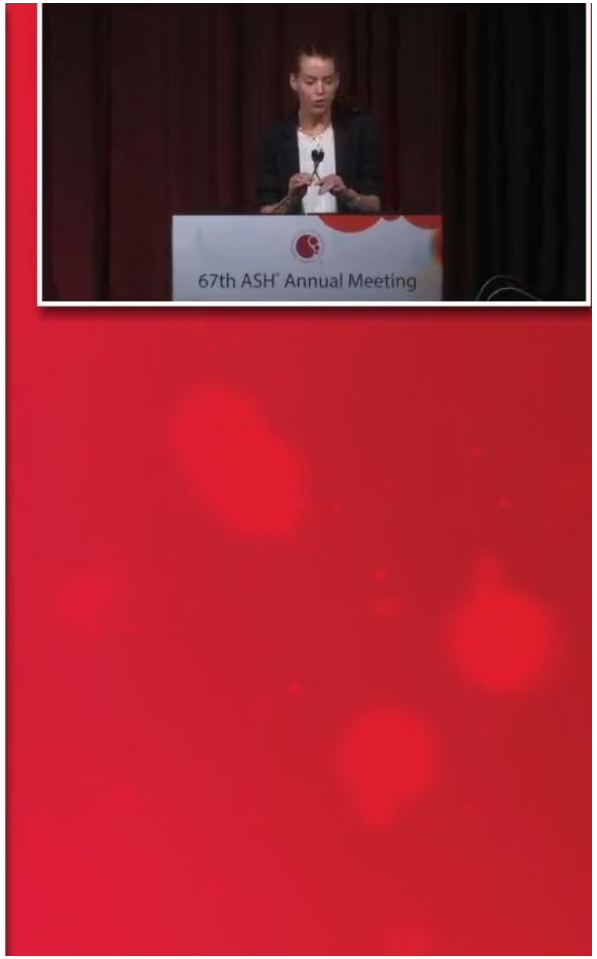
- First clinical application of base-editing;
- Good CART clinical safety profile and anticipated toxicity;
- Anti-leukemic response in 82% patients;
- High risk of infections due to augmented LD/CD7 CART effect;
- Subsequent allo-HCT ensured normal donor-derived T-cell / immune reconstitution;
- Phase 1 study extended for 2 years.

#815: Roddie: CD19 CAR-T for SLE

Obecabtagene autoleucel (obe-cel), a CD19-targeting chimeric antigen receptor (CAR) T-cell therapy, in patients with severe, refractory systemic lupus erythematosus (SLE) in the Phase I CARLYSLE study: initial safety, preliminary efficacy, pharmacokinetics, and biomarker results

Claire Roddie[1,2], Maria Leandro[1,2], Ben Parker[3], Eleni Tholouli[4], David Jayne[5], Ben Uttenthal[5], Josefina Cortés-Hernández[6], Pere Barba[6], José Andrés Román Ivorra[7], Yanqing Hu[8], Wolfram Brugger[9], Silvia Basilico[10], Davide Germano[10], Ruth Pepper[1,2,11]

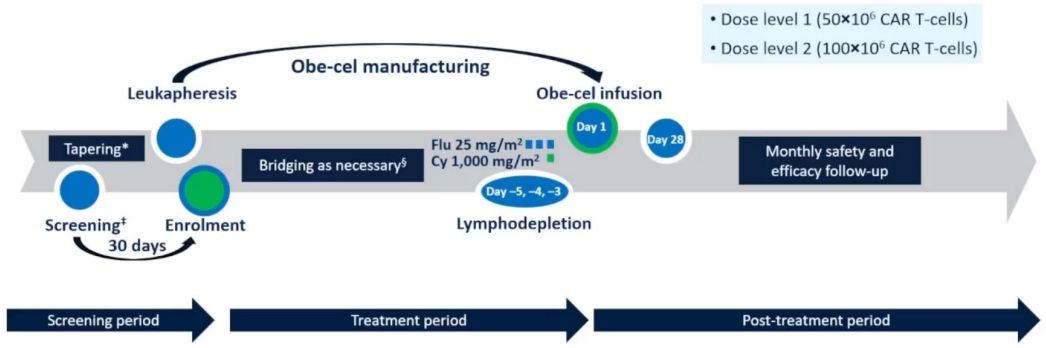
[1]University College London, London, UK; [2]University College London Hospital, London, UK; [3]NIHR Manchester CRF, Manchester Royal Infirmary, Manchester, UK; [4]Manchester Royal Infirmary, Manchester, UK; [5]Cambridge University Hospitals NHS Foundation Trust, Cambridge, UK; [6]Hospital Universitari Vall d'Hebron-Universitat Autónoma de Barcelona, Barcelona, Spain; [7]Hospital Universitari I Politècnic La Fe, Valencia, Spain; [8]Autolus Therapeutics, Rockville, MD, USA; [9]Autolus Therapeutics, Munich, Germany; [10]Autolus Therapeutics, Basel, Switzerland; [11]Royal Free Hospital NHS Trust, London, UK.



#815: Roddie: CD19 CAR-T for SLE

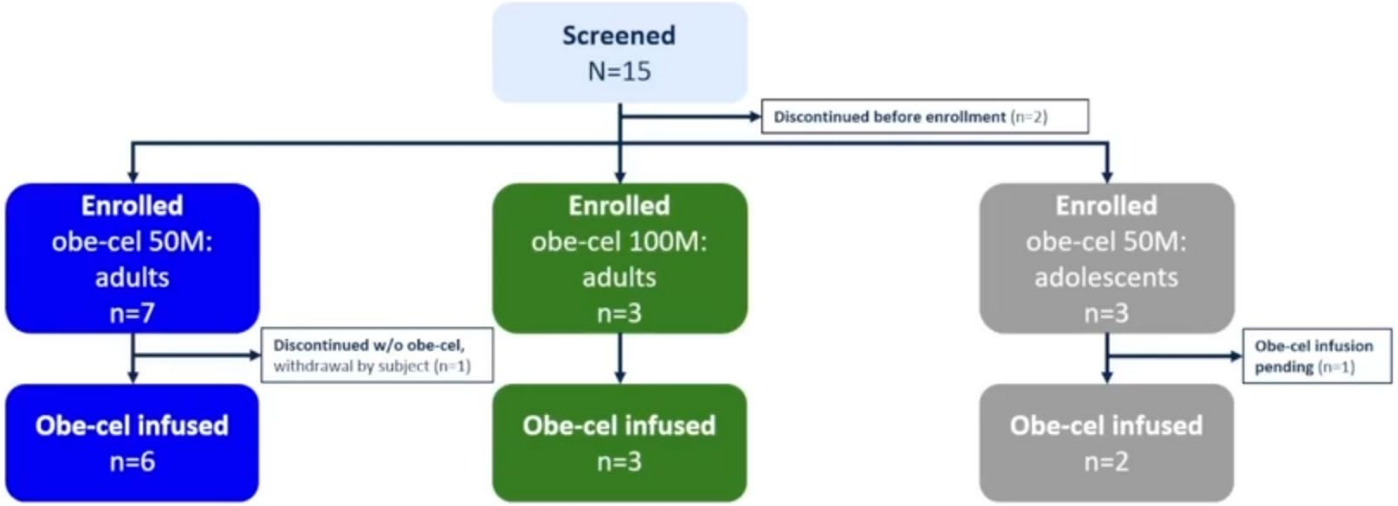
CARLYSLE study design and dose administration

Obe-cel was infused at a single target dose of 50×10^6 or 100×10^6 CAR T-cells



Patient disposition

Nine adult patients were infused with obe-cel at the 50M (n=6) or 100M (n=3) dose



- Adult patients infused with 50×10^6 CAR T-cells had a median follow-up of 11.4 months (range: 8.5–16.3)*
- Adult patients infused with 100×10^6 CAR T-cells had a median follow-up of 3.3 months (range: 3.1–7.2)*

#815: Roddie: CD19 CAR-T for SLE

Safety outcomes

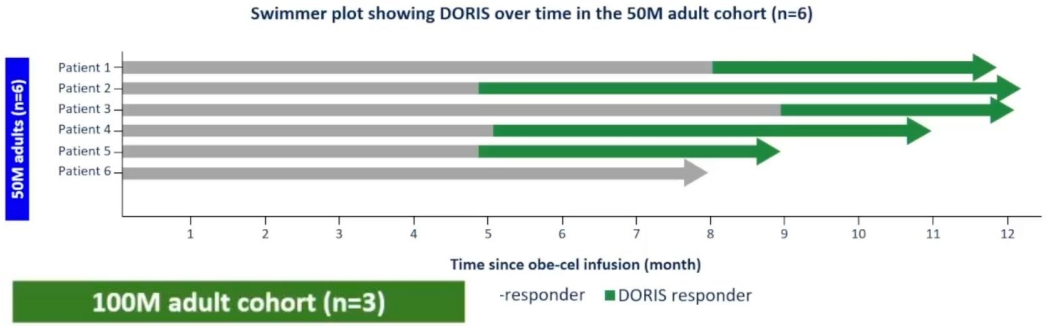
Obe-cel was well tolerated in all patients, with no ICANS or Grade ≥2 CRS

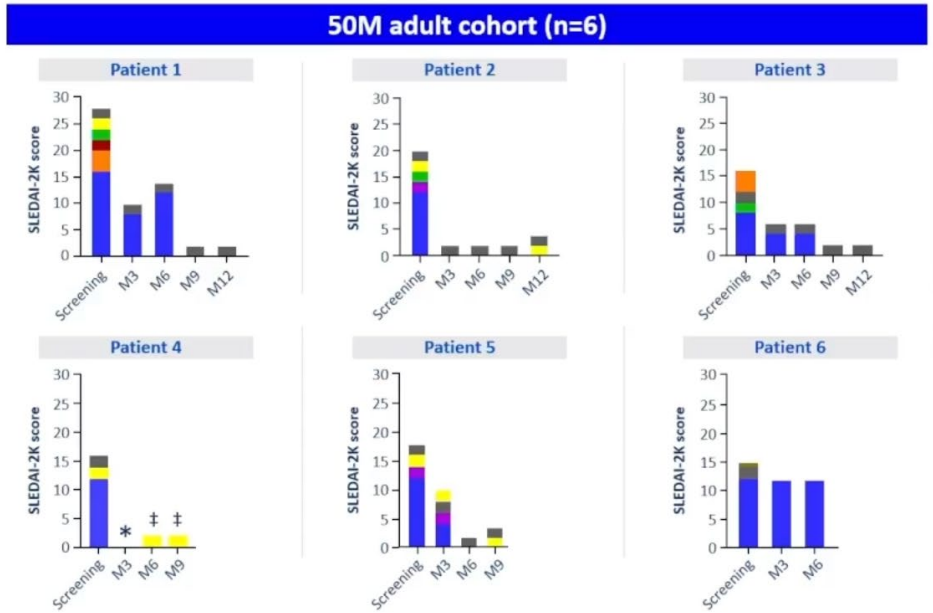
	Infused adult patients, 50M (n=6)		Infused adult patients, 100M (n=3)	
	Any grade, n (%)	Grade ≥3, n (%)	Any grade, n (%)	Grade ≥3, n(%)
CRS	3 (50.0)	0	3 (100)	0
ICANS	0	0	0	0
Any treatment-emergent adverse event				
Neutropenia				
Infection				
Hypertension*				
Anemia				
Febrile neutropenia				
Thrombocytopenia				
Liver injury				

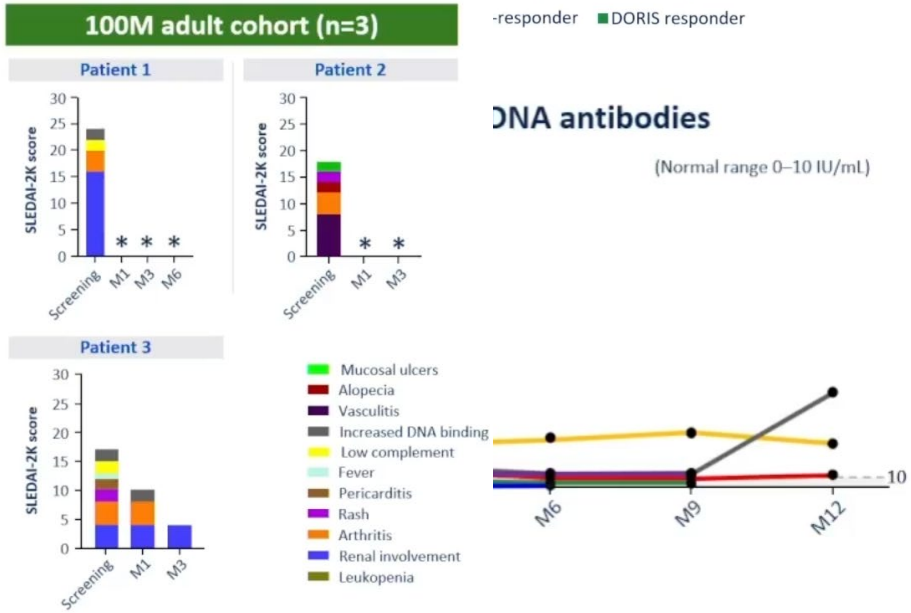
- One case of liver injury was observed, v
  medication-related toxicity (anti-infecti

Efficacy outcomes: DORIS and renal response

Five (83.3%) patients in the 50M adult cohort achieved DORIS with a median onset of 5.1 months (range: 4.9–8.9)





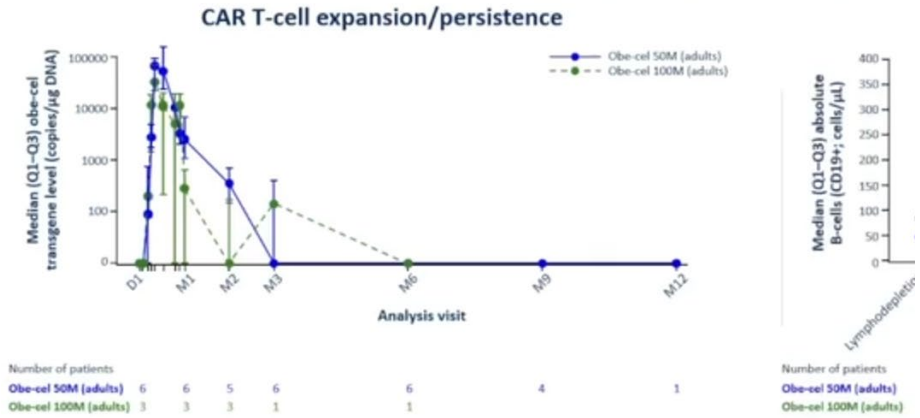


#815: Roddie: CD19 CAR-T for SLE

CAR T-cell kinetics and B-cell depletion

Robust and rapid CAR T-cell expansion and B-cell depletion were observed

Median (Q1–Q3) in CAR T-cell expansion/persistence and B-cell depletion over time



B-cell phenotypes following repopulation (50M adult cohort)

Early findings suggest that obe-cel induces a reset of the B-cell population

Patient	Day –5	Month 3	Month 6	Month 9	Month 12
#1	No sample available	No B-cells			
#2					
#3		No B-cells			
#4					Visit not reached
#5					
#6		No B-cells	No B-cells	Visit not reached	

Transitional
Naïve
Pre-switched memory
Switched memory
Double negative memory
Plasmablasts

- Overall, >90% of reconstituted B-cells at the time of recovery were transitional or naïve

Klinische Studien Zelltherapie Tübingen

Entity	Description	Status
Allogeneic SCT/GVHD	**A Randomised, Open-label, Multicentre, Phase 3 Trial of First-line Treatment with Mesenchymal Stromal Cells MC0518 Versus Best Available Therapy in Adult and Adolescent Subjects with Steroid-refractory Acute Graft-versus-host Disease After Allogeneic Haematopoietic Stem Cell Transplantation (IDUNN Trial)**	**open** Infos: Prof. Dr. Bethge
Lymphomas/ALL	**A phase I/II dose finding and efficacy study of MB-CART-CD19/CD22 in patients with relapsed/refractory B-cell malignancies**	**open** Infos: Prof. Dr. Bethge
Autoimmune Diseases	**An open-label phase I/IIa, multicentre, interventional single-arm trial of MBCART19.1 in patients with refractory SLE**	**open** Info: Prof. Dr. Henes
	Use of MB-CART19 also for autoimmune diseases: Basjet (SLE, MS, myositis)	**planned** Info: Prof. Dr. Henes
	A phase I/II safety, tolerability and preliminary efficacy study of UKT-CART-19.1 in patients with primary progressive multiple sclerosis	**planned** Info: Dr. Malte Rörden
Lymphomas	**Phase-II-Studie zur Bewertung der Wirksamkeit und Sicherheit von MB-CART2019.1 im Vergleich zur Standardtherapie bei Teilnehmern mit rezidiviertem/refraktärem diffus-großzelligem B-Zell-Lymphom (R-R DLBCL) | DALY 2-EU**	**open** Infos: Prof. Dr. Bethge
Lymphomas/ Car-T cells	**Phase II study to evaluate the efficacy and safety of MB-CART2019.1 compared with standard therapy in participants with relapsed/refractory diffuse large B-cell lymphoma (RR DLBCL) |** DALY 2-EU	**reopened** Info: Prof. Dr. Bethge

und am Ende....

Vielen Dank für Ihre Aufmerksamkeit

Universitätsklinikum Tübingen