

# THE LANCET

## Haematology

### Supplementary appendix

This appendix formed part of the original submission and has been peer reviewed. We post it as supplied by the authors.

Supplement to: DeZern AE, Eapen M, Wu J, et al. Haploidentical bone marrow transplantation in patients with relapsed or refractory severe aplastic anaemia in the USA (BMT CTN 1502): a multicentre, single-arm, phase 2 trial. *Lancet Haematol* 2022; published online July 27. [https://doi.org/10.1016/S2352-3026\(22\)00206-X](https://doi.org/10.1016/S2352-3026(22)00206-X).

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Supplementary Table 1:

**Chronic GVHD listing**

Eight patients reported chronic GVHD reported, maximum severity and GVHD prophylaxis, treatment for these patients are listed in below table.

<b>Maximum Chronic GVHD Severity</b>	<b>GVHD prophylaxis taken at 1 year</b>	<b>GVHD treatment taken at 1 year</b>
mild	Sirolimus	Systemic corticosteroids
mild	none	Topical corticosteroids
mild	Tacrolimus	Systemic corticosteroids
mild	none	Topical corticosteroids
mild	none	none
mild	Tacrolimus	Topical corticosteroids, Systemic corticosteroids
moderate	Tacrolimus	none
mild	Tacrolimus, beclomethasone	none

Supplementary Table 2: Infections by Engraftment Status

	Total		Patients With GF		Patients Without GF	
	N	%	N	%	N	%
<b># Patients Transplanted</b>	31		5		26	
<b># Patients with Infections</b>	19	61.3%	5	100.0%	14	53.8%
<b># Patients with Infection Reports</b>						
=1	7		2		5	
=2	2		1		1	
=3	2		0		2	
=4	3		0		3	
=5	3		0		3	
>=6	2		2		0	
<b># Total Infection Events</b>	64		24		40	
Grade 2	52	81.2%	20	83.3%	32	80.0%
Grade 3	12	18.8%	4	16.7%	8	20.0%
<b>Maximum Severity by Patient</b>						
None	12	38.7%	0	0.0%	12	46.2%
Grade 2	12	38.7%	1	20.0%	11	42.3%
Grade 3	7	22.6%	4	80.0%	3	11.5%
<b>Infection Type (# of Patients)</b>						
Bacterial	26	(13)	9	(3)	17	(10)
Viral	32	(12)	12	(2)	20	(10)
Fungal	3	(3)	3	(3)	0	(0)
Protozoal	0	(0)	0	(0)	0	(0)
Other	3	(2)	0	(0)	3	(2)
<b>Non-Microbial Infections (# of Patients)</b>	7	(4)	3	(2)	4	(2)
<b>Incidence of CMV (# of Patients)</b>	22.6% ( 7 )		40.0% ( 2 )		19.2% ( 5 )	
<b>Incidence of EBV (# of Patients)</b>	9.7% ( 3 )		20.0% ( 1 )		7.7% ( 2 )	
<b>Incidence of PTLD (# of Patients)</b>	6.5% ( 2 )		0.0% ( 0 )		7.7% ( 2 )	
<b>Cumulative Incidence of Grades 2-3infections at 1 year</b>	58.1% (95% CI: 38.4%, 73.5%)		100% (95% CI: 47.8%, 100%)		50% (95% CI: 29.3%, 67.6%)	

Supplementary Table 3: Health Related Quality of Life (HR-QoL) assessments

HR-QoL Measurement	Change at Day 100 from baseline			Change at 6 Months from baseline			Change at 1 Year from baseline		
	N	Mean (SD)	P-Value	N	Mean (SD)	P-Value	N	Mean (SD)	P-Value
SFH MCS	14	4.8 (14.0)	0.220	14	6.0 (10.2)	0.047	9	2.2 (10.7)	0.562
SFH PCS	14	4.0 (13.8)	0.292	14	6.6 (11.6)	0.053	9	10.4 (6.6)	0.002*
PedsQL	7	11.4 (16.2)	0.111	8	9.5 (15.4)	0.123	6	18.7 (11.1)	0.009*

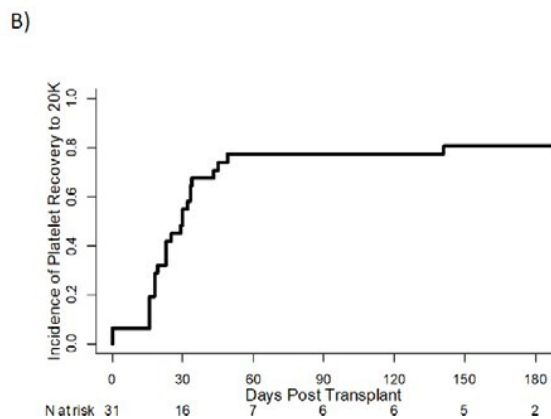
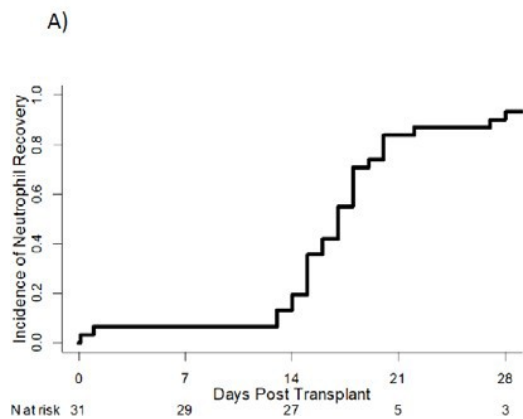
\*P values <0.01 are statistically significant. P-value from paired t-test.

HR-QoL data were analyzed for changes in mean score from baseline measurement and performed as an exploratory analysis, given the small sample size. A paired Student t test was used to assess changes from baseline to each posttransplant time point (day 100, 6 months, and 1 year). Only P values of <.01 were considered significant for the analyses, given the multiple comparisons.

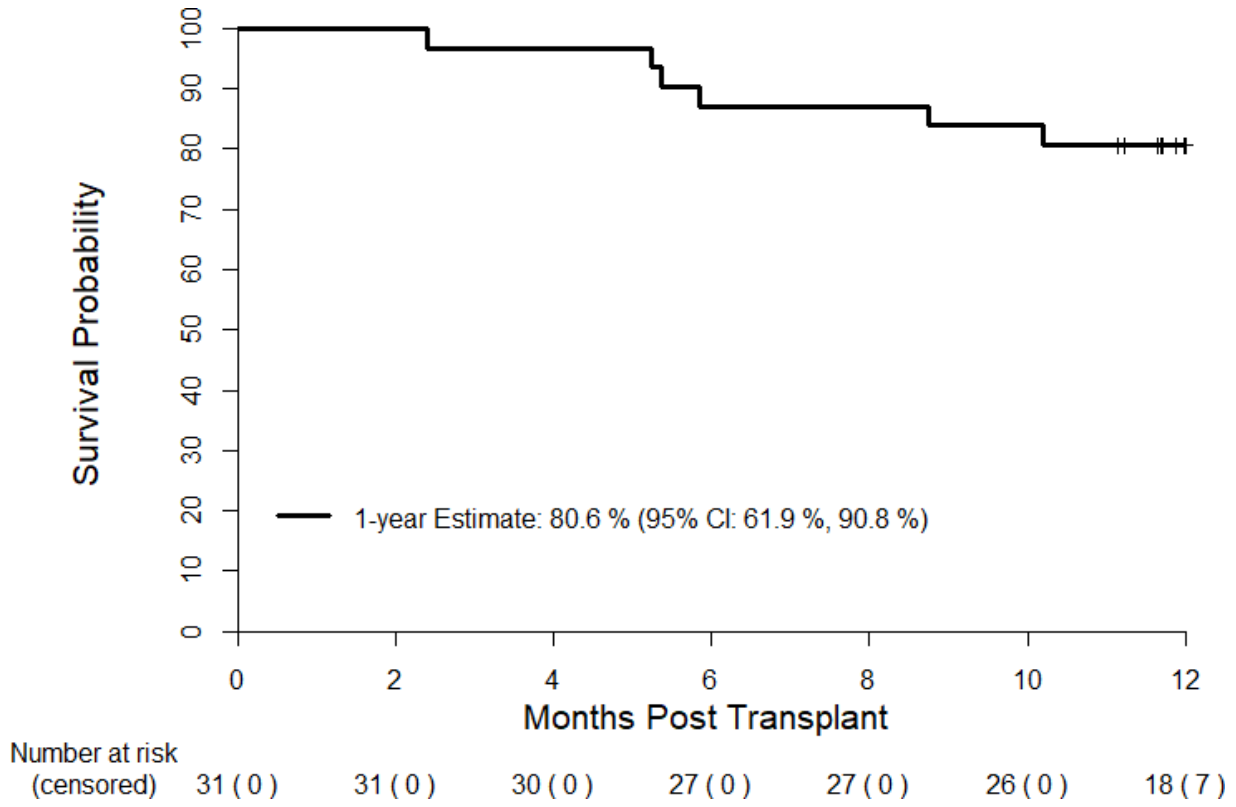
Supplementary Table 4: Accrual by Site

Site	PI Name	Total Accrual
All Children's Hospital	Oshrine	.
BMT Group of Georgia	Solh	3
Baylor College of Med/The Methodist Hospital	Sasa	.
Children's Hospital Los Angeles	Pulsipher	2
Children's Hospital at Oakland	Lalefar	.
Children's Mercy Hospital and Clinics	Goyal	.
Children's National Medical Center	Abraham, Davila-Saldana	1
City of Hope National Medical Center	Karanes, Sahebi	3
Duke University Medical Center	Horwitz, Parikh, Kurtzberg	1
Fred Hutchinson Cancer Research Center	Burroughs, Mallhi, Mielcarek	2
H. Lee Moffitt Cancer Center	Perez, Pidala	.
Hackensack University Medical Center	Gillio	.
Indiana University Med Center/Riley Hospital	Delgado, Skiles	.
Johns Hopkins University	DeZern	8
Karmanos Cancer Institute		1
MD Anderson Cancer Research Center	Ciurea, Bashir, Patel	.
Medical College of Wisconsin	Talano	4
Miami Children's Health System	Godder	3
Oregon Health Sciences University	Shereck	.
Penn state/Milton S Hershey Medical Center	Greiner	.
Roswell Park Cancer Institute	Bambach	.
Stanford Hospital and Clinics	Arai, Muffly	1
Texas Transplant Institute	Quigg	.
University of Florida College of Medicine	Wingard	1
University of Louisville		.
University of Michigan Medical Center	Vander Lugt	.
University of Pennsylvania Hospital Center	Hexner	1
Washington University of St. Louis	Westervelt	1

# Supplementary Figure 1

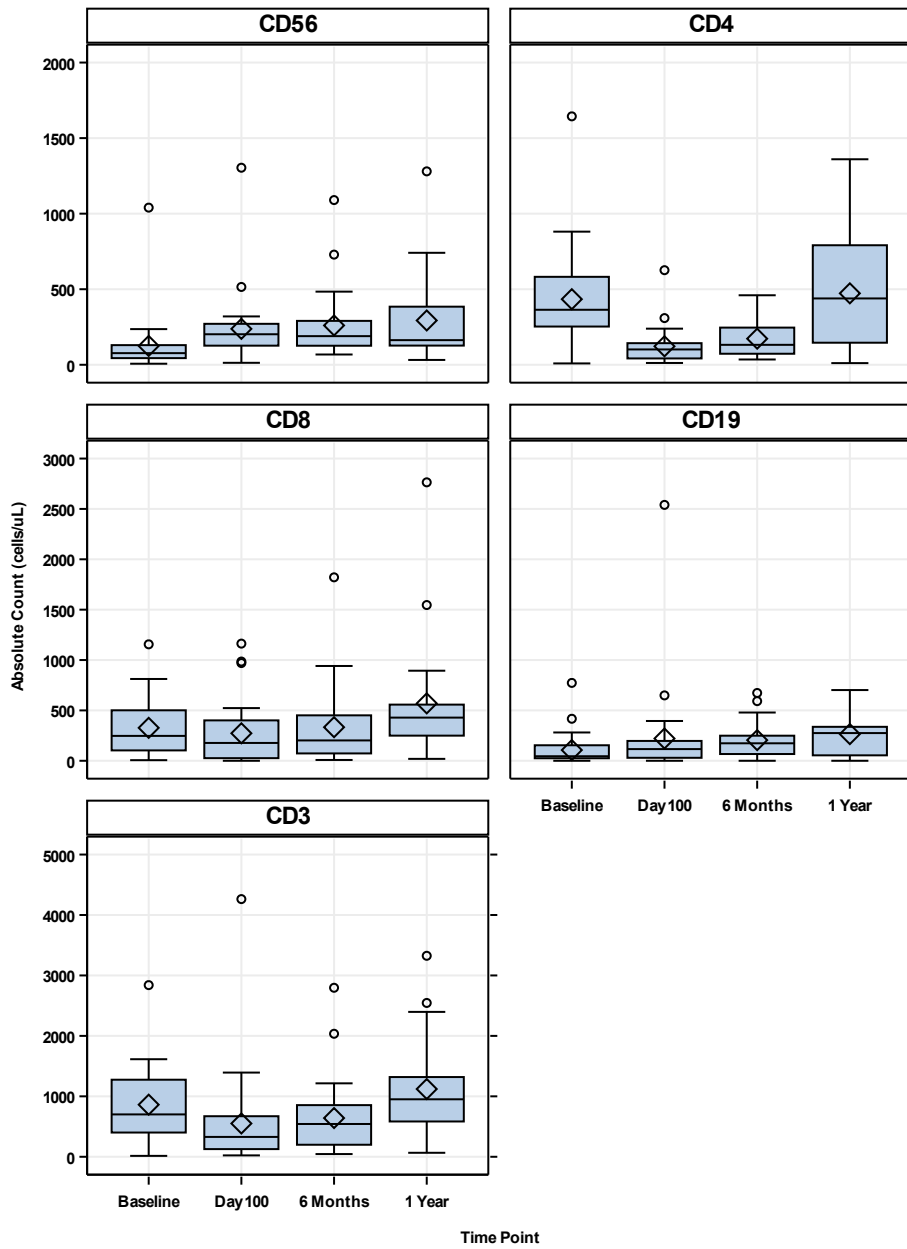


SUPPLEMENTARY FIGURE 2





SUPPLEMENTARY FIGURE 3:



**BMT CTN Protocol 1502 Site List**

<b>Center</b>	<b>Total Accr ual #</b>	<b>PI</b>
Johns Hopkins/SKCCC	8	Amy DeZern, MD, MHS
Medical College of Wisconsin/Children's Hospital of Wisconsin	4	Julie-An Talano, MD
Blood & Marrow Transplant Program at Northside Hospital	3	Melhem Solh, MD
City of Hope National Medical Center	3	Chatchada Karanes, MD
Miami Children's Hospital	3	Jorge Ricardo Galvez Silva, MD
Children's Hospital Los Angeles	2	Mike Pulsipher, MD
Fred Hutchinson Cancer Research Center	2	Lauri Burroughs, MD, Kanwaldeep Mallhi, MD, Joachim Deeg, MD
Children's National Medical Center	1	Blachy Davila-Saldana, MD
Duke University Medical Center	1	Mitchell Horwitz, MD, Joanne Kurtzberg, MD
Karmanos Cancer Institute/Children's Hospital of Michigan	1	Sureyya Savasan, MD
Stanford Hospital and Clinics	1	Sally Arai, MD, Rajni Agarwal, MD
University of Florida College of Medicine (Shands)	1	John Wingard, MD
University of Pennsylvania Cancer Center	1	Elizabeth Hexner, MD
Washington University/Barnes Jewish Hospital	1	Peter Westervelt, MD, PhD
<b>Total</b>	<b>32</b>	