Supplementary Appendix

This appendix has been provided by the authors to give readers additional information about their work.

SUPPLEMENTARY APPENDIX

This appendix has been provided by the authors to give readers additional information about their work.

Supplement to: Wagner JE, Eapen M, Carter S, et al. Cord Blood Transplant for Leukemia: Randomization of One versus Two Units. [Journal Citation]

TABLE OF CONTENTS

List of Primary Investigators and Institutions

Aleksandra Petrovic, MD, All Children's Hospital, St Petersburg, FL
Kirk R. Schultz, MD, British Columbia Children's Hospital, Vancouver, BC, Canada
Kuang-Yueh Chiang, MD, Children's Healthcare of Atlanta, Atlanta, GA
Leslie Lehman, MD, Children's Hospital Boston, Boston, MA
Roger Giller, MD, Children's Hospital of Denver, Denver, CO
Nancy Bunin, MD, Children's Hospital of Philadelphia, Philadelphia, PA
Peter Shaw, MD, Children's Hospital at Westmead, Sydney, Australia
Lolie Yu, MD, Children's Hospital of New Orleans, New Orleans, LA
Mark Walters, MD, Children's Hospital at Oakland, Oakland, CA
Victor Aquino, MD, Children's Medical Center of Dallas, Dallas, TX
Jignesh Dalal, MD, Children's Mercy Hospital and Clinics, Kansas City, MO
David Jacobsohn, MD, Children's National Medical Center, Washington, D.C.
Joseph Rosenthal, MD, City of Hope National Medical Center, Duarte, CA
Gretchen Eames, MD, Cook Children's Medical Center, Fort Worth, TX
Joanne Kurtzberg, MD, Duke University Medical Center, Durham, NC
Colleen Delaney, MD, Fred Hutchinson Cancer Research Center, Seattle, WA
Paul Haut, MD, Indiana University Med Center/Riley Hospital, Indianapolis, IN
Roland Chu, MD, Karmanos Cancer Institute, Detroit, MI
David Margolis, MD, Medical College of Wisconsin, Milwaukee, WI
Michelle P. Hudspeth, MD, Medical University of South Carolina, Charleston, SC
Thomas Gross, MD, Nationwide Children's Hospital, Columbus, OH
Joyce Sandler, MD, Nemours Children's Clinic, Jacksonville, FL
Fevzi Ozkaynak, MD, New York Medical College, Valhalla, NY
Eneida Nemecek, MD, Oregon Health Sciences University, Portland, OR
Roberta Adams, MD, Phoenix Children's Hospital, Phoenix, AZ
Michael Pulsipher, MD, Primary Children's/University of Utah, Salt Lake City, UT
Andrew Dietz, MD, Rady Children's Hospital, San Diego, CA
Donna Wall, MD, Texas Transplant Institute, San Antonio, TX
Alyssa Reddy, MD, University of Alabama at Birmingham, Birmingham, AL
Biljana Horn, MD, University of California, San Francisco Pediatric, San Francisco, CA
John Wingard, MD, University of Florida College of Medicine, Gainesville, FL
Alexandra Cheerva, MD, University of Louisville, Louisville, KY
Martin Andreansky, MD, University of Miami, Miami, FL
Edward Peres, MD, University of Michigan Medical Center, Ann Arbor, MI
John Wagner, MD, University of Minnesota, Minneapolis, MN
Gail Megason, MD, University of Mississippi Medical Center, Jackson, MS
Haydar Frangoul, MD, Vanderbilt University, Nashville, TN
Kamar Godder, MD, Virginia Commonwealth University, Richmond, VA

Table S1: Multivariate Analysis for Survival and DFS

A. Overall Survival*

	P-value	Hazard Ratio (95% confidence interval)
Treatment	0.20	
Single UCB transplant		1.00
Double UCB transplant		1.34 (0.86 – 2.09)
Disease	0.03	
Acute myeloid leukemia		1.00
Acute lymphoid leukemia		0.57 (0.36 – 0.91)
Other leukemia/MDS		0.48 (0.22 – 1.03)

*P-value for interaction test between treatment and disease = 0.38

B. Disease-free Survival*

	P-value	Hazard Ratio (95% confidence interval)
Treatment	0.08	
Single UCB transplant		1.00
Double UCB transplant		1.48 (0.95 – 2.29)
Disease	0.03	
Acute myeloid leukemia		1.00
Acute lymphoid leukemia		0.59 (0.38 – 0.94)
Other leukemia/MDS		0.45 (0.21 – 0.97)
Race	0.03	
White		1.00
Non – White		1.68 (1.05 – 2.67)
HLA-match score	0.03	
6/6 and 5/6 HLA-match score		1.00
4/6 and 3/6 HLA-match score		0.60 (0.38 – 0.95)

*P-value for interaction test between treatment and disease (p= 0.34), race (p=0.10) and HLA-match (p=0.60)

HLA-match for double UCB transplants was assigned the worst HLA-match score between the UCB unit and recipient. For example, if unit 1 was 6/6 HLA-matched and unit 2, 4/6 HLA-matched, the transplant was assigned HLA-match score = 4/6

Table S2: Immune Reconstitution Evaluation

	VISIT	Double UCB			Single UCB			Wilcoxon P-Value
		Patients Evaluated	Mean (std)	Median (IQR)	Patients Evaluated	Mean (std)	Median (IQR)	
CD3 (cells/uL)	Day 100	76	494.3 (1011.5)	240.0 (110.0,417.0)	90	407.9 (417.8)	295.0 (173.0,444.0)	.16
	Day 180	68	656.9 (705.9)	431.5 (187.5,822.0)	79	752.5 (919.9)	394.0 (275.0,840.0)	.50
	Day 365	66	1193.0 (976.8)	977.5 (524.0,1714.0)	72	1320.0 (1115.8)	1003.0 (565.0,1822.0)	.56
CD4 (cells/uL)	Day 100	76	212.8 (304.3)	142.0 (86.0,273.0)	90	238.8 (214.3)	191.0 (106.0,318.0)	.14
	Day 180	68	354.7 (337.9)	254.0 (111.0,472.0)	79	374.9 (329.9)	290.0 (184.0,487.0)	.40
	Day 365	67	711.4 (599.0)	542.0 (293.0,1113.0)	73	771.6 (598.6)	631.0 (363.0,1040.0)	.38
CD8 (cells/uL)	Day 100	76	259.2 (728.1)	41.5 (23.0,133.0)	90	142.9 (236.3)	55.5 (28.0,127.0)	.47
	Day 180	68	286.5 (458.6)	94.5 (34.0,336.0)	79	333.3 (682.0)	91.0 (44.0,288.0)	.88
	Day 365	66	471.6 (517.9)	349.0 (146.0,589.0)	73	507.6 (636.0)	340.0 (169.0,707.0)	.99
CD19 (cells/uL)	Day 100	75	324.4 (533.0)	102.0 (1.0,398.0)	86	297.4 (470.5)	76.5 (8.0,443.0)	.68
	Day 180	67	603.6 (770.9)	448.0 (54.0,842.0)	77	606.1 (631.3)	444.0 (99.0,834.0)	.67
	Day 365	66	936.6 (826.4)	846.0 (341.0,1307.0)	70	886.2 (684.6)	852.5 (335.0,1325.0)	.99
CD56+/CD16+ (cells/uL)	Day 100	72	324.1 (339.3)	238.0 (138.5,374.5)	83	341.2 (307.5)	297.0 (119.0,483.0)	.42
	Day 180	64	287.9 (230.9)	244.5 (132.0,405.5)	71	300.3 (247.7)	230.0 (157.0,370.0)	.98
	Day 365	63	254.4 (241.6)	228.0 (126.0,320.0)	67	268.1 (580.8)	164.0 (127.0,280.0)	.26

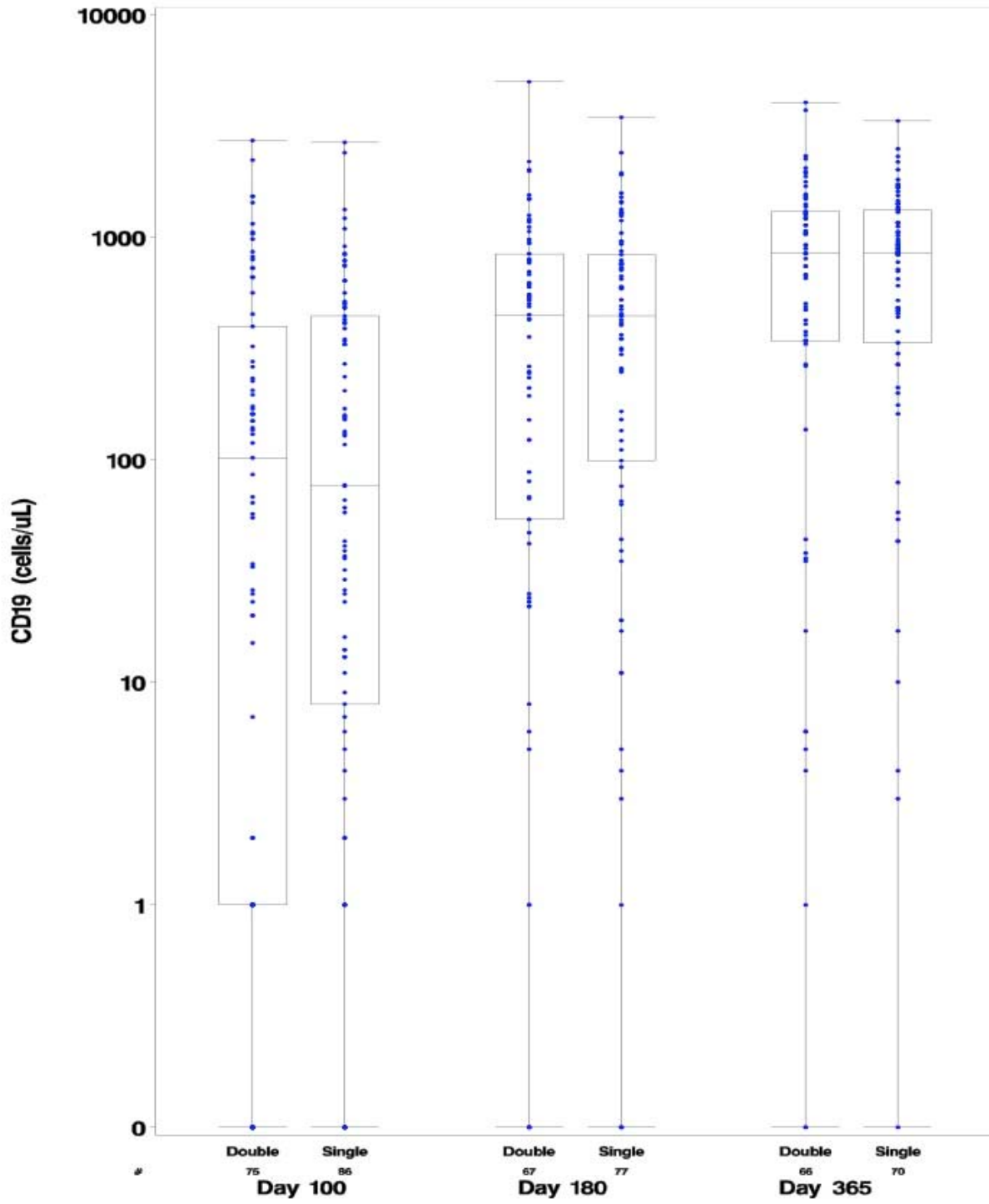Std=standard deviation; IQR=interquartile range.

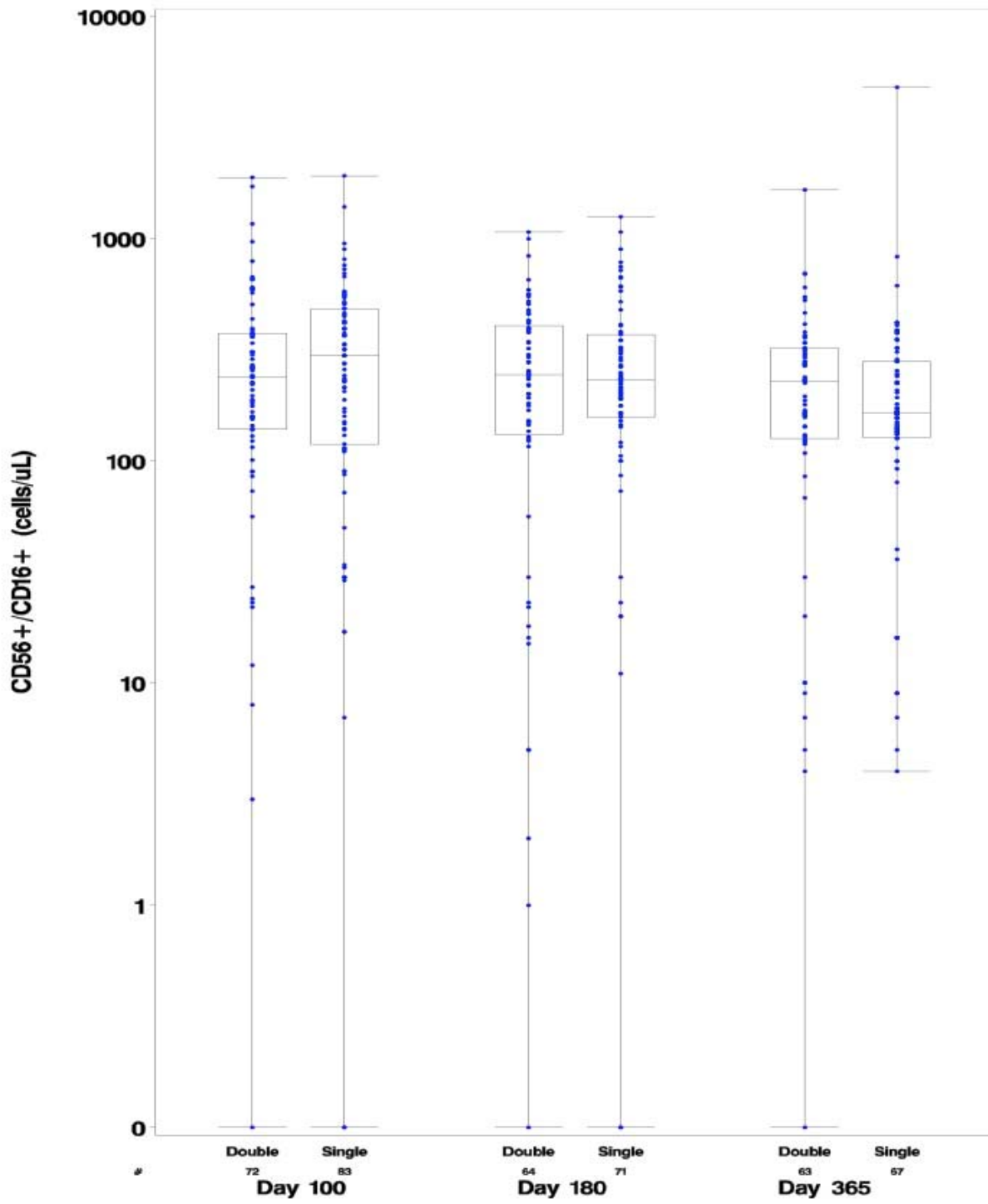**Figure S1. Immune reconstituion**
Panel A
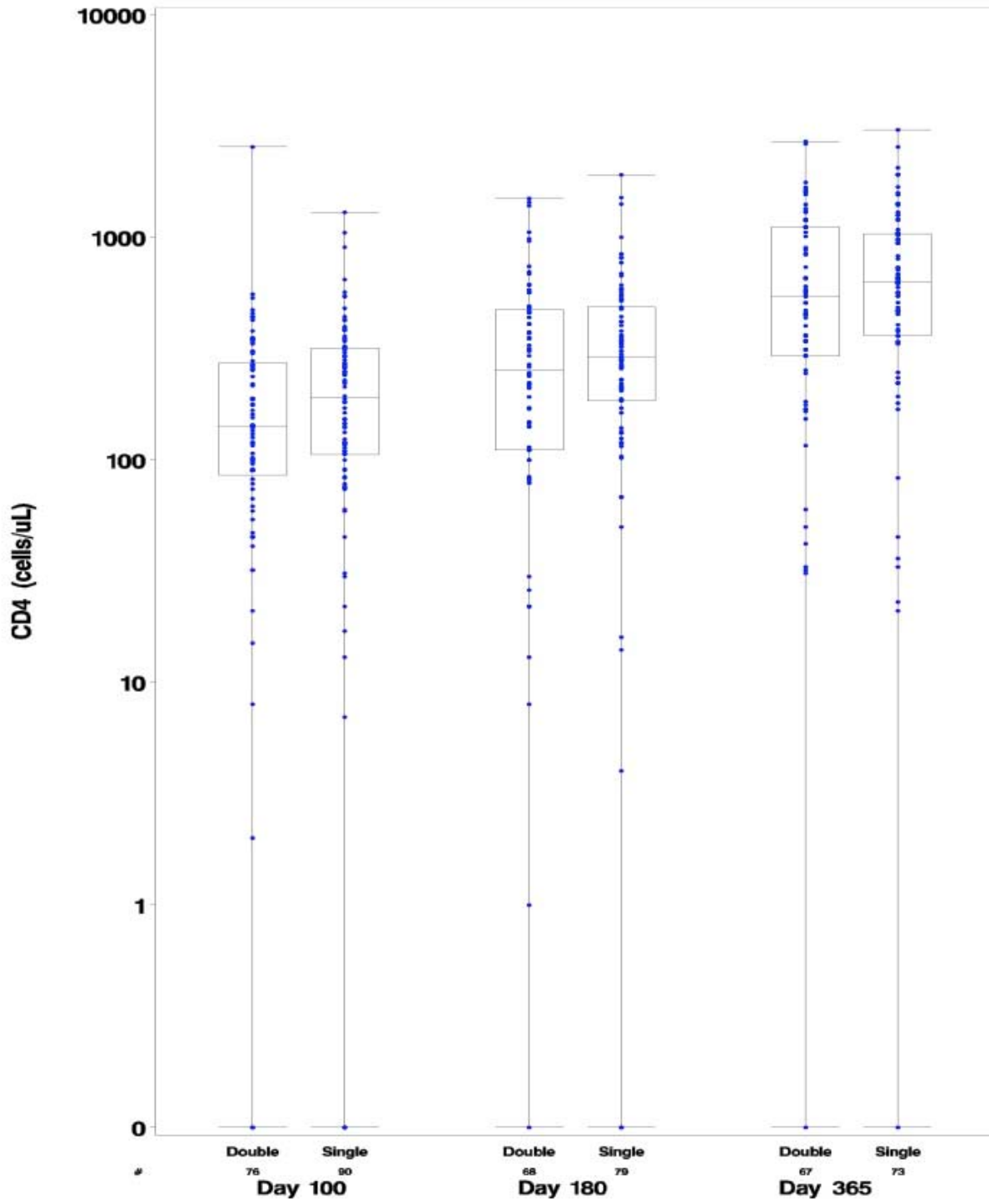
Panel B
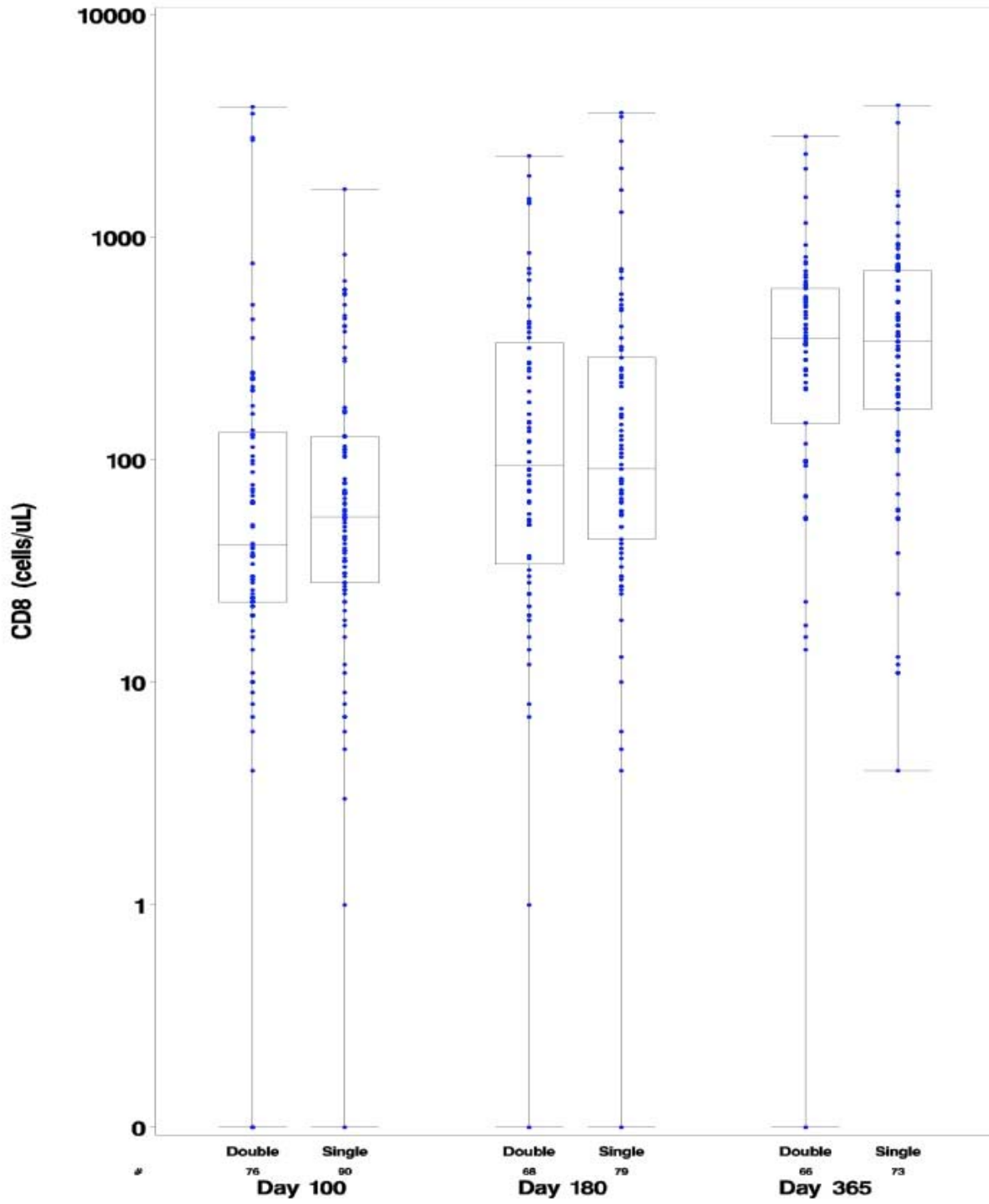
Panel C

Panel D

Distribution of cell populations by treatment arm (double=double UCBT; single=single UCBT) as assessed on days 100, 180 and 365. Y axis shows absolute cell counts per microliter peripheral blood on a logarithmic scale. CD4 cells (Panel A), CD8 cells (Panel B), CD19 cells (Panel C), and CD56/CD16 cells (Panel D). Side-by-side box plots show the individual measurements (dots) and the 25-75% quartiles at the very top and bottom of the box with the median indicated within the box, and range (horizontal bars at ends of data points).