Vor Bio Clinical Update

EHA 2023

June 9, 2023

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Today’s Agenda

Agenda	Speaker
Introductory Remarks	Robert Ang, Vor Bio President & CEO
VBP101 Clinical Trial Update & Results	Eyal Attar, MD, Vor Bio Chief Medical Officer
Investigator Perspective	Guenther Koehne, MD, PhD, Deputy Director and Chief of Blood & Marrow Transplant and Hematologic Oncology, Miami Cancer Institute of Baptist Health South Florida
Closing Remarks	Robert Ang, Vor Bio President & CEO
Q&A	

Introductory Remarks

Robert Ang, President & CEO

Vor's Vision: Cure Blood Cancers Through Cell & Genome Engineering

Unique approach
of protected eHSC transplants enabling post-transplant targeted therapy

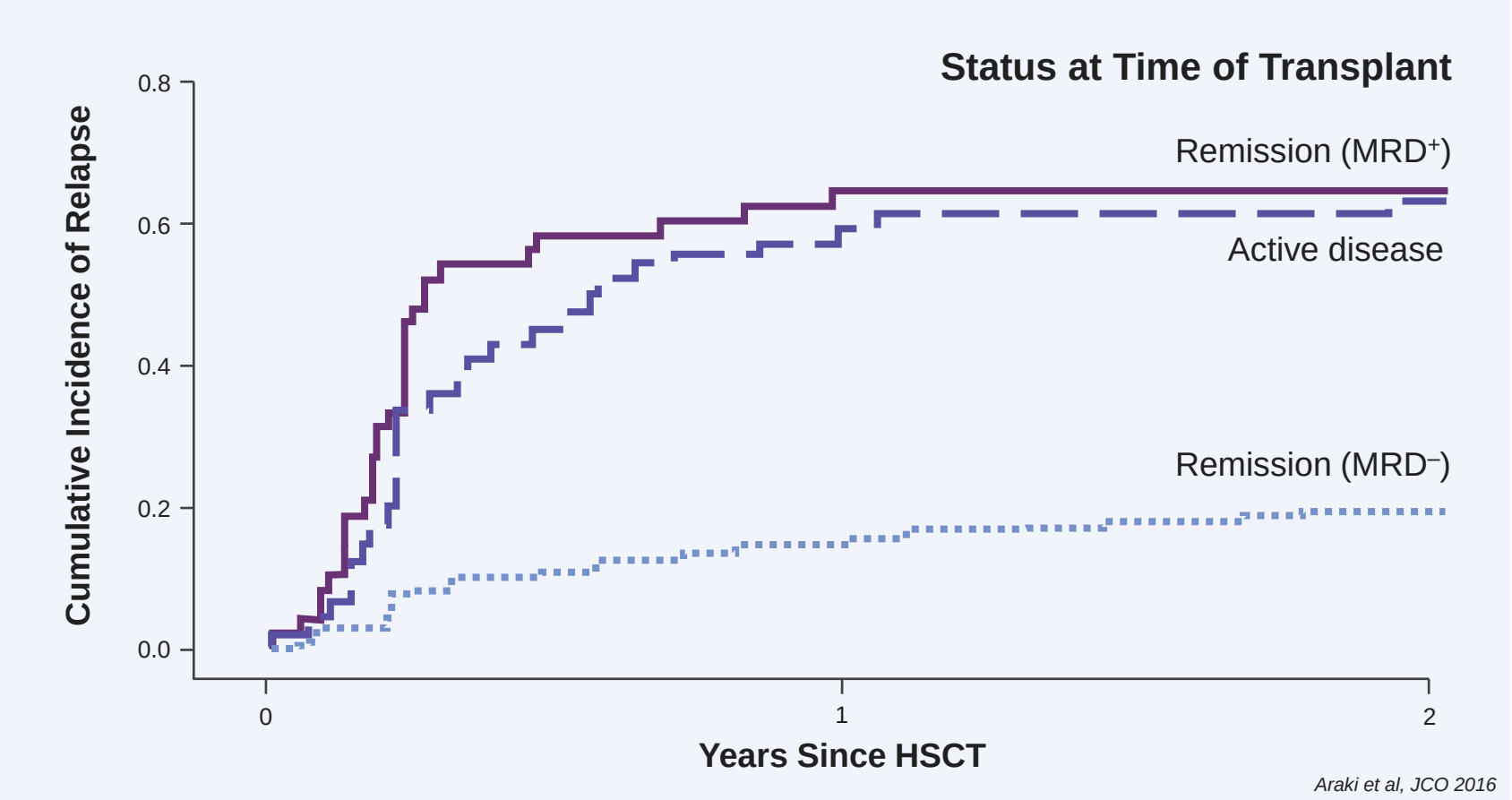
Clinical proof of concept
of trem-cel eHSC demonstrated in patients with AML

VCAR33^{ALLO}
IND cleared

Fully integrated in-house
GMP manufacturing capability

$210M
in cash, cash equivalents and marketable securities as of
March 31, 2023

Relapse is the #1 Issue With Transplant



Araki et al, JCO 2016

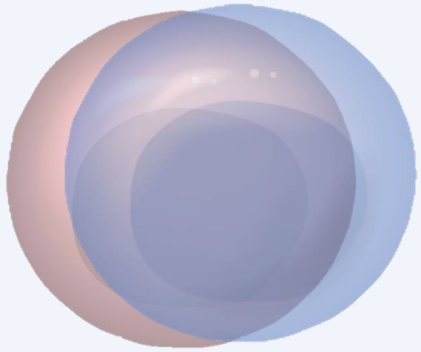
For relapsed patients, 2-year survival is <20%

Schmid et al, Blood 2012

Standard of care is watchful waiting since treatment will damage the transplant

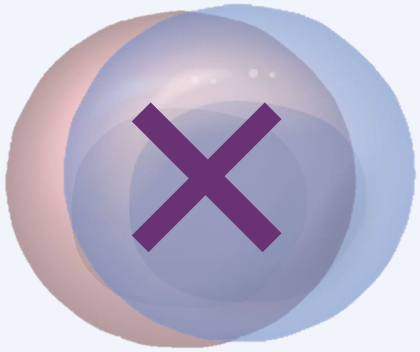
Changing the Thinking on Tumor Targeting

Biology: Overlapping Targets



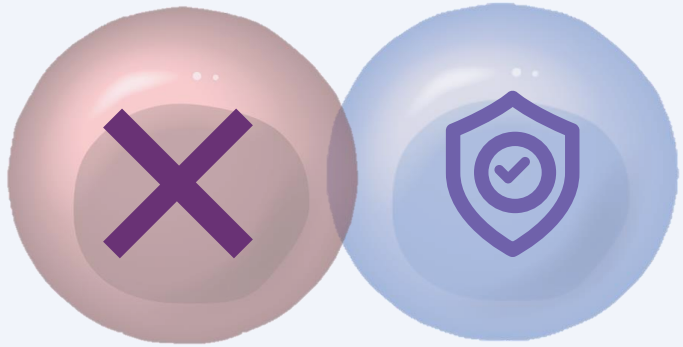
Cancer antigens also expressed on healthy cells

Problem: On-target Toxicity



Limits treatment opportunities leading to poor outcomes

Solution: Protected Transplants

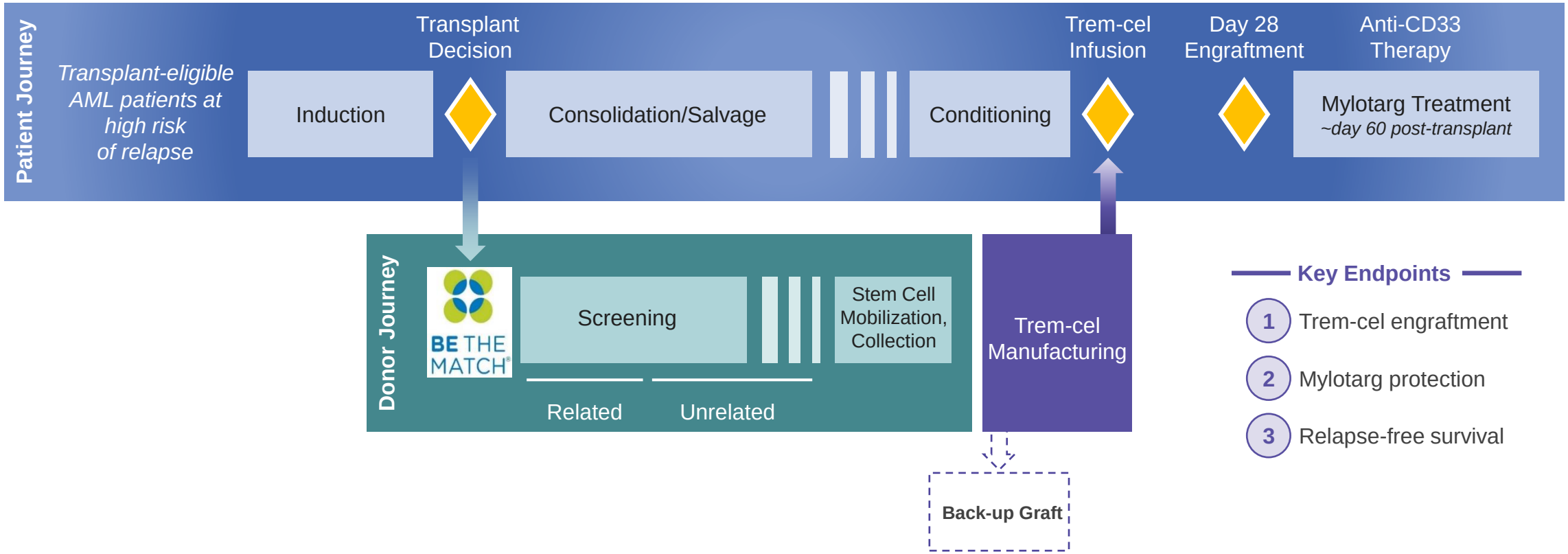


Treatment-resistant transplants allowing therapies to be cancer-specific

VBP101 Clinical Trial Update

Eyal Attar, MD, Chief Medical Officer

VBP101: Trem-cel (VOR33) + Mylotarg Phase 1/2a Clinical Trial



Key Endpoints

1. Trem-cel engraftment
2. Mylotarg protection
3. Relapse-free survival

Clinical Trial Sites

- MSKCC (NY)
- Hackensack/Theurer Cancer Ctr. (NJ)
- Miami Cancer Inst. (FL)
- UC San Diego Cancer Ctr. (CA)
- CWRU/Seidman Cancer Ctr. (OH)
- Hôpital Maisonneuve-Rosemont (Montreal)
- The National Cancer Institute (MD)
- WashU Siteman Cancer Ctr. (MO)
- Fred Hutchinson Cancer Ctr. (WA)

Enrolling High Risk Patients with Complex Cytogenetics

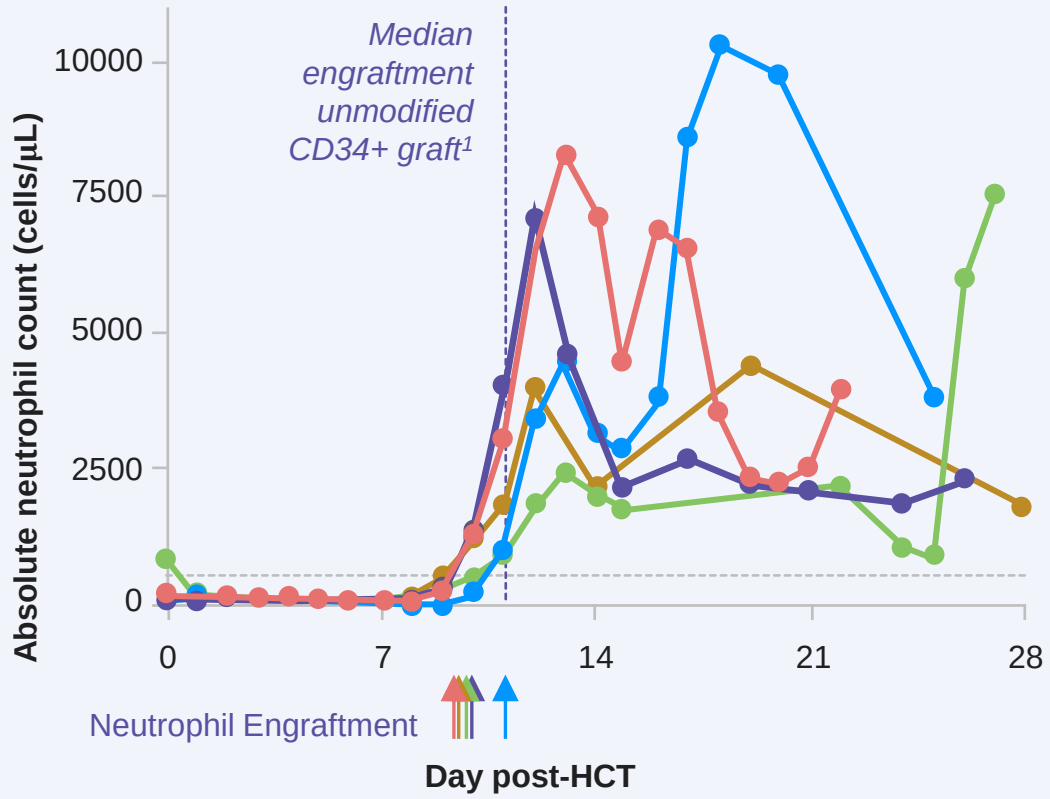
Pt	Age/ Sex	Disease and Genetics	Weight	Donor, Dose, *CD33* gene-editing efficiency
1	64/F	• AML-MRC • Highly complex (adverse) cytogenetics, CR2, TP53 mutation • MRD: 1.8%	69.9 kg	• 10/10 HLA MUD • 7.6 × 10^6 CD34 cells/kg • 88% *CD33* gene editing
2	32/M	• AML after myeloid sarcoma resected from abdomen • Inv 16 and +22, t(3;3)	120.7 kg	• 10/10 HLA MUD • 3.2 × 10^6 CD34 cells/kg • 87% *CD33* gene editing
3	55/F	• AML-MRC • DNMT3A, IDH2 and SMC1A mutations	114.1 kg	• 10/10 HLA MUD • 2.6 × 10^6 CD34 cells/kg • 80% *CD33* gene editing
4	68/M	• AML-MRC • Complex cytogenetics, active disease, NRAS, ZRSR2, TET2 mutations • MRD: 16%	72.4 kg	• 10/10 HLA MSD • 5.8 × 10^6 CD34 cells/kg • 89% *CD33* gene editing
5	66/M	• Secondary AML • KIT D816V, CBL, SRSF2, RUNX1/2, BCORL1 mutations	102.1 kg	• 10/10 HLA MUD • 4.6 × 10^6 CD34 cells/kg • 85% *CD33* gene editing

Data compiled from EDC, lab reports and PI/site reports, pending full source data verification

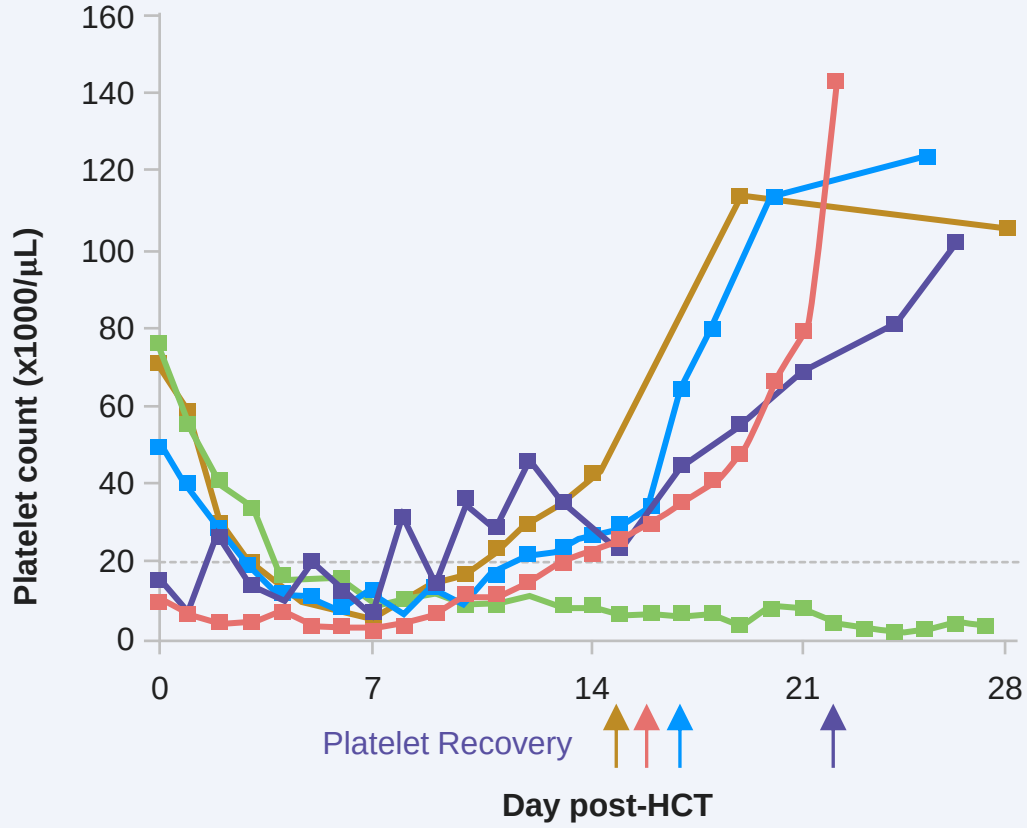
MRC = myelodysplasia-related changes, MRD = Measurable Residual Disease, MUD = Matched Unrelated Donor, MSD = Matched Sibling Donor

Trem-Cel Engrafted Similar to Non-Edited Transplants

Neutrophil engraftment



Platelet recovery



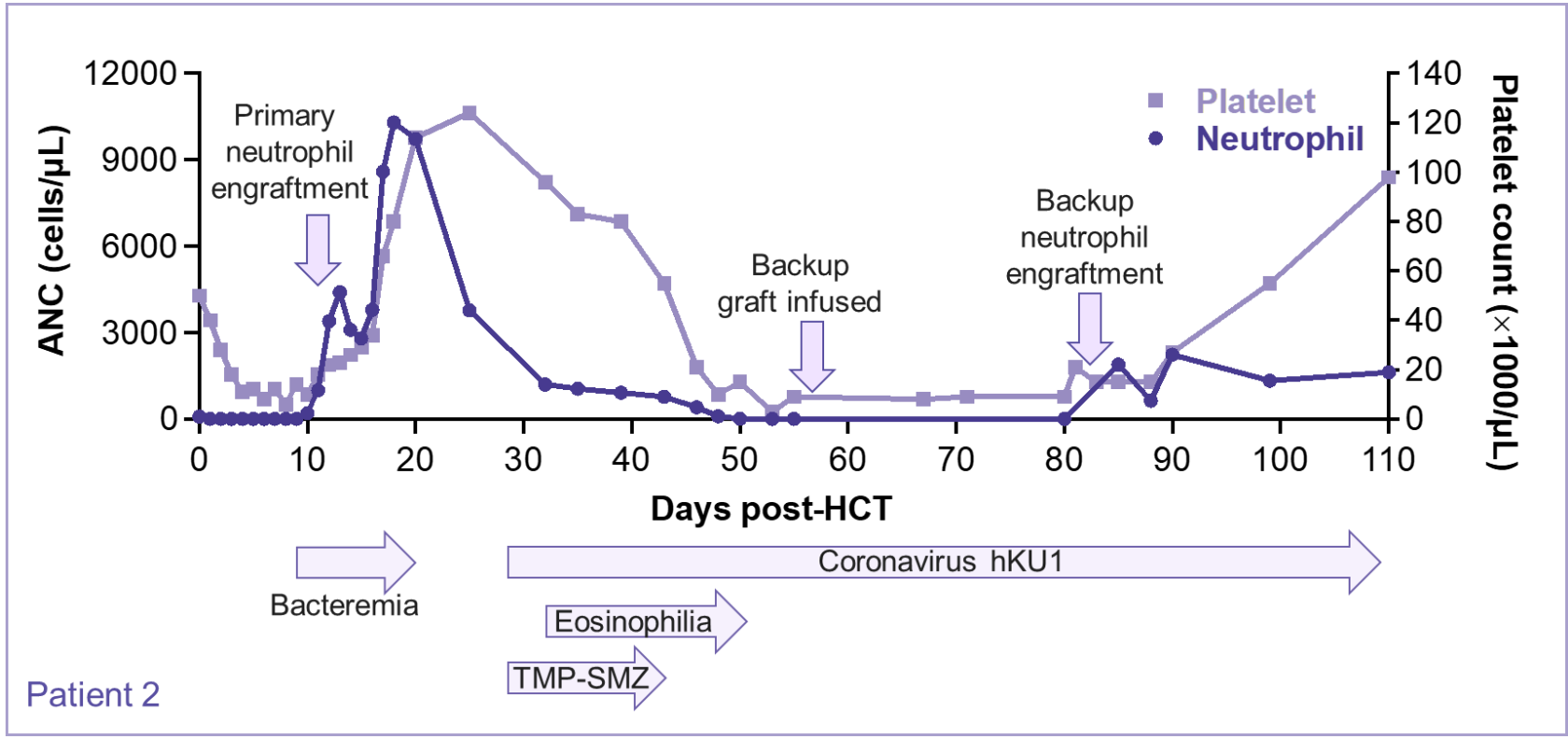
Patient 1 | Patient 2 | Patient 3 | Patient 4 | Patient 5

[1]Luznik L. et al. J Clin Oncol 2022;40(4):356–368. Data compiled from EDC, Lab Reports and PI/site reports, pending full source data verification

Patient 2: Clinical Course

Summary

- Primary neutrophil engraftment: D+11
- Patient developed cytopenias after D+28 in the setting of coronavirus hKU1, trimethoprim-sulfamethoxazole (TMP-SMZ) exposure, and subsequent eosinophilia
- Backup graft infused: D+57
- Neutrophil engraftment and platelet recovery at days 26 and 30 respectively following back-up infusion



Data compiled from EDC, Lab Reports and PI/site reports, pending full source data verification

Patient 3: Clinical Course

- Neutrophil engraftment at D+10; platelets are still recovering
- A platelet-reactive antibody was identified, and the patient is being treated for immune thrombocytopenia
- D+100 BM biopsy showed normal cellularity with normal megakaryocytes
- As of D+117 the platelet count was 15,000/μL independent of transfusions

Data compiled from EDC, Lab Reports and PI/site reports, pending full source data verification

Reported Clinical Safety Events

Pt	Related Serious Adverse Event (SAE) or Adverse Event (AE)	Trem-cel related	Mylotarg-related	Grade
1	**Gastrointestinal:** Nausea and vomiting		Y	1-2
2	**Hematologic:** Secondary graft failure	Y		4
3	**Dermatologic:** Full-body maculopapular rash (Gr 2 skin acute GVHD/resolved)	Y		2
	Hematologic: Neutropenia	Y		3
	Other: Dyspnea and fatigue, petechia	Y		1 - 2

Patients 4 and 5 had no related AEs to report as of data cutoff date: 23 May 2023

Data compiled from EDC, Lab Reports and PI/site reports, pending full source data verification

Stable Edit and Donor Chimerism Across Lineages

Patient	Bulk		Monocytes (CD14+ CD15+)		NK cells (CD16+ CD56+)		B cell (CD19+)	
Transplant D+28								
	DC%	CD33 GE%	DC%	CD33 GE%	DC%	CD33 GE%	DC%	CD33 GE%
1	100	95.2	100	95	100	92.1	QNS	QNS
2	94	88.9	100	94.5	99	92.3	100	91.8
3	100	86.6	100	87.9	100	89.0	100	89.4
4	100	pending	100	pending	100	pending	100	pending
Transplant D+60								
1	100	95.9	100	95.6	100	94.9	100	95.6
3	100	87.9	100	90.1	100	90.1	100	88.5

Data compiled from EDC, Lab Reports and PI/site reports, pending full source data verification

CD33 GE%= CD33 Gene Editing Efficiency %, DC%= Donor Chimerism %, QNS = Quantity Not Sufficient

Flow Cytometry Confirms Low CD33 Expression

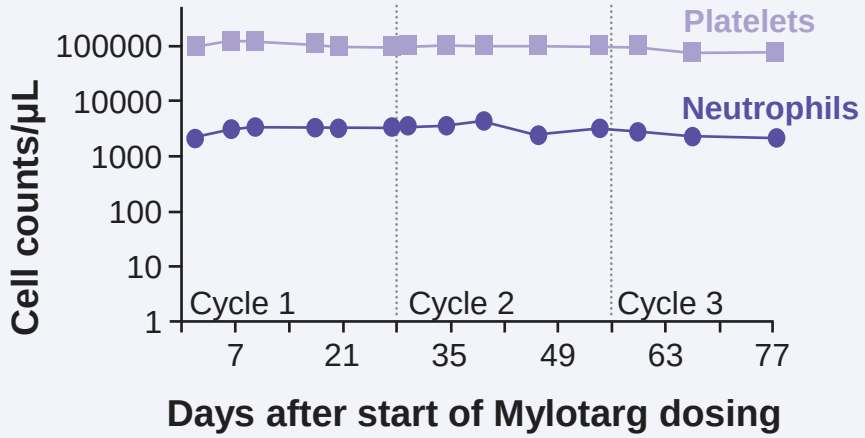
CD33-negative expression by flow (%)								
Patient	Monocyte		Myeloid		Monocyte		Myeloid	
	Transplant D+28				Transplant D+60			
	PB	BM	PB	BM	PB	BM	PB	BM
1	94	92	95	95	94	90	96	91
2	93	91	99	98	NC	NC	NC	NC
3	82	80	86	86	87	85	92	89
4	90	90	95	94	TBD	TBD	TBD	TBD

Data compiled from EDC, Lab Reports and PI/site reports, pending full source data verification

Patient 5 has not yet had D+28 evaluation. BM= bone marrow, PB=peripheral blood, NC=not collected, TBD=to be determined

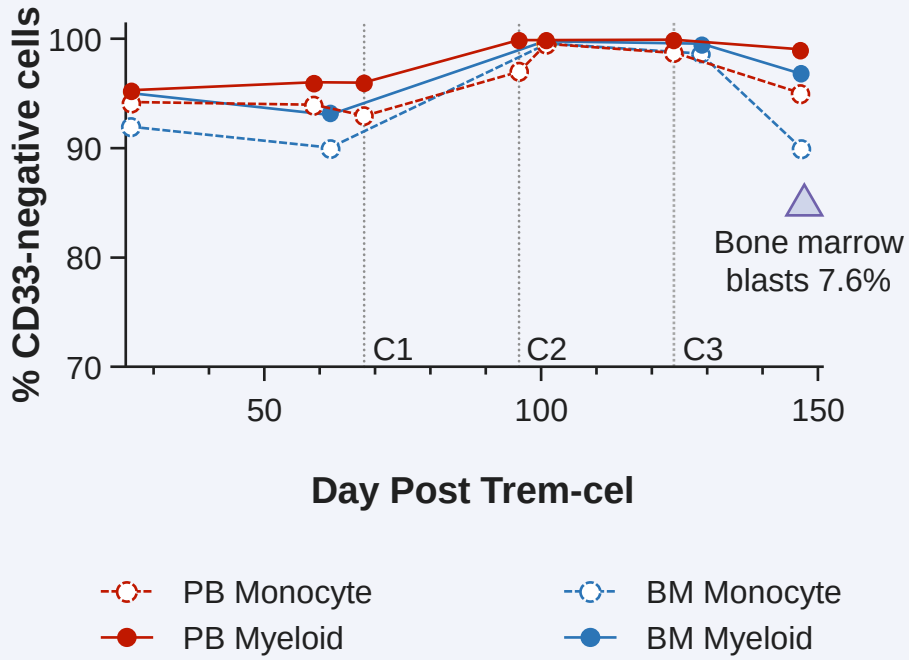
Patient 1: Heme Protection after Mylotarg Dosing

Neutrophil and platelet counts



Days Post-HCT (Post-Mylotarg)	BM MRD %
D+60 (Pre-Mylotarg)	<0.1
D+101 (5d post-cycle 2)	0.3
D+147 (23d post-cycle 3)	7.6

CD33-negative cells (flow)



Data compiled from EDC, Lab Reports and PI/site reports, pending full source data verification

Relapsed CD33+ AML overlapped normal cell populations at D+147 time point

Conclusions

Engraftment

All patients achieved primary engraftment

High levels of CD33-negative hematopoiesis

Safety

Data support CD33 dispensability

Patients 2 and 3 successfully supported through cytopenias

Protection

Patient 1 demonstrated durable heme protection through multiple Mylotarg cycles

Manufacturing

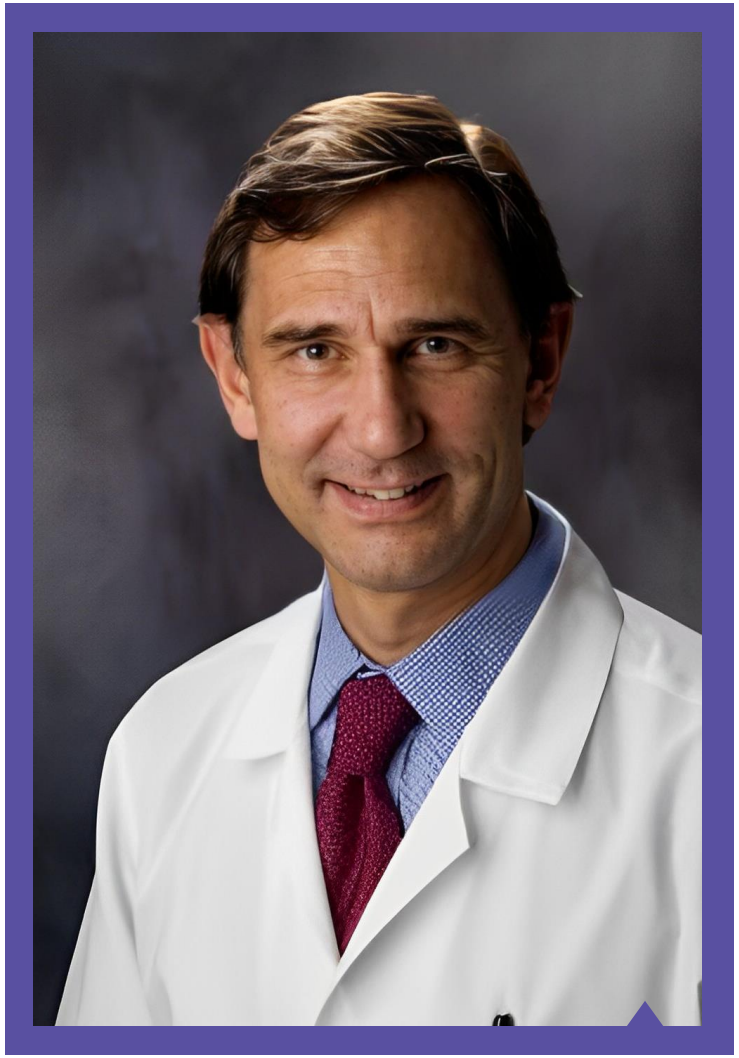
Successful runs and high editing efficiency for all patients

Clinical Execution

Strong investigator enthusiasm

Demand exceeds enrollment stagger

Investigator Perspective



Guenther Koehne, MD, PhD

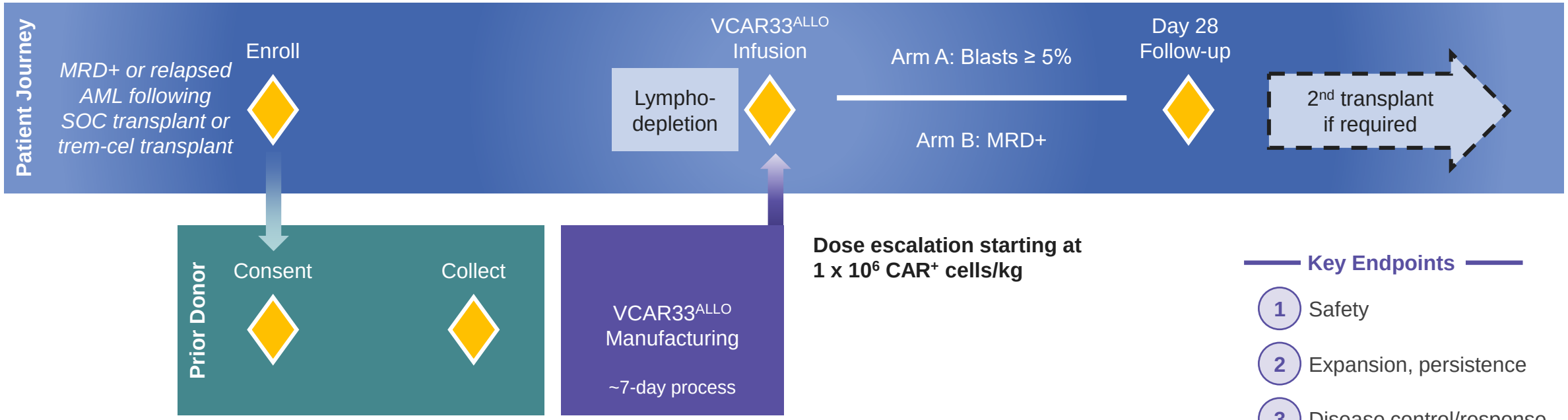
Deputy Director and Chief of Blood &
Marrow Transplant and Hematologic Oncology
Miami Cancer Institute of Baptist Health South Florida

Closing Remarks

Robert Ang, President & CEO

Breaking News: FDA Clearance of VCAR33^{ALLO} IND Application

VBP301: VCAR33^{ALLO} Phase 1/2 Clinical Trial



Key Clinical Catalysts over Next 12 Months

Clinical Progress

Expect additional VBP101 data at year-end 2023

- Robust enrollment and strong investigator enthusiasm
- Additional engraftment data
- Additional heme protection data following Mylotarg treatment

FDA cleared VCAR33^{ALLO} IND

VOR

Q&A

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