



Clinical & Corporate Update

December 9, 2024



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Today's Agenda

Agenda

Speaker

Introductory Remarks

Robert Ang, MBBS, MBA, President & CEO

VBP101 Clinical Update and Regulatory Update

Eyal Attar, MD, Chief Medical Officer

Closing Remarks

Robert Ang, MBBS, MBA, President & CEO

Perspective on VBP101

Guenther Koehne, MD, PhD, Deputy Director and Chief of Blood & Marrow Transplant and Hematologic Oncology at Miami Cancer Institute of Baptist Health South Florida

Q&A

Robert Ang, MBBS, President & CEO
Eyal Attar, MD, Chief Medical Officer
Han Choi, MD, LLM, Chief Financial Officer
Guenther Koehne, MD, PhD

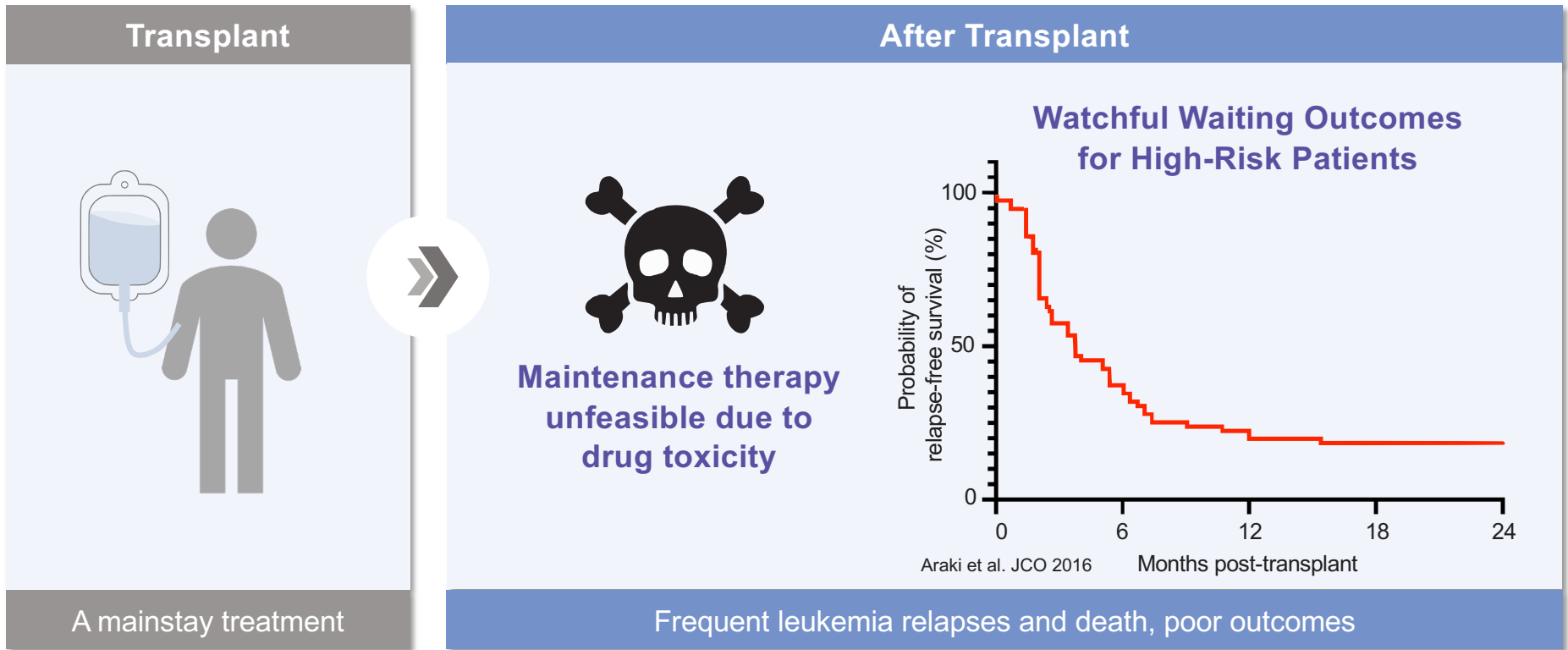


Introductory Remarks

Robert Ang, MBBS, MBA, President & CEO

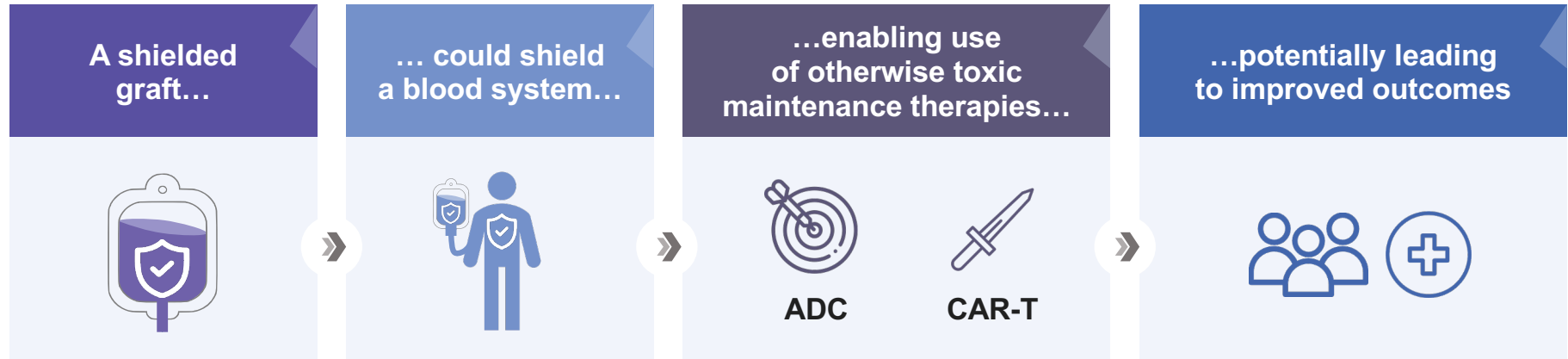


Even After Transplant, High-Risk AML Has Poor Outcomes





What If Shielding Could Lead to Improved Outcomes?

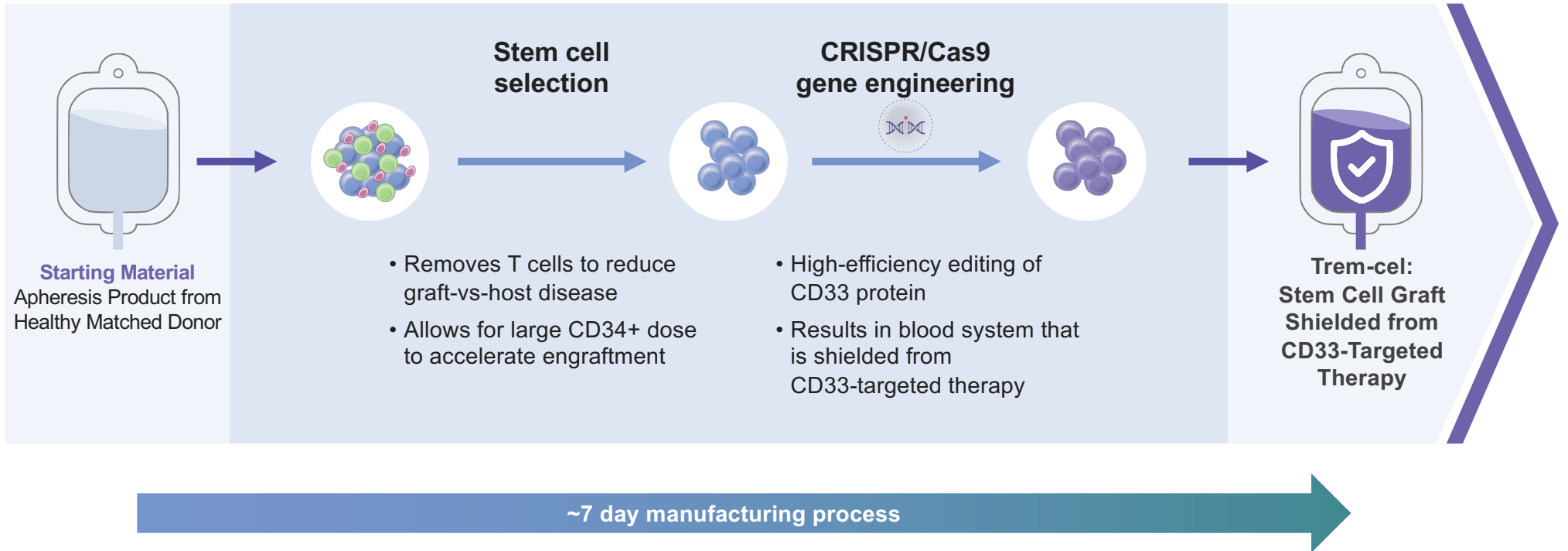


Required Shielded Graft Attributes

- ✓ **Engraftment**
Reliably reconstitute the blood system
- ✓ **Shielding**
Protect against otherwise toxic therapies
- ✓ **Therapeutic Index**
Optimize efficacy and safety of maintenance therapies
- ✓ **Patient Benefit**
Prolong relapse-free survival



What is Trem-Cel?

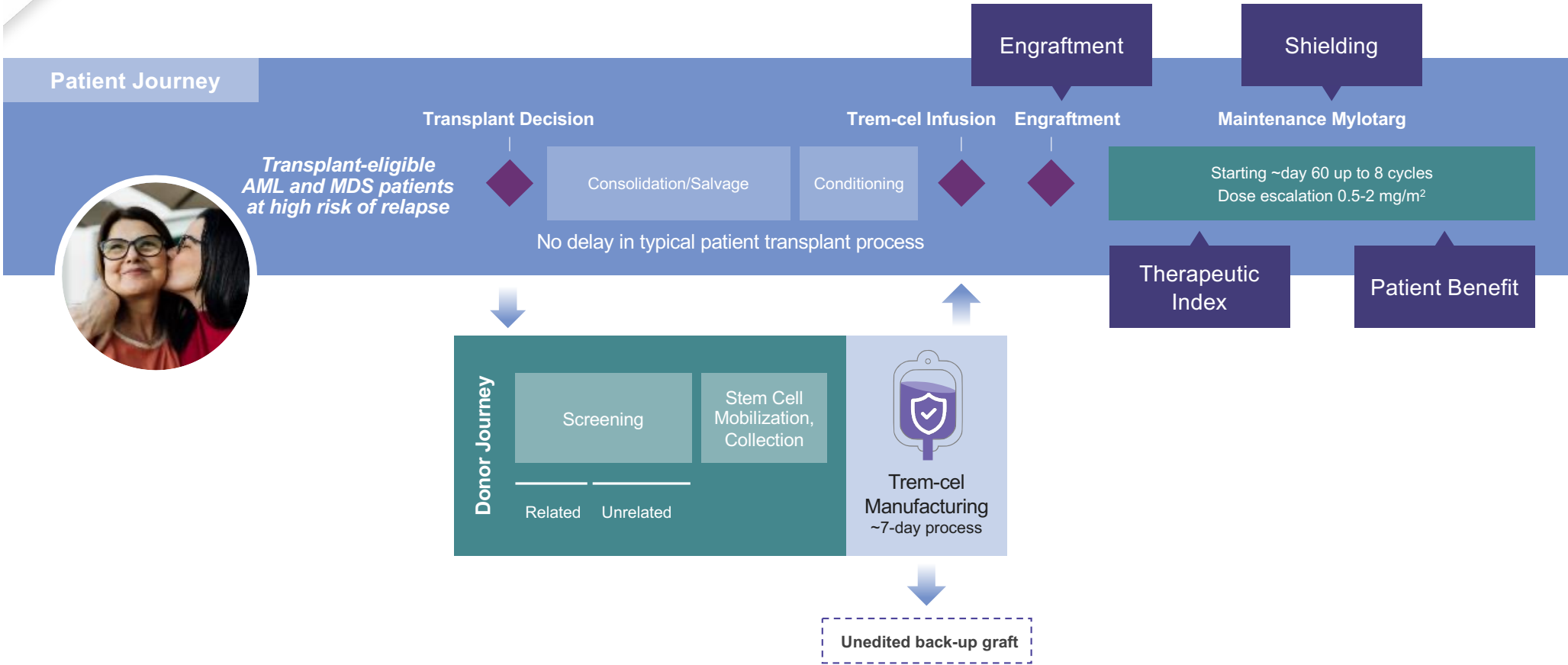




VBP101 Clinical Update

Eyal Attar, MD, Chief Medical Officer

VBP101: Trem-cel Phase 1/2a Clinical Trial





Trem-cel Achieved Timely Engraftment

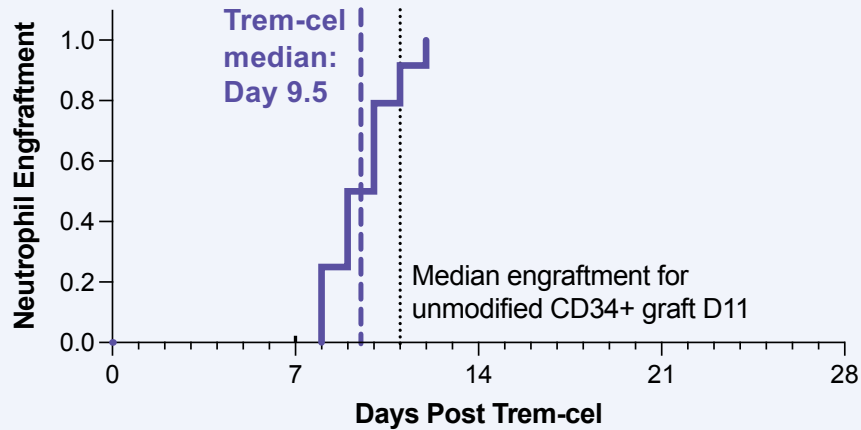
✓ Engraftment

✓ Shielding

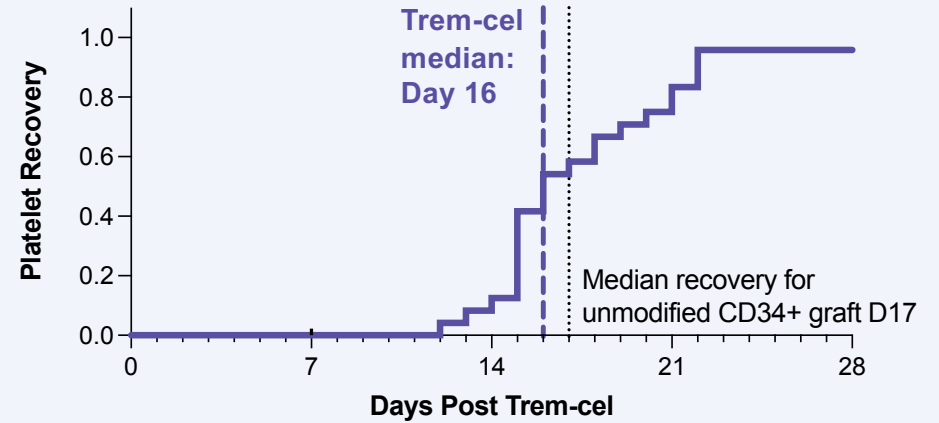
✓ Therapeutic Index

✓ Patient Benefit

Neutrophil Engraftment (n=25)



Platelet Engraftment (n=25)



✓ High CD33 editing efficiency (median 90%, range 71-94%)

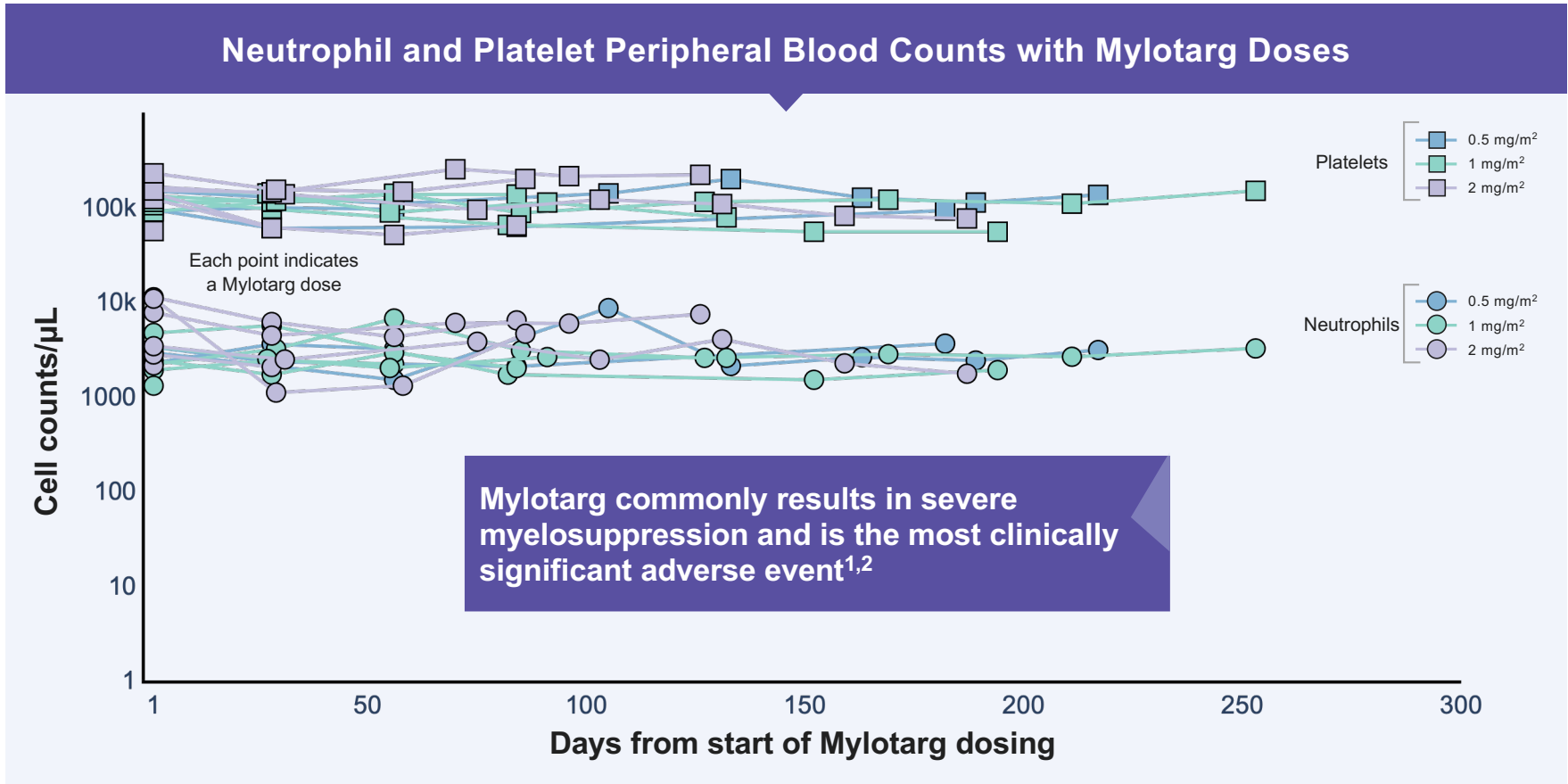
✓ 100% neutrophil engraftment

✓ 100% achieved full myeloid chimerism at D28

Data cut-off: 1-NOV-2024



Trem-cel Demonstrated Shielding Across Mylotarg Doses

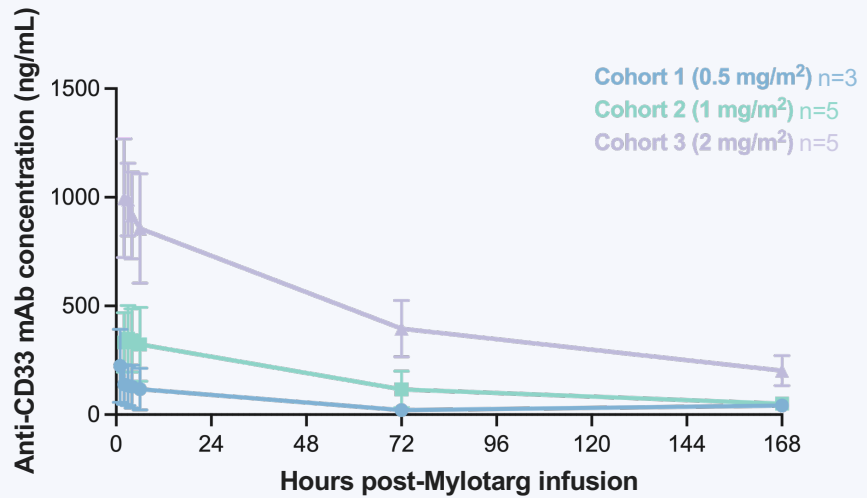


11 1. Sievers et al. Blood 1999 2. Mylotarg prescribing information
Data cut-off: 1-NOV-2024

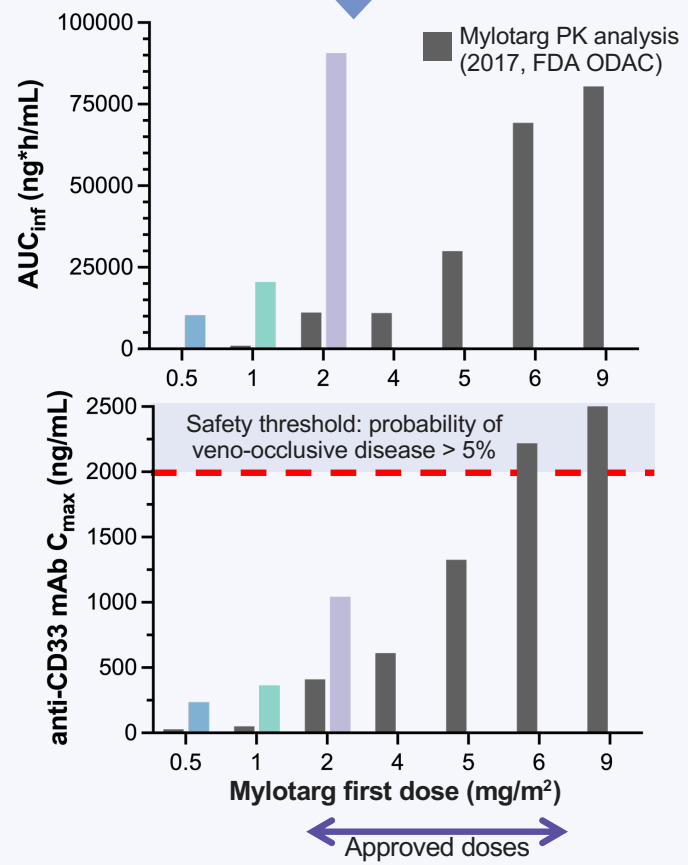


Trem-cel Enabled Broadened Therapeutic Index for Mylotarg

Mylotarg Pharmacokinetic Profile



C_{max} and AUC Across Mylotarg Doses





Baseline Risk Factor Demographics for AML Patients: VBP101 vs. Comparators

Disease Characteristic	VBP101 AML ITT (n=24)	VBP101 AML Treated with Mylotarg (n=15)	Araki MRD+ Cohort ⁽²⁰¹⁶⁾ (n=76)	Jentzsch Adverse Risk Cohort ⁽²⁰²²⁾ (n=271)
Cytogenetics Risk ELN 2022				
Favorable	8%	13%	3%	N/A
Intermediate	33%	27%	58%	N/A
Adverse	58%	60%	39%**	100%*
Other AML Risk Factors				
TP53 mutation	33%	40%	NR	NR
Secondary AML ^a	42%	33%	42%	49%
Disease Burden Status				
Remission (MRDneg)	75%	73%	N/A	20%
MRD+ (>0.1-<5% blasts by flow)	13%	20%	100%*	13%
Active disease (≥5% blasts)	13%	7%	N/A	32%***
AML Disease Status				
CR1	63%	60%	67%	61%
CR2	25%	33%	33%	7%
Relapsed or refractory	13%	7%	0	32%***
Adverse Risk Features (Adverse ELN/molecular/cytogenetic, Secondary AML, MRD or active disease, CR2 or Relapsed/Refractory), n (%)				
1	11 (46%)	6 (40%)		
2 or more	13 (54%)	9 (60%)		

*Selected comparison cohort (n) from published studies. **Adverse cytogenetics. ***Includes partial remission, relapsed, refractory. Jentzsch values for disease burden status do not total 100% due to data not reported.

^aDefined as AML with myelodysplasia-related change and therapy-related AML, NR=not reported, N/A=not applicable

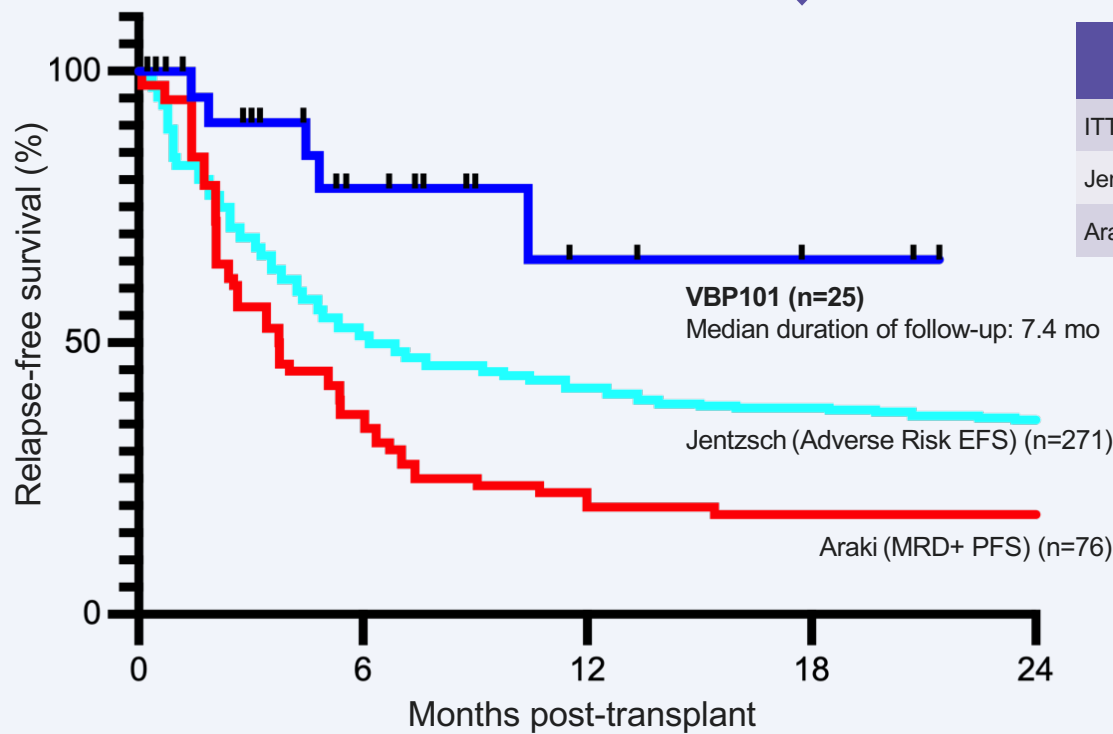
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Trem-cel+Mylotarg RFS Appears Favorable vs Published High-Risk AML Comparators

- ✓ Engraftment
- ✓ Shielding
- ✓ Therapeutic Index
- ✓ Patient Benefit

Relapse-Free Survival of VBP101 (intention-to-treat) vs Araki and Jentzsch (historical controls)



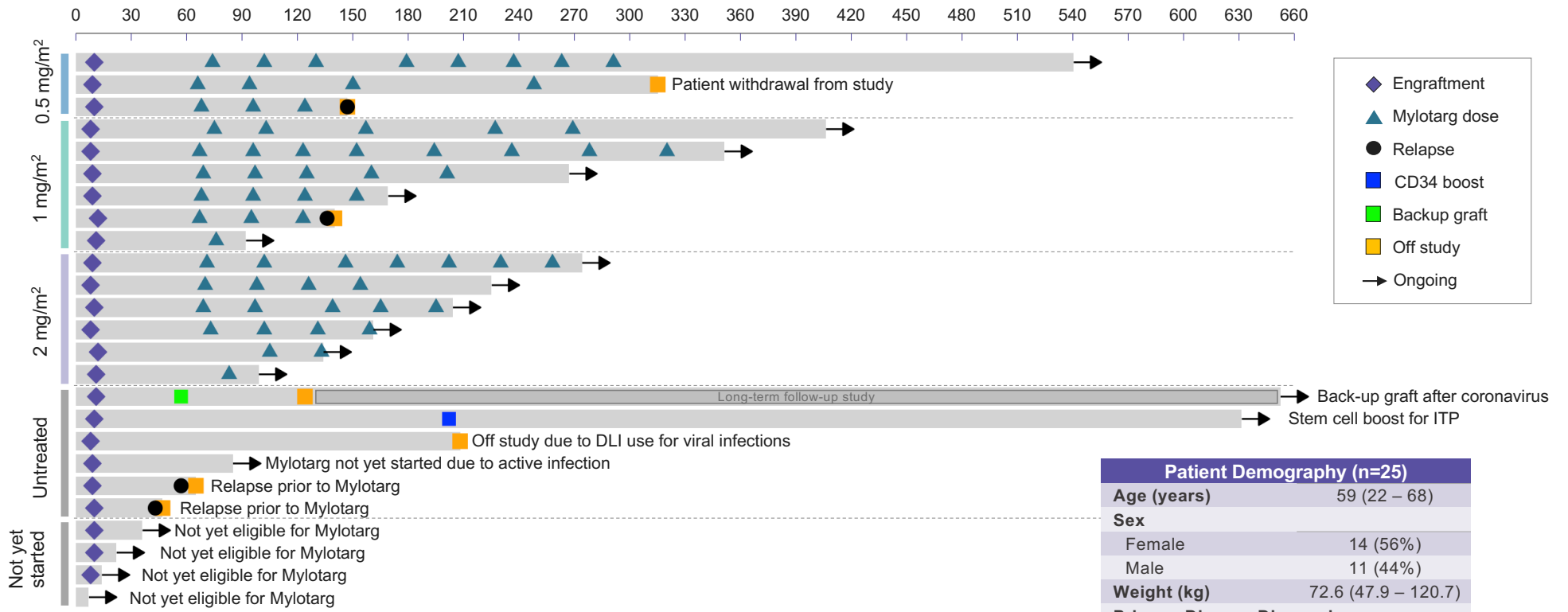
	Median RFS (mo)	P value vs VBP101*	Hazard Ratio* (HR)	HR 95% CI*
ITT	Not reached			
Jentzsch	6.2	0.02	0.36	0.21-0.64
Araki	3.8	0.0004	0.23	0.14-0.40

- Four relapses observed: (all CD33 positive at relapse)
 - 2/4 relapsed prior to Mylotarg treatment
 - 3/4 transplanted with active disease; 1/4 with MRD
 - 4/4 adverse risk cytogenetics
- One patient died off-study due to complications of viral infection

VBP101 data cut-off: 1-NOV-2024. Adapted from Fig 2B MRD+ PFS line from Araki et al. JCO 2016; Adapted from Fig 1C, ELN 2022 Adverse risk EFS line from Jentzsch et al. Blood Cancer Journal 2022. * = individual comparison to VBP101 using log-rank Mantel-Cox test. Data not from head-to-head trial.



Low Rate of Relapse (2/15) Among Patients Receiving Mylotarg



Patient Demography (n=25)	
Age (years)	59 (22 – 68)
Sex	
Female	14 (56%)
Male	11 (44%)
Weight (kg)	72.6 (47.9 – 120.7)
Primary Disease Diagnosis	
AML	24 (96%)
MDS	1 (4%)

ITP: idiopathic thrombocytopenic purpura or similar immune-mediated thrombocytopenia
 Data cut-off: 1-NOV-2024



Any Grade Treatment Adverse Events After Receiving Mylotarg (n=15)

Adverse Event	Grade 1	Grade 2	Grade 3	Grade 4
Hematologic				
Anemia	-	1/15 (7%)	3/15 (20%)	-
Autoimmune hemolytic anemia	-	-	1/15 (7%)	-
Leukopenia	-	-	1/15 (7%)	-
Lymphocyte count decreased	1/15 (7%)	-	-	-
Lymphopenia	-	-	1/15 (7%)	-
Neutropenia	-	2/15 (13%)	3/15 (20%)	-
Platelet count decreased	-	-	2/15 (13%)	-
Thrombocytopenia	-	1/15 (7%)	1/15 (7%)	1/15 (7%) ^a
Hepatobiliary				
ALT increased	2/15 (13%)	1/15 (7%) ^b	-	-
AST increased	1/15 (7%)	-	1/15 (7%) ^b	-
Biliary colic	1/15 (7%)	-	-	-
Alk Phos increased	3/15 (20%)	-	-	-
Blood bilirubin increased	1/15 (7%)	-	-	-
LDH increased	2/15 (13%)	-	-	-
Cholecystitis	-	2/15 (13%)	-	-
Veno-occlusive disease	1/15 (7%) ^c	-	-	-

^aFollowing adverse event, patient continued to receive multiple cycles of Mylotarg

^bALT/AST elevation attributed to fluconazole toxicity and resolved after discontinuation

^cMild grade late-onset veno-occlusive disease occurred 97 days after 0.5 mg/m² Mylotarg dose. Predisposing factors included azole toxicity, concurrent norovirus infection and gram-negative bacteremia.

ALT = Alanine aminotransferase; AST = Aspartate aminotransferase; Alk Phos = blood alkaline phosphatase; LDH = blood lactate dehydrogenase

Data cut-off: 1-NOV-2024



Clinical Update Summary

- Robust neutrophil engraftment, platelet recovery and full donor myeloid chimerism
- Consistent shielding from Mylotarg-related cytopenias during repeated 0.5, 1, and 2 mg/m² doses
- Immune reconstitution, multilineage chimerism, and safety profile similar to unedited CD34-selected grafts
- Broadened Mylotarg therapeutic index following trem-cel
- Preliminary data suggesting improved RFS compared to published groups of AML patients at high risk of relapse post-HCT

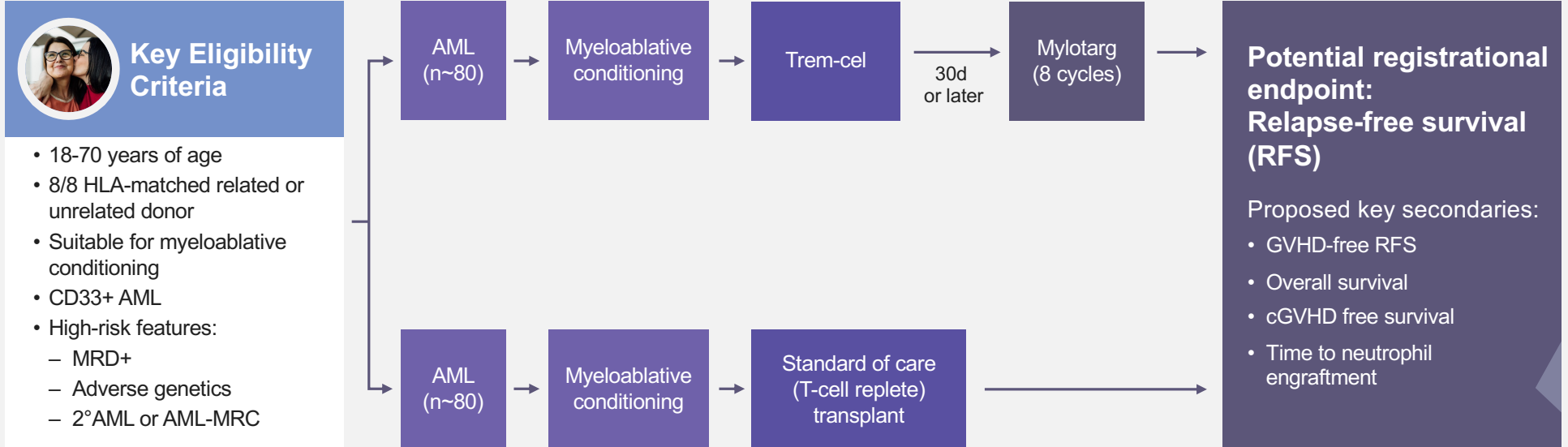


Trem-cel + Mylotarg Regulatory Update



Potential Registrational Trial Design for Trem-cel/Mylotarg

Patient Journey





Summary of FDA Response to Type C Meeting Request

- Agreement that trem-cel engrafts neutrophils and platelets and has a similar safety profile to unedited CD34+ grafts
- Agreement with the trem-cel-Mylotarg registrational clinical trial design with respect to study population, control arm, primary endpoint, stratification factors, and statistical design
- Agreement to provide further updates to FDA from the VBP101 trial alongside submission of the full registrational clinical trial protocol



Closing Remarks

Robert Ang, MBBS, MBA, President & CEO



Vor Bio Unique Approach to Potentially Cure Blood Cancers



Trem-cel, a first-in-class investigational* shielded stem cell transplant

- Reliable engraftment, robust shielding of the blood system
- Platform therapy addressing >\$1B potential market opportunity



Trem-cel + Mylotarg combination

- Broadened Mylotarg therapeutic index and early evidence of patient benefit prolonging RFS
- Supportive feedback from FDA on registrational trial design



VCAR33^{ALLO} and VADC45

- Offer multiple additional potential therapeutic options as targeted therapies in AML and in oncology, gene therapy, and autoimmune disorders





Perspective on VBP101

Guenther Koehne, MD, PhD

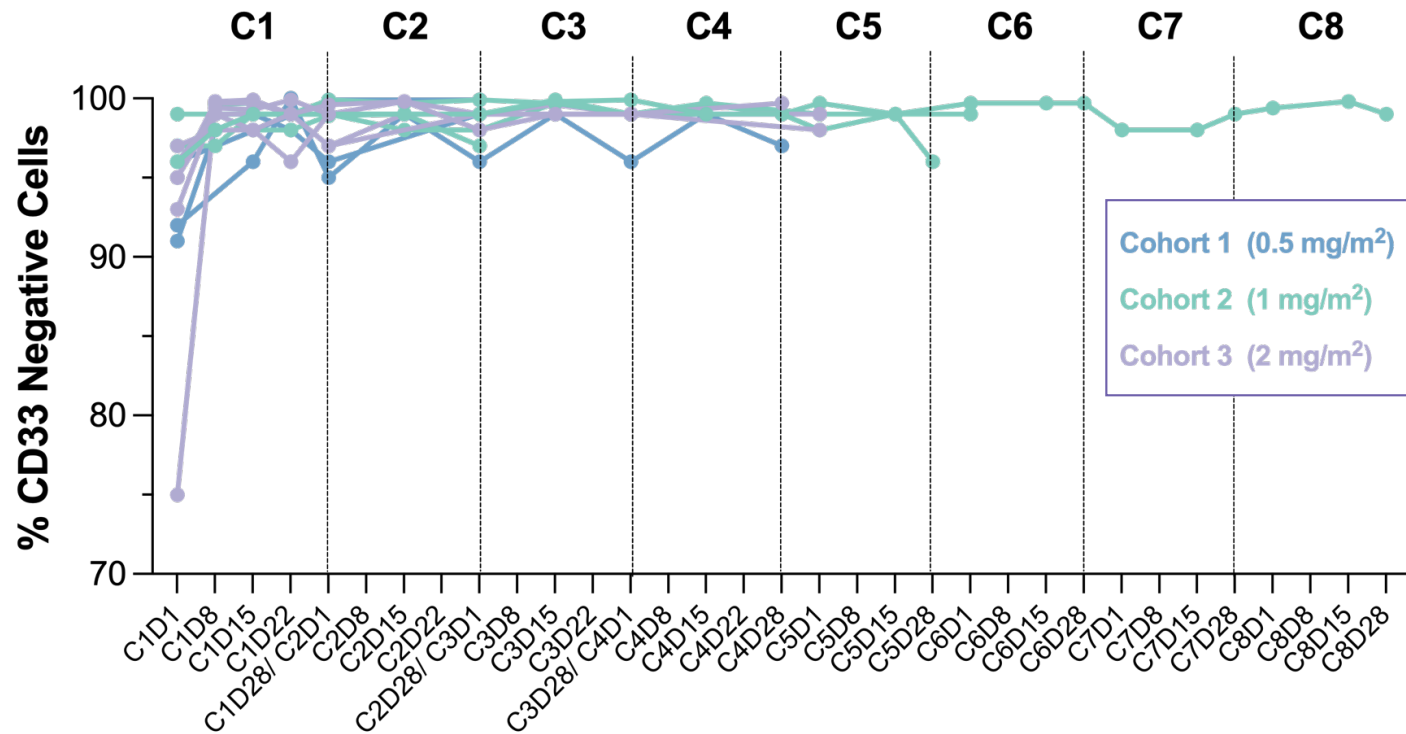


Q&A



CD33 Negative Cells Enriched with Mylotarg Doses

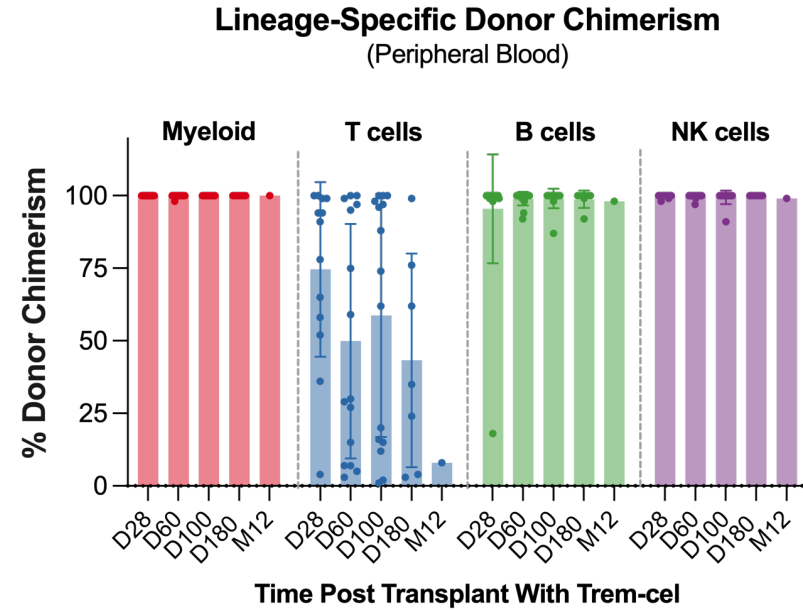
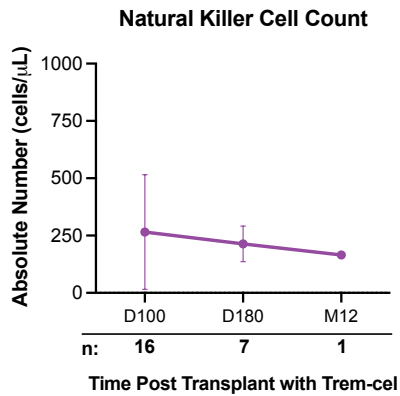
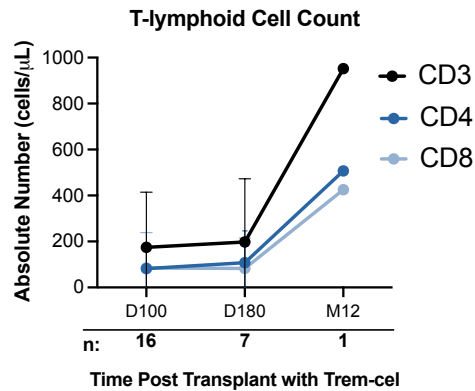
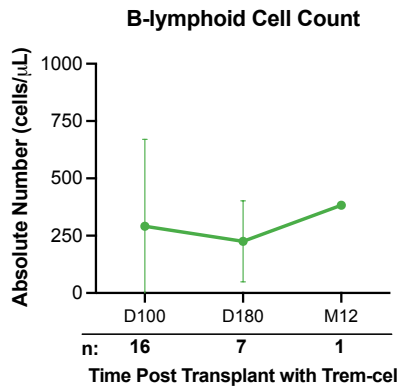
Loss of CD33 Expression on Myeloid Cells (Peripheral Blood, n=20)



Data cut-off: 1-NOV-2024



Immune Reconstitution, Full and Sustained Myeloid Chimerism, and CD33-negative Myeloid Cells Are Observed



% CD33-Negative Analysis	Drug Product*	D28*	D60*
NGS Gene Editing	90 (78-94) n=18	94 (85-98) n=19	94 (86-97) n=16
Flow Cytometry	N/E	93 (74-98) n=22	94 (78-99) n=19

*Mean % (range), Editing and flow data from peripheral blood monocytes and myeloid cells respectively
N/E: not evaluated

Data cut-off: 1-NOV-2024. Reference unedited CD34-selected reconstitution: Goldberg et al Leuk and Lymph 58 (217); Llauroador et al. Transplantation and Cellular Therapy 27 (2021)



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