UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
WASHINGTON, D.C. 20549

FORM 8-K

CURRENT REPORT
Pursuant to Section 13 or 15(d)
of the Securities Exchange Act of 1934

Date of Report (Date of earliest event reported): January 5, 2024

Vor Biopharma Inc.
(Exact name of registrant as specified in its Charter)

Delaware	**001-39979**	**81-1591163**
(State or Other Jurisdiction of Incorporation)	**(Commission File Number)**	**(IRS Employer Identification No.)**

100 Cambridgepark Drive	
Suite 101	
Cambridge, Massachusetts	**02140**
(Address of Principal Executive Offices)	**(Zip Code)**

Registrant's telephone number, including area code: (617) 655-6580

(Former Name or Former Address, if Changed Since Last Report)

Check the appropriate box below if the Form 8-K filing is intended to simultaneously satisfy the filing obligation of the registrant under any of the following provisions (see General Instructions A.2. below):

☐ Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)

☐ Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12)

☐ Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b))

☐ Pre-commencement communications pursuant to Rule 13e-4(c) under the Exchange Act (17 CFR 240.13e-4(c))

Securities registered pursuant to Section 12(b) of the Act:

Title of each class	Trading Symbol(s)	Name of each exchange on which registered
Common Stock, $0.0001 par value per share	VOR	Nasdaq Global Select Market

Indicate by check mark whether the registrant is an emerging growth company as defined in Rule 405 of the Securities Act of 1933 (§230.405 of this chapter) or Rule 12b-2 of the Securities Exchange Act of 1934 (§240.12b-2 of this chapter).

Emerging growth company ☒

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act. ☐

Item 8.01 Other Events

On January 5, 2024, Vor Biopharma Inc. (the "Company") updated its investor presentation (the "Corporate Presentation"). The Corporate Presentation is available on the Company's website and is filed as Exhibit 99.1 hereto and incorporated herein by reference.

Item 9.01 Financial Statements and Exhibits

Exhibit No.	Description
99.1	Corporate Presentation, dated January 5, 2024
104	Cover Page Interactive Data File (embedded within the Inline XBRL document)

SIGNATURES

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned hereunto duly authorized.

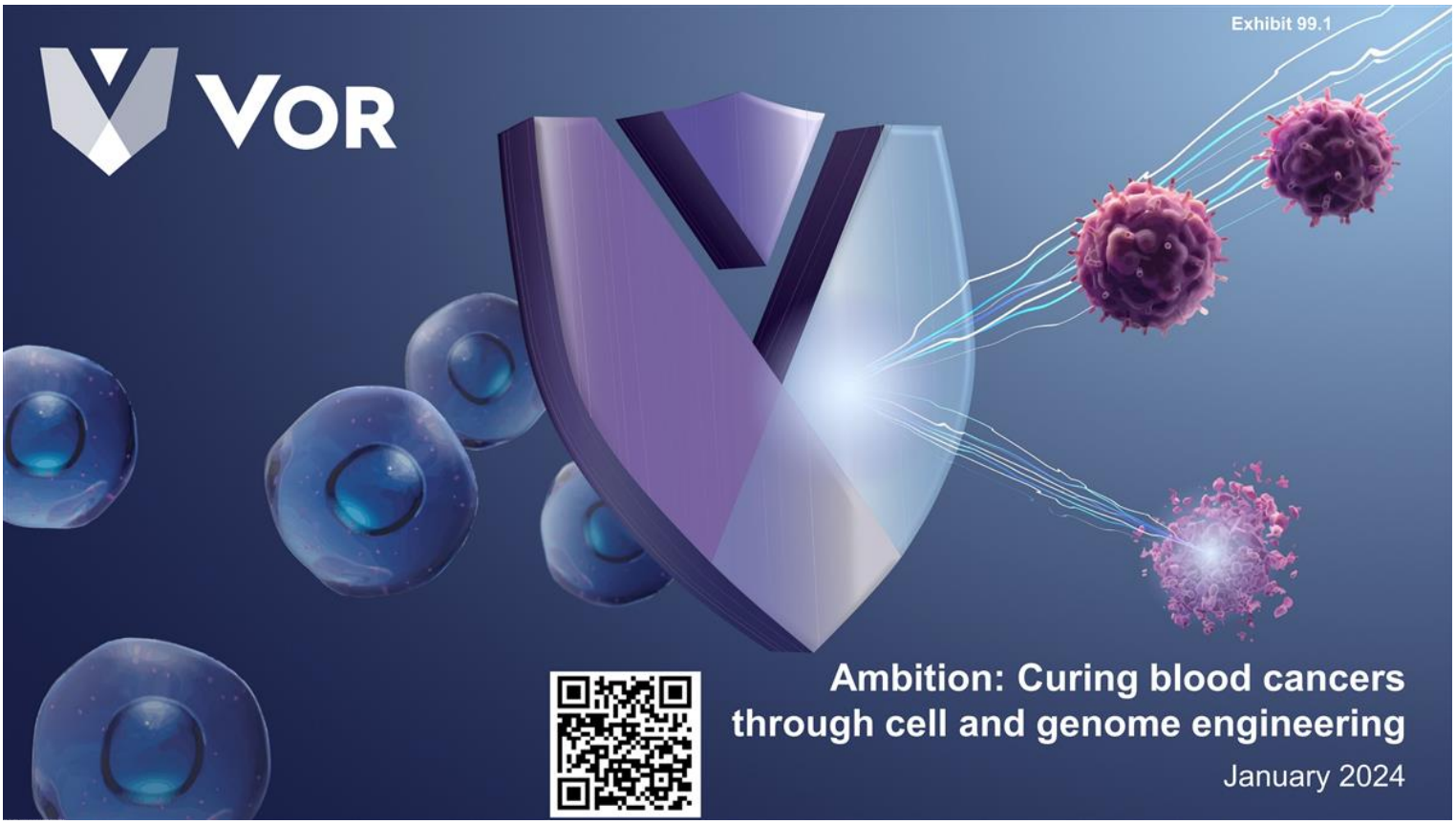
Vor Biopharma Inc.

By: /s/ Robert Ang
Robert Ang
Chief Executive Officer

Date: January 5, 2024

Exhibit 99.1

VOR



Ambition: Curing blood cancers through cell and genome engineering

January 2024

Disclaimer

This presentation (the "Presentation") contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995 about Vor Biopharma Inc. ("Vor," "Vor Bio" or the "Company"). The words "aim," "anticipate," "believe," "can," "could," "design," "enable" "estimate," "expect," "intend," "may," "ongoing," "plan," "potential," "project," "should," "target," "towards," "will," and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. Forward-looking statements in this Presentation include those regarding the feasibility of a trem-cel (formerly VOR33) transplant to be successfully manufactured, to engraft normally, to maintain blood counts following treatment with Mylotarg following allogeneic hematopoietic cell transplant and to be well tolerated, the potential of VCAR33^{ALLO} in combination with trem-cel as a Treatment System, the potential of trem-cel to enable targeted therapies in the post-transplant setting including Mylotarg and CD33-targeted CAR-Ts, the potential of Vor Bio's platform, Vor Bio's plans, strategies, expectations and anticipated milestones for its preclinical and clinical programs, the availability and timing of results from preclinical studies and clinical trials, the timing of regulatory filings, the expected safety profile of Vor Bio's product candidates, cash runway and expected capital requirements, and its plans and expectations related to the Company's manufacturing and facilities. Vor Bio may not actually achieve the plans, intentions, or expectations disclosed in these forward-looking statements, and you should not place undue reliance on these forward-looking statements. Actual results or events could differ materially from the plans, intentions and expectations disclosed in these forward-looking statements as a result of various factors, including: uncertainties inherent in the initiation, completion of, and availability and timing of results from, preclinical studies and clinical trials and clinical development of Vor Bio's product candidates; whether preclinical data or interim results from a clinical trial will be predictive of the final results of the trial or the results of future trials; the uncertainty of regulatory approvals to conduct trials or to market products; the success of Vor Bio's in-house manufacturing capabilities and efforts; and availability of funding sufficient for its foreseeable and unforeseeable operating expenses and capital expenditure requirements. The interim data for trem-cel presented in this Presentation is based on five patients and future results for these patients or additional patients may not produce the same or consistent results. These and other risks are described in greater detail under the caption "Risk Factors" included in Vor Bio's most recent annual or quarterly report and in other reports it has filed or may file with the Securities and Exchange Commission. Any forward-looking statements contained in this Presentation speak only as of the date of this Presentation, and Vor Bio expressly disclaims any obligation to update any forward-looking statements, whether because of new information, future events or otherwise, except as may be required by law.

Certain information contained in this Presentation relates to or is based on studies, publications, surveys and other data obtained from third party sources and Vor Bio's own internal estimates and research. While we believe these third-party sources to be reliable as of the date of this Presentation, we have not independently verified, and make no representation as to the adequacy, fairness, accuracy or completeness of any information obtained from third party sources. In addition, there can be no guarantee as to the accuracy or reliability of any assumptions or limitations that may be included in such third-party information. While we believe our own internal research is reliable, such research has not been verified by any independent source.

Our Vision: Cure Blood Cancers Through Cell and Genome Engineering

Unique approach

shielded stem cell transplants enabling targeted therapy

Positive clinical proof of concept

demonstrated in AML with CD33-deleted trem-cel* transplants

VCAR33^{ALLO}

Fully owned CD33-directed transplant donor CAR-T

Multiple sites activated

In-house GMP manufacturing facility

Four modular clean rooms for clinical supply

$160M

as of Sept. 30, 2023

Cash runway into 2H 2025

*tremtelectogene empogeditemcel, formerly VOR33
$160M in cash, cash equivalents and marketable securities as of September 30, 2023.

Current AML Disease State and Standard of Care

Standard of Care: Replace Diseased Bone Marrow with Transplanted Healthy Donor Cells

1 Conditioning

Toxic therapies to kill existing bone marrow

2 Harvest

Mobilize and collect stem cells from matched healthy donor

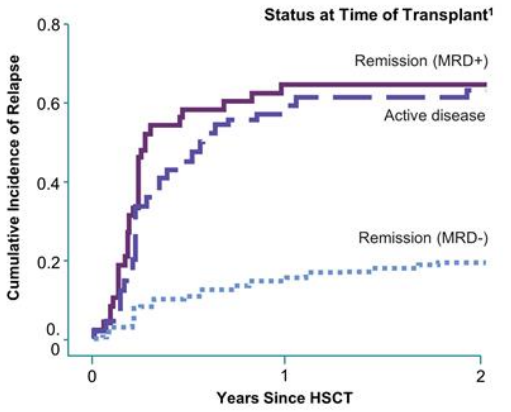
3 Transplant

Infuse stem cells into patient to restore the blood system

4 Watchful Waiting

Monitor for relapse; any follow-up treatment will damage the transplant

Despite transplantation, relapse is still common in AML patients



MRD: measurable residual disease; SOC: standard of care; HSCT: Hematopoietic Stem Cell Transplant
1 Araki et al, JCO 2016

Clear Unmet Need in AML

~20,000

People in the U.S. diagnosed with AML annually[1]

~4,000

AML Transplants per year (U.S.)[2]

50%

Increase in # of AML transplants over the last 10 years[2]

~40%

Post-transplant relapse, with <20% two-year survival[3,4]

1 American Cancer Society 2023
2 Current use and outcome of hematopoietic stem cell transplantation: CIBMTR summary slides 2022
3 Araki et al, JCO 2016
4 Schmid et al, Blood 2012

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: Shielded Transplants

Shielded transplants allowing therapies to be cancer-specific

The Vision: eHSC + CAR-T Treatment Systems

Trem-cel	Trem-cel	Multiplex-edited HSCs
Shielded CD33-deleted HSC transplant ⊕	Shielded CD33-deleted HSC transplant ⊕	Next-gen shielded HSC transplant ⊕
Mylotarg	**VCAR33**	**Multi-specific CAR-Ts**
CD33-directed antibody-drug conjugate	CD33-directed transplant donor CAR-T therapy	Multi-targeted CAR-T therapy
Clinical proof of concept • Engraftment • Heme protection	**VCAR33^{ALLO}** • Healthy donor source, stemlike phenotype • Tolerized to new marrow **VCAR33^{AUTO}** • Phase 1/2 NMDP-sponsored trial	**Addresses tumor heterogeneity and potential escape mechanisms**

Expanding Pipeline Driven by Innovative Platform

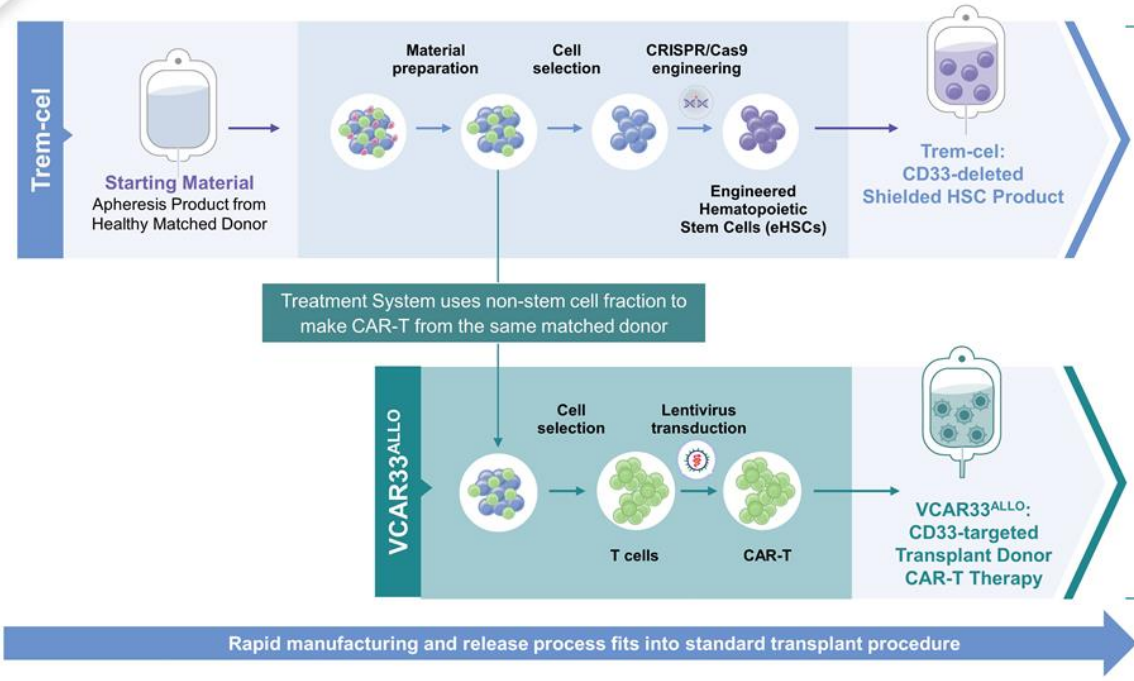
Description			Preclinical		Clinical	
Program / Trial	Modality	Indication	Discovery/ Validation	IND-Enabling	Phase 1/2	Phase 2/3
Trem-cel + Mylotarg / VBP101	Shielded CD33-deleted transplant + CD33-directed ADC	AML				
		MDS, MPN				
VCAR33^{ALLO} (allogeneic) / VBP301	CD33-directed transplant donor CAR-T	AML Post-transplant				
VCAR33^{AUTO} (autologous)	CD33-directed autologous CAR-T	AML Bridge-to-transplant	NMDP-sponsored trial*			
Trem-cel + VCAR33 Treatment System	Shielded CD33-deleted transplant + CD33-directed transplant donor CAR-T	AML				
CD33-CLL1 Treatment System	Multiplex-edited HSCs + Multi-specific CAR-T	AML				

Discovery Platform

- Leveraging our proprietary Vor Bio platform, we are exploring additional surface targets such as CD123, EMR2, and CD5 including multiplex genome engineering approaches where multiple surface targets are removed.
- We are conducting ongoing discovery efforts in commonly transplanted hematologic malignancies.

AML: acute myeloid leukemia; HSCs: hematopoietic stem cells; MDS: myelodysplastic syndrome; MPN: myeloproliferative neoplasm
** The VCAR33 construct is being studied in a Phase 1/2 clinical trial sponsored by the National Marrow Donor Program ("NMDP"), and the timing of data release is dependent on the investigators conducting the trial.*

Proprietary Dual Cell Product Potential



Unique In-house Manufacturing:

Multi-product GMP facility in Cambridge, MA

Four independent clean rooms and on-site QA/QC

Trem-cel + Mylotarg

Trem-cel

Shielded CD33-deleted HSC transplant

⊕

Mylotarg

CD33-directed antibody-drug conjugate

Clinical proof of concept

- Engraftment
- Heme protection

Trem-cel

Shielded CD33-deleted HSC transplant

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VCAR33

CD33-directed Transplant Donor CAR-T therapy

VCAR33^{ALLO}

- Healthy donor source, stemlike phenotype
- Tolerized to new marrow

VCAR33^{AUTO}

- Phase 1/2 NMDP-sponsored trial

Multiplex-edited HSCs

Next-gen shielded HSC transplant

⊕

Multi-specific CAR-Ts

Multi-targeted CAR-T therapy

Addresses tumor heterogeneity and potential escape mechanisms

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

Patient Journey

Transplant-eligible AML patients at high risk of relapse

Transplant Decision

Consolidation/Salvage

Conditioning

No delay in typical patient transplant process

Infusion

Engraftment

Maintenance Mylotarg

Starting ~day 60 up to 4 cycles dose escalation 0.5-2 mg/m^2

If relapse occurs:

- Induction-course Mylotarg
- VCAR33^{ALLO} (VBP301)
- Alternative treatments

Donor Journey

- Screening
  - Related
  - Unrelated
- Stem Cell Mobilization, Collection
- Trem-cel Manufacturing ~7-day process

Unedited back-up graft

Key Endpoints

01 **Trem-cel engraftment**

02 **Heme protection from Mylotarg**

03 **Relapse-free survival**

VOR

Patient Characteristics

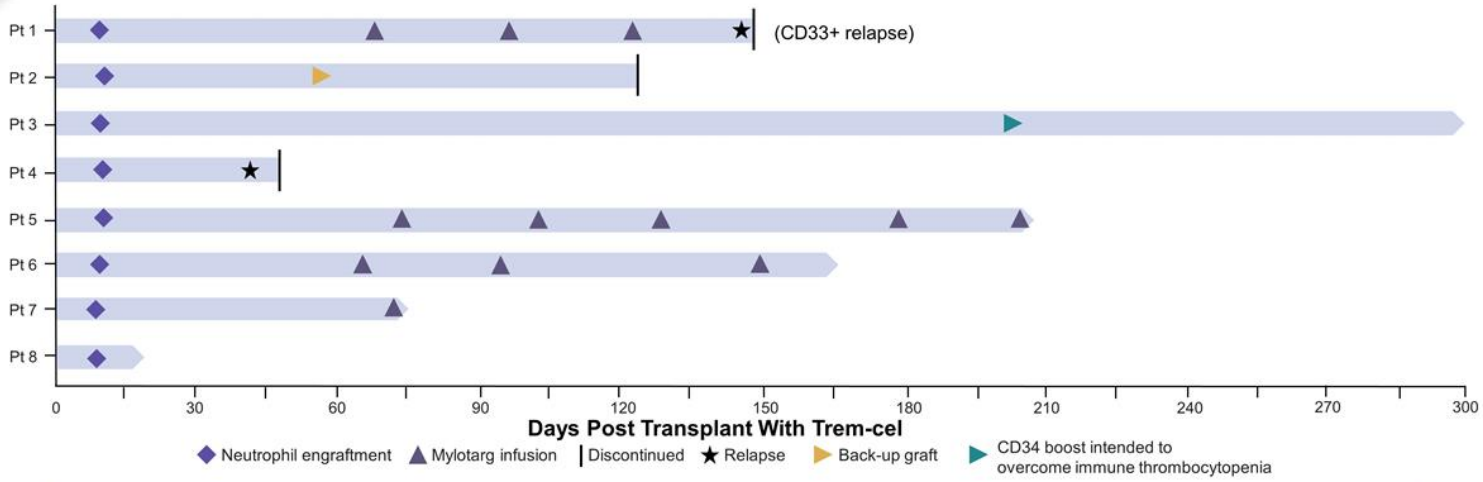
Pt	Age/ Sex	Disease and Genetics	Weight	Donor, Dose, *CD33* gene-editing efficiency
1	64/F	AML-MRC Highly complex cytogenetics; CR2; TP53 mutation MRD: 1.8%	69.9 kg	10/10 HLA MUD 7.6 × 10^6 CD34 cells/kg, 88% *CD33* gene editing
2	32/M	AML after myeloid sarcoma resected from abdomen Inv 16 and +22, t(3;3)	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
6	63/F	AML-MRC Highly complex cytogenetics; TP53, NRAS, WT1 mutations	66.2 kg	10/10 HLA MUD 5.7 × 10^6 CD34 cells/kg, 91% *CD33* gene editing
7	67/M	AML with recurrent abnormalities CR2; NPM1, TET2, EZH2, SETBP1, PIGA mutations	72.8 kg	10/10 HLA MUD 9.4 × 10^6 CD34 cells/kg, 87% *CD33* gene editing
8	57/M	AML (myelomonocytic) with nml karyotype CR2 (CRi/CRp)	68.9kg	10/10 HLA MUD 9.5 × 10^6 CD34 cells/kg, 91% *CD33* gene editing

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

All patients received myeloablative conditioning with busulfan/melphalan/fludarabine/rabbit anti-thymocyte globulin (ATG), with exception for patient #3, who received equine ATG.

 Data Cutoff: 4 Dec 2023. Presented data from EDC and site/PI communication; pending full source verification

Patient Clinical Timelines (Patients 1-8)



Patients Ineligible for Mylotarg:

Patient 2

Secondary graft failure in context of prior sepsis, TMP-SMZ/possible DRESS and persistent hKU1 coronavirus infection. Graft failure resolved after back-up graft given.

Patient 3

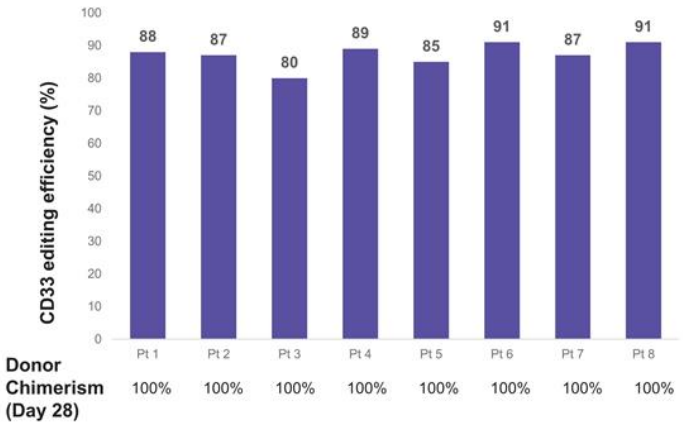
Immune thrombocytopenia. Resolving after treatment with IVIg, steroids, rituximab, CD34 boost.

Patient 4

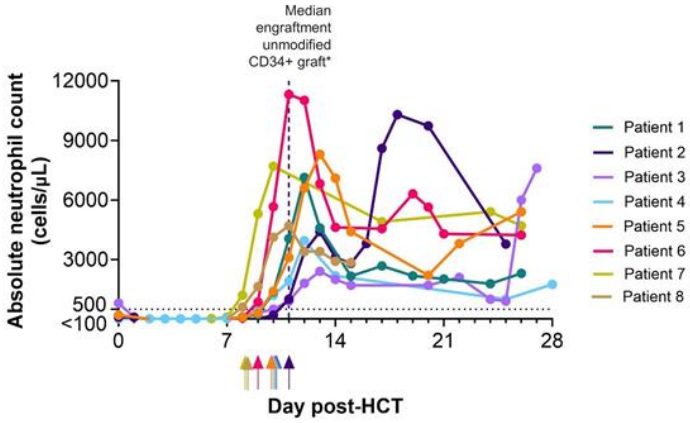
CNS and systemic relapse prior to Mylotarg dosing.

Proof of Concept: Successful Engraftment of CD33-Deleted HSCs

Highly Efficient Removal of CD33 from Donor HSCs



Timely Post-transplant Neutrophil Engraftment

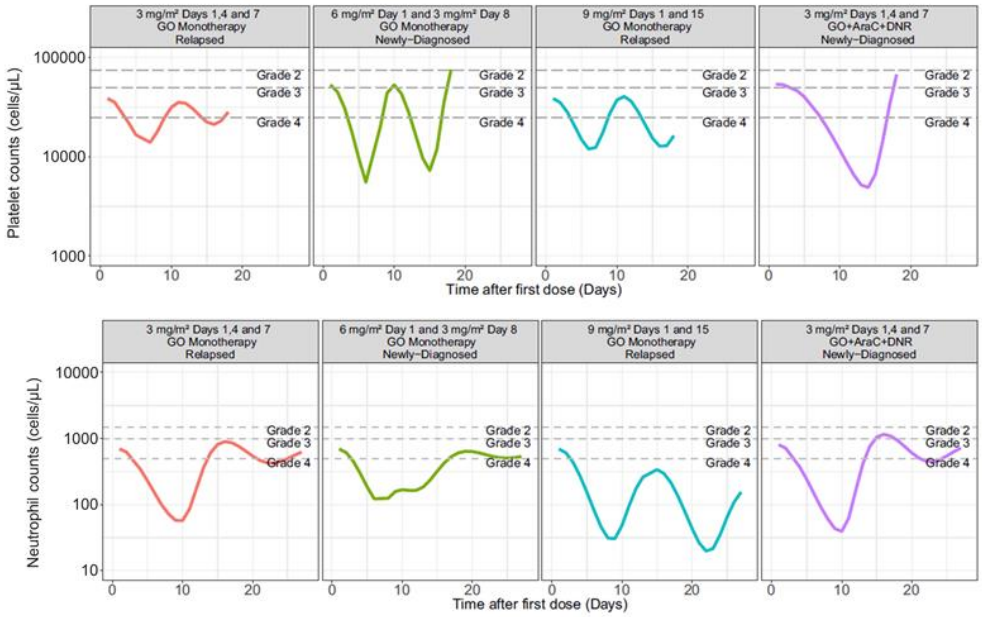


Arrows indicated day of individual patient neutrophil engraftment

Neutrophil engraftment = 3 days ≥ 500 cells/µL

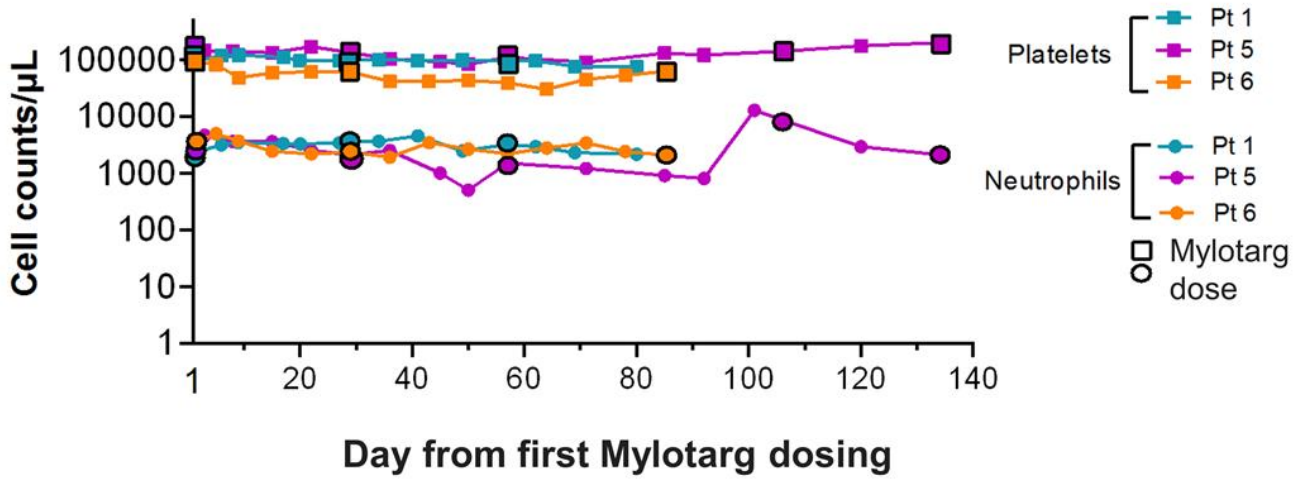
*Luznik L. et al. J Clin Oncol 2022;40(4):356–368

Mylotarg Causes Deep Cytopenias Across Various Regimens



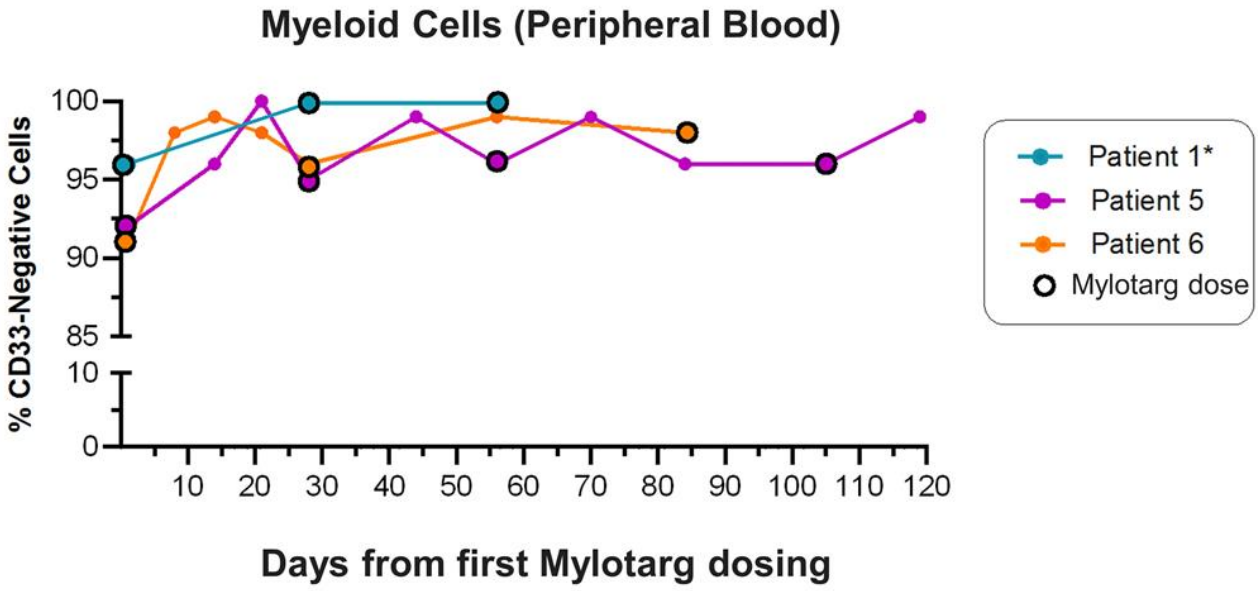
Fostvedt et al. Clin Pharm Thera 2019;106(5):1006-1017

Evidence of Protective Effect from Mylotarg at 0.5 mg/m^2



Mylotarg C1 Start: Pt 1 D+68; Pt 5 D+74; Pt 6 D+66 post-HCT

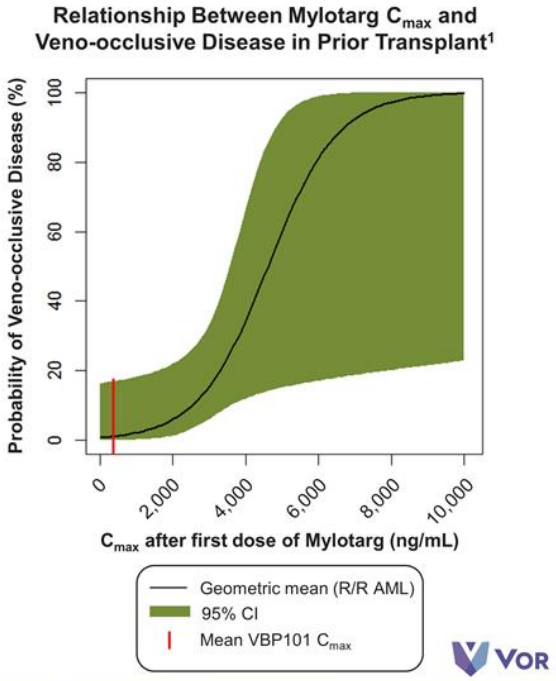
Enrichment of CD33-negative Cells following Mylotarg



Patients 1, 5, 6: PK after 1st Dose of Maintenance Mylotarg

Pharmacokinetics

	VBP101	Relapsed/Refractory AML Population (Mylotarg Phase 1 Study 0903A1-101-US)[1]					
Parameter	Mean +/- SD 0.5 mg/m²	0.25 mg/m²	0.5 mg/m²	1 mg/m²	2 mg/m²	4 mg/m²	5 mg/m²
C_{max} (ng/mL)	236 (+/- 151)	15	28	50	411	611	1,325
AUC_{inf} (Hr*ng/mL)	10,890 (+/- 13958)	82	468	943	11,110	10,970	29,980



[1]Mylotarg ODAC 2017

VCAR33

Trem-cel

Shielded CD33-deleted HSC transplant

⊕

Mylotarg

CD33-directed antibody-drug conjugate

Clinical proof of concept

- Engraftment
- Heme protection

Trem-cel

Shielded CD33-deleted HSC transplant

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VCAR33

CD33-directed Transplant Donor CAR-T therapy

VCAR33^{ALLO}

- Healthy donor source, stemlike phenotype
- Tolerized to new marrow

VCAR33^{AUTO}

- Phase 1/2 NMDP-sponsored trial

Multiplex-edited HSCs

Next-gen shielded HSC transplant

⊕

Multi-specific CAR-Ts

Multi-targeted CAR-T therapy

Addresses tumor heterogeneity and potential escape mechanisms

A New Way of Generating CAR-T Therapy

Traditional Approaches

Autologous cells
(derived from patient)

Exhausted, depleted T cells

High manufacturing failure

Allogeneic cells
(off-the-shelf)

Poor expansion and persistence

Poorer clinical durability

Vor Bio Approach

Transplant Donor Cells

VOR

T cells exactly matched to patient's new immune system, more likely to persist

Stem-like, CAR-T cells more likely to expand and less prone to exhaustion

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

Patient Journey

MRD+ or relapsed AML following SOC transplant or trem-cel transplant

Enroll

Lymphodepletion

VCAR33^{ALLO} Infusion

Arm A: Blasts ≥ 5%

Arm B: MRD+

Day 28 Follow-up

2nd transplant if required

3x3 dose escalation starting at 1 x 10^6 CAR+ cells/kg

Prior Transplant Donor

Consent

Collect

VCAR33^{ALLO} Manufacturing

~7-day process

Key Endpoints

01 Safety

02 Expansion, persistence

03 Disease control/response

*Multiple active clinical sites that overlap with VBP101 trem-cel Phase 1/2a clinical study

VOR

VCAR33^{AUTO} Shows Signs of Activity; VCAR33^{ALLO} Potentially More Active

VCAR33^{AUTO} (NCI CD33CART)

- Autologous starting material
- 6-site IST
- Young adults and children (median 16 y, range 1-35)
- Academic manufacturing process
- Accepted for oral presentation at ASH
  - N=24 enrolled, 19 infused
  - Manageable tox (n=4 with CRS ≥ Grade 3)

Dose (CAR^+ cells/kg)	Total	3×10^5	1×10^6	3×10^6	1×10^7
# infused	19	3	3	7	6 (resp assess in 5)
# with CR, (%)	2 (11%)	0 (0%)	0 (0%)	0 (0%)	2 (40%)

Data from ASH 2023 Abstract: https://ash.confex.com/ash/2023/webprogram/Paper179667.html

VCAR33^{ALLO}

- Transplant donor starting material
- IND cleared in June, multiple sites opened
- Targeting ~12 sites
- Streamlined manufacturing process with objective of stem like cell phenotype
- Allows trem-cel patients to enroll
- Starting dose 1×10^6 CAR^+ cells/kg

Moving Beyond Proof of Concept to Pivotal

Targeting Short Registrational Pathway

Fast Track granted on basis of trem-cel heme protection

- Exploring heme protection endpoints with agency

High unmet need in AML

- Precedence for single-arm pivotal trials
- CR and CR/CRh are approvable endpoints

R/R AML Single Arm Pivotal Trials

Agent	Indication	# pts	Endpoint
Ivosidenib *IDH1, Agios*	R/R AML	174	CR 25% CRh 8%[1]
Enasidenib *IDH2, Agios*	R/R AML	199	CR 19% CRh 4%[2]
Gilteritinib *FLT3, Astellas*	R/R AML	138	CR 12% CRh 9%[3]
Revumenib *KMT2Ar, Syndax*	R/R AML	57	CR 18% CRh 5%[4]
Mylotarg *ADC, Pfizer*	R/R AML	57	CR 26%[5]

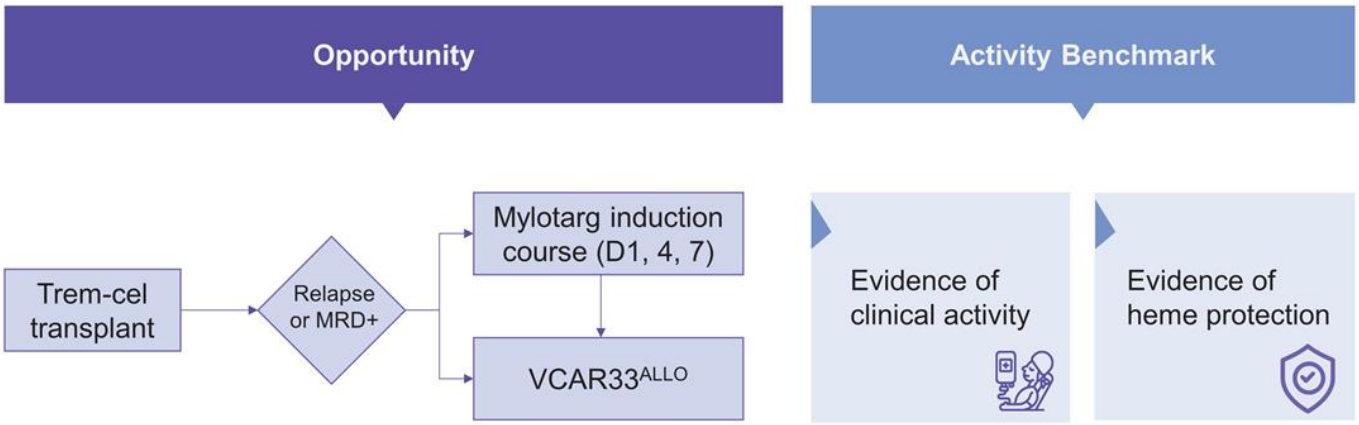
CR: Complete Remission CRh: Complete remission with partial hematologic recovery

1. Norsworthy KJ, et. al. FDA Approval Summary: Ivosidenib for Relapsed or Refractory Acute Myeloid Leukemia with an Isocitrate Dehydrogenase-1 Mutation. Clin Cancer Res. 2019 Jun 1;25(11):3205-3209.
2. https://www.fda.gov/drugs/resources-information-approved-drugs/fda-granted-regular-approval-enasidenib-treatment-relapsed-or-refractory-aml
3. Pulte ED, et. al. FDA Approval Summary: Gilteritinib for Relapsed or Refractory Acute Myeloid Leukemia with a *FLT3* Mutation. Clin Cancer Res. 2021 Jul 1;27(13):3515-3521.
4. https://cms.syndax.com/wp-content/uploads/2023/12/Aldoss-2023-AUGMENT-101-3.pdf. Per company, NDA initiated with FDA under RTOR program.
5. https://labeling.pfizer.com/showlabeling.aspx?id=9548

Trem-cel and VCAR33: Defining Success in 2024

Based on discussions with KOLs and our clinical investigators, even a single patient with clinical activity and heme protection may provide validation of the approach

Expect clinical validation in 2024



Significant Clinical Progress and Upcoming Milestones

	Progress to Date	Upcoming Milestones
Trem-cel	Trem-cel can be reliably manufactured with efficient CD33 deletion (87% average) CD33 appears biologically dispensable in regard to engraftment (8/8) Trem-cel provides hematologic protection from acute Mylotarg toxicity (3/3)	Mylotarg dosing escalated to next dose level of 1.0 mg/m^2 Multiple therapeutic options enabled for patients who relapse following trem-cel transplant: • Induction-course Mylotarg • VCAR33^{ALLO}
VCAR33	Potentially superior transplant donor cell source IND cleared; multiple sites active Trem-cel patients are eligible to enroll VCAR33^{AUTO} (CD33CART) showed activity at highest dose level and manageable safety in NCI study[1]	Preliminary VCAR33^{ALLO} safety and efficacy data

[1]ASH 2023 Abstract: https://ash.confex.com/ash/2023/webprogram/Paper179667.htm

Next-generation Approaches

Trem-cel

Shielded CD33-deleted HSC transplant

⊕

Mylotarg

CD33-directed antibody-drug conjugate

Clinical proof of concept

- Engraftment
- Heme protection

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Multiplex-edited HSCs

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⊕

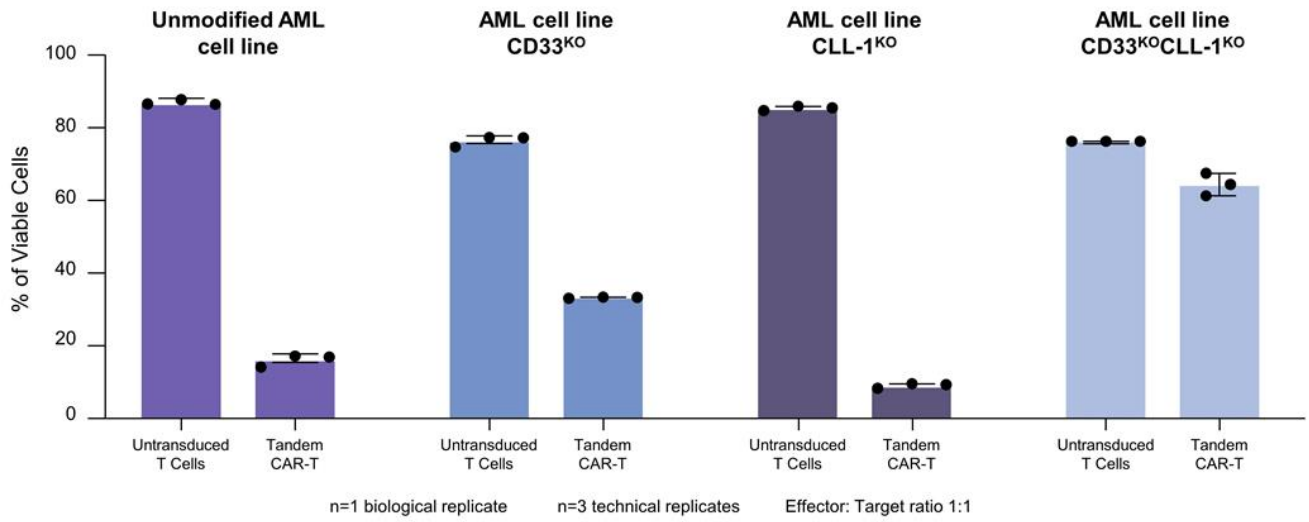
Multi-specific CAR-Ts

Multi-targeted CAR-T therapy

Addresses tumor heterogeneity and potential escape mechanisms

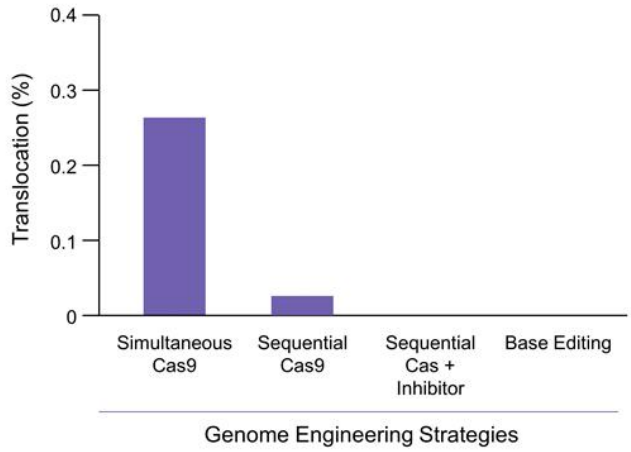
In Vitro Proof of Concept for Multi KO Target Cell + Multi-Specific CAR-T

CD33 and CLL-1 Dual-CAR-T Active Against Wild Type and Single Knock-out Target Cells

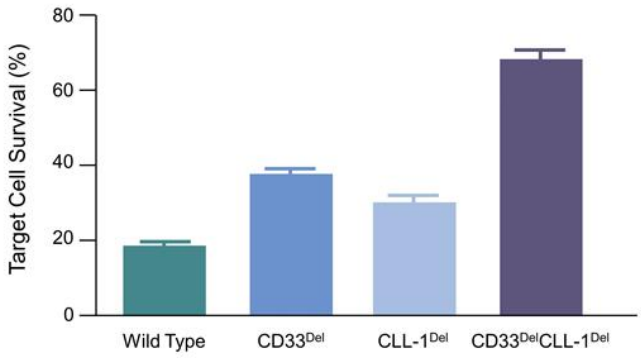


Multiplex Editing: Proprietary Capabilities Minimize Translocations and Protects from CAR-T

Minimized Translocation Rate



Cell Protection from CAR-T Killing



Effector : Target = 1:1

Our Vision: Cure Blood Cancers Through Cell and Genome Engineering

Unique approach

shielded stem cell transplants enabling targeted therapy

Positive clinical proof of concept

demonstrated in AML with CD33-deleted trem-cel* transplants

VCAR33^{ALLO}

Fully owned CD33-directed transplant donor CAR-T

Multiple sites activated

In-house GMP manufacturing facility

Four modular clean rooms for clinical supply

$160M

as of Sept. 30, 2023

Cash runway into 2H 2025

*tremtelectogene empogeditemcel, formerly VOR33
$160M in cash, cash equivalents and marketable securities as of September 30, 2023.

www.vorbio.com

Experienced and Passionate Leadership Team

Robert Ang, MBBS, MBA
President and CEO

Eyal Attar, MD
Chief Medical Officer

Tirtha Chakraborty, PhD
Chief Scientific Officer

Nathan Jorgensen, PhD MBA
Chief Financial Officer

Tania Philipp
Chief People Officer

Robert Pietrusko, PharmD
Chief Regulatory & Quality Officer

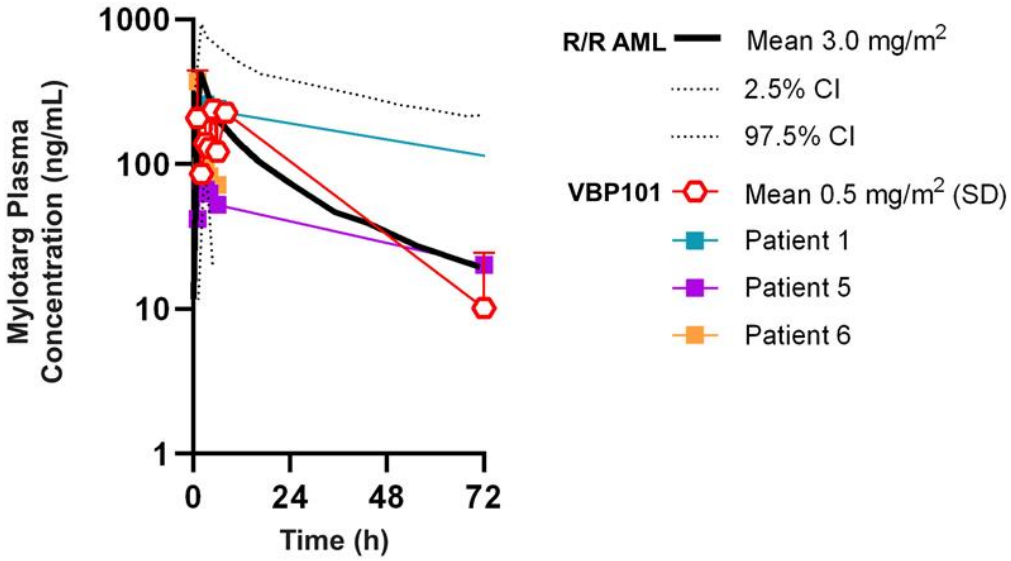
John King, MBA
Chief Commercial Officer & Head of Business Development

David Phillips, MBA
Senior Vice President, Head of Quality

Samir Vattompadam, MS
Senior Vice President, Portfolio Strategy and Program Management

Deep Cell & Gene Therapy Expertise

Mylotarg at 0.5 mg/m^2 Equivalent to ~3 mg/m^2 in the Context of CD33-negative Hematopoiesis



Data from 3.0 mg/m^2 mean (+/- ci) data digitized from simulations presented in Hibma et al, 2019

Safety Events Reported as Possibly Related to Either Trem-cel or Mylotarg (AE ≥ Grade 3 or any Grade SAE)

Adverse Event	Max Grade	Related to Trem-cel (# of events)	Related to Mylotarg (# of events)	SAE (# of events)
Anemia	3	1	—	—
Neutropenia	3	1	—	—
Thrombocytopenia	3	2	—	—
Graft Failure	4	1	—	1
Platelet count decreased	3	—	1	—
Platelet count decreased, worsening	3	1	1	—
Worsening maculopapular rash of whole body	2	1	—	1

For Mylotarg dosing:

- No dose-limiting toxicity criteria met
- No increase in liver function tests above upper limit of normal
- No observed sinusoidal obstruction syndrome / veno-occlusive disease

Data as of Oct. 31, 2023, presented at HSCT[2]

Potential Reimbursement Pathways

Shielded Stem Cell Transplants

MS-DRG 014 applies to
allogeneic bone marrow transplants

Section 108 (2020) provides new cost-based reimbursement for 100% of stem cell acquisition and processing

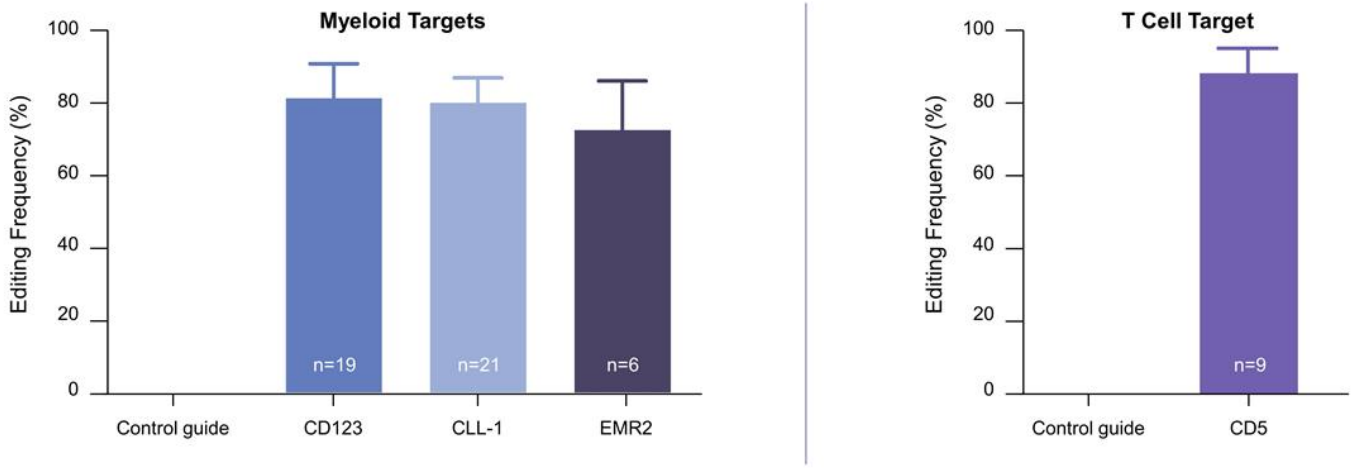
Omisirge® (recently FDA-approved HSC product) priced at $338,000

CAR-T Therapies

As of 2020, new MS-DRG 018 created for CAR-T therapy with base reimbursement rate of $247,939

High Editing Frequency for Next-Generation Targets

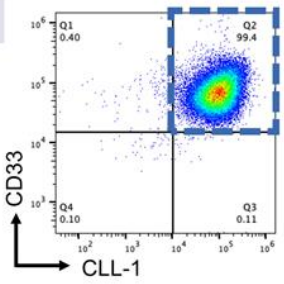
CD34+ Editing Frequency



Multiplex Editing Strategies Achieve Highly Efficient Double Knock-out

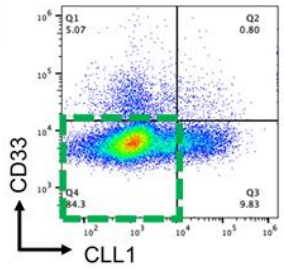
Sequential Cas9 Editing

Mock



$CD33^{+}CLL\text{-}1^{+}$
Double Pos
99.4%

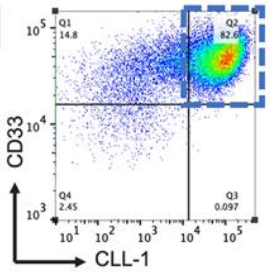
Edited



$CD33^{-}CLL\text{-}1^{-}$
Double KO
84.3%

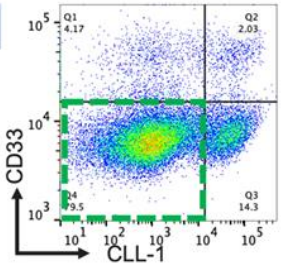
Base Editing

Mock



$CD33^{+}CLL\text{-}1^{+}$
Double Pos
82.6%

Edited



$CD33^{-}CLL\text{-}1^{-}$
Double KO
79.5%

VOR