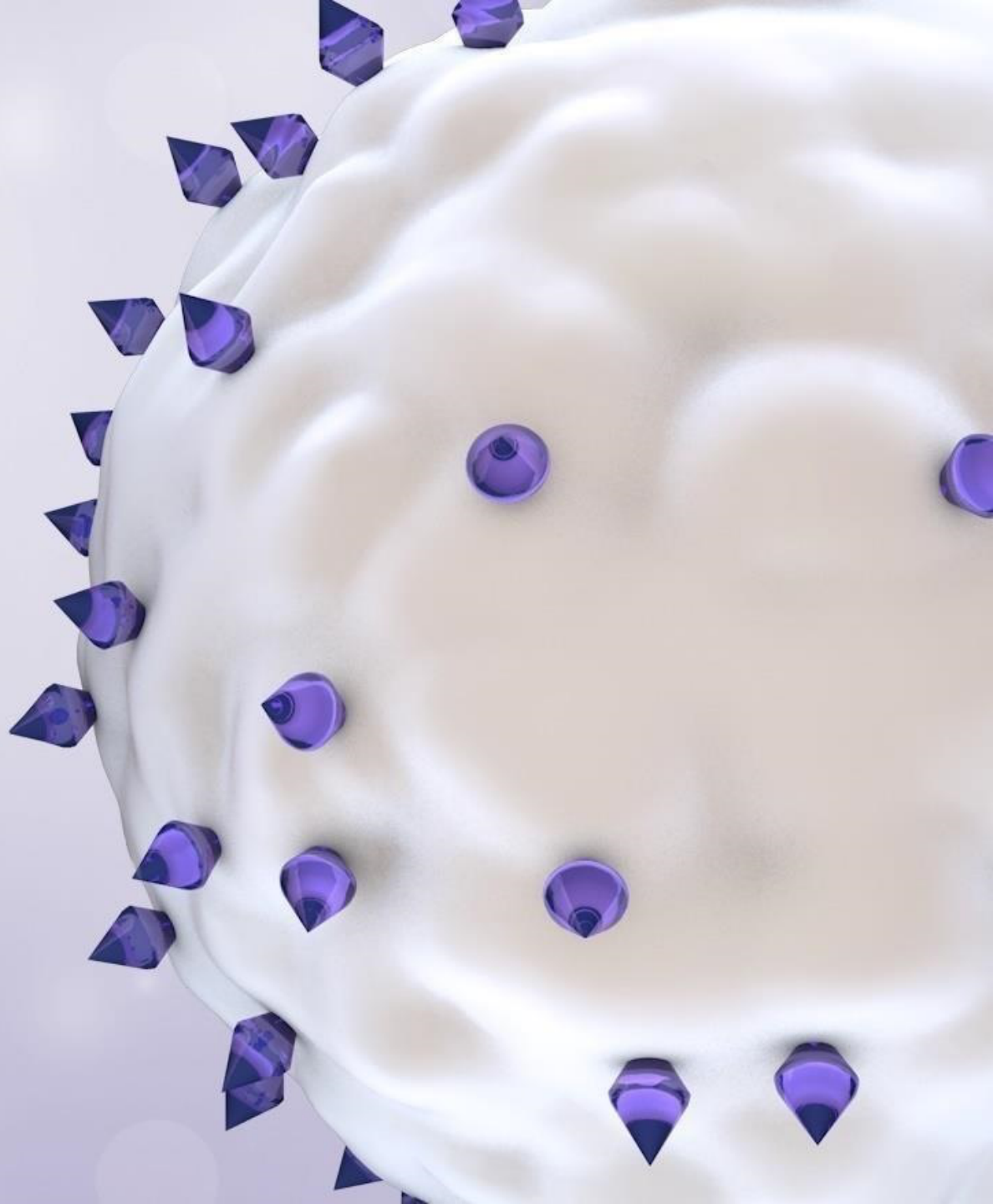
VOR

Change the normal | Expose the cancer

Corporate Presentation

December 2020



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Vor Bio: Engineering the Patient to Better Fight Their Cancers

Changing the Paradigm of Cancer Thinking

- Clinical-stage cell therapy company with fundamentally different approach to cancer targets
  - ✓ Proprietary engineered hematopoietic stem cell transplant (eHSC) platform
  - ✓ Unlocking potential of targeted therapies with curative intent
  - ✓ VOR33 eHSC completed IND-enabling studies; CD33 CAR-T in Phase 1/2
  - ✓ Single company solution combining our eHSC and CAR-T into Treatment System
- Platform with broad potential across hematological malignancies
- Experienced and proven management team
- Recent $110M Series B financing from blue-chip investors

Vor Senior Team Members

Robert Ang, MBBS, MBA
President and CEO

- CBO, Neon Therapeutics
- SVP, BD Bavarian Nordic
- VP Med Affairs & BD, Cadence Pharmaceuticals
- Frazier Healthcare Ventures, BCG

Tirtha Chakraborty, PhD
Chief Scientific Officer

- VP Cell Therapy Research, Sana Biotechnology
- Head of Hematology and Program Lead, CRISPR Therapeutics
- Group Leader mRNA Sciences, Moderna Therapeutics

Nathan Jorgensen, PhD
Chief Financial Officer

- Healthcare Portfolio Sr Manager, Qatar Investment Authority (QIA)
- Snr Research Analyst, Calamos Investments
- Research Analyst, Stifel Nicolaus
- Scientist, Columbia University

Sadik Kassim, PhD
Chief Technology Officer

- Exec Director Process Design, Kite
- CSO, Mustang Bio
- Head of Analytical Development, Novartis CAR-T
- Process development, NCI CAR-T, TCR, TIL

John King
Chief Commercial Officer

- CCO, Ra Pharma
- VP U.S. Neurology Business Unit, Head of Global LAL-D Franchise, Head of Global Hematology Franchise, Alexion
- Product Director of Enbrel, Wyeth Pharmaceuticals

Amy Mendel, JD
Chief Legal Officer

- SVP IP, Ohana Biosciences
- SVP IP, Evelo Biosciences
- VP and Assoc GC, IP & Licensing, Ziopharm Oncology

Tania Philipp
VP, Head of People

- VP HR, Tango Therapeutics
- Exec Director HR, Bavarian Nordic
- Director HR, Mendel Biotechnology
- Assoc Director HR, Sunesis

Bob Pietrusko, PharmD
Chief Regulatory & Quality Officer

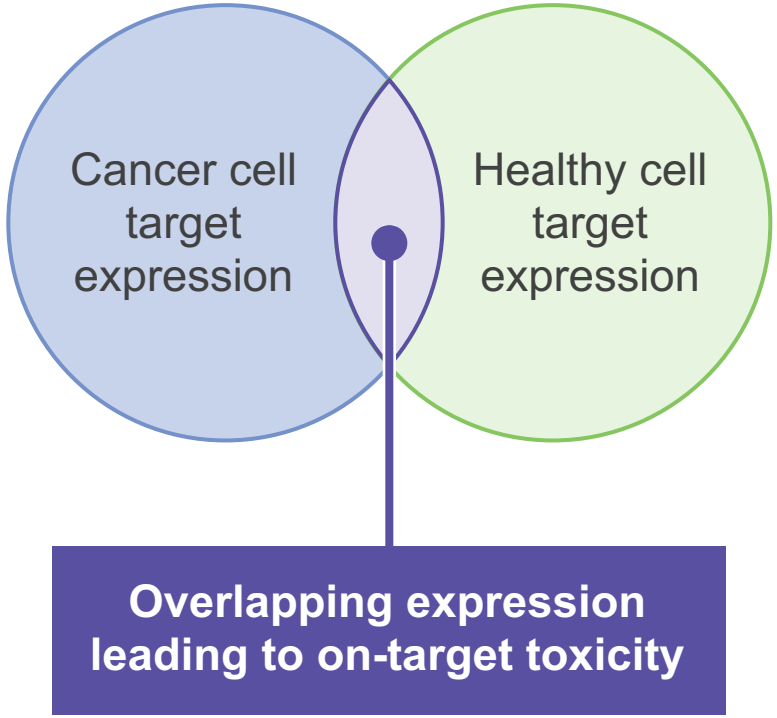
- SVP Reg & QA, Voyager
- VP Reg & Quality, ViroPharma
- SVP Reg, Millennium Pharmaceuticals
- VP Anti-infective and Anti-viral, SmithKline Beecham

Christopher Slapak, MD
Chief Medical Officer

- VP Early Phase Development Oncology, Eli Lilly and Co., SVP Imclone
- Ass't Professor, Dana-Farber Cancer Institute
- Assoc Clin Professor of Medicine and Pharmacology, Indiana Univ School of Medicine

Traditional Tumor Target Paradigm Limited by On-Target Toxicity

The Challenge of Targeted Therapies:
To identify and kill tumor cells while avoiding or minimizing damage to healthy cells

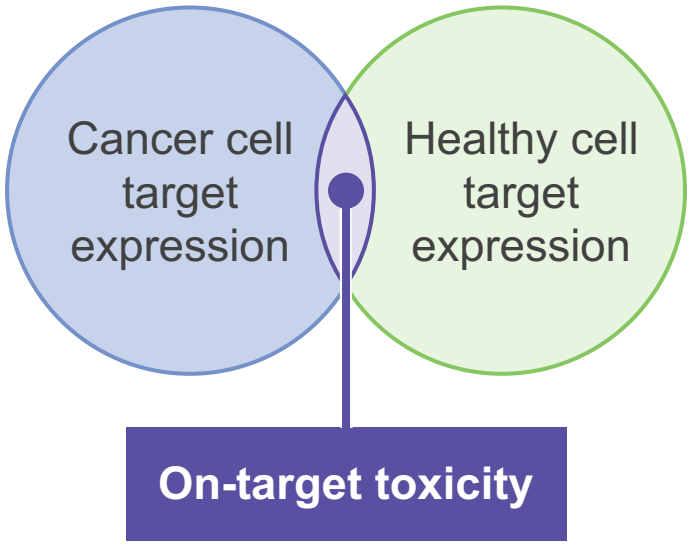


CD33-Targeted Therapies

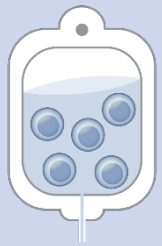
Company	Drug Name	Modality	Status
Pfizer	Mylotarg	Antibody-drug conjugate	Marketed On-target toxicity with neutropenia and thrombocytopenia
Seattle Genetics	SGN-CD33A	Antibody-drug conjugate	Failed in Phase 3 due to safety issues
Actinium	Actimab-A	Radio ligand	Single agent failed in Phase 2 due to safety issues
Immunogen	IMGN779	Antibody-drug conjugate	Discontinued in Phase 2
Amgen	AMG 330, AMG 673	Bispecific antibodies	In Phase 1 development
Janssen	JNJ-67571244	Bispecific antibody	In Phase 1 development

The Vor Platform: Changing the Tumor Target Paradigm

Traditional Paradigm



Vor Platform

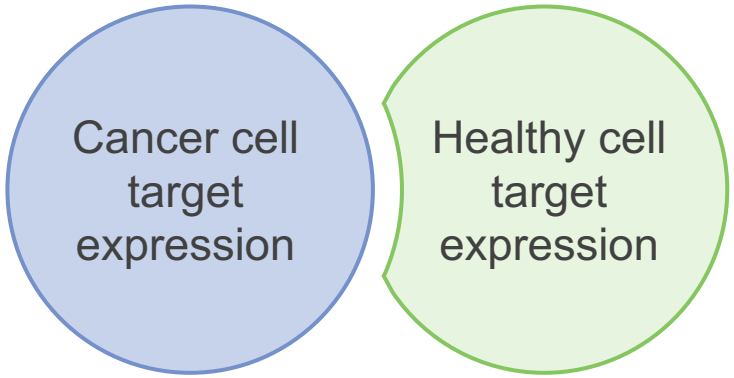


Hematopoietic Stem Cells

Genome Engineering

Targeted Therapies

Vor Paradigm: Engineered HSCs

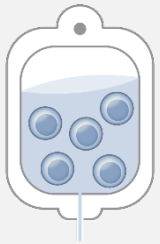


- Designed to unlock the true potential of targeted therapies
- Scalable platform potentially applicable across hematological malignancies and beyond

Engineering the Patient to Make Treatment-Resistant Transplant

Matched Healthy Donor

Current Standard of Care
following induction & myeloablation



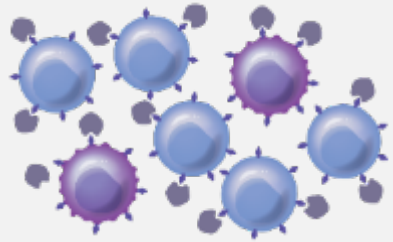
Traditional Hematopoietic Stem Cells (HSCs)

TRANSPLANT

Patient

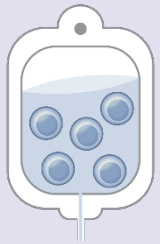
TARGETED THERAPY

Susceptible Marrow Limiting Treatment Opportunities



Editing Opportunity

Vor Engineering Process removing or modifying surface targets



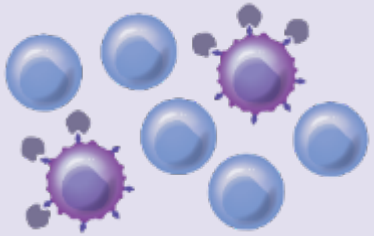
Engineered HSCs (**eHSCs**)

TRANSPLANT

Engineered Patient: Recipient with engrafted eHSCs

TARGETED THERAPY

Treatment-resistant Marrow Unlocking Potential of Targeted Therapies



AML: Large Unmet Need

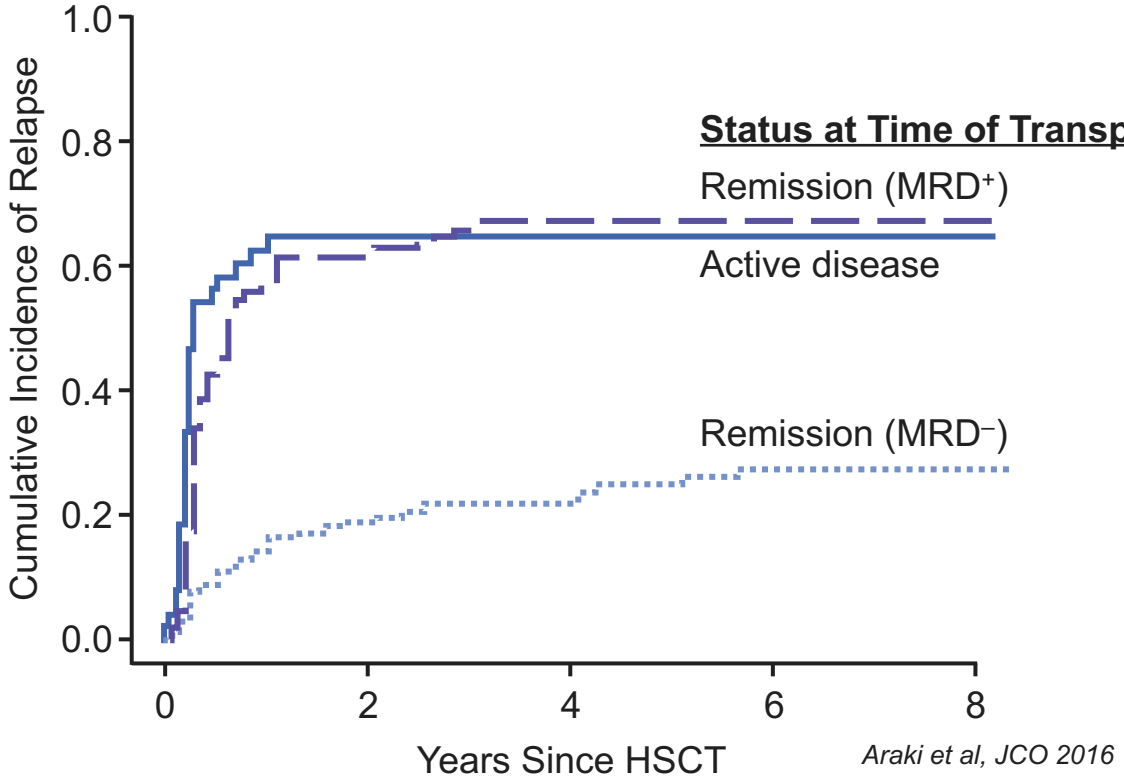
MOST common form of acute leukemia in adults

42,500 new diagnoses each year in the United States, Europe, and Japan

Average five-year survival rate is less than **30%**

For healthier patients, standard of care is intensive chemotherapy followed by **hematopoietic stem cell transplant (HSCT)**

Post-Transplant AML Relapse

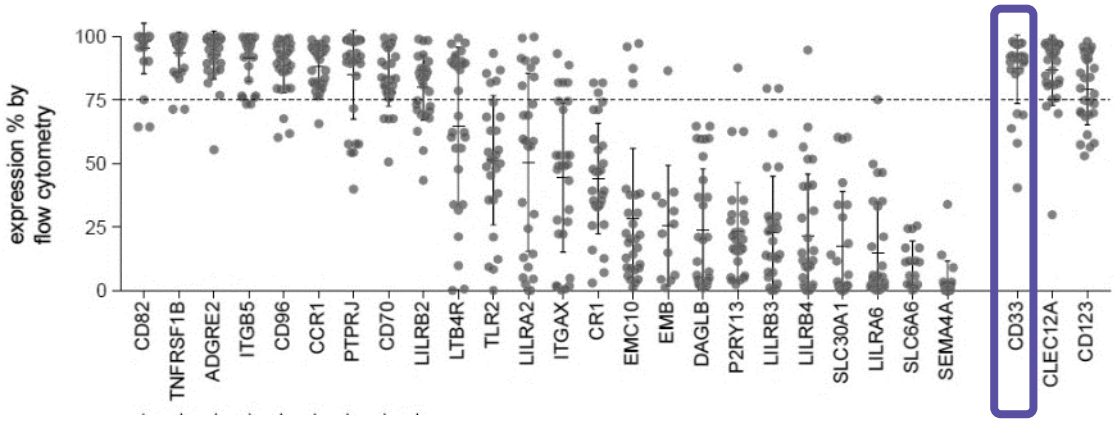


Araki et al, JCO 2016

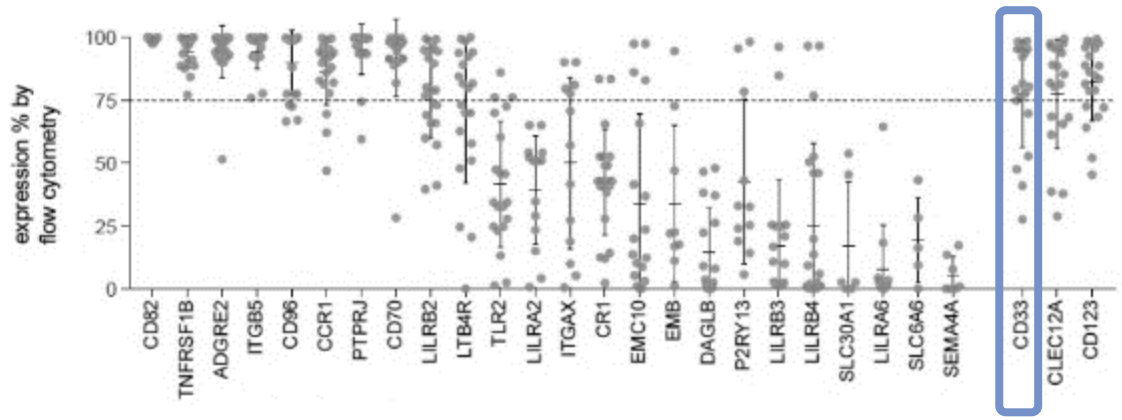
In relapsed patients, 2-year survival rate is <20%

CD33 Among the Most Attractive Targets in AML

Expression in Bulk AML Population



Expression in Leukemic $CD34^{+}CD38^{-}$ Cells ("Leukemic Stem Cells")



Perna, Sadelain et al, Cancer Cell 2017

Establishing eHSC as Standard of Care and Enabling Treatment Combos

Near-Term Development

VOR33 – $CD33^{Del}$ Allogeneic eHSC

- Expected initial engraftment and protection data within 12 months of FPI – potentially validating for VOR33 and broader eHSC approach

CD33-Directed CAR-T

- Currently enrolling ped Ph1/2 R/R AML study
- Planning adult Ph1 R/R AML study
- In process development using allogeneic cells

Potential Future Development

VOR33/CD33 CAR-T Treatment System

eHSC Transplant	Companion Therapeutic
VOR33	**CD33 CAR-T**

Other Possible Novel Treatment Combinations

Potential New Standard of Care	Companion Therapeutics
eHSC Transplants	• Bispecific T cell engagers • CAR-NKs • Antibody-drug conjugates

VOR33 (CD33^{Del} eHSC): Proof of Concept

		Validation from two independent labs	
	Mukherjee lab	Gill lab	Kiem lab
Institution	Columbia University	Penn Medicine	Fred Hutch
Protect cells *in vitro*	✓	✓	✓
Protect cells *in vivo*	✓	✓	✓
Preserve cell populations and function	✓	✓	✓
Safety in non-human primates	N/A	✓	N/A
	Borot et al, PNAS 2019	*Kim et al, Cell 2018*	*Humbert et al, Leukemia 2018*

Vor Bio Scientific Founder
Dr. Siddhartha Mukherjee

- Associate Professor of Medicine in the Division of Hematology and Oncology at Columbia University
- Laboratory research focus: biology of normal and malignant blood development, with focus on AML
- Author of *The Emperor of All Maladies: A Biography of Cancer* and *The Gene: An Intimate History*

Strongest Supportive Evidence: Human Genetics

65 individuals with homozygous loss-of-function mutations in CD33 gene

in Genome Aggregation Database

gnomAD

BROAD INSTITUTE

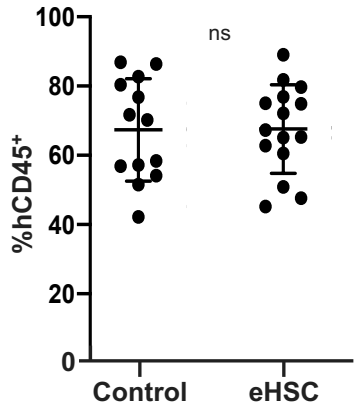
http://gnomad.broadinstitute.org

VOR

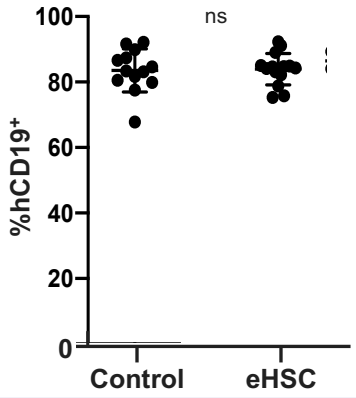
VOR33 ($CD33^{Del}$ eHSCs): No Observed Impact on Cell Populations or Function

Xeno-transplant Mouse Model: 16-week Bone Marrow

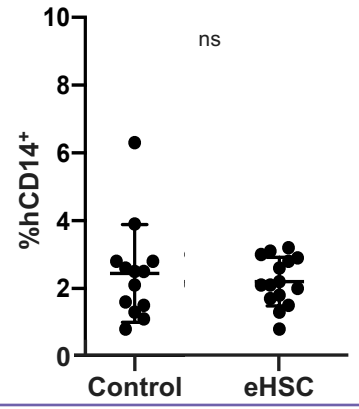
No change in nucleated bone marrow cell frequency

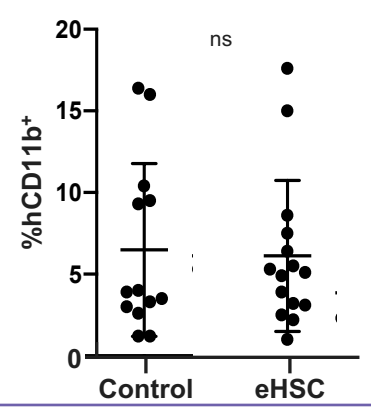


No change in B cell frequency



No change in myeloid cell frequency

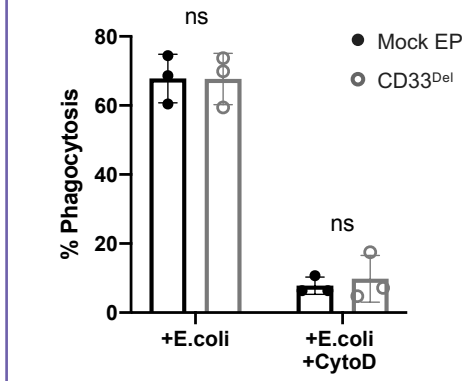




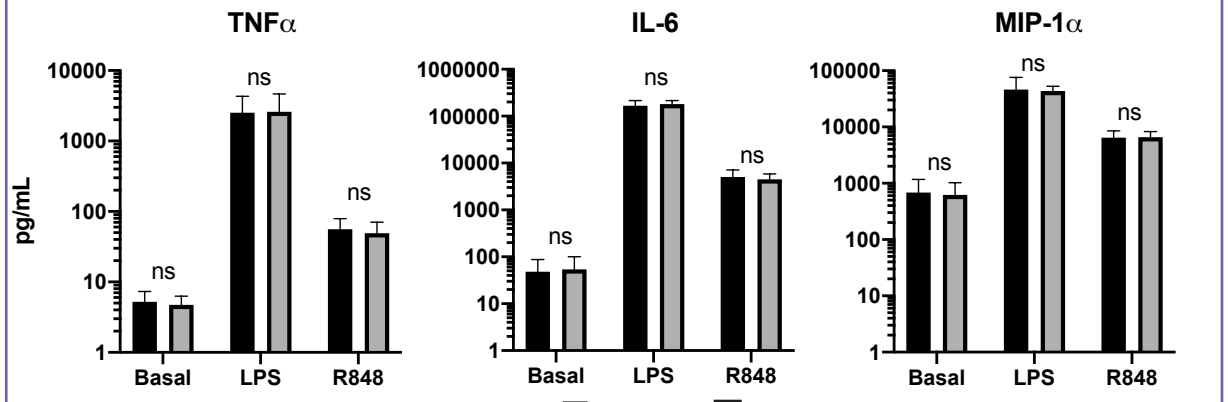
n=15 per group

In Vitro Cell Function Assays

No change in phagocytosis



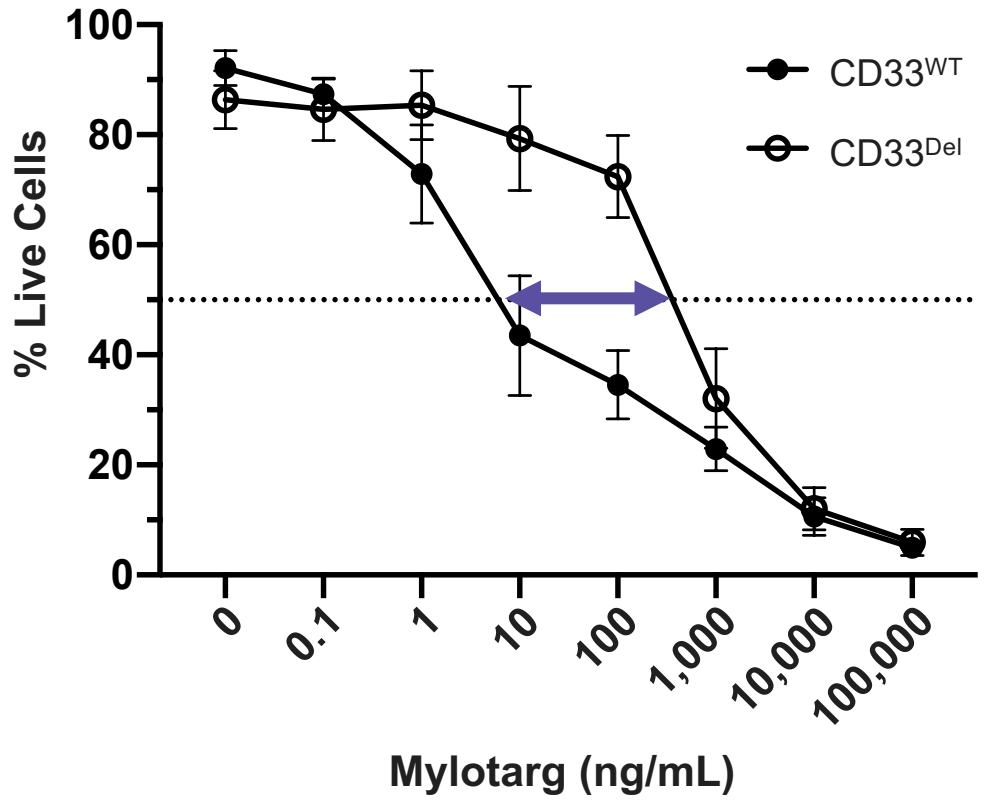
No change in cytokine production



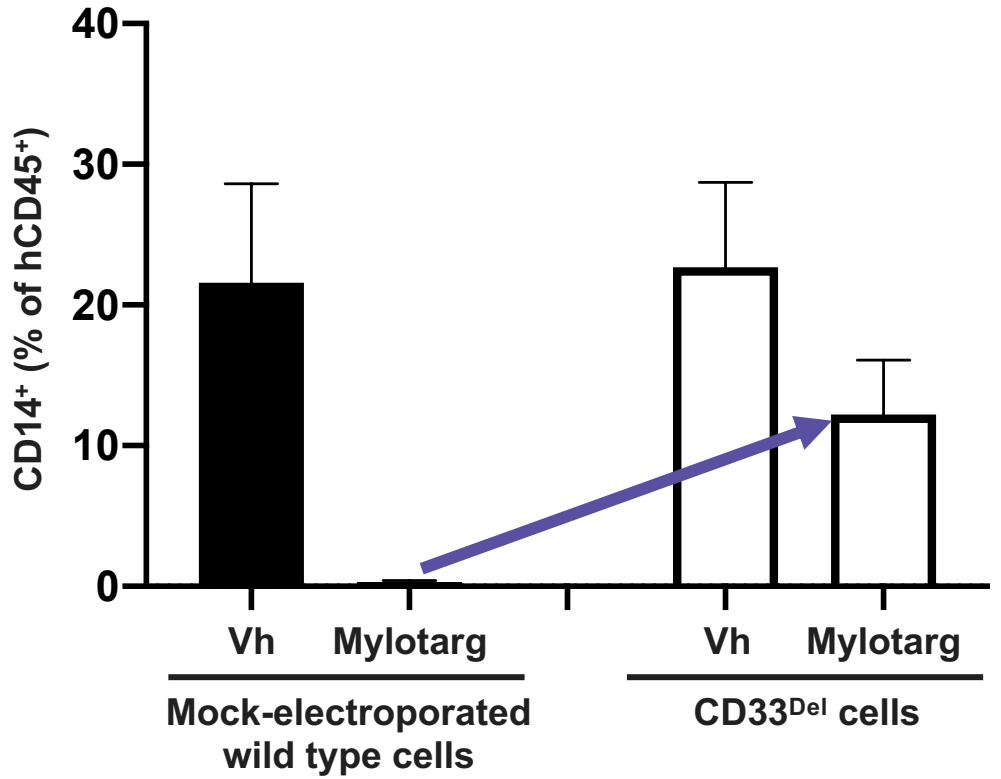
n=3 per group; ns=not significant (p>0.05)

VOR33 (CD33^{Del} eHSCs): Resistance to CD33 Therapy

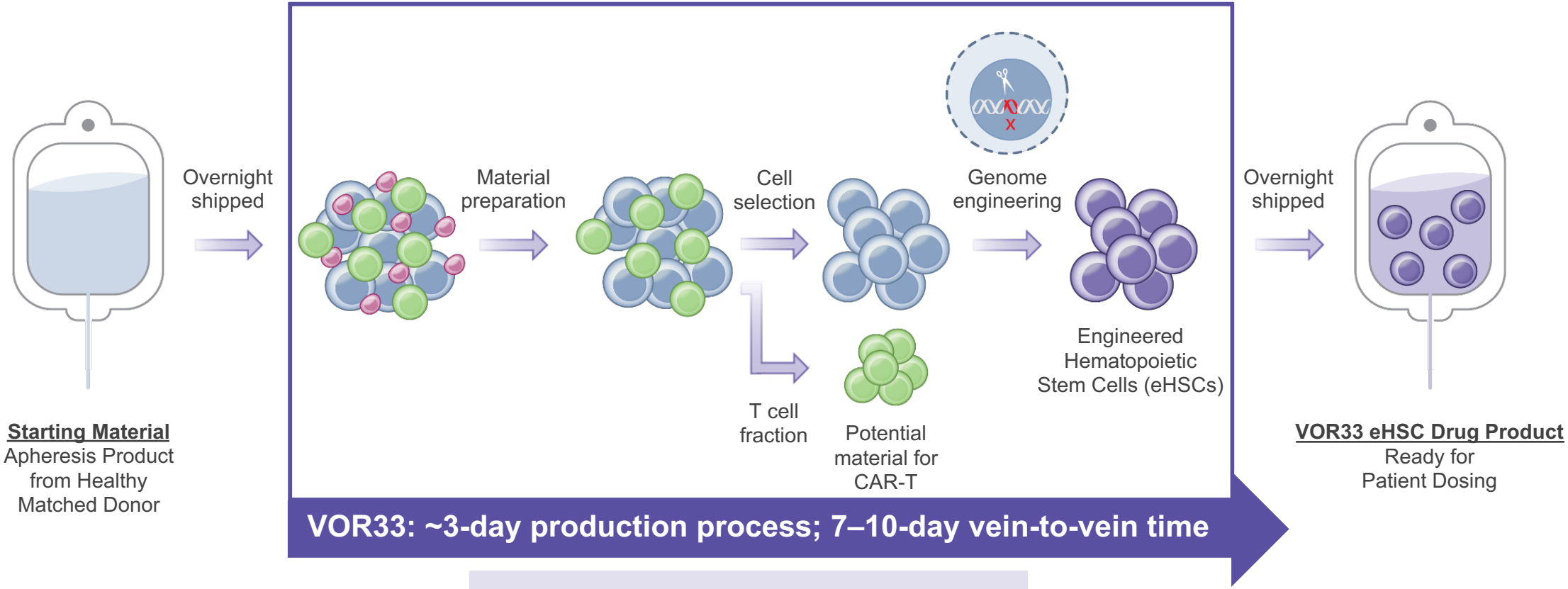
In Vitro



In Vivo



VOR33: Streamlined Cell Manufacturing Process



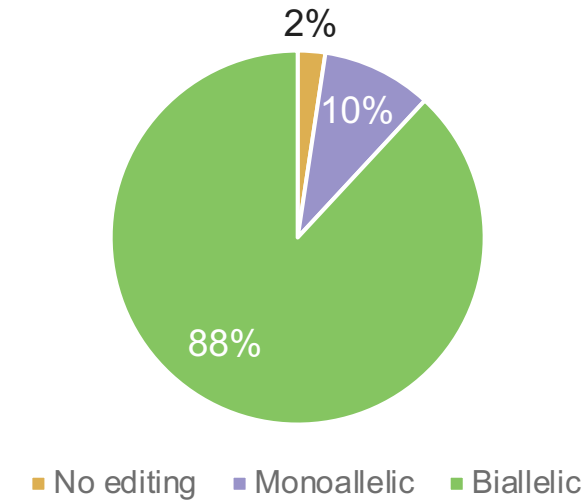
- ✓ No new genetic material nor viral vectors
- ✓ No cell expansion

CD33 Can Be Efficiently and Reproducibly Removed

Efficient CD33 Removal

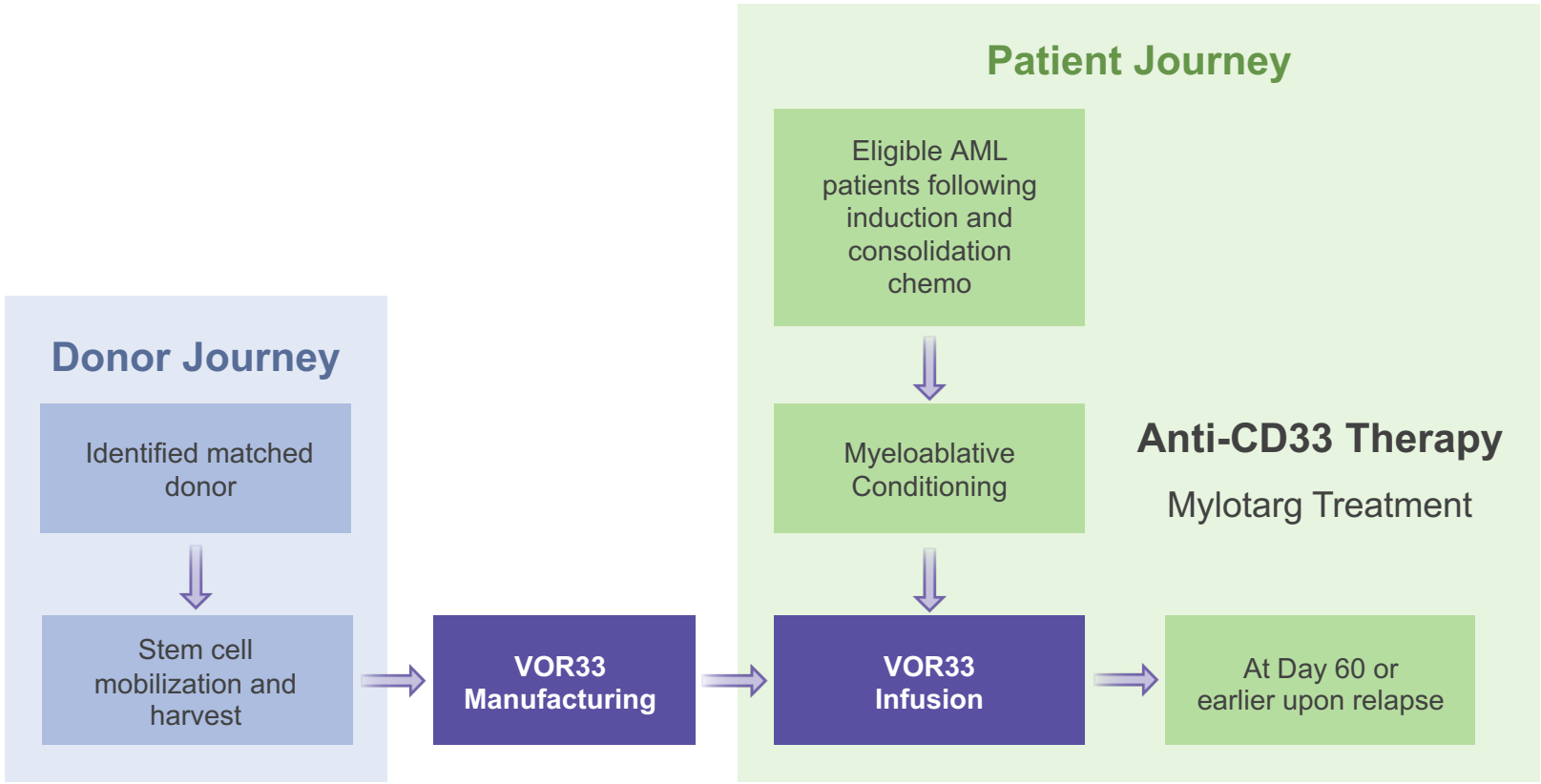
- Enriched stem cells from six independent healthy donors
- Two separate lots of gRNA

Largely Biallelic Removal



- Allelic editing analysis in single cell derived colonies
- 84 total colonies analyzed

VOR33 First-in-Human Clinical Study Outline



Three key endpoints

1. VOR33 engraftment
2. Mylotarg protection
3. Relapse-free survival

Expected Clinical Trial Sites:

- MSKCC (NY)
- Fred Hutchinson Cancer Ctr. (WA)
- Hackensack/Theurer Cancer Ctr. (NJ)*
- Miami Cancer Inst. (FL)*
- CWRU/Seidman Cancer Ctr. (OH)*
- UC San Diego Cancer Ctr. (CA)*
- Hopital Maisonneuve-Rosemont (Montreal)

* Use central IRBs and willing to begin process prior to IND approval

CD33-Directed CAR-T

CD33 CAR Construction

Same antibody binder used in SGN-CD33A (vadastuximab talirine) from Seattle Genetics

CD28 costimulatory domain achieved superior preclinical activity vs. 4-1BB

Devised by renowned T cell expert Dr. Terry Fry

Exclusively licensed from the National Institutes of Health

Development Strategy

Evaluation of Bridge-to-Transplant Monotherapy Safety and Preliminary Efficacy

- Multi-site Phase 1/2 pediatric trial in R/R AML
  - Current sponsor: Center for International Blood and Marrow Transplant Research
- Phase 1 adult trial in R/R AML (in planning)

Evaluation of VOR33/CD33 CAR-T Treatment System

- VOR33 eHSC transplant
- Administer CAR-T in post-transplant setting with curative intent

Vor AML Platform – Potential US Reimbursement Pathways

Medicare

eHSC

Carve-out for actual cost of stem cell acquisition and processing

OR

New Technology Add-on Payment (NTAP)

NEW 2021 Inpatient Prospective Payment Systems (IPPS) Ruling for Medicare

CAR-T

MS-DRG 018 (or new MS-DRG)

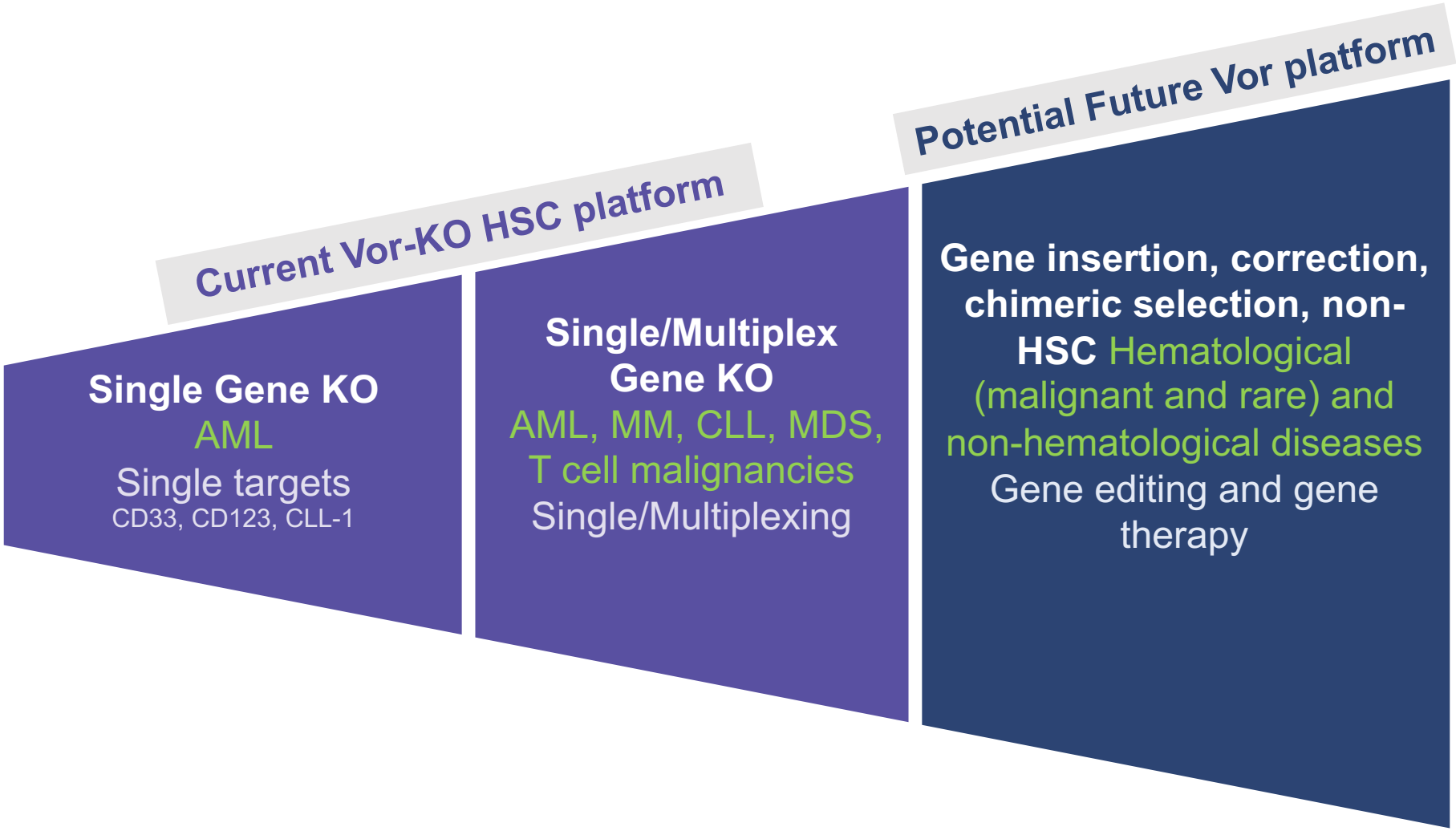
+/-

New Technology Add-on Payment (NTAP)

Commercial Payors

Negotiated case rate, incremental carve-out, or outcomes-based agreement

Vor's Technology-Driven Pipeline Vision



CD123 or CLL-1-Edited eHSCs: Normal Differentiation and Function, Target-specific Protection

Engineering

✓ Over 80% editing efficiency

Engraftment

✓ No change in long-term engraftment in mouse models

✓ No change in multilineage leukocyte distribution

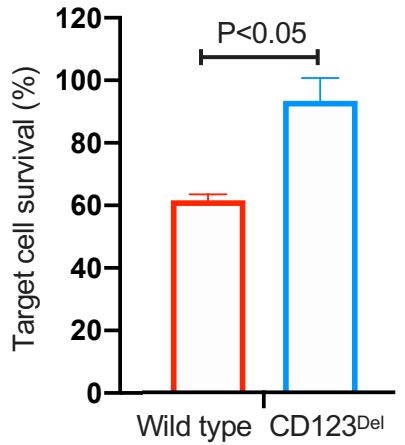
Function

✓ No change in phagocytosis

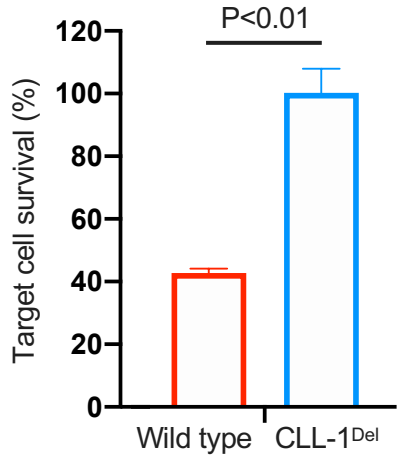
✓ No change in IL-6 production

✓ No change in TNF-α production

Protection from CD123 CAR-T



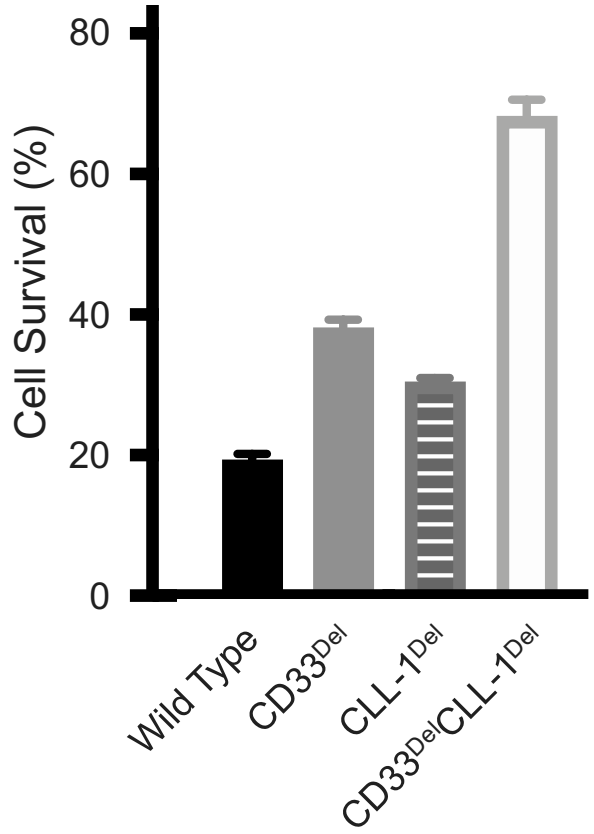
Protection from CLL-1 CAR-T



CD33/CLL-1 Multiplex Editing Results in Protection from CAR-T Cytotoxicity

- Dual editing of CD33 and CLL-1 in the same HSPCs via sequential CRISPR/Cas9 editing
- *In vitro* application of dual CAR-Ts
  - Anti-CD33
  - Anti-CLL-1

Protection from Dual CAR-T Killing



Effector : Target = 1:1

$152M Raised from Blue-Chip Investors

- $42M Series A round announced Jan 2019
- $110M Series B round announced July 2020

RACapital

5AM VENTURES

Fidelity INVESTMENTS

SARISSA CAPITAL MANAGEMENT LP

PagsGroup

CUP OSAGE UNIVERSITY PARTNERS

ALEXANDRIA VENTURE INVESTMENTS

Johnson & Johnson INNOVATION — JJDC —

PURETECH HEALTH

NOVARTIS NOVARTIS INSTITUTES FOR BIOMEDICAL RESEARCH

VOR

Vor Bio: Engineering the Patient to Better Fight Their Cancers

Changing the Paradigm of Cancer Treatment

- Clinical-stage cell therapy company with fundamentally different approach to cancer targets
  - ✓ Proprietary engineered hematopoietic stem cell transplant (eHSC) platform
  - ✓ Unlocking potential of targeted therapies with curative intent
  - ✓ VOR33 eHSC completed IND-enabling studies; CD33 CAR-T in Phase 1/2
  - ✓ Single company solution combining our eHSC and CAR-T into Treatment System
- Platform with broad potential across hematological malignancies
- Experienced and proven management team
- Recent $110M Series B financing from blue-chip investors

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