



Vor Bio Reports Third Quarter 2024 Financial Results and Provides Company Update

November 7, 2024

- Company to present trem-cel + Mylotarg clinical data update at American Society of Hematology (ASH) 2024 annual meeting
- Han Choi, M.D., LL.M, seasoned healthcare institutional investor, appointed as Chief Financial Officer

CAMBRIDGE, Mass., Nov. 07, 2024 (GLOBE NEWSWIRE) -- Vor Bio (Nasdaq: VOR), a clinical-stage cell and genome engineering company, today reported financial results for the three-month period ended September 30, 2024, and provided a corporate update.

"We are pleased with our operational and clinical execution this quarter and look forward to sharing additional clinical data since our September data update at the ASH annual meeting in December," said Dr. Robert Ang, Vor Bio's President and Chief Executive Officer.

Corporate Updates

Trem-cel + Mylotarg (VBP101) Clinical Trial

- 25 patients have been dosed with trem-cel and 6 patients have now received the third dose level of Mylotarg at 2 mg/m².
- On September 5, 2024, the Company provided a clinical update on 18 patients treated with trem-cel in the VBP101 study. The data demonstrated:
 - Reliable engraftment, robust platelet recovery, and high CD33 editing efficiency with full myeloid chimerism at Day 28.
 - Shielding of the blood system, with maintained neutrophil and platelet counts across multiple Mylotarg doses of 0.5, 1, and 2 mg/m².
 - Broadened therapeutic index for Mylotarg with drug exposure represented by AUC which is related to efficacy, consistent with labeled Mylotarg doses, and with maximal concentrations, measured by C_{max} and related to veno-occlusive disease, well below known toxic range.
 - Early evidence suggesting patient benefit as measured by relapse-free survival when compared to published high-risk AML comparators¹.
- The Company plans to share a further clinical data update in a poster presentation at the ASH Annual Meeting on Sunday, December 8, from 6-8pm PT. (*Poster Session 616. Acute Myeloid Leukemias: Investigational Drug and Cellular Therapies. Publication number 2873*). The poster will be made available on Vor Bio's website when the ASH embargo lifts at 9am PT on the day of the presentation and the Company plans to host a webcast to review the data on Monday, December 9th at 5am PT/8am ET.

Trem-cel is a shielded transplant in development for patients with acute myeloid leukemia (AML) and myelodysplastic syndromes (MDS), in which healthy transplant donor cells are genetically engineered by removing CD33, with the potential to shield healthy cells and enable targeted therapies post-transplant such as Mylotarg and CAR-T therapy.

VCAR33^{ALLO} (VBP301) Clinical Trial

- Dosing is ongoing in the VBP301 study with continued strong enrollment.
- On September 5, 2024, the Company announced encouraging in vivo CAR-T expansion data from three patients treated to date, all at the lowest dose of 1 x 10⁶ CAR+ cells/kg.

VBP301, a Phase 1/2, multicenter, open-label, first-in-human study of VCAR33^{ALLO}, is a transplant donor-derived anti-CD33 CAR-T cell therapy for patients with AML who have relapsed following a standard-of-care or trem-cel transplant.

Preclinical Research Updates

VADC45

In September 2024, the Company announced a new preclinical asset, VADC45, which has several potential opportunities in oncology, gene therapy, and autoimmune disorders. VADC45 is an antibody-drug conjugate (ADC) designed to target the CD45 protein, a well-validated target for a wide variety of blood cancers with clinical proof of concept. The linker-payload used in VADC45 has also been clinically validated. Vor Bio is currently progressing IND-enabling studies to enable future Phase 1 studies.

Preclinical data presentations further validate Vor Bio's robust platform

ESGCT 2024

In October 2024, the Company presented [preclinical data](#) at the European Society of Gene and Cell Therapy Congress on the development and characterization of its novel CD33/CLL-1-directed CAR-T approach in AML.

ASH 2024

Additional analyses from the most comprehensive single cell AML atlas known to date (more than 400,000 cells from 26 AML patients and 10 healthy donors) were accepted for presentation at the ASH 2024 annual meeting in December. These data provide an unprecedented view of AML immunophenotypic heterogeneity, both across patients and within patients across clinical timepoints.

Abstract Title: *Multimodal Atlas of Paired Diagnosis and Relapse AML Reveals Surface Antigens for Multi-Specific Immunotherapy*

Format: Poster presentation

Session Name: Acute Myeloid Leukemias: Biomarkers and Molecular Markers in Diagnosis and Prognosis

Session date and time: Saturday, Dec 7, 5:30-7:30pm PT

New Chief Financial Officer appointed

Han Choi, M.D., LL.M., was appointed as Vor Bio's new Chief Financial Officer. Dr. Choi brings more than 25 years of experience in investment management, business development, and corporate strategy within the pharmaceutical and biotechnology sectors to Vor Bio. His extensive expertise in forming corporate partnerships, managing complex transactions, and developing capital markets strategies will be pivotal as the Company advances its clinical pipeline and strengthens its leadership in cell and genome engineering.

Upcoming Milestones

- Trem-cel + Mylotarg clinical data update planned at ASH 2024 annual meeting

Third Quarter 2024 Financial Results

- **Cash Position:** Cash, cash equivalents and marketable securities were \$62.8 million as of September 30, 2024, which is projected to fund operations into the second half of 2025.
- **Research & Development (R&D) Expenses:** R&D expenses for the third quarter of 2024 were \$21.8 million, compared to \$27.6 million for the third quarter of 2023. The decrease of \$5.8 million was due to a decrease in license payments, preclinical expenses, and manufacturing starting materials, offset in part by an increase in clinical trial costs to support our trem-cel and VCAR33^{ALLO} programs.
- **General & Administrative (G&A) Expenses:** G&A expenses for the third quarter of 2024 were \$6.7 million, compared to \$7.7 million for the third quarter of 2023. The decrease of \$1.0 million was primarily due to a decrease in professional fees and stock-based compensation expense.
- **Net Loss:** Net loss for the third quarter of 2024 was \$27.6 million, compared to \$33.2 million for the third quarter of 2023.

Condensed Consolidated Balance Sheet Data (Unaudited) (in thousands)

	September 30, 2024	December 31, 2023
Cash, cash equivalents and marketable securities	\$ 62,809	\$ 137,175
Total assets	115,991	198,126
Total liabilities	43,036	47,402
Total stockholders' equity	72,955	150,724

Condensed Consolidated Statement of Operations (Unaudited) (in thousands, except share and per share data)

	Three Months Ended September 30,	
	2024	2023
Operating expenses:		
Research and development	\$ 21,817	\$ 27,606
General and administrative	6,696	7,710
Total operating expenses	\$ 28,513	\$ 35,316
Loss from operations	\$ (28,513)	\$ (35,316)
Other income:		
Interest income	954	2,126
Total other income	954	2,126
Net loss	\$ (27,559)	\$ (33,190)

Net loss per share attributable to common stockholders, basic and diluted	\$ (0.40)	\$ (0.49)
Weighted-average common shares outstanding, basic and diluted	<u>68,465,801</u>	<u>67,607,713</u>

About Vor Bio

Vor Bio is a clinical-stage cell and genome engineering company that aims to change the standard of care for patients with blood cancers by engineering hematopoietic stem cells to enable targeted therapies post-transplant. For more information, visit: www.vorbio.com.

Forward-Looking Statements

This press release contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995. The words “aim,” “anticipate,” “can,” “continue,” “could,” “design,” “enable,” “expect,” “initiate,” “intend,” “may,” “on-track,” “ongoing,” “plan,” “potential,” “should,” “target,” “update,” “will,” “would,” 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 press release include Vor Bio’s statements regarding the potential of its product candidates to positively impact quality of life and alter the course of disease in the patients it seeks to treat, the timing and pace of patient enrollment and dosing in clinical trials and the availability of data therefrom, the potential of trem-cel to enable targeted therapies in the post-transplant setting including Mylotarg and CD33-targeted CAR-Ts, its potential upcoming milestones, its cash runway and expected capital requirements, and other statements that are not historical fact. 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 and completion of preclinical studies and clinical trials and clinical development of Vor Bio’s product candidates; availability and timing of results from preclinical studies and clinical trials; whether interim results from a clinical trial will be predictive of the final results of the trial or the results of future trials; uncertainties regarding 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 and Vor Bio’s ability to continue as a going concern. 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 press release speak only as of the date hereof, 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.

Contact:

Investors & Media
Sarah Spencer
+1 857-242-6076
sspencer@vorbio.com