Vor Bio Reports Fourth Quarter and Full Year 2024 Financial Results and Provides Company Update

March 20, 2025

- *VCAR33 clinical data update planned for first half of 2025 and trem-cel + Mylotarg clinical data update planned for second half of 2025*
- *Anticipate initiation of the first trem-cel+VCAR33 Treatment System clinical trial in second half of 2025*
- *$55.6 million private placement completed in December 2024*

CAMBRIDGE, Mass., March 20, 2025 (GLOBE NEWSWIRE) -- Vor Bio (Nasdaq: VOR), a clinical-stage cell and genome engineering company, today reported financial results for the three-month period and full year ended December 31, 2024, and provided a business update.

“We continue to make significant strides in advancing our novel cell and genome engineering platform. The continued progress in our clinical trials reinforces our confidence in the potential of trem-cel and VCAR33 to transform the treatment landscape,” said Dr. Robert Ang, Vor Bio’s President and Chief Executive Officer. “With a strengthened financial position from recent investment by new investor Reid Hoffman and existing investor RA Capital Management, we are well-positioned to drive our mission forward and deliver meaningful impact to patients.”

Corporate Updates

Trem-cel + Mylotarg (VBP101) Clinical Trial

Trem-cel is a shielded transplant in development for patients with AML and MDS, in which healthy transplant donor cells are genetically engineered by removing CD33, with the potential to enable targeted therapies such as Mylotarg and CD33-targeted CAR-T therapy post-transplant, while avoiding on-target toxicities.

- The latest data update from VBP101, the Phase 1/2a clinical trial of trem-cel + Mylotarg, was an encore presentation of data presented at ASH 2024 and took place at the TANDEM Meetings of ASTCT and CIBMTR on February 15, 2025. The data released included 25 patients treated with trem-cel of which 15 had received Mylotarg (six at the 2 mg/m^2 dose) as of the data cut-off date of November 1, 2024. The data demonstrated durable engraftment, shielding from Mylotarg on-target toxicity, a broadened Mylotarg therapeutic window, and early evidence of improved relapse-free survival compared to published high-risk AML comparators[1].
- Patients are now being treated in this study at the recommended Phase 2 dose of Mylotarg at 2 mg/m^2.
- Patients receiving a trem-cel transplant who become measurable residual disease (MRD) positive or relapse have the option to receive Mylotarg or enroll in VBP301 and receive VCAR33.
- The Company expects to report further follow-up data from patients receiving Mylotarg in the the second half of 2025.

[1] Araki et al. JCO 2016; Jentzsch et al. Blood Cancer Journal 2022.

VCAR33 (VBP301) Clinical Trial

VBP301, a Phase 1/2, multicenter, open-label, first-in-human study of VCAR33, 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.

- Dosing is ongoing in the VBP301 study with continued strong enrollment, and the next clinical data update is planned for first half of 2025.
- The Company previously announced encouraging in vivo CAR-T expansion data from three patients treated to date, all at the lowest dose of 1 x 10^6 CAR+ cells/kg.

Trem-cel+VCAR33 Treatment System

The combination of a trem-cel transplant followed by VCAR33 in the maintenance setting has the potential to transform patient

outcomes in AML and establish a new standard of care for patients with high risk of relapse. The trem-cel+VCAR33 Treatment System would utilize cells from the same healthy donor for both trem-cel and VCAR33, potentially prolonging persistence and optimizing CAR-T expansion.

The Company anticipates initiating a Phase 1 clinical trial with the trem-cel+VCAR33 Treatment System in the second half of 2025.

$55.6 Million Financing Completed and New Board Member Appointed

In December 2024 the Company entered into a securities purchase agreement for a private investment in public equity financing (PIPE) that resulted in gross proceeds of approximately $55.6 million. The PIPE was led by new investor, Reid Hoffman, and included participation from existing investor and Vor Bio's largest stockholder, RA Capital Management.

In addition, as part of the transaction, life sciences and technology investor and entrepreneur Mr. Erez Kalir was appointed to a newly created seat on Vor Bio's Board of Directors. Mr. Kalir brings a wealth of experience as an investor, entrepreneur, and thought leader with a deep focus on life sciences and technology.

Upcoming Milestones

- VCAR33 clinical data update expected in the first half of 2025
- Trem-cel clinical data update expected in the second half of 2025
- Anticipate initiation of a Phase 1 trem-cel+VCAR33 Treatment System clinical trial in the second half of 2025

Fourth Quarter and Full Year 2024 Financial Results

- **Cash Position:** Cash, cash equivalents and marketable securities were $91.9 million as of December 31, 2024, which is projected to fund operations into the first quarter of 2026.
- **Research & Development (R&D) Expenses:** R&D expenses for the fourth quarter of 2024 were $25.3 million, compared to $20.9 million for the fourth quarter of 2023, and for the year ended December 31, 2024, were $93.3 million, compared to $94.3 million for the year ended December 31, 2023. The quarter over quarter increase in R&D expenses was primarily attributable to an increase in clinical trial costs to support our trem-cel and VCAR33 programs, offset in part by a decrease in preclinical activities.

- **General & Administrative (G&A) Expenses:** G&A expenses for the fourth quarter of 2024 were $6.0 million, compared to $7.2 million for the fourth quarter of 2023, and for the year ended December 31, 2024, were $27.9 million, compared to $31.7 million for the year ended December 31, 2023. The quarter over quarter decrease in G&A expenses was primarily attributable to a decline in stock-based compensation and legal and consulting fees.

- **Net Loss:** Net loss for the fourth quarter of 2024 was $30.7 million, compared to $26.3 million for the fourth quarter of 2023, and for the year ended December 31, 2024, was $116.9 million, compared to $117.9 million for the year ended December 31, 2023.

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, plans for a registrational trial of trem-cel, 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

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

	December 31, 2024	December 31, 2023
Cash, cash equivalents and marketable securities	$ 91,926	$ 137,175
Total assets	142,891	198,126
Total liabilities	46,227	47,402
Total stockholders' equity	96,664	150,724

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

	Twelve Months Ended December 31,	
	2024	2023
Operating expenses:		
Research and development	$ 93,310	$ 94,315
General and administrative	27,875	31,721
Total operating expenses	$ 121,185	$ 126,036
Loss from operations	$ (121,185)	$ (126,036)
Other income:		
Interest income	4,271	8,173
Total other income	4,271	8,173
Net loss	$ (116,914)	$ (117,863)
Net loss per share attributable to common stockholders, basic and diluted	$ (1.70)	$ (1.75)
Weighted-average common shares outstanding, basic and diluted	68,705,639	67,191,973