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

May 9, 2024

- *On-track for trem-cel and VCAR33^{ALLO} clinical updates in the second half of 2024*
- *Trem-cel trial expanded to include patients with myelodysplastic syndromes (MDS); Mylotarg™ dosing has advanced to the third cohort*

CAMBRIDGE, Mass., May 09, 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 March 31, 2024, and provided a business update.

“We are pleased with the strong enthusiasm from investigators to enroll patients in both of our clinical trials and are encouraged by the number of VCAR33^{ALLO} CAR-T trial sites that are now active, many of which overlap with our trem-cel sites,” said Dr. Robert Ang, Vor Bio’s President and Chief Executive Officer. “We are fully focused on execution of these clinical trials and are looking forward to our next substantive data update.”

Corporate Updates

Strong progress with VCAR33^{ALLO}

- Enrollment progress continues with multiple patients dosed in the first half of 2024.
- 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 acute myeloid leukemia (AML) who have relapsed following a standard-of-care or trem-cel transplant.

VCAR33^{ALLO} is manufactured from lymphocytes collected from the patient’s original transplant donor, generating a CAR-T cell therapy that is exactly matched to the recipient’s engrafted blood system. By using healthy transplant donor cells as the starting material to produce VCAR33 ^{ALLO}, the CAR-T cells have a more stem-like phenotype, leading to greater potential for expansion, persistence, and anti-leukemia activity compared to a product derived from a patient’s own lymphocytes.

Trem-cel trial expanded to include patients with MDS, and dose escalation of Mylotarg is proceeding

- 15 patients have been dosed with trem-cel and additional trem-cel engraftment, and hematologic protection data from higher doses of Mylotarg are expected in the second half of 2024.
- Patients are now receiving the third dose level of Mylotarg at 2.0 mg/m^2.
- The trem-cel clinical trial has been expanded to include patients diagnosed with MDS. Approximately 1,250 stem cell transplants occur annually in the US for patients with MDS[1] and Vor Bio’s approach represents an important advancement in potentially transforming treatment of these blood cancers.
- Trem-cel is a shielded transplant in development for patients with AML, 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.

1. HRSA Transplant Activity Report (Table 10 By specific disease – 2020 data).

Accomplished oncology and cancer immunotherapy R&D executive joins Board
As previously announced, the Company has appointed Fouad Namouni, M.D. to its Board of Directors. Dr. Namouni currently serves as President of Research & Development at Blueprint Medicines and brings significant industry experience and expertise to Vor Bio's Board.

Upcoming Milestones

- Trem-cel clinical data update expected in the second half of 2024
- VCAR33^{ALLO}clinical data update expected in the second half of 2024

First Quarter 2024 Financial Results

- **Cash Position:** Cash, cash equivalents and marketable securities were $107.5 million as of March 31, 2024, which is projected to fund operations into the second half of 2025.

- **Research & Development (R&D) Expenses:** R&D expenses for the first quarter of 2024 were $24.3 million, compared to $21.9 million for the first quarter of 2023. The increase of $2.4 million was primarily attributable to an increase in costs

related to our trem-cel and VCAR33^{ALLO} clinical programs.

- **General & Administrative (G&A) Expenses:** G&A expenses for the first quarter of 2024 were $8.0 million, compared to $8.5 million for the first quarter of 2023. The decrease of $0.5 million was primarily attributable to a decrease in consulting and legal expenses, partially offset by an increase in personnel-related costs.

- **Net Loss:** Net loss for the first quarter of 2024 was $30.8 million, compared to $28.4 million for the first quarter of 2023.

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

	March 31, 2024	December 31, 2023
Cash, cash equivalents and marketable securities	$ 107,479	$ 137,175
Total assets	167,030	198,126
Total liabilities	43,995	47,402
Total stockholders' equity	123,035	150,724

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

	Three Months Ended March 31,	
	2024	2023
Operating expenses:		
Research and development	$ 24,322	$ 21,915
General and administrative	8,004	8,507
Total operating expenses	$ 32,326	$ 30,422
Loss from operations	$ (32,326)	$ (30,422)
Other income:		
Interest income	1,522	1,989
Total other income	1,522	1,989
Net loss	$ (30,804)	$ (28,433)
Net loss per share attributable to common stockholders, basic and diluted	$ (0.45)	$ (0.43)
Weighted-average common shares outstanding, basic and diluted	68,030,966	66,265,703

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 expected safety profile of its product candidates, its intentions to use 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, its potential upcoming milestones, and its cash runway and expected capital requirements. 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. 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