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

August 8, 2024

- *On-track for trem-cel + Mylotarg™ and VCAR33^{ALLO} clinical updates in the second half of 2024*

CAMBRIDGE, Mass., Aug. 08, 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 June 30, 2024, and provided a corporate update.

"We are pleased with the progress we have made with enrollment of both the trem-cel + Mylotarg and VCAR33 ^{ALLO} clinical trials during the quarter," said Dr. Robert Ang, Vor Bio's President and Chief Executive Officer. "Every year, thousands of patients with AML and MDS relapse post-transplant with no safe and effective treatment options. Our approach of shielded transplants may provide the first opportunity to deliver curative treatments post-transplant. We look forward to our next clinical update in the second half of this year."

Corporate Updates

Trem-cel trial continues to enroll rapidly and patients are receiving Mylotarg at the third dose level

- 21 patients have been dosed with trem-cel and patients are now receiving the third dose level of Mylotarg at 2.0 mg/m^2.
- The next data update is expected in the second half of 2024 and will include engraftment and hematologic protection data, and additional Mylotarg pharmacokinetic analyses. Several patients have been followed out for more than one year, and the Company also expects to provide a clinical update on these patients.
- 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.

Continued progress with VCAR33^{ALLO}

- Several patients have been dosed with VCAR33^{ALLO} in the VBP301 study with enrollment continuing, and initial data is still expected in the second 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 AML who have relapsed following a standard-of-care or trem-cel transplant.

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

Second Quarter 2024 Financial Results

- **Cash Position:** Cash, cash equivalents and marketable securities were $85.9 million as of June 30, 2024, which is projected to fund operations into the second half of 2025.
- **Research & Development (R&D) Expenses:** R&D expenses for the second quarter of 2024 were $21.8 million, compared to $23.9 million for the second quarter of 2023. The decrease of $2.1 million was due to timing of purchases of manufacturing starting materials for our VCAR33^{ALLO} program and a decrease in preclinical expenses, 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 second quarter of 2024 were $7.2 million, compared to $8.3 million for the second quarter of 2023. The decrease of $1.1 million was primarily due to a decrease in consulting and legal expenses, partially offset by an increase in personnel costs.
- **Net Loss:** Net loss for the second quarter of 2024 was $27.8 million, compared to $30.0 million for the second quarter of 2023.

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

	June 30,	December 31,

	2024	2023
Cash, cash equivalents and marketable securities	$ 85,938	$ 137,175
Total assets	141,588	198,126
Total liabilities	43,508	47,402
Total stockholders' equity	98,080	150,724

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

	Three Months Ended June 30,	
	2024	2023
Operating expenses:		
Research and development	$ 21,823	$ 23,897
General and administrative	7,212	8,277
Total operating expenses	$ 29,035	$ 32,174
Loss from operations	$ (29,035)	$ (32,174)
Other income:		
Interest income	1,196	2,195
Total other income	1,196	2,195
Net loss	$ (27,839)	$ (29,979)
Net loss per share attributable to common stockholders, basic and diluted	$ (0.41)	$ (0.45)
Weighted-average common shares outstanding, basic and diluted	68,299,170	67,033,150

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, 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