Vor Bio Reports Fourth Quarter and Full Year 2023 Financial Results and Provides Company Update

March 20, 2024

- *Trem-cel and VCAR33^{ALLO} clinical trial data expected in the second half of 2024*
- *VCAR33^{ALLO} granted Fast Track and Orphan Drug Designation by U.S. Food & Drug Administration*
- *Cash runway extends into second half of 2025*

CAMBRIDGE, Mass., March 20, 2024 (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, 2023, and provided a business update.

“We are very pleased with the foundational progress we made in 2023. Building on this, we expect 2024 to be an exciting year in which we demonstrate the Vor Bio approach to be instrumental in reducing the disease burden of AML, a devastating cancer where we desperately need effective treatment options,” said Dr. Robert Ang, Vor Bio’s President and Chief Executive Officer.

Corporate Updates

Multiple patients expected to be dosed with VCAR33^{ALLO} in the first half of 2024; initial data anticipated in 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. The first patient was dosed in January 2024, and multiple patients are expected to be dosed in the first half of 2024. We anticipate initial data in the second half of 2024.
- The U.S. Food & Drug Administration (FDA) has granted Fast Track Designation and Orphan Drug Designation to VCAR33^{ALLO}. The FDA Fast Track process aims to facilitate the development and expedite the review of drugs that treat serious conditions and fill an unmet medical need. Orphan Drug Designation entitles companies to development incentives including tax credits for clinical testing, prescription drug user fee exemptions and seven-year marketing exclusivity in the event of regulatory approval.

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.

VBP101 clinical trial of trem-cel enrolling steadily, with the next data readout expected in the second half of 2024

- Dose escalation of Mylotarg to 1.0 mg/m^2 has commenced with multiple patients now treated.
- Patients receiving a trem-cel transplant who become measurable residual disease (MRD) positive or relapse have the option to receive induction-course Mylotarg or VCAR33^{ALLO}.
- The Company expects to report further engraftment and protection data from the VBP101 clinical trial in the second half of 2024.
- The latest data update from VBP101, the Phase 1/2a clinical study of trem-cel, was presented at the ASH Annual Meeting on December 10, 2023, showing primary neutrophil engraftment in all eight patients and hematologic protection from acute Mylotarg toxicity through repeat doses.

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.

Continued progress on dual-targeted CAR-T and multiplex engineering approach

The Company continues to make progress with a dual-specific CAR-T targeting CD33 and CLL-1, two of the most promising antigens in AML. This is being paired with a hematopoietic stem cell (HSC) multiplex engineering approach, which could allow removal or modification of these two genes. The Company has demonstrated *in vitro* proof of concept for this approach.

IND-enabling work is progressing for the Company’s dual-specific CAR-T with key *in vivo* proof-of-concept experiments underway.

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

Fourth Quarter and Full Year 2023 Financial Results

- **Cash Position:** Cash, cash equivalents and marketable securities were $137.2 million as of December 31, 2023, which is projected to fund operations into the second half of 2025.

- **Research & Development (R&D) Expenses:** R&D expenses for the fourth quarter of 2023 were $20.9 million, compared to $17.1 million for the fourth quarter of 2022, and for the year ended December 31, 2023, were $94.3 million, compared to $64.6 million for the year ended December 31, 2022. The increase in R&D expenses was attributable primarily to an increase in clinical trial, manufacturing and personnel expenses, and the execution of our non-exclusive license agreement with Editas Medicine.

- **General & Administrative (G&A) Expenses:** G&A expenses for the fourth quarter of 2023 were $7.2 million, compared to $7.7 million for the fourth quarter of 2022, and for the year ended December 31, 2023, were $31.7 million, compared to $28.9 million for the year ended December 31, 2022. The increase in G&A expenses was primarily attributable to an increase in personnel expenses, including an increase in share-based compensation expense.

- **Net Loss:** Net loss for the fourth quarter of 2023 was $26.3 million, compared to $23.9 million for the fourth quarter of 2022, and for the year ended December 31, 2023, was $117.9 million, compared to $92.1 million for the year ended December 31, 2022.

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 an 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, its potential upcoming milestones, 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; expectations for 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.

Condensed Consolidated Balance Sheet Data
(in thousands)

	December 31, 2023	December 31, 2022
Cash, cash equivalents and marketable securities	$ 137,175	$ 230,245
Total assets	198,126	299,366
Total liabilities	47,402	48,759
Total stockholders' equity	150,724	250,607

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

	Three Months Ended December 31,		Twelve Months Ended December 31,	
	2023	2022	2023	2022
Operating expenses:				
Research and development	$ 20,897	$ 17,062	$ 94,315	$ 64,550

General and administrative	7,227	7,663	31,721	28,868
Total operating expenses	$ 28,124	$ 24,725	$ 126,036	$ 93,418
Loss from operations	$ (28,124)	$ (24,725)	$ (126,036)	$ (93,418)
Other income:				
Interest income	1,863	814	8,173	1,324
Total other income	1,863	814	8,173	1,324
Net loss	$ (26,261)	$ (23,911)	$ (117,863)	$ (92,094)
Net loss per share attributable to common stockholders, basic and diluted	$ (0.39)	$ (0.53)	$ (1.75)	$ (2.33)
Weighted-average common shares outstanding, basic and diluted	67,839,463	45,394,089	67,191,973	39,551,420

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