Vor Bio Reports Second Quarter 2023 Financial Results and Provides Company Update

August 10, 2023

- *Next trem-cel clinical data update expected by year-end 2023*
- *VCAR33^{ALLO} IND cleared by U.S. FDA; Rapidly activating sites toward study initiation*
- *Secured non-exclusive license to Cas9 gene-edited HSCs*

CAMBRIDGE, Mass., Aug. 10, 2023 (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, 2023, and provided a business update.

"We are pleased with the progress we have made during the quarter. The recent clearance of our IND for VCAR33 ^{ALLO} represents a significant step forward in our vision of creating a Treatment System for acute myeloid leukemia which may enable more potent and durable responses in the post-transplant setting without on-target toxicity," said Dr. Robert Ang, Vor Bio's President and Chief Executive Officer. "We also look forward to sharing further engraftment and Mylotarg hematologic protection data from our VBP101 clinical study by year-end."

Corporate Updates

Trem-cel clinical trial (VBP101) continues to actively enroll patients; next data update expected by year-end 2023

- Clinical data presented at the European Hematology Association 2023 meeting by Dr. Guenther Koehne, a clinical investigator for the VBP101 study, showed primary neutrophil engraftment in the first five patients treated and demonstrated consistent manufacturing of trem-cel with high CD33 editing efficiency.
- The Company plans to provide its next VBP101 clinical update by year-end 2023. The data set is expected to include engraftment data for additional patients receiving a trem-cel transplant and further Mylotarg™ hematologic protection data.
- Following a planned safety review of initial patients receiving trem-cel, the enrollment stagger requiring engraftment prior to transplanting subsequent patients was removed and is expected to increase the pace of enrollment.

Trem-cel is a genome-edited allogeneic hematopoietic cell transplant (HCT) that is lacking the CD33 protein. It is designed to replace standard of care transplants for patients suffering from AML and potentially other blood cancers. Trem-cel has the potential to enable powerful targeted therapies in the post-transplant setting including Mylotarg and CD33-targeted CAR-Ts.

VCAR33^{ALLO} IND cleared by the U.S. Food & Drug Administration (FDA)

- The U.S. FDA has cleared the Company's Investigational New Drug (IND) application for VCAR33 ^{ALLO}, a T-cell therapy derived from allogeneic healthy donors using a chimeric antigen receptor (CAR) specifically binding to CD33.
- The Phase 1/2 VBP301 clinical trial will enroll patients who have relapsed following allogeneic stem cell transplant, which uses lymphoid cells harvested from the original donor as starting material for the drug product.
- Many of the planned clinical trial sites for VBP301 overlap with VBP101, which should optimize clinical trial site initiation.
- The protocol allows for patients who have received a trem-cel transplant on the VBP101 study to enroll onto VBP301 and receive VCAR33^{ALLO}. This may provide valuable early insights into the potential of the Company's Treatment System combining trem-cel and VCAR33^{ALLO} to enable a more potent therapy and durable responses post-transplant.

In-house clinical manufacturing of VCAR33^{ALLO} ready to be initiated; First engineering runs in preparation for Trem-cel tech transfer completed

- The Company recently completed Current Good Manufacturing Practices (cGMP) qualification activities at its new in-house manufacturing facility. The site can now initiate clinical manufacturing of VCAR33^{ALLO} for the VBP301 clinical trial.
- The first engineering runs required in preparation for tech transfer of trem-cel to Vor Bio's in-house facility have been completed. The Company is on-track to commence in-house trem-cel manufacturing in 2023 and will continue to leverage a third-party to provide manufacturing redundancy.

Secured worldwide non-exclusive license to Cas9 gene-edited HSCs from Editas Medicine, Inc.
The company secured a worldwide non-exclusive license from Editas Medicine for ex-vivo Cas9 gene-edited HSC therapies for the treatment and/or prevention of hematological malignancies. The license provides access to key intellectual property for the continued development and commercialization of edited HSCs including trem-cel (VOR33), with the option to elect additional product candidate targets within the next five years.

Upcoming Milestones

- VBP101 clinical data update including engraftment data for additional patients and further Mylotarg hematologic protection expected by year-end 2023
- In-house manufacturing of trem-cel operational by year-end 2023

Second Quarter 2023 Financial Results

- **Cash Position:** Cash, cash equivalents and marketable securities were $186.9 million as of June 30, 2023, which is projected to fund operations into the first quarter of 2025.

- **Research & Development (R&D) Expenses:** R&D expenses for the second quarter of 2023 were $23.9 million, compared to $15.3 million for the second quarter of 2022. The increase in R&D expenses was primarily due to an increase in clinical, manufacturing and consulting expenses of $4.7 million as a result of the ongoing trem-cel clinical trial and the development of our VCAR33 programs, an increase in personnel expenses of $2.5 million, and an increase in facility costs from our laboratory and cGMP manufacturing facility expansion of $1.4 million.

- **General & Administrative (G&A) Expenses:** G&A expenses for the second quarter of 2023 were $8.3 million, compared to $6.5 million for the second quarter of 2022. The increase in G&A expense was primarily due to an increase in personnel costs of $1.4 million, including an increase in stock-based compensation expense of $1.0 million, and an increase in facilities and other expenses of $0.4 million.

- **Net Loss:** Net loss for the second quarter of 2023 was $30.0 million, compared to $21.7 million for the second quarter of 2022.

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

	June 30, 2023	December 31, 2022
Cash, cash equivalents and marketable securities	$ 186,902	$ 230,245
Total assets	250,986	299,366
Total liabilities	46,536	48,759
Total stockholders' equity	204,450	250,607

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

	Three Months Ended June 30,		Six Months Ended June 30,	
	2023	2022	2023	2022
Operating expenses:				
Research and development	$ 23,897	$ 15,333	$ 45,812	$ 30,613
General and administrative	8,277	6,459	16,784	13,979
Total operating expenses	$ 32,174	$ 21,792	$ 62,596	$ 44,592
Loss from operations	$ (32,174)	$ (21,792)	$ (62,596)	$ (44,592)
Other income:				
Interest income	2,195	133	4,184	196
Total other income	2,195	133	4,184	196
Net loss	$ (29,979)	$ (21,659)	$ (58,412)	$ (44,396)
Net loss per share attributable to common stockholders, basic and diluted	$ (0.45)	$ (0.58)	$ (0.88)	$ (1.19)
Weighted-average common shares outstanding, basic and diluted	67,033,150	37,437,063	66,651,547	37,365,647

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 in clinical trials and the availability of data therefrom, the success and timing of manufacturing clinical supply for its product candidates at its in-house manufacturing facility, the potential of overlapping clinical trial sites for VBP301 and VBP101 to optimize clinical trial site initiation, its intentions to use VCAR33^{ALLO} in combination with trem-cel as a Treatment System and the potential benefits of such 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; 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.

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