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

November 7, 2023

- *Next trem-cel (VOR33) clinical data update expected by Relapse After Transplant and Cellular Therapy (HSCT^2) Conference Nov 10-11*
- *Three oral abstracts and two poster presentations accepted at ASH 2023 Dec 9-12*

CAMBRIDGE, Mass., Nov. 07, 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 September 30, 2023, and provided a business update.

"We look forward to imminent data releases by HSCT ^{2} and ASH demonstrating progress across our clinical programs and platform. Following successful clearance of our IND application in June, we are pleased that the VBP301 clinical trial of VCAR33^{ALLO} is now actively enrolling patients," said Dr. Robert Ang, Vor Bio's President and Chief Executive Officer.

Corporate Updates

VBP101 trem-cel clinical data update expected by the ASTCT-EBMT 6th International Conference on Relapse After Transplant and Cellular Therapy (HSCT^2), Nov 10-11

- The planned update will include engraftment data for additional patients receiving a trem-cel transplant and further data from patients treated with multiple doses of Mylotarg^{TM}.
- The data was selected for an oral presentation at ASH 2023.

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 with acute myeloid leukemia (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^{AUTO} (CD33CART) data released by PTCTC in ASH abstract supports Vor Bio's VCAR33 ^{ALLO}

- Data from the Pediatric Transplantation and Cellular Therapy Consortium (PTCTC)[1] regarding the ongoing Phase 1/2 pediatric bridge-to-transplant trial evaluating CD33CART (also known as VCAR33^{AUTO}) in adolescent and young adult relapsed/refractory AML patients was recently released in an abstract accepted for oral presentation at ASH 2023. Nineteen patients were treated in the Phase 1 portion of the study. The data showed that 2 out of 5 (40%) evaluable patients achieved complete remission at the highest dose level (DL4, 1 x 10^7 CAR$^+$ cells/kg), suggesting that this is a potentially active CAR-T product candidate. Four of 19 total patients treated had cytokine release syndrome (CRS) ≥ Grade 3.
- Vor Bio's VCAR33 ^{ALLO} uses the same CAR-T construct used in CD33CART. However, VCAR33^{ALLO} uses a potentially superior T cell source from healthy transplant donors, which are likely to have a more stem-like phenotype and greater potential for expansion, persistence, and anti-leukemic activity compared to a product derived from autologous sources.
- Enrollment is ongoing in VBP301, a Phase 1/2, multicenter, open-label, first-in-human study of VCAR33^{ALLO}, a transplant donor-derived anti-CD33 CAR-T cell therapy in patients with post-transplant relapsed or refractory AML.

Continuing to build industry-leading engineered HSC platform

- The preclinical and process development research that supported Vor Bio's Investigational New Drug (IND) application for trem-cel demonstrating the potential for next generation transplants in AML using CRISPR/Cas9 CD33-edited hematopoietic stems cells was published in *Molecular Therapy - Methods & Clinical Development* online on October 12, 2023. The full manuscript can be accessed HERE.
- Data presented at the SITC 38th Annual Meeting, held November 3-5, 2023, in San Diego, CA showed potent *in vitro* and *in vivo* cytolytic activity of Vor Bio's antigen-specific CLL-1-directed CAR-T cells, supporting further clinical development of the company's lead CLL-1 CAR candidate either as a stand-alone treatment or in combination with its eHSC platform.
- Data presented at the 30th Annual ESGCT Congress, held October 24-27, 2023, highlighted Vor Bio's advanced computational biology expertise, showing development of a proprietary computational workflow known as GUMM (Genotyping Using Mixture Models) that interprets single-cell DNA sequencing data from gene editing experiments and confirms the edits were correctly made. GUMM addresses existing analytical challenges in using single-cell DNA sequencing by streamlining the genotyping process, adopting an automated data-driven approach which requires minimal

user intervention.

Expanding scientific advisory board

The Company is adding academic pioneers in engineering stem cell transplants to protect against on-target toxicity to its scientific advisory board.

- Saar Gill, MD, PhD, Scientific Co-Director, Cell Therapy and Transplant Program and Associate Professor of Medicine (Hematology-Oncology) at the Hospital of the University of Pennsylvania.
- Pietro Genovese, PhD, Assistant Professor in Pediatrics at the Harvard Medical School and Principal Investigator at the Gene Therapy Program at Dana-Farber/Boston Children's Cancer and Blood Disorders Center.

Upcoming Milestones

- VBP101 clinical data update including engraftment data for additional patients and further Mylotarg hematologic protection expected by HSCT^2, Nov 10-11
- In-house manufacturing of trem-cel operational by year-end 2023

Third Quarter 2023 Financial Results

- **Cash Position:** Cash, cash equivalents and marketable securities were $160.1 million as of September 30, 2023, which is projected to fund operations into the first quarter of 2025.

- **Research & Development (R&D) Expenses:** R&D expenses for the third quarter of 2023 were $27.6 million, compared to $16.9 million for the third quarter of 2022. The increase in R&D expenses of $10.7 million was primarily due to an increase in external research and development costs of $5.1 million driven by the execution of our non-exclusive license agreement with Editas Medicine and continued development of our platform, an increase in external manufacturing expenses of $1.8 million, an increase in clinical expenses of $0.8 million related to our ongoing trem-cel clinical trial and the development of our VCAR33 programs, and an increase in personnel expenses of $1.9 million.

- **General & Administrative (G&A) Expenses:** G&A expenses for the third quarter of 2023 were $7.7 million, compared to $7.2 million for the third quarter of 2022. The increase in G&A expense of $0.5 million was primarily due to an increase in personnel costs of $0.8 million, including an increase in stock-based compensation expense of $0.3 million, offset by a decrease in facilities and other expenses of $0.3 million.

- **Net Loss:** Net loss for the third quarter of 2023 was $33.2 million, compared to $23.8 million for the third quarter of 2022.

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

	September 30, 2023	December 31, 2022
Cash, cash equivalents and marketable securities	$ 160,098	$ 230,245
Total assets	223,023	299,366
Total liabilities	48,242	48,759
Total stockholders' equity	174,781	250,607

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2023	2022	2023	2022
Operating expenses:				
Research and development	$ 27,606	$ 16,875	$ 73,418	$ 47,488
General and administrative	7,710	7,226	24,494	21,205
Total operating expenses	$ 35,316	$ 24,101	$ 97,912	$ 68,693
Loss from operations	$ (35,316)	$ (24,101)	$ (97,912)	$ (68,693)
Other income:				
Interest income	2,126	313	6,310	509
Total other income	2,126	313	6,310	509
Net loss	$ (33,190)	$ (23,788)	$ (91,602)	$ (68,184)
Net loss per share attributable to common stockholders, basic and diluted	$ (0.49)	$ (0.63)	$ (1.37)	$ (1.81)

Weighted-average common shares outstanding, basic and diluted	67,607,713	38,009,022	66,973,771	37,582,463

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, 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, the potential superiority of the T-cell source of VCAR33^{ALLO} compared to VCAR33^{AUTO}, 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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