Vor Bio Reports Second Quarter 2022 Financial Results and Provides Company Update

August 11, 2022

- *Initial clinical data for patients treated with Vor Bio's engineered HSC candidate VOR33 on track for Q4 2022*
- *In-house manufacturing facility in Cambridge, MA operational in Q4 2022*
- *Data presented at EHA successfully demonstrates in-vivo engraftment of multiplex edited HSCs for next-generation AML treatment*
- *Cash runway extended into Q1 2024*

CAMBRIDGE, Mass., Aug. 11, 2022 (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, 2022, and provided a business update.

"We are on track to disclose first-in-human clinical data for our lead product candidate VOR33 in Q4 this year," said Dr. Robert Ang, Vor Bio's President and Chief Executive Officer. "We believe this data will provide clinical proof-of-concept for VOR33 and our proprietary platform, which has the potential to transform outcomes for patients with blood cancers."

Corporate Highlights

Vor Bio's in-house clinical manufacturing facility in Cambridge, MA headquarters to become operational in Q4 2022 . The facility is designed to support clinical manufacturing for both Vor Bio's eHSC and CAR-T product pipeline and reduce the time and cost required to manufacture cell therapy clinical candidates.

Successfully demonstrated engraftment of multiplex edited Hematopoietic Stem Cells (HSCs). Pre-clinical data recently presented at the European Hematology Association Congress, June 9-12, demonstrated that multiplex deletion of CD33 and CLL-1 from human CD34+ hematopoietic stem and progenitor cells (HSPCs) maintained cell function and persisted long-term post engraftment *in vivo*, with a high-level of editing, no counterselection, and minimal translocation risk when compared to unedited control cells. In addition, genetically modifying HSPCs to remove select cell surface targets did not appear to impair function and these dual engineered cells showed significant protection from targeted immunotherapy *in vitro*. A link to the presentation can be found on the Company's website at: https://www.vorbio.com/publications/

Program Updates

VOR33: VOR33 is the Company's lead product candidate consisting of genome-edited HSPCs that have been engineered to lack the CD33 protein. It is designed to replace standard of care transplants for patients suffering from acute myeloid leukemia (AML) and potentially other blood cancers. VOR33 has been granted Orphan Drug and Fast Track designations from the U.S. Food and Drug Administration.

- The Company is on track to share initial clinical proof-of-concept data in Q4 of 2022 from VBP101, its Phase 1/2a multicenter, open-label, first-in-human study of VOR33 in patients with AML who are at risk of relapse. Successful engraftment would provide important validation for Vor Bio's platform.
- Patients receive Mylotarg™, an antibody-drug conjugate, around day 60 post-transplant. Initial data on VOR33's ability to provide hematologic protection from Mylotarg toxicities is expected to follow shortly after initial engraftment data.
- The clinical trial continues to recruit patients with six trial sites currently active.
- The enrollment of the first three patients in VBP101 is staggered such that prior to treating the next patient, each patient must demonstrate neutrophil recovery at 28 days after Hematopoietic Cell Transplantation (HCT) and VOR33 infusion. Additionally, dose escalation of Mylotarg, which is being executed using a 3+3 design, is staggered for each of the first three patients and the first patient at each dose escalation.

VCAR33 Programs: The VCAR33 programs are chimeric antigen receptor T (CAR-T) cell therapy candidates designed to target CD33, a clinically-validated target for AML.

VCAR33^{ALLO} uses allogeneic healthy donor-derived cells and is Vor Bio's lead VCAR33 program in which the Company plans to submit an investigational new drug (IND) application in the first half of 2023 to support a Phase 1/2 clinical trial for patients with relapsed/refractory AML. The scientific community has an increasing appreciation for the value of stem-like cell phenotype in CAR-T approaches, and HLA-matched healthy donor cells have a potentially superior cell phenotype with improved persistence and *in vivo* expansion capability.

VOR33 + VCAR33 Treatment System: The combination of VOR33 followed by treatment with VCAR33 in the post-transplant setting could potentially transform patient outcomes and offer the potential for cures for patients who have limited treatment options. The VOR33 + VCAR33 Treatment System will use the same healthy donor allogenic cell source for both VOR33 and VCAR33. The Company's development pathway is to collect initial data on VOR33 from the VBP101 clinical trial and initial clinical data from the VCAR33^{ALLO} program prior to IND submission for this Treatment System.

VOR33-CLL1 + VCAR33-CLL1 Treatment System: The Company continues to make progress on preclinical and IND-enabling studies for its multiplex-edited program. Knocking out CD33 and CLL-1 through gene editing offers a promising new approach to treating patients with AML using

Vor Bio's novel eHSC platform, which can be combined with a multi-specific CAR-T approach.

Upcoming Milestones

- Initial VOR33 clinical data on-track for Q4 of 2022
- In-house clinical manufacturing facility on-track to be operational in Q4 2022
- VCAR33^{ALLO} IND submission expected in the first half of 2023
- Data from the Phase 1/2 National Marrow Donor Program (NMDP)-sponsored trial evaluating VCAR33^{AUTO} expected in 2022
- VOR33 + VCAR33 Treatment System IND submission expected following initial clinical data from the VBP101 clinical trial and the VCAR33^{ALLO} program

Second Quarter 2022 Financial Results

- **Cash Position:** Cash, cash equivalents and investments were $151.1 million as of June 30, 2022, which is anticipated to fund operations into the first quarter of 2024.

- **Research & Development (R&D) Expenses:** R&D expenses for the second quarter of 2022 were $15.3 million, compared to $13.0 million for the second quarter of 2021. The increase in R&D expenses was primarily due to an increase in employee headcount necessary to support the growth of our R&D efforts and in facility costs and other expenses as a result of our laboratory and cGMP manufacturing facility expansion.

- **General & Administrative (G&A) Expenses:** G&A expenses for the second quarter of 2022 were $6.5 million, compared to $5.4 million for the second quarter of 2021. The increase in G&A expenses was primarily due to increases to personnel-related costs and stock-based compensation, professional fees and facility costs and other expenses as a result of our corporate headquarters office expansion.

- **Capital Expenditures and Facility Expansion:** During the second quarter of 2022, spend increased due to one-time capital expenditures and advancements related to the build-out of our laboratory and in-house clinical manufacturing facility.

- **Net Loss:** Net loss for the second quarter of 2022 was $21.7 million, compared to $18.4 million for the second quarter of 2021.

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," "believe," "continue," "could," "estimate," "expect," "intend," "may," "plan," "potential," "project," "should," "target," "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 its upcoming milestones, including timing with respect to initial clinical data for VOR33, submission of an IND for the VCAR33^{ALLO} program, receipt of data from the Phase 1/2 NMDP-sponsored trial evaluating VCAR33^{AUTO}, the submission of an IND for the VOR33 + VCAR33 Treatment System and its in-house clinical manufacturing facility being operational, as well as its cash, cash equivalents and investments, 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 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)

	June 30, 2022	December 31, 2021
Cash, cash equivalents and marketable securities	$ 151,088	$ 207,469
Total assets	224,615	242,590

Total liabilities	49,641	26,327
Total stockholders' equity	174,974	216,263

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

	Three Months Ended June 30,		Six Months Ended June 30,	
	2022	2021	2022	2021
Operating expenses:				
Research and development	$ 15,333	$ 12,970	$ 30,613	$ 21,911
General and administrative	6,459	5,410	13,979	10,199
Total operating expenses	21,792	18,380	44,592	32,110
Loss from operations	(21,792)	(18,380)	(44,592)	(32,110)
Other income (expense):				
Interest income	133	10	196	17
Total other income	133	10	196	17
Net loss	$ (21,659)	$ (18,370)	$ (44,396)	$ (32,093)
Cumulative dividends on redeemable convertible preferred stock	-	-	-	(1,228)
Net loss attributable to common stockholders	$ (21,659)	$ (18,370)	$ (44,396)	$ (33,321)
Net loss per share attributable to common stockholders, basic and diluted	$ (0.58)	$ (0.50)	$ (1.19)	$ (1.13)
Weighted-average common shares outstanding, basic and diluted	37,437,063	36,843,087	37,365,647	29,593,814

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