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

May 12, 2022

- *Initial clinical data for Vor Bio's engineered HSC candidate VOR33 expected in 2H 2022*
- *Data presented at Keystone Symposia demonstrates significant advancement of novel multiplex editing platform*
- *In-house manufacturing facility in Cambridge, MA operational in 2022*

CAMBRIDGE, Mass., May 12, 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 March 31, 2022, and provided a business update.

"We remain fully focused on generating first-in-human clinical data from VBP101, our Phase 1/2a clinical trial of VOR33 in patients with relapsed/refractory AML, which will provide important proof of concept for our proprietary platform and has the potential to transform outcomes for patients with blood cancers," said Dr. Robert Ang, Vor Bio's President and Chief Executive Officer. "We also continue to make progress on our allogeneic healthy donor-derived CAR-T therapy VCAR33^{ALLO}, which remains on track for IND submission in 1H 2023, and look forward to the opening of our new in-house eHSC and CAR-T clinical manufacturing facility later this year."

Corporate Highlights

Data showcased at Keystone Symposia demonstrates success of several multiplex editing approaches. Data recently presented at the Keystone Symposia Precision Genome Engineering meeting, April 27-May 1, included two posters and a workshop discussion featuring data on multiplex gene editing in human hematopoietic stem cells using various genome editing technologies including base editing. The data show efficient removal of multiple surface antigens with high on-target editing and undetectable translocations, reinforcing the potential of Vor Bio's novel platform and approach.

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

The Company continues to make progress in manufacturing innovation with several recent and upcoming data presentations.

- A poster presentation at the International Society of Cell Therapy (ISCT), May 4-7, evaluated a novel automated negative T cell selection technology, allowing for selection of untouched T cells which resulted in high T cell purity and the potential for successful generation of CAR-T therapies in a highly time efficient manner.
- A poster presentation at the upcoming American Society of Gene & Cell Therapy (ASGCT) 25th Annual Meeting, May 16-19, demonstrates the use of a novel, next generation cGMP enabling, electroporation platform potentially enabling better and more predictive translation from R&D through to clinical scale manufacturing of gene modified hematopoietic stem and progenitor cells.

Links to these presentations are located in the Medical & Scientific Events section of the Company's website.

Program Updates

VOR33: VOR33 is the Company's lead product candidate consisting of genome-edited hematopoietic stem and progenitor cells that have been engineered to lack the CD33 protein. It is designed to replace the standard of care in transplant settings for patients suffering from AML and potentially other blood cancers. VOR33 has been granted Orphan Drug designation and Fast Track designation from the U.S. Food and Drug Administration.

- VBP101 is a Phase 1/2a multicenter, open-label, first-in-human study of VOR33 in participants with AML who are at risk of relapse.
- The clinical trial continues to actively recruit patients and the Company is on track to share initial clinical data from the VBP101 trial in the second half of 2022.

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 a newly announced program in which Vor Bio 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.

VOR33 + VCAR33 Treatment System: The combination of VOR33 followed by treatment with VCAR33^{ALLO} in the post-transplant setting may transform patient outcomes and offer the potential for cures for patients that have limited treatment options. The VOR33 + VCAR33 Treatment System utilizes the same healthy donor allogenic cell source for both VOR33 and VCAR33^{ALLO}. The Company's development pathway for this novel-novel treatment 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 expected in the second half of 2022
- In-house clinical manufacturing facility fully operational in 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

First Quarter 2022 Financial Results

- **Cash Position:** Cash, cash equivalents and investments were $181.6 million as of March 31, 2022, which is anticipated to fund operations into the fourth quarter of 2023.
- **Research & Development (R&D) Expenses:** R&D expenses for the first quarter of 2022 were $15.3 million, compared to $8.9 million for the first quarter of 2021. The increase in R&D expenses was primarily due to an expansion of our clinical and process development studies, an increase in employee headcount necessary to support the growth of our R&D efforts and an increase in facility costs and other expenses.
- **General & Administrative (G&A) Expenses:** G&A expenses for the first quarter of 2022 were $7.5 million, compared to $4.8 million for the first quarter of 2021. The increase in G&A expenses was primarily due to increases in employee headcount, professional fees and facility costs and other expenses.
- **Net Loss:** Net loss for the first quarter of 2022 was $22.7 million, compared to $13.7 million for the first 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 expecting initial clinical data for VOR33 in the second half of 2022, expecting the submission of an IND for the VCAR33^{ALLO} program in the first half of 2023, expecting data from the Phase 1/2 NMDP-sponsored trial evaluating VCAR33^{AUTO} in 2022, expecting the submission of an IND for the VOR33 + VCAR33 Treatment System following initial clinical data from the VBP101 clinical trial and the VCAR33^{ALLO} program and its in-house clinical manufacturing facility being operational in 2022, and its cash, cash equivalents and investments being able to fund its operations into the fourth quarter of 2023. 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)

	March 31, 2022	December 31, 2021
Cash, cash equivalents and investments	$ 181,617	$ 207,469
Total assets	234,269	242,590
Total liabilities	39,822	26,327
Total stockholders' equity	194,447	216,263

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

	Three Months Ended March 31,	
	2022	2021
Operating expenses:		
Research and development	$ 15,280	$ 8,941
General and administrative	7,520	4,789
Total operating expenses	22,800	13,730
Loss from operations	(22,800)	(13,730)
Other income (expense):		
Interest income	63	7
Total other income	63	7
Net loss	$ (22,737)	$ (13,723)
Cumulative dividends on redeemable convertible preferred stock	-	(1,228)
Net loss attributable to common stockholders	$ (22,737)	$ (14,951)
Net loss per share attributable to common stockholders, basic and diluted	$ (0.61)	$ (0.67)
Weighted-average common shares outstanding, basic and diluted	37,293,438	22,263,994

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