Vor Bio Reports Fourth Quarter and Full Year 2021 Financial Results and Provides Company Update

March 14, 2022

- *Initial clinical data for Vor Bio's engineered HSC candidate VOR33 expected in 2H 2022*
- *New program VCAR33^{ALLO} using healthy donor-derived T cells IND submission expected in 1H 2023*
- *Cash runway extended into Q4 2023 providing additional financial flexibility and optionality*

CAMBRIDGE, Mass., March 14, 2022 (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, 2021, and provided a business update.

"2022 is a pivotal year for Vor Bio with a number of catalysts that will continue to validate the potential of our platform," said Dr. Robert Ang, Vor Bio's President and Chief Executive Officer. "We are focused on recruitment and enrollment of patients in VBP101, the Phase 1/2a trial of VOR33, our lead eHSC candidate for patients with acute myeloid leukemia (AML). Beyond VOR33, we are advancing a number of additional exciting programs generated from our platform, including our VCAR33^{ALLO} program which uses allogenic healthy donor cells, a potentially superior cell phenotype compared to autologous approaches. We are excited to generate first-in-human clinical data from VBP101 which we expect will support Vor Bio's unique approach to improve the lives of cancer patients."

Corporate Highlights

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.

- 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.
- COVID-19-related issues delayed trial site readiness resulting in enrollment delays, which are being mitigated by the opening of additional trial sites. The Company now plans to share initial clinical data from the VBP101 trial in the second half of 2022 which will include enrollment progress and initial engraftment data. Successful neutrophil engraftment would demonstrate important proof-of-concept for Vor Bio's platform. Initial data on VOR33 protecting from Mylotarg toxicities is expected to follow shortly after initial engraftment data, with patients receiving Mylotarg within 60 days of transplant.
- In December 2021, the U.S. Food and Drug Administration (FDA) granted VOR33 Orphan-drug designation (ODD). Orphan-drug designation is granted by the FDA to a drug or biologic intended to treat a rare disease or condition that affects fewer than 200,000 individuals in the U.S. ODD granted therapies entitle 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.
- In September 2021, VOR33 received Fast Track designation for the treatment of AML from the U.S. FDA allowing for potential facilitated development and expedited review process.

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 is now made up of two programs with different cell sources.

- **VCAR33^{AUTO}** uses autologous cells from each patient, and is being studied in an ongoing Phase 1/2 clinical trial sponsored by the National Marrow Donor Program (NMDP) in young adult and pediatric patients with relapsed/refractory AML in a bridge-to-transplant study. The Company anticipates that NMDP will share data from this study in 2022.
- **VCAR33^{ALLO}** uses allogeneic healthy donor-derived cells. There has been an increasing appreciation for the value of cell phenotype in CAR-T approaches, and HLA-matched healthy donor cells are a potentially superior cell phenotype with improved persistence and in vivo expansion capability. VCAR33^{ALLO} 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}. Following ongoing discussions with the FDA and alongside improved scientific understanding of the differences in T-cell sources, the Company now plans 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 the Treatment System. Vor Bio believes this approach will be a superior development pathway for this novel-novel treatment combination.

VOR33-CLL1 + VCAR33-CLL1 Treatment System: Vor Bio continues to make progress on this new multiplex-edited program, previously announced in November 2021. 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. The Company plans to share preclinical data on this approach at upcoming scientific meetings in 2022.

Vor Bio's in-house manufacturing facility in Cambridge, MA headquarters to be 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.

Multiple scientific presentations presented at ASH and SITC. Vor Bio presented multiple poster presentations demonstrating the depth of science behind the Company's engineered HSCs and antigen-directed immunotherapy treatments at the Society for Immunotherapy of Cancer's (SITC) 36th Annual Meeting held in November 2021 and at the 63rd American Society of Hematology (ASH) Annual Meeting & Exposition held in December of 2021. Links to these posters and presentations are located in the Medical & Scientific Events section of the Company's website.

Fourth Quarter and Full Year 2021 Financial Results

- **Cash Position:** Cash, cash equivalents and investments were $207.5 million as of December 31, 2021, which now is anticipated to fund operations into the fourth quarter of 2023.

- **Research & Development (R&D) Expenses:** R&D expenses for the fourth quarter of 2021 were $12.7 million, compared to $11.3 million for the fourth quarter of 2020 and for the year ended 2021 were $47.5 million, compared to $31.6 million for the year ended 2020. The increase in R&D expenses was primarily due to an expansion of our clinical and preclinical 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 fourth quarter of 2021 were $5.6 million, compared to $4.3 million for the fourth quarter of 2020 and for the year ended 2021 were $21.5 million, compared to $11.7 million for the year ended 2020. 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 fourth quarter of 2021 was $18.3 million, compared to $15.7 million for the fourth quarter of 2020 and for the year ended 2021 was $68.9 million, compared to $43.3 million for the year ended 2020.

Upcoming Milestones

- Initial VOR33 clinical data expected in the second half of 2022
- VCAR33^{ALLO} IND submission expected in the first half of 2023
- Data from the Phase 1/2 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
- In-house manufacturing facility operational in 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 "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 manufacturing facility being operational in 2022, and the 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)

	December 31, 2021	December 31, 2020
Cash, cash equivalents and investments	$ 207,469	$ 48,539
Total assets	242,590	75,908
Total liabilities	26,327	27,637
Convertible preferred stock	-	107,336
Total stockholders' equity (deficit)	216,263	(59,065)

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

	Three Months Ended December 31,		Year Ended December 31,	
	2021	2020	2021	2020
Operating expenses:				
Research and development	$ 12,693	$ 11,330	$ 47,529	$ 31,618
General and administrative	5,613	4,333	21,489	11,748
Total operating expenses	18,306	15,663	69,018	43,366
Loss from operations	(18,306)	(15,663)	(69,018)	(43,366)
Other income (expense):				
Interest income	54	-	119	29
Total other income	54	-	119	29
Net loss and comprehensive loss	$ (18,252)	$ (15,663)	$ (68,899)	$ (43,337)
Cumulative dividends on redeemable convertible preferred stock	-	(2,170)	(1,228)	(5,925)
Net loss attributable to common stockholders	$ (18,252)	$ (17,833)	$ (70,127)	$ (49,262)
Net loss per share attributable to common stockholders, basic and diluted	$ (0.49)	$ (55.10)	$ (2.10)	$ (230.57)
Weighted-average common shares outstanding, basic and diluted	37,088,835	323,650	33,433,214	213,658

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