Vor Reports Third Quarter 2021 Financial Results and Provides Company Update

November 10, 2021

- *VOR33 on track with initial clinical data expected in the first half of 2022*
- *Announced pipeline expansion with first multi-targeted Treatment System simultaneously targeting CD33 and CLL-1 for AML*

CAMBRIDGE, Mass., Nov. 10, 2021 (GLOBE NEWSWIRE) -- Vor Biopharma (Nasdaq: VOR), a clinical-stage cell and genome engineering company, today reported financial results for the three-month period ended September 30, 2021, and provided a business update.

“Our main focus during the third quarter of 2021 has been on site activation and patient recruitment in the Phase 1/2a trial of VOR33 for patients with AML. The initial data from this trial, expected in the first half of 2022, is intended to provide first-in-human demonstration of CD33 biological redundancy,” said Robert Ang, M.B.B.S., MBA, Vor’s President and Chief Executive Officer. “In addition to advancing our lead program, we are excited to expand our pipeline to include multiplexing and the nomination of our first multiplex-edited eHSC candidate and first multi-specific CAR-T candidate. We also continue to make progress on the build-out of our internal manufacturing facility which will support the development of our pipeline as we work towards our mission of curing blood cancers through cell and genome engineering.”

Corporate Highlights

- **Continued progress on study enrollment and site activation for clinical trial of VOR33 in patients with AML.** 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 offer an alternative to the standard of care in transplant settings for patients suffering from AML and potentially other blood cancers.
    - The Phase 1/2a trial is actively enrolling and recruiting patients. The Company plans to report initial clinical data in the first half of 2022, which will include enrollment progress and initial engraftment on patients enrolled to date. Neutrophil engraftment by day 28 is the primary endpoint of the trial.
    - In September, VOR33 received Fast Track designation for the treatment of AML from the U.S. Food and Drug Administration (FDA) allowing for potential facilitated development and expedited review process.
- **Announced first multi-targeted Treatment System comprising VOR33-CLL1 multiplex-edited eHSC therapy and VCAR33-CLL1 multi-specific CAR-T therapy.** The Company continues to make progress on editing multiple antigens with its eHSC platform. These multiplex-edited eHSCs will allow Vor to push the frontiers of CAR-T therapies. Knocking out CD33 and CLL-1 through gene editing offers a promising new approach to treating patients with AML using Vor’s novel eHSC platform. The Company’s research demonstrates that multiplex genome editing of allogeneic hematopoietic stem cells may represent another exciting strategy to efficiently and safely edit multiple genes in blood stem cells, allowing the potential use of multi-targeted blood cancer therapies.
- **Expanded existing license agreement with Columbia University with addition of an exclusive license to certain base editing technologies.** The agreement supports further advancement of Vor’s novel and proprietary platform for cell and genome engineering including the Company’s multiplex editing programs.
- **Multiple scientific presentations highlighting progress on Vor’s platform at key medical conferences in the second half of 2021.** In October, Vor presented data on its novel eHSC platform at the virtual 28th Annual Congress of the European Society of Gene & Cell Therapy (ESGCT) and plans to present additional posters at the Society for Immunotherapy of Cancer's (SITC) 36th Annual Meeting and 63rd American Society of Hematology (ASH) Annual Meeting & Exposition being held in the fourth quarter. The data presented at these conferences will continue to demonstrate the depth of science behind Vor’s engineered HSCs and antigen-directed immunotherapy treatments. In addition to this data, the Company will present data at ASH supporting the rationale behind the selection of CD33 and CLL-1 as its first multiplex candidate target.
- **Leadership team strengthened with the addition of Dr. Veit Schmelmer as Senior Vice President of Program and Alliance Management.** Dr. Schmelmer brings more than 25 years of experience leading the development of novel therapies for cancer, autoimmune disorders and infectious diseases at leading biopharmaceutical companies. His experience working with HSCs will be helpful as Vor continues to progress its novel eHSC therapeutic candidates through clinical development and to market.
- **Continued progress on build-out of our in-house manufacturing facility at Cambridge, MA headquarters to be operational in 2022**. The facility has been designed to support clinical manufacturing for Vor’s product pipeline and will enable the company to achieve flexible manufacturing capacity and to reduce the time and cost required to manufacture

complex cell and genomic engineering clinical candidates.

Third Quarter 2021 Financial Results

- **Cash Position:** Cash, cash equivalents and investments were $226 million as of September 30, 2021, which is anticipated to fund operations into mid-2023.
- **Research & Development (R&D) Expenses:** R&D expenses for the third quarter of 2021 were $12.9 million, compared to $8.1 million for the third quarter of 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 third quarter of 2021 were $5.7 million, compared to $3.6 million for the third quarter of 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 third quarter of 2021 was $18.6 million, compared to $11.8 million for the third quarter of 2020.

Upcoming Milestones

- On track to report initial VOR33 clinical data in the first half of 2022
- Initial VCAR33 monotherapy clinical data expected in 2022
- In-house manufacturing facility operational in 2022
- File an investigational new drug (IND) application for VOR33/VCAR33 Treatment System in the second half of 2022

About Vor Biopharma

Vor Biopharma 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 Biopharma's statements regarding its upcoming milestones, including reporting initial VOR33 Phase 1/2a clinical trial in the first half of 2022 the submission of an IND for the VOR33/VCAR33 Treatment System in the second half of 2022, the timing of the release of data from the VCAR33 clinical trial, which is dependent on the investigator's timing of data release, and the timing of the operationalization of its internal manufacturing facility in 2022. Vor Biopharma 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 Biopharma'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 Biopharma'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 Biopharma 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)

	September 30, 2021	December 31, 2020
Cash, cash equivalents and investments	$ 225,968	$ 48,539
Total assets	258,632	75,908
Total liabilities	25,675	27,637
Convertible preferred stock	-	107,336
Total stockholders' equity (deficit)	232,957	(59,065)

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

	Three Months Ended September 30, 2021		Nine Months Ended September 30, 2021	
	2021	2020	2021	2020
Operating expenses:				
Research and development	$ 12,925	$ 8,142	$ 34,836	$ 20,288

General and administrative	5,677	3,643	15,876	7,415
Total operating expenses	18,602	11,785	50,712	27,703
Loss from operations	(18,602)	(11,785)	(50,712)	(27,703)
Other income (expense):				
Interest income	48	-	65	29
Total other income	48	-	65	29
Net loss and comprehensive loss	$ (18,554)	$ (11,785)	$ (50,647)	$ (27,674)
Cumulative dividends on redeemable convertible preferred stock	-	(2,185)	(1,228)	(3,755)
Net loss attributable to common stockholders	$ (18,554)	$ (13,970)	$ (51,875)	$ (31,429)
Net loss per share attributable to common stockholders, basic and diluted	$ (0.50)	$ (61.23)	$ (1.62)	$ (177.84)
Weighted -average common shares outstanding, basic and diluted	36,934,311	228,144	32,067,535	176,726

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