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

November 10, 2022

- *Initial clinical data for VOR33 on track for Q4 2022*
- *Eyal C. Attar, M.D. appointed as Chief Medical Officer*
- *Initiated in-house clinical manufacturing at Cambridge, MA headquarters*

CAMBRIDGE, Mass., Nov. 10, 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 September 30, 2022 and provided a business update.

“We continue to actively enroll patients in our lead clinical trial, VBP101 and look forward to sharing important initial clinical proof of concept data from this study by year end,” said Dr. Robert Ang, Vor Bio’s President and Chief Executive Officer. “We are excited that our new in-house clinical manufacturing facility is now operational and our IND for VCAR33 is on-track for submission in the first half of 2023. We remain fully focused on our mission to transform the standard of care for patients with blood cancers.”

Corporate Highlights

Eyal C. Attar, M.D. appointed as Chief Medical Officer. Eyal C. Attar, M.D., has joined Vor Bio as Chief Medical Office. Dr. Attar brings more than 20 years of demonstrated clinical experience including extensive background in hematologic malignancies, with previous roles as a clinician, bench researcher and in clinical development.

Initiated in-house clinical manufacturing at Cambridge, MA headquarters. The new facility provides critical operational and strategic control of manufacturing enabling more efficient development of the Company’s novel cell and genome engineering platform and pipeline of engineered hematopoietic stem cell (eHSC) and CAR-T product candidates. The facility will initially manufacture clinical supply to support the IND for $VCAR33^{ALLO}$ with plans to technology transfer the production of VOR33 to the site.

Successfully demonstrated potential of base and sequential multiplex editing of HSCs for next-generation acute myeloid leukemia (AML) treatment. Preclinical data presented at the European Society of Gene & Cell Therapy (ESGCT) 29th Congress, October 11-14, demonstrated that multiplex deletion of myeloid antigens CD33 and CLL-1 or CD33 and CD123 in human HSCs resulted in long-term engraftment and persistence of editing. These data showed that Vor Bio’s novel platform enabled efficient removal of multiple target antigens from HSCs while managing off-target and translocation events. The presentations can be found on the Company’s website at: https://www.vorbio.com/publications/

Screening and initial validation of single domain CAR binders for CAR-T cell therapies. Research presented at the Society for Immunotherapy of Cancer (SITC), November 8-12, identified novel CAR binders that may yield promising candidates for future *in vitro* and *in vivo* screening of primary CAR-T cell therapies against AML. The presentation can be found on the Company’s website at: https://www.vorbio.com/publications/

VOR33 and VCAR33 granted ATMP status by EMA. VOR33 and VCAR33 received Advanced Therapy Medicinal Product (ATMP) classification by the European Medicines Agency (EMA). ATMPs are medicines for human use that are based on genes, tissues or cells that offer groundbreaking new opportunities for the treatment of disease and injury. ATMP classification confers certain benefits to company sponsors, designed to expedite the research, development, and Marketing Authorisation of these products.

Program Updates

VOR33: VOR33, is a genome-edited hematopoietic stem cell transplant (HSCT), that is lacking 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 the potential to enable powerful targeted therapies in the post-transplant setting including CD33-targeted CAR-Ts.

- 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.
- The clinical trial continues to recruit patients with nine trial sites currently active.

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.
- $VCAR33^{AUTO}$ 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 no longer expects data from this study to be reported in 2022 and timing of data

release is dependent on the investigators conducting the trial.

Upcoming Milestones

- Initial VOR33 clinical data on-track for Q4 of 2022
- VOR33 engraftment and hematologic protection data updates expected in 2023
- VCAR33^{ALLO} IND submission expected in the first half of 2023

Third Quarter 2022 Financial Results

- **Cash Position:** Cash, cash equivalents and investments were $136.1 million as of September 30, 2022, which is anticipated to fund operations into the first quarter of 2024.

- **Research & Development (R&D) Expenses:** R&D expenses for the third quarter of 2022 were $16.9 million, compared to $12.9 million for the third quarter of 2021. The increase in R&D expense was primarily due to increased personnel expenses including an increase in stock compensation expense, facility costs from our laboratory and cGMP manufacturing facility expansion and clinical and manufacturing expenses as a result of the ongoing VOR33 clinical trial.

- **General & Administrative (G&A) Expenses:** G&A expenses for the third quarter of 2022 were $7.2 million, compared to $5.7 million for the third quarter of 2021. The increase in G&A expense was primarily due to increased personnel expenses, including an increase in stock compensation expense.

- **Net Loss:** Net loss for the third quarter of 2022 was $23.8 million, compared to $18.6 million for the third 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,” “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 its potential upcoming milestones, 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; 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.

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

	September 30, 2022	December 31, 2021
Cash, cash equivalents and marketable securities	$ 136,067	$ 207,469
Total assets	206,373	242,590
Total liabilities	47,639	26,327
Total stockholders' equity	158,734	216,263

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2022	2021	2022	2021
Operating expenses:				
Research and development	$ 16,875	$ 12,925	$ 47,488	$ 34,836

General and administrative	7,226	5,677	21,205	15,876
Total operating expenses	$ 24,101	$ 18,602	$ 68,693	$ 50,712
Loss from operations	$ (24,101)	$ (18,602)	$ (68,693)	$ (50,712)
Other income (expense):				
Interest income	313	48	509	65
Total other income	313	48	509	65
Net loss	$ (23,788)	$ (18,554)	$ (68,184)	$ (50,647)
Cumulative dividends on redeemable convertible preferred stock	-	-	-	(1,228)
Net loss attributable to common stockholders	$ (23,788)	$ (18,554)	$ (68,184)	$ (51,875)
Net loss per share attributable to common stockholders, basic and diluted	$ (0.63)	$ (0.50)	$ (1.81)	$ (1.62)
Weighted-average common shares outstanding, basic and diluted	38,009,022	36,934,311	37,582,463	32,067,535

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