Vor Bio Reports Fourth Quarter and Full Year 2022 Financial Results and Provides Company Update

March 23, 2023

- *Initial clinical data supports founding vision that engineered hematopoietic stem cells can enable treatment options after AML transplant*
- *Additional trem-cel data expected by year-end 2023; VCAR33^{ALLO} on track for IND submission in first half of 2023*
- *$116M financing extends expected cash runway into Q1 2025*

CAMBRIDGE, Mass., March 23, 2023 (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, 2022, and provided a business update.

"We are encouraged with the initial proof of concept demonstrated in patients treated in our VBP101 study," said Dr. Robert Ang, Vor Bio's President and Chief Executive Officer. "We remain focused on rapid enrollment and plan to share additional clinical data later this year. Our IND for VCAR33^{ALLO} is on-track for submission in the first half of 2023 which, together with trem-cel, has the potential to transform outcomes for patients with blood cancers."

Corporate Highlights

Initial VBP101 clinical data represents an important milestone for Vor Bio's founding vision and further validates the Company's novel platform. Clinical data presented at the 2023 TANDEM meetings (Transplantation & Cellular Therapy Meetings of ASTCT ™and CIBMTR®) in February 2023 demonstrated sustained hematopoiesis in the first patient treated with trem-cel five months (147 days) post-transplant and Mylotarg (gemtuzumab ozogamicin) was well-tolerated through three cycles of treatment at the initial dose level of 0.5 mg/m^2. Mylotarg first-dose pharmacokinetics revealed 0.5 mg/m^2 achieved Cmax and AUC parameters equivalent to Mylotarg doses of 1-2 and 4-5 mg/m^2, respectively, potentially due to the decreased CD33 antigen sink. CD33 deletion was observed in donor cells of myeloid and lymphoid origin which were both enriched following Mylotarg, suggesting that CD33 is expressed early in hematopoietic differentiation and that Mylotarg treatment effectively removes CD33-positive cells. Due to detectable measurable residual disease (MRD), the patient was moved to other therapies following administration of the third dose of Mylotarg and subsequently relapsed with CD33+ blasts.

A second patient successfully received a trem-cel transplant and showed robust cell recovery with neutrophil engraftment occurring at Day 11 and platelet recovery on Day 17. Trem-cel was well tolerated in both patients.

$116 million financing extends Company's expected cash runway into Q1 2025. In December 2022, Vor Bio announced the pricing of an underwritten offering and a private placement, with combined gross proceeds of approximately $115.8 million. Vor Bio intends to use the net proceeds from the financing primarily to fund the continued clinical development of pipeline programs and for working capital and general corporate purposes.

GMP qualification of in-house clinical manufacturing facility underway. Current Good Manufacturing Practices (cGMP) qualification activities at the new facility are well underway, and the Company is on track to begin clinical manufacturing of VCAR33^{ALLO} post IND submission. The Company plans to commence trem-cel production at the in-house facility in 2023.

Strategic additions to Clinical and Scientific Advisory Board. As the Company continues to evolve toward providing next-generation transplants for patients, it is building out a world-class Clinical and Scientific Advisory Board comprised of luminaries in the field who can provide the Company with deep advisory expertise in genome engineering, hematopoietic stem cell (HSC) biology, cancer immunotherapy and clinical development of therapies to treat blood cancers. Scientific and Clinical Advisors currently include Siddhartha Mukherjee, MD, DPhil; Hans-Peter Kiem, MD, PhD; Malcolm K Brenner, MD, PhD; Steven Devine, MD; Rob Soiffer, MD; Eric Sievers, MD; and Yi-Bin Chen, MD.

For more information, visit our website at: https://www.vorbio.com/about/scientific-clinical-advisors/.

Program Updates

Trem-cel (formerly VOR33): Trem-cel is a genome-edited allogeneic 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. Trem-cel has the potential to enable powerful targeted therapies in the post-transplant setting including CD33-targeted CAR-Ts.

- The Company continues to actively enroll patients into the VBP101 clinical study and is progressing as planned with dose escalation of Mylotarg per the 3+3 schema in the protocol.
- An encore poster presentation of VBP101 data has been accepted at the 49th Annual Meeting of the EBMT to be held in Paris, France, April 23-26, 2023.
- The Company expects to share new data from additional patients transplanted with trem-cel and treated with Mylotarg at scientific/medical forums by year-end 2023.

VCAR33^{ALLO}: VCAR33^{ALLO} is a T-cell therapy derived from allogeneic healthy donors using a chimeric antigen receptor (CAR) specifically binding to

CD33.

- The Company is on-track to submit an IND in the first half of 2023.
- The initial clinical trial will focus on patients who have relapsed following allogeneic stem cell transplant, where T cells harvested from the original donor are used as starting material for the drug product.
- The Company intends to evaluate VCAR33^{ALLO} in combination with trem-cel as a Treatment System, aiming at prolonged remissions or cures following transplant.

Upcoming Milestones

- VCAR33^{ALLO} IND submission expected in the first half of 2023
- Additional trem-cel engraftment and hematologic protection data updates expected by year-end 2023

Fourth Quarter and Full Year 2022 Financial Results

- **Cash Position:** Cash, cash equivalents and investments were $230.2 million as of December 31, 2022, which is projected to fund operations into the first quarter of 2025.
- **Research & Development (R&D) Expenses:** R&D expenses for the fourth quarter of 2022 were $17.1 million, compared to $12.7 million for the fourth quarter of 2021, and for the year ended December 31, 2022, were $64.6 million, compared to $47.5 million for the year ended December 31, 2021. The increase in R&D expenses was primarily due to an increase in personnel expenses, including an increase in stock compensation expense, an increase in facility costs from our laboratory and cGMP manufacturing facility expansion, and an increase in clinical, manufacturing and consulting expenses as a result of the ongoing trem-cel clinical trial and the development of the VCAR33^{ALLO} program.
- **General & Administrative (G&A) Expenses:** G&A expenses for the fourth quarter of 2022 were $7.7 million, compared to $5.6 million for the fourth quarter of 2021, and for the year ended December 31, 2022, were $28.9 million, compared to $21.5 million for the year ended December 31, 2021. The increase in G&A expense was primarily due to increased personnel expenses, including an increase in in stock compensation expense, an increase in facilities and other expenses as a result of our corporate headquarters office expansion, and an increase in professional fees.
- **Net Loss:** Net loss for the fourth quarter of 2022 was $23.9 million, compared to $18.3 million for the fourth quarter of 2021, and for the year ended December 31, 2022, was $92.1 million, compared to $68.9 million for the year ended December 31, 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 the potential of its product candidates to positively impact quality of life and alter the course of disease in the patients it seeks to treat, the timing of patient enrollment in clinical trials and the availability of data therefrom, the timing of regulatory filings, the expected safety profile of its product candidates, the potential cGMP qualification of its manufacturing facility and the success and timing of manufacturing clinical supply for its product candidates, its intentions to use VCAR33^{ALLO} in combination with trem-cel as a Treatment System, its potential upcoming milestones, its intended use of proceeds from capital raising activities, 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
(in thousands)

	December 31, 2022	December 31, 2021
Cash, cash equivalents and marketable securities	$ 230,245	$ 207,469
Total assets	299,366	242,590
Total liabilities	48,759	26,327

Total stockholders' equity	250,607	216,263

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

	Three Months Ended December 31,		Twelve Months Ended December 31,	
	2022	2021	2022	2021
Operating expenses:				
Research and development	$ 17,062	$ 12,693	$ 64,550	$ 47,529
General and administrative	7,663	5,613	28,868	21,489
Total operating expenses	$ 24,725	$ 18,306	$ 93,418	$ 69,018
Loss from operations	$ (24,725)	$ (18,306)	$ (93,418)	$ (69,018)
Other income (expense):				
Interest income	814	54	1,324	119
Total other income	814	54	1,324	119
Net loss	$ (23,911)	$ (18,252)	$ (92,094)	$ (68,899)
Cumulative dividends on redeemable convertible preferred stock	-	-	-	(1,228)
Net loss attributable to common stockholders	$ (23,911)	$ (18,252)	$ (92,094)	$ (70,127)
Net loss per share attributable to common stockholders, basic and diluted	$ (0.53)	$ (0.49)	$ (2.33)	$ (2.10)
Weighted-average common shares outstanding, basic and diluted	45,394,089	37,088,835	39,551,420	33,433,214

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