Vor Reports First Quarter 2021 Financial Results and Provides Company Update

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Successful completion of Initial Public Offering raising $203.4 million in gross proceeds

CAMBRIDGE, Mass., May 06, 2021 (GLOBE NEWSWIRE) -- Vor Biopharma (Nasdaq: VOR or the Company) today reported financial results for the three-month period ended March 31, 2021, and provided an update on its business, including the announcement of the second regulatory authority to allow development of VOR33 in acute myeloid leukemia (AML) patients with the Canadian clinical trial application (CTA) clearance.

"The Vor team remains focused on delivering upon multiple clinical and operational milestones," said Robert Ang, MBBS, MBA, Vor's President and Chief Executive Officer. "We have established a strong foundation to generate several important catalysts over the next 12 to 24 months which will demonstrate the potential of Vor's novel platform. With respect to our lead clinical program, VOR33 in patients suffering from acute myeloid leukemia, we remain on track to enroll the first patient in our Phase 1/2a trial in the second quarter of 2021."

Corporate Highlights

- **Health Canada clears the CTA for VOR33 clinical trial.** In April 2021, the Company received a No Objection Letter from Health Canada for its CTA to conduct a clinical trial for Vor's lead engineered hematopoietic stem cell (eHSC) product candidate, VOR33. This CTA clearance represents the second regulatory authority to allow development of VOR33 in AML patients following the U.S. Food and Drug Administration's clearance of Vor's investigational new drug (IND) application for VOR33 in AML in January 2021. The Canadian CTA utilizes the same protocol as cleared under the IND in the U.S. and data from these patients can be used with data from U.S.-treated patients to expedite development. Vor expects to initiate a first-in-human Phase 1/2a trial of VOR33 in AML patients in combination with Mylotarg™ (gemtuzumab ozogamicin) by enrolling the first patient in the second quarter of 2021. The Company believes VOR33, if successful, has significant potential to improve clinical outcomes for malignant cancers beyond AML.
- **Vor to present on VOR33 manufacturing scale-up and in-depth genomic characterization at the American Society of Gene & Cell Therapy (ASGCT) Annual Meeting, taking place virtually May 11-14, 2021.** On May 11, 2021 at 7:00 PM ET, the Company will conduct an oral presentation titled *VOR33: A Clinic-Ready CRISPR/Cas9 Engineered Hematopoietic Stem Cell Transplant for the Treatment of Acute Myeloid Leukemia.* The study outlined the pre-clinical journey of VOR33 and the manufacturing scale-up for its clinical development. Also on May 11, 2021, between 8:00 AM and 10:00 AM ET, the Company will have a poster presentation titled *Rigorous Assessment of Off-Target Editing by CRISPR/Cas9 in VOR33, an Engineered Hematopoietic Stem Cell Transplant for the Treatment of Acute Myeloid Leukemia.* The in-depth genomic characterization included evaluation of homology sequence based as well as sequence homology independent off-target assessment through deep sequencing of genome-edited DNA. These studies confirmed that CD33-edited HSCs, generated at clinical scale, had no significantly detectable genotoxic risk.
- **Successful completion of Initial Public Offering raising $203.4 million in gross proceeds.** In February 2021, the Company completed its IPO, raising $203.4 million in gross proceeds before deducting underwriting discounts, commissions and other offering expenses.

First Quarter 2021 Financial Results

- **Cash and Cash Equivalents:** Vor finished the first quarter of 2021 with $262.6 million in cash and cash equivalents, compared with $48.5 million as of December 31, 2020. Cash and cash equivalents on March 31, 2021 includes the $186.3 million in net proceeds from the Company's IPO completed in February 2021 as well as $45.4 million in net proceeds from the completion of its Series B preferred stock financing in January 2021.

- **R&D Expenses:** Research and development expenses increased to $8.9 million in the first quarter of 2021, up from $6.2 million in the first quarter of 2020. The increase in research and development expenses was primarily due to an increase in employee headcount necessary to support the growth of our research and development efforts and increases in facilities and other expenses.

- **G&A Expenses:** General and administrative expenses totaled $4.8 million in the first quarter of 2021, up from $1.7 million in the first quarter of 2020, primarily due to an increase in employee headcount, increased professional fees, and an increase in facilities and other expenses.

- **Net Loss:** Vor reported a net loss of $13.7 million in the first quarter of 2021, compared with a net loss of $7.8 million for the first quarter of 2020.

Upcoming Milestones

- Enroll first patient in VOR33 Phase 1/2a trial in the second quarter of 2021
- Initial VOR33 human engraftment and protection data expected in late 2021 or first half of 2022
- Initial VCAR33 monotherapy clinical proof-of-concept data expected in 2022, depending on investigator's timing of data release
- File IND for VOR33/VCAR33 Treatment System in the second half of 2022, following data from our VOR33 clinical trial and the National Marrow Donor Program's Phase 1/2 clinical trial studying the VCAR33 construct

About Vor Biopharma

Vor Biopharma is a cell therapy company that aims to transform the lives of cancer patients by pioneering engineered hematopoietic stem cell (eHSC) therapies to create next-generation, treatment-resistant transplants that unlock the potential of targeted therapies. By removing biologically redundant proteins from eHSCs, we design these cells and their progeny to be treatment-resistant to complementary targeted therapies, thereby enabling these therapies to selectively destroy cancerous cells while sparing healthy cells. For more information please visit www.vorbio.com.

Forward-Looking Statements

This press release contains forward-looking statements within the meaning the Private Securities Litigation Reform Act of 1995. The words "believe," "continue," "could," "estimate," "expect," "intend," "may," "milestones," "plan," "potential," "project," "should," "target," "upcoming," "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 statements regarding the Company's initiation the VOR33 Phase 1/2a clinical trial by enrolling the first patient in the second quarter of 2021, the release of data from such trial, the submission of an IND for the VOR33/VCAR33 Treatment System and the timing of the release of data from the VCAR33 clinical trial. The Company 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 the Company'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 the Company's 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 the Company's most recent Quarterly Report on Form 10-Q, which is on file with the Securities and Exchange Commission (SEC), and in other filings that the Company make with the SEC in the future. Any forward-looking statements contained in this press release speak only as of the date hereof, and the Company 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.

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Condensed Consolidated Balance Sheets
(in thousands, unaudited)

	March 31, 2021	December 31, 2020
Cash and cash equivalents	$ 262,567	$ 48,539
Total assets	292,939	75,908
Total liabilities	25,752	27,637
Convertible preferred stock	-	107,336
Total stockholders' equity (deficit)	267,187	(59,065)

Condensed Consolidated Statement of Operations
(unaudited)

(in thousands, except share and per share amounts)	Three Months Ended March 31, 2021	2020
Operating expenses:		
Research and development	$ 8,941	$ 6,171
General and administrative	4,789	1,707
Total operating expenses	13,730	7,878
Loss from operations	(13,730)	(7,878)
Other income (expense):		
Interest income	7	29
Total other income	7	29
Net loss and comprehensive loss	$ (13,723)	$ (7,849)
Cumulative dividends on redeemable convertible preferred stock	(1,228)	(715)
Net loss attributable to common stockholders	$ (14,951)	$ (8,564)
Net loss per share attributable to common stockholders, basic and diluted	$ (0.67)	$ (59.06)
Weighted-average common shares outstanding, basic and diluted	22,263,994	145,016