Vor Reports Fiscal Year 2020 Financial Results

March 25, 2021

IND accepted for VOR33, Vor's lead eHSC product candidate for treatment of AML

Completed initial public offering raising $203.4 million in total gross proceeds

CAMBRIDGE, Mass., March 25, 2021 (GLOBE NEWSWIRE) -- Vor Biopharma (Nasdaq: VOR) (the Company), a cell therapy company pioneering engineered hematopoietic stem cell (eHSC) therapies combined with targeted therapies for the treatment of cancer, today reported financial results for the year ended December 31, 2020, and provided an update on its business.

"We are very pleased with our continued exceptional scientific, clinical and financial progress," noted Robert Ang, MBBS, MBA, Vor's President and Chief Executive Officer. "Earlier this year, the FDA accepted our IND for our lead eHSC product candidate VOR33, and with the completion of our successful initial public offering, Vor is now in a strong financial position to change the paradigm around targeted oncology treatment and hematopoietic stem cell transplants."

Corporate Highlights

- **Investigational new drug (IND) application accepted for VOR33, Vor's lead eHSC product candidate for treatment of acute myeloid leukemia (AML).** The Company is developing VOR33 as an eHSC product candidate to potentially replace the standard of care in the transplant setting. Vor intends 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 malignancies beyond AML.
- **Successfully completed an initial public offering (IPO).** In February 2020, the Company completed its IPO, raising $203.4 million in gross proceeds before deducting underwriting discounts and commissions and other offering expenses.
- **Vor strengthens platform with CAR-T license and collaborations.** To complement VOR33 and lay groundwork for the VOR33/VCAR33 Treatment System, Vor licensed a CD33-targeted CAR-T construct, VCAR33, from the National Institutes of Health. The Company also announced collaborations with two next-generation gene-editing companies, Arbor Biotechnologies and Metagenomi.

Full Year 2020 Financial Results

- **Cash and Cash Equivalents:** Vor ended 2020 with $48.5 million in cash and cash equivalents, compared with $6.5 million as of December 31, 2019. Cash at December 31, 2020 does not include the $186.3 million in net proceeds from our IPO completed in February 2021 and $45.4 million in net proceeds from the completion of our Series B preferred stock financing in January 2021.

- **R&D Expenses:** Research and development expenses increased to $31.6 million for the year ended December 31, 2020, up from $6.2 million for the prior year. The increase in research and development expenses was primarily due to an increased level of expenses associated with external preclinical studies, consulting fees, laboratory supplies costs and increased employee headcount.

- **G&A Expenses:** General and administrative expenses totaled $11.7 million for the year ended December 31, 2020, up from $4.2 million for the prior year, 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 $43.3 million for the year ended December 31, 2020, compared with a net loss of $10.8 million for the prior year.

Potential 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/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," "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 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 Annual Report on Form 10-K for the year ended December 31, 2020, 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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Consolidated Balance Sheet Data
(in thousands)

	December 31,	
	2020	**2019**
Cash and equivalents	$ 48,539	$ 6,466
Total assets	75,908	9,826
Total liabilities	27,637	2,186
Convertible preferred stock	107,336	25,069
Total stockholders' equity (deficit)	(59,065)	(17,429)

Consolidated Statement of Operations

	Twelve Months Ended December 31,	
(in thousands, except share and per share amounts)	**2020**	**2019**
Operating expenses:		
Research and development	$ 31,618	$ 6,200
General and administrative	11,748	4,217
Total operating expenses	43,366	10,417
Loss from operations	(43,366)	(10,417)
Other income (expense):		
Interest income	29	154
Interest expense related to convertible notes	-	(608)
Change in fair value of derivative liabilities	-	32
Total other income (expense)	29	(422)
Net loss and comprehensive loss	$ (43,337)	$ (10,839)
Cumulative dividends on redeemable convertible preferred stock	(5,925)	(1,773)
Net loss attributable to common stockholders	$ (49,262)	$ (12,612)

Net loss per share attributable to common stockholders, basic and diluted	$ (230.57)	$ (109.70)
Weighted-average common shares outstanding, basic and diluted	213,658	114,961