# Vor Reports Second Quarter 2021 Financial Results and Provides Company Update

August 9, 2021

- *On track to report initial VOR33 clinical data in the first half of 2022*
- *Announced collaborations with Abound Bio and Janssen to develop novel treatment systems for hematologic malignancies*
- *Initiated build-out of in-house clinical manufacturing facility*

CAMBRIDGE, Mass., Aug. 09, 2021 (GLOBE NEWSWIRE) -- Vor Biopharma (Nasdaq: VOR or the Company), a cell and genome engineering company, today reported financial results for the three-month period ended June 30, 2021, and provided a business update.

"Vor is pioneering engineered hematopoietic stem cell (eHSC) therapies combined with targeted cancer therapies, and this quarter, we made several important advancements towards the clinic, and expanded our platform as we continue our mission to change the treatment paradigm in oncology," said Robert Ang, MBBS, MBA, Vor's President and Chief Executive Officer. "We are making progress in our VOR33 Phase 1/2a trial with site initiation activities well underway and we remain on track to release initial clinical data in the first half of 2022. We are proud to have begun construction on our own manufacturing facility, which is another critical milestone in our journey to develop novel treatment systems for patients facing acute myeloid leukemia and other serious blood cancers."

**Corporate Highlights**

- **Expect to enroll first patient in VOR33 acute myeloid leukemia (AML) Phase 1/2a clinical trial in the next few months.** VOR33 is the Company's lead product candidate, consisting of CRISPR genome-edited hematopoietic stem and progenitor cells that have been engineered to lack the CD33 protein. It is designed to replace the standard of care in transplant settings for patients suffering from AML and potentially other hematologic malignancies. The primary endpoint assessing the safety of VOR33 is the incidence of successful engraftment at 28 days. The Company remains on track to report initial clinical data in the first half of 2022.

- **Signed collaborations with Abound Bio and Janssen, expanding Vor's platform to explore additional companion therapy opportunities for eHSCs.** A key strategic initiative for Vor is to pair future engineered hematopoietic stem cell (eHSC) product candidates with companion therapeutics beyond VCAR33.

    - In June, the Company entered into a multi-year strategic collaboration with Abound Bio, a biotechnology company generating novel antibody-based biological therapeutics for cancer, led by world-renowned pioneers in antibody and cell therapy research. Under the terms of the collaboration with Abound Bio, the companies aim to develop single and multi-targeted chimeric antigen receptor T-cell (CAR-T) treatments.

    - In July, the Company formed a collaboration with Janssen Biotech, Inc. (Janssen), one of the Janssen Pharmaceutical Companies of Johnson & Johnson. Under the terms of the collaboration with Janssen, Vor will investigate the combination of its eHSC platform with one of Janssen's bispecific antibodies into a novel treatment system for AML.

- **Initiated build-out of clinical manufacturing facility.** The facility, located at Vor's corporate headquarters in Cambridge, MA, is designed to support clinical manufacturing for Vor's cell therapy programs, including both eHSCs and CAR-T therapeutic candidates, and to be current Good Manufacturing Practice (cGMP) compliant. Vor recently hired clinical manufacturing veterans Joseph Amico Sr. and Michael Pinaud to expand an already industry-leading CMC team and support the build-out of its manufacturing facility. By integrating research, manufacturing and quality control testing capabilities under one roof, Vor aims to achieve flexible manufacturing capacity and to reduce the complexity, time, and cost required to manufacture cell therapy candidates.

- **Building a world-class cell and genome engineering company with the appointment of a new Chairman and promotion of Chief People Officer.** The Company's Board of Directors appointed Matthew R. Patterson to the role of Chairman. Mr. Patterson joined the board in 2020 and brings nearly 30 years of senior leadership experience having most recently served as co-founder and Chief Executive Officer of Audentes Therapeutics, Inc., which was acquired by Astellas Pharma in January 2020. He succeeds Kush M. Parmar, MD, PhD, Managing Partner at 5AM Ventures, who will continue his service to Vor as a member of its Board of Directors. To further support the Company's rapid growth, Tania Philipp was promoted to Chief People Officer where she will continue to help build an industry-leading team with expertise across

multiple disciplines, including scientific research, clinical development and manufacturing.

- **Strong balance sheet to fund operations at least into the first quarter of 2023.** Vor ended the quarter with cash, cash equivalents and investments of $244.6 million that will support operations well beyond important clinical catalysts.

**Second Quarter 2021 Financial Results**

- **Cash Position:** Cash, cash equivalents and investments were $244.6 million as of June 30, 2021.

- **Research & Development (R&D) Expenses:** R&D expenses for the second quarter of 2021 were $13.0 million, compared to $6.0 million for the second quarter of 2020. The increase in R&D expenses was primarily due to an increase in clinical and preclinical studies, an increase in employee headcount necessary to support the growth of our R&D efforts and an increase in facilities and other expenses.

- **General & Administrative (G&A) Expenses:** G&A expenses for the second quarter of 2021 were $5.4 million, compared to $2.1 million for the second quarter of 2020. The increase in G&A expenses was primarily due to an increase in employee headcount, increase in professional fees and an increase in facilities and other expenses.

- **Net Loss:** Net loss for the second quarter of 2021 was $18.4 million, compared to $8.0 million for the second 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, depending on investigator's timing of data release
- 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 cell and genome engineering company that aims to transform the lives of cancer patients by pioneering an engineered hematopoietic stem cell (eHSC) therapeutic platform that unlocks 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 cancer cells while sparing healthy cells.

**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 the Company's statements regarding building-out an internal clinical manufacturing facility at its headquarters, including the capabilities of such facility, and its upcoming milestones, including enrolling a first patient in its VOR33 Phase 1/2a clinical trial within the next few months and reporting initial clinical data from this trial in the first half of 2022, 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 annual or quarterly report and in other reports the Company 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 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.

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

| | June 30, 2021 | December 31, 2020 |
|---|---|---|
| Cash, cash equivalents and investments | $ 244,634 | $ 48,539 |
| Total assets | 275,143 | 75,908 |
| Total liabilities | 25,035 | 27,637 |
| Convertible preferred stock | - | 107,336 |
| Total stockholders' equity (deficit) | 250,108 | (59,065) |

**Condensed Consolidated Statement of Operations**

**(in thousands, except share and per share data)**

| | Three Months Ended June 30, 2021 | | Six Months Ended June 30, 2021 | |
|---|---|---|---|---|
| | 2021 | 2020 | 2021 | 2020 |
| Operating expenses: | | | | |
| Research and development | $ 12,970 | $ 5,975 | $ 21,911 | $ 12,146 |
| General and administrative | 5,410 | 2,065 | 10,199 | 3,772 |
| Total operating expenses | 18,380 | 8,040 | 32,110 | 15,918 |
| Loss from operations | (18,380) | (8,040) | (32,110) | (15,918) |
| Other income (expense): | | | | |
| Interest income | 10 | - | 17 | 29 |
| Total other income | 10 | - | 17 | 29 |
| Net loss and comprehensive loss | $ (18,370) | $ (8,040) | $ (32,093) | $ (15,889) |
| Cumulative dividends on redeemable convertible preferred stock | - | (855) | (1,228) | (1,570) |
| Net loss attributable to common stockholders | $ (18,370) | $ (8,895) | $ (33,321) | $ (17,459) |
| Net loss per share attributable to common stockholders, basic and diluted | $ (0.50) | $ (56.85) | $ (1.13) | $ (115.83) |
| Weighted -average common shares outstanding, basic and diluted | 36,843,087 | 156,454 | 29,593,814 | 150,735 |

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