Vor Bio Doses First AML Patient with VCAR33 (ALLO) and Provides Corporate Update

January 17, 2024

- *First patient dosed in company's second clinical program with initial data expected in second half of 2024*
- *Cash runway extended into second half of 2025 with clinical trials on track*

CAMBRIDGE, Mass., Jan. 17, 2024 (GLOBE NEWSWIRE) -- Vor Bio (Nasdaq: VOR), a clinical-stage cell and genome engineering company, announced today it has dosed the first patient in VBP301, its Phase 1/2, multicenter, open-label, first-in-human study of VCAR33^{ALLO}. The Company has extended its cash runway into the second half of 2025.

"We are pleased to start 2024 with strong execution and we look forward to sharing initial clinical data from the VBP301 trial later this year," said Dr. Eyal Attar, Vor Bio's Chief Medical Officer. "We are excited to bring to AML patients, for the first time, a healthy transplant donor-derived CAR-T developmental therapy which may overcome shortfalls seen with either autologous or allogeneic off-the-shelf approaches."

VBP301 Clinical Trial/VCAR33^{ALLO}

The first patient with relapsed/refractory acute myeloid leukemia (AML) has been dosed with VCAR33^{ALLO} in the VBP301 clinical trial, a significant milestone demonstrating that VCAR33^{ALLO} can be successfully manufactured in Vor Bio's in-house manufacturing facility.

VCAR33^{ALLO} is manufactured from lymphocytes collected from the patient's original transplant donor, generating a CAR-T cell product that is exactly matched to the recipient's engrafted blood system. By using healthy transplant donor cells as the starting material to produce VCAR33 ^{ALLO}, the CAR-T cells have a more stem-like phenotype, leading to greater potential for expansion, persistence, and anti-leukemia activity compared to a product derived from a patient's own lymphocytes.

While this first patient had relapsed after a standard-of-care transplant, patients who have relapsed after a trem-cel transplant are also eligible to enroll in the VBP301 protocol and to receive VCAR33^{ALLO}. The ability to treat relapsed trem-cel transplant patients with VCAR33^{ALLO} may provide valuable early insights into the potential of the Company's trem-cel + VCAR33 Treatment System, which pairs VCAR33 after trem-cel to reduce the risk of relapse or treat evidence of relapse. Initial data from VBP301 is expected in the second half of 2024.

Corporate Update

The Company has further extended its cash runway into the second half of 2025 through an internal review and prioritization process and will continue to invest in its platform and clinical programs while strategically prioritizing late-stage programs and managing employee growth to preserve cash.

About AML

AML is the most common type of acute leukemia in adults and one of the deadliest and most aggressive blood cancers, affecting 20,000 newly diagnosed patients each year in the United States. Approximately half of patients with AML who receive a hematopoietic cell transplant suffer a relapse of their leukemia where their two-year survival rates are less than 20%. Transplanted hematopoietic stem cells are fragile following transplant, preventing the use of potentially curative treatment options.

About VCAR33^{ALLO}

VCAR33^{ALLO} is a CD33-directed CAR-T cell therapy made from healthy cells obtained from the same donor from which the patient was previously transplanted. VBP301 is a Phase 1/2, multicenter, open-label, first-in-human (FIH) study of VCAR33^{ALLO} in patients with relapsed or refractory AML after standard-of-care transplant or a trem-cel transplant. The trial is designed to test the hypothesis that a CD33-targeted CAR-T derived from a healthy donor can be safely administered to a patient with AML who has relapsed after transplant and that the CAR-T can demonstrate anti-leukemia activity. For more information, visit: https://www.clinicaltrials.gov/study/NCT05984199.

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 and pace of patient enrollment in clinical trials and the availability of data therefrom, the expected safety profile of its product candidates, the potential of trem-cel to enable targeted therapies in the post-transplant setting including Mylotarg and CD33-targeted CAR-Ts, the potential of the Company's Treatment System, and the Company's expected cash runway. 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; whether successful engraftment and platelet recovery will ultimately lead to efficacy of trem-cel; the uncertainty of 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.

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