Successful Primary Engraftment of Trem-cel in First Five AML Patients Demonstrates Promise of Vor Bio's Platform

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- *Patients transplanted with trem-cel achieved primary neutrophil engraftment and high levels of myeloid donor chimerism.*
- *Strong investigator enthusiasm and continued robust enrollment; additional data updates expected by year-end 2023.*
- *U.S. FDA clears VCAR33^{ALLO} IND.*
- *Conference call scheduled for today, June 9 at 8:30am ET.*

CAMBRIDGE, Mass., June 09, 2023 (GLOBE NEWSWIRE) -- Vor Bio (Nasdaq: VOR), a clinical-stage cell and genome engineering company, today presented updated clinical data from patients treated in VBP101, its Phase 1/2a multicenter, open-label, first-in-human study of trem-cel (formerly VOR33) in patients with acute myeloid leukemia (AML). These data were presented by Guenther Koehne, MD, PhD, Deputy Director and Chief of Blood & Marrow Transplant and Hematologic Oncology at Miami Cancer Institute of Baptist Health South Florida in a poster presentation at the European Hematology Association (EHA) 2023 Congress in Frankfurt, Germany.

"Based on this interim update of five patients treated, we remain confident in the potential of our approach to enable targeted therapies post-transplant. Investigator enthusiasm is strong and proposal of patients for enrollment in the study currently exceeds the enrollment stagger. We look forward to sharing further engraftment and hematologic protection data from additional patients treated by year-end," said Eyal Attar, MD, Vor Bio Chief Medical Officer.

"The results being presented today are very encouraging. The unmet medical need, particularly for high-risk AML, is significant and this approach, if approved, could potentially transform outcomes for these patients," said Dr. Koehne.

The time for neutrophil engraftment in all five patients treated with trem-cel (10-11 days) was similar to unedited transplants, suggesting that CD33 may be biologically dispensable. All patients achieved high levels of myeloid donor chimerism by day 28.

After achieving timely neutrophil engraftment and platelet recovery, patient 2 experienced secondary graft failure coincident with a detected coronavirus hKU1 infection and following administration of antimicrobial agents including trimethoprim-sulfamethoxazole, both of which may be associated with graft failure. A backup graft was administered, and neutrophil engraftment and platelet recovery were observed.

Patient 3 achieved timely neutrophil engraftment at Day 10, however platelets are still recovering. A platelet-reactive antibody was identified, and the patient is being treated for autoimmune thrombocytopenia. Platelets are steadily increasing and are at 15,000 per µL independent of transfusions.

Patients 4 and 5 achieved normal neutrophil engraftment and platelet recovery, providing further confidence in the Company's platform and approach. These patients have not experienced any unexpected adverse events to date.

Cell doses for all five patients were successfully manufactured and met release criteria, and all had a high level of CD33 editing efficiency.

As previously reported, neutrophil and platelet cell counts were maintained in patient 1 who received multiple Mylotarg doses at 0.5 mg/m^2, suggesting protection from Mylotarg-induced hematotoxicity.

U.S. FDA clears VCAR33^{ALLO} IND

The Company also announced today that the U.S. Food and Drug Administration (FDA) has cleared its Investigational New Drug (IND) application for VCAR33^{ALLO}, a T-cell therapy derived from allogeneic healthy donors using a chimeric antigen receptor (CAR) specifically binding to CD33. VCAR33^{ALLO} is planned to be studied in the VBP301 clinical trial, which 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.

Conference Call & Webcast Information

Members of the Vor Bio management team, joined by Dr. Guenther Koehne, will conduct a live conference call and webcast today at 8:30 am Eastern Time.

Listeners can register for the webcast via this LINK.

Analysts wishing to participate in the Q&A session should use this LINK.

A replay of the webcast will be available via the investor section of the Company's website at www.vorbio.com approximately two hours after the call's conclusion.

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 (HCT) suffer a relapse of their leukemia, with two-year survival rates of less than 20%, and relapse rates are higher for patients with certain adverse risk features. The fragility of engrafted hematopoietic stem cells prevents treatment following transplant, giving the cancer a chance to return.

About the VBP101 Clinical Trial
VBP101 is a Phase 1/2a, multicenter, open-label, first-in-human study of trem-cel (VOR33) in participants with AML who are undergoing human leukocyte antigen (HLA)-matched allogeneic HCT. Trem-cel is an allogeneic CRISPR/Cas9 genome-edited hematopoietic stem and progenitor cell (HSPC) therapy product, lacking the CD33 protein. It is being investigated for participants with $CD33^+$ AML at high risk for relapse after HCT to allow post-HCT targeting of residual $CD33^+$ acute AML cells using Mylotarg (gemtuzumab ozogamicin) without toxicity to engrafted cells. Participants undergo a myeloablative HCT with matched related or unrelated donor CD34-selected HSPCs engineered to remove CD33 expression (trem-cel drug product). Mylotarg is given after engraftment for up to four cycles. The primary endpoint is the incidence of successful engraftment, defined as the first day of 3 consecutive days of absolute neutrophil count (ANC) 500 cells/mm^2 by day 28. Part 1 of this study is evaluating the safety of escalating Mylotarg dose levels to determine the maximum tolerated dose (MTD) and recommended Phase 2 dose. Part 2 will expand the number of participants to evaluate the Mylotarg recommended Phase 2 dose. For more information, visit: https://clinicaltrials.gov/ct2/show/NCT04849910

About Trem-cel
Tremtelectogene empogeditemcel (trem-cel), formerly VOR33, is a genome-edited hematopoietic stem and progenitor allogeneic donor product candidate where CD33 has been deleted using genome engineering. Transplant with trem-cel is designed to replace standard of care transplants for patients suffering from 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-T cells.

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. 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; 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.

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