Vor Bio Presents Positive Data Update on Trem-cel at HSCT² Conference

November 9, 2023

- *Primary neutrophil engraftment of trem-cel achieved in all seven patients treated to date*
- *All three patients receiving multiple Mylotarg™ doses exhibited hematologic protection, further validating Vor Bio's approach*
- *Conference call today, November 9 at 4:30 PM ET*

CAMBRIDGE, Mass., Nov. 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 (VOR33) in patients with acute myeloid leukemia (AML). Guenther Koehne, MD, PhD, Deputy Director and Chief of Blood & Marrow Transplant and Hematologic Oncology at Miami Cancer Institute of Baptist Health South Florida will present these data on November 10, 4:15PM PST, in an oral presentation at the ASTCT/EBMT 6th International Conference on Relapse After Transplant and Cellular Therapy (HSCT²) in Los Angeles, California.

"With this additional hematologic protection data in multiple patients over multiple doses of Mylotarg, we are now seeing the promise of our approach come to light as a potential next-generation therapeutic option for AML patients," said Eyal Attar, MD, Vor Bio's Chief Medical Officer. "Now with VCAR33^{ALLO} in the clinic, we have the potential for an even better combination with trem-cel with the goal of providing durable responses or even a potential cure for AML."

"The results being presented further validate Vor Bio's platform and approach. We are excited to see that trem-cel has worked as expected in these patients, providing them with hematologic protection and exposing their leukemia to targeted therapy," said Dr. Koehne.

Additional data demonstrated successful engraftment of trem-cel in all seven patients

Primary neutrophil engraftment occurred in all seven patients treated to date with trem-cel with a median time to engraftment of 10 days, further providing evidence that CD33 is biologically dispensable. Additionally, platelet recovery occurred at a median of 15.5 days, excluding one patient with previously documented anti-platelet antibodies (immune thrombocytopenia).

Hematologic protection and CD33-negative donor cell enrichment demonstrated in three patients treated with multiple cycles of Mylotarg

All three patients treated with Mylotarg experienced hematologic protection from deep cytopenias through repeat doses, suggesting that trem-cel transplants shielded patients' healthy cells from the on-target toxicity (myelosuppression) typically seen with Mylotarg treatment. The hematological protection exhibited provides support that dose escalation of Mylotarg is warranted and highlights the potential to dose CD33-targeted CAR-T therapy without expected hematologic toxicity.

Mylotarg first-dose pharmacokinetics for the three patients treated showed that the 0.5 mg/m^2 dose was within the exposure range measured for the therapeutic dose of Mylotarg in relapsed/refractory AML patients, potentially due to the decreased CD33 antigen sink in trem-cel patients. In all three patients, the percentage of CD33-negative donor cells increased following Mylotarg administration, suggesting that Mylotarg treatment at the first cohort level of 0.5 mg/m^2 was pharmacologically active and enriched for CD33-edited donor cells.

Next steps in the VBP101 study include dose escalation of Mylotarg to 1.0 mg/m^2 and providing treatment opportunities for trem-cel patients who become measurable residual disease positive or relapse, such as induction-course Mylotarg and VCAR33^{ALLO}.

VCAR33^{AUTO} (CD33CART) study further validates Vor Bio's allogeneic CAR-T

As previously announced, the Pediatric Transplantation and Cellular Therapy Consortium (PTCTC) released data in an ASH abstract from its Phase 1/2 study (NCT03971799)[1] of CD33CART (also known as VCAR33^{AUTO}), which uses the same CAR-T construct as the Company's VCAR33 ^{ALLO}. The data show that 2 of 5 (40%) evaluable patients treated at the highest dose level achieved complete remission with a manageable safety profile (four out of 19 evaluable patients had cytokine release syndrome ≥ Grade 3). This data further validates Vor Bio's approach of using transplant donor cells as CAR-T starting material that are healthy, stem-like, and exactly matched to the patient's immune system.

Conference Call & Webcast Information

Vor Bio management will host a live webcast today at 4:30 PM ET.

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; whether results from preclinical studies and clinical trials of $VCAR33^{AUTO}$ will be replicated or superior in those of $VCAR33^{ALLO}$; 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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[1] Sponsored by the National Marrow Donor Program (NMDP) and Center for International Blood and Marrow Transplant Research (CIBMTR). Funding by St. Baldrick’s Foundation.