



First AML Patient Successfully Transplanted with Vor Bio's Investigational Trem-cel (VOR33) and Tolerated Mylotarg™

December 7, 2022

- Trem-cel (formerly VOR33) successfully manufactured and engrafted normally
- Blood counts successfully maintained following post-transplant treatment with Mylotarg
- Conference call scheduled for today, December 7 at 8:00am ET

CAMBRIDGE, Mass., Dec. 07, 2022 (GLOBE NEWSWIRE) -- Vor Bio (Nasdaq: VOR), a clinical-stage cell and genome engineering company, today announced initial clinical data from VBP101, its Phase 1/2a multicenter, open-label, first-in-human study of tremtelectogene empogeditemcel or "trem-cel" (formerly VOR33) in patients with acute myeloid leukemia (AML). The data observed from the first treated patient support the potential of a trem-cel transplant to be successfully manufactured, to engraft normally, and to maintain blood counts following treatment with the CD33-targeted therapy Mylotarg. The clinical trial continues to enroll patients and additional data are expected in 2023.

"These early engraftment data represent the first time genome engineering has been used to genetically alter donor cells by removing an antigen present on blood cells, thereby allowing treatment using a CD33 targeted therapy while protecting normal blood cells," said Dr. Robert Ang, Vor Bio's President and Chief Executive Officer. "These encouraging data represent the first clinical validation of our platform to potentially enable next-generation transplants for patients with blood cancers. We look forward to sharing additional data updates in 2023."

Trem-cel Displayed Normal Engraftment

A product dose of 7.6×10^6 CD34⁺ viable cells/kg, with a CD33 editing efficiency of 88% was manufactured. Following myeloablative conditioning, trem-cel was infused with no infusion reactions. The patient achieved neutrophil engraftment 10 days post-transplant which was within expectations for CD34-enriched transplants. Platelet recovery was observed on Day 22. Hematopoietic cell sub-population reconstitution was robust with over 90% of peripheral blood cells negative for CD33 expression, and 100% donor chimerism was achieved. These data provide proof-of-concept that trem-cel can engraft as expected and that CD33 does not appear to be biologically necessary for engraftment and hematopoietic reconstitution.

Mylotarg Tolerated at Initial Dose Level

The patient received Mylotarg at a dose of 0.5 mg/m². At this dose, Mylotarg saturates CD33 antigen in patients with relapsed/refractory AML¹, and in the original Phase 1 trial of Mylotarg², neutropenia was observed across dose levels starting at 0.25mg/m² within 14 days of infusion. No treatment related adverse events and no liver enzyme changes were observed through day 20 following Mylotarg dosing. No negative impacts to neutrophil and platelet counts were observed through day 20, suggesting tolerability at this initial dose level.

"The unmet medical need for AML is significant and hematopoietic cell transplant is the best hope for these patients," said Brenda Cooper, M.D., Professor of Medicine in the Cellular Therapy Program at University Hospitals, Seidman Cancer Center, and an investigator in the VBP101 study. "Early treatment data in the first patient show that trem-cel can engraft normally and maintain normal hematopoiesis following Mylotarg dosing, which typically causes severe cytopenias. These data support the promise of this approach."

¹ Mylotarg ODAC 2017

² Sievers 1999 Blood 93:3678

Conference Call & Webcast Information

Members of the Vor Bio management team, joined by Dr. Brenda Cooper, will conduct a live conference call and webcast today at 8:00 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 in participants with AML who are undergoing human leukocyte antigen (HLA)-matched allogeneic hematopoietic cell transplant (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 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/mm² 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 feasibility of a trem-cel transplant to be successfully manufactured, to engraft normally, to maintain blood counts following treatment with Mylotarg following allogeneic hematopoietic cell transplant and to be well tolerated, the potential of Vor Bio’s platform, and timing expectations for additional release of clinical data. 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; 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. The interim data presented in this press release is based on one patient and future results for this patient or additional patients may not produce the same or consistent results. 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.

Contact:

Investors & Media
Sarah Spencer
+1 857-242-6076
sspencer@vorbio.com