Multiple Clinical and Preclinical Presentations at ASH 2023 Highlight Vor Bio's Novel eHSC and CAR-T Platform

November 2, 2023

- *Three oral and two poster presentations accepted by ASH*
- *Company to host call featuring Sarah K. Tasian, MD, Associate Professor of Pediatrics at the University of Pennsylvania School of Medicine and Chief of the Hematologic Malignancies Program in the Division of Oncology at Children's Hospital of Philadelphia, to discuss abstracts today, November 2, at 9.30 AM ET.*

CAMBRIDGE, Mass., Nov. 02, 2023 (GLOBE NEWSWIRE) -- Vor Bio (Nasdaq: VOR), a clinical-stage cell and genome engineering company, today announced that preclinical and clinical data supporting the Company's novel platform and approach for treating acute myeloid leukemia (AML), will be presented at the 65th American Society of Hematology (ASH) Annual Meeting & Exposition, being held from December 9-12, 2023, in San Diego, CA.

"These three oral and two poster presentations represent further preclinical and clinical validation of our platform and reflect the significant progress we continue to make as we develop truly novel next generation transplants that have the potential to improve the lives of patients with blood cancers," said Dr. Robert Ang, Vor Bio's President and Chief Executive Officer.

VBP101 Clinical Data Update
An abstract providing a clinical update from the VBP101 clinical trial (NCT048499910), a Phase 1/2a multicenter, open-label, first-in-human study of trem-cel (VOR33) in patients with AML, was accepted by ASH for oral presentation. This data supports robust neutrophil engraftment of trem-cel and provides evidence of hematologic protection from Mylotarg^{TM}, a CD33-targeted antibody drug conjugate.

An updated data release from VBP101 is expected by the Relapse After Transplant and Cellular Therapy (HSCT²) conference taking place November 10-11, 2023.

CD33CART Study Clinical Data Update
The Pediatric Transplantation and Cellular Therapy Consortium (PTCTC) released data providing a clinical update on a Phase 1/2 study of CD33CART (NCT03971799)[1], an autologous CAR-T therapy targeting CD33 (also referred to as VCAR33^{AUTO}) which uses the same CAR-T construct as VCAR33^{ALLO}. Nineteen pediatric and young adult patients with relapsed/refractory AML with a median age of 16 years were infused in the Phase 1 portion of the study. This data shows that as of the cutoff date of June 1, 2023, 2 of 5 (40%) evaluable patients treated at the highest dose level (DL4, 1 x 10^7 CAR$^+$ cells/kg) achieved complete remission. Transient CD33CART expansion was detected in 11 (58%) subjects across all doses tested and in all 6 (100%) subjects evaluated at DL4, as of the cutoff date. Four out of 19 evauable patients treated had cytokine release syndrome (CRS) ≥ Grade 3. The study is being led by Nirali Shah, MD, MHSc, Head, Hematologic Malignancies Section, Pediatric Oncology Branch, National Cancer Institute and Richard Aplenc, MD, PhD, MSCE, Professor of Pediatrics, Children's Hospital of Philadelphia (CHOP). This abstract was accepted by ASH for oral presentation.

Sarah K. Tasian, MD, is a co-investigator on the PTCTC-supported clinical trial and led the preclinical testing and translation of CD33CART with Terry Fry, MD, at the University of Colorado.

Dr. Tasian commented: "The interim results from our CD33 CAR T cell immunotherapy clinical trial are very encouraging. CD33CART is clearly an active therapy based upon our data to date, and the expansion phase of the study will provide additional critical safety and efficacy data. Our results provide a compelling foundation for Vor Bio's approach using the same construct for their VBP301 study."

Vor Bio's VCAR33 ^{ALLO} uses the same CAR-T construct used in CD33CART. However, VCAR33^{ALLO} uses a potentially superior T cell source from healthy transplant donors, which the Company believes are likely to have a more stem-like phenotype and greater potential for expansion, persistence, and anti-leukemic activity compared to a product derived from autologous sources.

Additional ASH Presentations
The Company also released data from a single cell analysis studying molecular signatures from 28 AML patients in various stages of AML progression. This data is the most comprehensive analysis to date on AML profiling. This abstract was accepted by ASH for oral presentation.

The preclinical collaboration between Vor Bio and Janssen yielded *in vitro* and *in vivo* xenotransplant data demonstrating that CD33-deleted allografts were synergistic with Janssen's CD33 directed immunotherapy candidate (JNJ-67571244), and maintained robust on-target cytotoxicity while reducing production of inflammatory cytokines associated with CRS. This abstract was accepted by ASH for poster presentation.

Lastly, a trial-in-progress poster will be presented on the Company's VBP301 clinical trial, a Phase 1/2 multicenter, open-label, first-in-human study of VCAR33^{ALLO} in patients with relapsed or refractory AML after allogeneic stem cell transplantation. This abstract was accepted by ASH for poster presentation.

Full details of the ASH 2023 presentations are as follows:

Vor Bio Clinical Abstracts

Abstract Title: *Trem-cel, a CRISPR/Cas9 Gene-Edited Allograft Lacking CD33, Shows Rapid Primary Engraftment with CD33-Negative Hematopoiesis in Patients with High-Risk Acute Myeloid Leukemia (AML) and Avoids Hematopoietic Toxicity During Gemtuzumab Ozogamicin (GO) Maintenance Post-Hematopoietic Cell Transplant (HCT)*
Format: Oral presentation
Session Name: Gene Therapies: New Approaches from Bench to Bedside
Session date and time: Sunday, December 10, 2023, 10:00 AM PST

Abstract Title: *Phase 1/2 Study of Donor-Derived Anti-CD33 Chimeric Antigen Receptor Expressing T Cells (VCAR33) in Patients with Relapsed or Refractory Acute Myeloid Leukemia after Allogeneic Hematopoietic Cell Transplantation*
Format: Trial in Progress Poster, #483
Session Name: Cellular Immunotherapies: Early Phase and Investigational Therapies: Poster III
Session date and time: Monday, December 11, 2023, 6:00 PM - 8:00 PM PST

PTCTC Clinical Abstract

Abstract Title: *CD33 CAR T-cells (CD33CART) for Children and Young Adults with Relapsed/Refractory AML: Dose-Escalation Results from a Phase I Multicenter Trial*
Format: Oral presentation
Session Name: Cellular Immunotherapies: Early Phase and Investigational Therapies: Novel Approaches to Enhance Cellular Therapies and Immune Responses in Leukemias and Lymphomas
Session date and time: Monday, December 11, 2023, 11:00 AM PST

Vor Bio Preclinical Abstracts

Abstract Title: *Multimodal Atlas of Paired Diagnosis and Relapse AML Samples Enables Novel Therapeutic Targeting of Surface Antigens*
Format: Oral presentation
Session Name: Acute Myeloid Leukemias: Biomarkers, Molecular Markers and Minimal Residual Disease in Diagnosis and Prognosis: Biomarkers and Therapeutics
Session date and time: Saturday, December 9, 2023, 2:15 PM PST
Abstract Summary: The most comprehensive single cell AML atlas known to date (More than 450,000 cells from 28 AML patients) was generated to identify potential differences in molecular signatures in AML tumor types at multiple stages of AML progression. This extensive AML profiling offers deep insight into cell surface changes during disease progression and reveals the potential for multi-targeted treatment strategies.

Abstract Title: *CD33-Deleted Hematopoietic Cells (trem-cel) are Protected from CD33xCD3 Bispecific Antibody Treatment and Produce Significantly Reduced Levels of Inflammatory Cytokines in Preclinical Studies*
Format: Poster, #3425
Session Name: Experimental Transplantation: Basic and Translational: Poster II
Session date and time: Sunday, December 10, 2023, 6:00 PM - 8:00 PM PST
Abstract Summary: Preclinical proof-of-concept data resulting from the Company's strategic collaboration with Janssen, demonstrated that the *CD33* deleted hematopoietic compartment was protected from Janssen's CD33 directed immunotherapy (JNJ-67571244) both in cytotoxicity assays and xenotransplantation studies, with reduction of inflammatory cytokines associated with CRS. These findings may enable the development of a next-generation AML treatment strategy by pairing a trem-cel transplant with a subsequent CD33-directed bispecific compound that could enhance the safety and effectiveness of the treatment while decreasing the harmful effect on the bone marrow.

Conference Call & Webcast Information
Members of the Vor Bio management team, joined by Sarah K. Tasian, MD, will conduct a live conference call and webcast to discuss the abstracts, today at 9:30 AM ET.

Listeners can register for the webcast via this LINK.

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

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, and the potential superiority of the T-cell source of $VCAR33^{ALLO}$ compared to $VCAR33^{AUTO}$. 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 results from preclinical studies and clinical trials of $VCAR33^{AUTO}$ will be replicated or superior in those of $VCAR33^{ALLO}$, 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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