Vor Bio Provides Clinical Update Further Validating Approach of Using Shielded Transplants to Deliver Targeted Therapies; Receives Supportive Feedback from FDA Regarding Registrational Trial Design

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- *Preliminary data suggests improved relapse-free survival compared to published groups of acute myeloid leukemia (AML) patients at high risk of relapse post-transplant*
- *Trem-cel + Mylotarg continue to demonstrate engraftment, shielding, and broadened therapeutic window*
- *Company has received supportive feedback from the FDA regarding a registrational clinical trial design*

CAMBRIDGE, Mass., Dec. 09, 2024 (GLOBE NEWSWIRE) -- Vor Bio (Nasdaq: VOR), a clinical-stage cell and genome engineering company, today announced updated clinical data from its ongoing Phase 1/2 VBP101 study of patients with relapsed/refractory AML receiving trem-cel followed by Mylotarg™. The data, which was presented in a poster at the American Society of Hematology (ASH) Annual Meeting on Sunday, December 8th, demonstrated durable engraftment, shielding from Mylotarg on-target toxicity, a broadened Mylotarg therapeutic window, and early evidence of improved relapse free survival compared to published high-risk AML comparators.

"With additional maturity, we are even more encouraged by this data and the potential of offering AML and MDS patients the opportunity to receive post-transplant maintenance therapy while still maintaining healthy blood count levels," said Dr. Eyal Attar, Vor Bio's Chief Medical Officer.

The data released today included 25 patients treated with trem-cel of which 15 had received Mylotarg (six at the 2 mg/m^2 dose) as of the data cut-off date of November 1, 2024. The data demonstrated:

- Preliminary evidence of improved relapse-free survival (median RFS not reached with median follow-up duration of 7.4 months) compared to published groups of AML patients at high risk of relapse post hematopoietic stem cell transplant (HCT)[1].
- Shielding of the blood system, with maintained neutrophil and platelet counts across multiple Mylotarg doses of 0.5, 1, and 2 mg/m^2.
- Broadened therapeutic index for Mylotarg when administered after trem-cel.
- Reliable engraftment, with 100% of patients achieving primary neutrophil engraftment (median 9.5 days), robust platelet recovery (median 16 days), and full myeloid donor chimerism at Day 28.
- Trem-cel continues to be manufactured with high CD33 editing efficiency (median 90%, range 71-94%).

Company received supportive feedback from the FDA in a Type C meeting

The Company had the opportunity to interact with the FDA regarding data from the trem-cel + Mylotarg study alongside a proposed registrational clinical trial synopsis. The FDA agreed that trem-cel engrafts neutrophils and platelets and has a similar safety profile to unedited CD34+ grafts. In addition, there was agreement with the trem-cel + Mylotarg registrational clinical trial design with respect to study population, control arm, primary endpoint, stratification factors, and statistical design. The Company agreed to provide further updates to the FDA alongside submission of the full clinical trial protocol.

Conference Call & Webcast Information

Vor Bio management, joined by Guenther Koehne, MD, PhD, an investigator on the VBP101 study and Deputy Director and Chief of Blood & Marrow Transplant and Hematologic Oncology at Miami Cancer Institute of Baptist Health South Florida, will host a live webcast today at 5:00 AM PT / 8:00 AM 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 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, including potential improvements in relapse-free survival, the timing of initiation of clinical trials, the potential of trem-cel to enable targeted therapies in the post-transplant setting including Mylotarg and CD33-targeted CAR-Ts while maintaining healthy blood count levels and change the standard of care for patients with blood cancers, the safety profile of trem-cel plus Mylotarg, the potential design of a registrational trial for trem-cel and plans for regulatory submissions for trem-cel. 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; uncertainties regarding 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 and Vor Bio’s ability to continue as a going concern. 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