Vor Bio Presents Novel Research Highlighting Opportunities and Challenges Facing Institutions Enrolling Patients in Cell and Gene Therapy Trials

February 13, 2025

- *Clinical trial complexity, logistical challenges associated with treatments, and resource constraints at trial sites cited as top factors that delay enrollment into cell and gene therapy (CGT) clinical trials*
- *Despite the increased time required to obtain informed consent, 100% of those surveyed continue to offer patients these innovative investigational treatment options*

CAMBRIDGE, Mass., Feb. 13, 2025 (GLOBE NEWSWIRE) -- Vor Bio (Nasdaq: VOR), a clinical-stage cell and genome engineering company, today presented novel research evaluating the patient experience and barriers to enrollment and participation in cell and gene therapy (CGT) trials. The data, which was presented at the TANDEM Meetings of ASTCT and CIBMTR, demonstrated the need to improve the process and experience of patients considering and enrolling in CGT trials.

“This original research emphasizes the unique challenges involved in offering and educating patients about cell and gene therapy trials and also highlights physician interest in the promise these novel therapies may provide to patients,” said Dr. Eyal Attar, Vor Bio’s Chief Medical Officer. “These learnings will help us improve the patient experience in our own trials and provide a roadmap for everyone involved in the clinical trial enrollment process as we strive to educate patients about these novel, yet often complicated, therapies.”

Despite the growing number of CGT trials, their complexity, logistical challenges including treatment duration and caregiver burden, and the significant resources they require represent important barriers that delay enrollment and limit patient access. The study was designed to investigate these key barriers by evaluating challenges facing patients, clinical staff, and institutions. The study conducted and analyzed surveys and interviews with 30 physicians, study coordinators, research nurses, and other clinicians who participate in the patient consent process, as well as patient advocacy partners.

Key findings from the data:

- Educating patients and enrolling them in CGT trials requires more time compared to non-CGT trials due to their complexity and patients’ need for additional reassurance or emotional support.
- Despite the extra time required to educate patients, this did not prevent any survey participants from offering these trials to patients, underscoring the transformative potential of these treatments.
- Study respondents reported that patients share many concerns about CGT trials, including the fear of unknown risks of gene editing and caregiver requirements.

The results reveal significant opportunities for institutions and trial sponsors to improve the patient experience, streamline the process, and enroll more patients in CGT trials. Clinical trial sponsors can address the complexity of cell and gene therapies and help patients better understand these options by providing more comprehensive educational content beyond the handouts typically shared. Additionally, as hospitals are often limited in how many CGT trials they can offer, adding a specialized CGT research team could potentially expand their capacity to offer more trials and treat more patients.

This research will inform Vor Bio’s communications approach for future clinical trials and how the Company can help support patients and partner institutions through the enrollment process. Vor Bio plans to continue this research to uncover additional learnings that may lead to further improvements in the patient experience and CGT clinical trial enrollment.

The study coauthors include staff at NMDP (National Marrow Donor Program), MDS Foundation, Memorial Sloan Kettering Cancer Center, University Hospitals Cleveland Medical Center, Miami Cancer Institute, and Stanford Medicine.

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