VOR33 Granted U.S. FDA Fast Track Designation for AML

September 9, 2021

CAMBRIDGE, Mass., Sept. 09, 2021 (GLOBE NEWSWIRE) -- Vor Biopharma (Nasdaq: VOR), a clinical-stage cell and genome engineering company, today announced that the U.S. Food and Drug Administration (FDA) has granted Fast Track designation to VOR33, the Company's lead engineered hematopoietic stem cell (eHSC) therapeutic candidate for the treatment of acute myeloid leukemia (AML).

VOR33 consists of CRISPR genome-edited hematopoietic stem and progenitor cells that have been engineered to lack CD33. Once infused, VOR33 is designed to protect patients' healthy cells from anti-CD33 therapies, such as VCAR33 or Mylotarg™ (gemtuzumab ozogamicin). VOR33 is intended to replace standard of care transplants for AML patients who are at high risk of relapse and has the potential to seamlessly integrate into current transplant settings.

"Receiving Fast Track designation is an important milestone for Vor, which signals the FDA's recognition of the serious and life-threatening medical condition of patients facing acute myeloid leukemia and the potential of VOR33 to address this unmet medical need," said Robert Pietrusko, PharmD, Vor's Chief Regulatory and Quality Officer. "We will continue to work closely with the FDA to expedite the development of VOR33, which is now actively enrolling in its Phase 1/2a clinical trial for AML patients who currently have limited treatment options. We continue to remain on-track to report VOR33's initial clinical data in the first half of 2022."

VOR33 is the lead product candidate of Vor's novel scientific platform, which has the mission to create next-generation, treatment-resistant transplants that unlock the potential of targeted cancer therapies by leveraging advances in cell therapy and gene editing. Vor is currently exploring the use of its genome engineered hematopoietic stem cell platform in combination with multiple therapeutic modalities.

Fast Track designation is intended to facilitate development and expedite review of products designed to treat serious and life-threatening conditions with unmet medical needs. The designation is granted upon the FDA's review of data that demonstrate this potential, along with a product development program that is adequately designed to address the unmet medical need. Therapeutic candidates receiving Fast Track designation may be eligible for priority review and accelerated approval if certain conditions are met.

About Vor Biopharma
Vor Biopharma is a clinical-stage cell and genome engineering company that aims to transform the lives of cancer patients by pioneering an engineered hematopoietic stem cell (eHSC) therapeutic platform that unlocks the potential of targeted therapies. By removing biologically redundant proteins from eHSCs, we design these cells and their progeny to be treatment-resistant to complementary targeted therapies, thereby enabling these therapies to selectively destroy cancer cells while sparing healthy cells.

Forward-Looking Statements
This press release contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995. The words "believe," "continue," "could," "estimate," "expect," "intend," "may," "plan," "potential," "project," "should," "target," "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 the Company's statements regarding the potential efficacy of VOR33, potential benefits VOR33 may receive in connection with its Fast Track designation and reporting initial clinical data from the VOR33 Phase 1/2a clinical trial in the first half of 2022. The Company 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 the Company'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 impact of the COVID-19 pandemic on the Company's business, including its preclinical studies and clinical trials and availability of funding sufficient for the Company's 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 the Company's most recent annual or quarterly report and in other reports the Company 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 the Company 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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