Vor To Present on VOR33 Manufacturing Scale-Up and Off-Target Editing at ASGCT

April 28, 2021

An oral presentation on successful scale-up of GMP-like VOR33 manufacturing and a poster presentation on off-target editing are scheduled for Tuesday, May 11

CAMBRIDGE, Mass., April 28, 2021 (GLOBE NEWSWIRE) -- Vor Biopharma (Nasdaq: VOR) is pleased to announce that Vor scientists will give two presentations documenting the development, manufacturing, and validation of its engineered hematopoietic stem cell (eHSC) clinical candidate VOR33 at the American Society of Gene & Cell Therapy (ASGCT) Annual Meeting, taking place virtually May 11-14, 2021.

“As we ready VOR33 for a Phase 1/2a trial, we are pleased to share the first consolidated review of the comprehensive research that underpins this cell therapy candidate and our wider eHSC platform,” said John Lydeard, PhD, scientific lead on the VOR33 program and Head of Target Discovery at Vor. “This tremendous progress with Vor’s genome engineering process and manufacturing scale-up brings us one step closer to potentially realizing the benefits of our powerful platform to treat patients with acute myeloid leukemia.”

The details of the presentations are below:

Oral Presentation
Title: *VOR33: A Clinic-Ready CRISPR/Cas9 Engineered Hematopoietic Stem Cell Transplant for the Treatment of Acute Myeloid Leukemia*
Presenter: John Lydeard
Session Title: Advances in Ex Vivo Modified Cell Therapies
Session Date/Time: Tuesday May 11, 2021 5:30 PM - 7:15 PM ET
Presentation Time: 7:00-7:15 PM ET
Abstract Number: 7

Poster Presentation
Title: *Rigorous Assessment of Off-Target Editing by CRISPR/Cas9 in VOR33, an Engineered Hematopoietic Stem Cell Transplant for the Treatment of Acute Myeloid Leukemia*
Session Title: Cell Therapy Product Engineering, Development or Manufacturing
Session Date/Time: Tuesday May 11, 2021 8:00 AM - 10:00 AM ET
Abstract Number: 858

About VOR33
VOR33 is Vor’s lead product candidate, consisting of eHSCs that we have engineered to lack the protein CD33, and is designed to replace the standard of care in transplant settings for patients suffering from AML and potentially other hematologic malignancies. Once the VOR33 cells have engrafted, we believe that patients can be treated with anti-CD33 therapies, such as Mylotarg® or, if approved by the FDA, Vor’s in-licensed CD33 chimeric antigen receptor T-cell (CAR-T) therapy candidate, with limited on-target toxicity, leading to durable anti-tumor activity and potential cures. In preclinical studies, we have observed that the removal of CD33 provided robust protection of VOR33 eHSCs from the cytotoxic effects of CD33-directed therapies, yet had no deleterious effects on the differentiation or function of hematopoietic cells.

About Vor Biopharma
Vor Biopharma is a cell therapy company that aims to transform the lives of cancer patients by pioneering engineered hematopoietic stem cell (eHSC) therapies to create next-generation, treatment-resistant transplants that unlock 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 cancerous cells while sparing healthy cells.

Forward Looking Statements

This press release contains forward-looking statements within the meaning 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 statements regarding Vor’s (the Company) platform’s potential to treat patients suffering from acute myeloid leukemia. 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 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 Annual Report on Form 10-K for the year ended December 31, 2020, which is on file with the Securities and Exchange Commission (SEC), and in other filings that the Company make with the SEC in the future. 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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