Vor to Present New Platform and Preclinical Data at ASH

November 4, 2021

- Knock out of CD123 or CLL-1 by CRISPR-Cas9 from Human Hematopoietic Stem Cells Maintains Hematopoietic Function and is Resistant to Targeted Therapies
- Multiplex Engineering of Human CD34+ HSPCs Enables Dual Gene Knock-out with High Engraftment Potential
- Dual-Mobilized Donor Derived CD33 CAR T-Cells as Potent and Effective AML Therapy in Preclinical Models

CAMBRIDGE, Mass., Nov. 04, 2021 (GLOBE NEWSWIRE) -- Vor Biopharma (Nasdaq: VOR), a clinical-stage cell and genome engineering company, today announced the upcoming presentation of new data from its novel engineered hematopoietic stem cell (eHSC) platform at the American Society of Hematology (ASH) 63rd Annual Meeting, taking place in Atlanta, Georgia from December 11-14, 2021.

“The data being presented at ASH underscore the potential of our novel cell and genome engineering platform to change the standard of care for patients living with blood cancers,” said Tirtha Chakraborty, Ph.D., Vor’s Chief Scientific Officer. “By knocking out either CD123 or CLL-1 through CRISPR-Cas9 gene editing, we are exploring a promising new path to treating patients with acute myeloid leukemia with our novel eHSC platform. In addition, our research demonstrating multiplex genome editing of allogeneic hematopoietic stem cells represents another exciting strategy to efficiently and safely edit multiple genes in blood stem cells, potentially enabling the use of multiple targeted blood cancer therapies.”

“In total, we believe these preclinical data, including results showing that T cells derived from the same HSC transplant donor are suitable to become cancer-targeting CAR-T cells, will enable Vor’s development of efficient and safe cell therapies to treat AML patients and support the potential of our novel platform approach.”

**ASH Annual Meeting Presentations**

**Poster Presentation**

**Title:** Knock out of CD123 or CLL-1 By CRISPR-Cas9 Editing from Human Hematopoietic Stem Cell Transplants Provide New Possibilities for Increasing Therapeutic Index and Safety for AML Treatment  
**Abstract Number:** 3818  
**Session Title:** 701. Experimental Transplantation: Basic and Translational: Poster III  
**Presenting Author:** Michelle Lin, Ph.D., Head of Preclinical Sciences and HSC Biology, Vor  
**Date:** Monday, December 13, 2021  
**Time:** 6:00pm ET - 8:00pm ET  
**Location:** Georgia World Congress Center, Hall B5

**Poster Presentation**

**Title:** Multiplex Engineering of Human CD34+ HSPCs Enables Dual Gene Knock-out while Maintaining High Engraftment Potential and Safety  
**Abstract Number:** 2939  
**Session Title:** 801. Gene Therapies: Poster II  
**Presenting Author:** Nipul Patel, Scientist, Vor  
**Date:** Sunday, December 12, 2021  
**Time:** 6:00pm ET - 8:00pm ET  
**Location:** Georgia World Congress Center, Hall B5

**Poster Presentation**

**Title:** G-CSF/Plerixafor Dual-Mobilized Donor Derived CD33 CAR T-cells as Potent and Effective AML Therapy in Pre-Clinical Models  
**Abstract Number:** 1716  
**Session Title:** 703. Cellular Immunotherapies: Basic and Translational: Poster I  
**Presenting Author:** Giacomo Canesin, Ph.D., Scientist, Vor  
**Date:** Saturday, December 11, 2021  
**Time:** 5:30 pm ET - 7:30pm ET  
**Location:** Georgia World Congress Center, Hall B5

The posters from these presentations will be available on the Vor corporate website on Monday, December 13, 2021 at 9:00am ET at https://ir.vorbio.com/news-and-events/events-and-presentations.

**About Vor Biopharma**

Vor Biopharma 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 “aims,” “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 Vor Biopharma’s statements regarding the potential efficacy of its platform and its therapies. Vor Biopharma 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 Biopharma’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 Vor Biopharma’s business, including its preclinical studies and clinical trials 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 the Vor Biopharma’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 Biopharma 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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