



## Vor Bio Successfully Demonstrates Multiplex Editing of Hematopoietic Stem Cells for Next-generation AML Treatment Presented at EHA

June 10, 2022

*Multiplex deletion of myeloid antigens CD33 and CLL-1 in human hematopoietic stem cells demonstrates potential of next-generation HSC transplants for treatment of acute myeloid leukemia*

*Dual edited hematopoietic stem and progenitor cells persisted long-term post engraftment, with minimum translocation risk*

CAMBRIDGE, Mass., June 10, 2022 (GLOBE NEWSWIRE) -- Vor Bio (Nasdaq: VOR), a clinical-stage cell and genome engineering company, today announced successful first of its kind dual editing of CD33 and CLL-1 in human hematopoietic stem cells (HSCs) demonstrating continued progress on its novel approach for the treatment of acute myeloid leukemia (AML). The data is being presented at the European Hematology Association Congress in Vienna, Austria.

The pre-clinical data demonstrates that multiplex deletion by CRISPR/Cas9 of CD33 and CLL-1 from human CD34+ hematopoietic stem and progenitor cells (HSPCs) maintained cell function and persisted long-term post engraftment *in vivo*, with a high-level of editing, no counterselection, and minimum translocation risk when compared to unedited control cells. In addition, genetically modifying HSPCs to remove select cell surface targets does not impair their function and these dual engineered cells showed significant protection from targeted immunotherapy *in vitro*.

"As most tumor antigens are also expressed on normal blood cells or bone marrow, traditional targeted immunotherapy increases the risk of severe cytopenia," explained Tirtha Chakraborty, Ph.D., Vor Bio's Chief Scientific Officer. "Our pre-clinical proof-of-concept data shows that the knockout of both CD33 and CLL-1 from allogeneic HSC grafts can restrict these antigens to only the patients' AML cells, thereby protecting the healthy HSCs and making them resistant to the toxic effects of targeted therapies. These data validate that CD33 and CLL-1 may both be independently biologically dispensable, where we envision our multiplex treatment system has the potential to avoid concerns regarding tumor heterogeneity or escape mechanisms."

AML is the most common type of acute leukemia in adults and is characterized by excessive proliferation of immature myeloid progenitor cells and their failure to properly differentiate into mature blood cells. Healthy donor HSC transplantation is the standard of care and currently around 40% of patients with AML who receive HSC transplantation suffer a relapse of their cancer, with two-year survival rates of less than 20%, highlighting the need for new therapeutic approaches for these patients.

Vor Bio is developing a first-in-class treatment approach consisting of gene-edited HSC transplants that are designed to be resistant to targeted therapies, enabling post-transplant use of powerful therapies such as CAR-Ts or other targeted immuno-therapies. This new approach has the potential to protect healthy cells from the damaging effects of cancer-targeted therapies, leaving the cancerous cells exposed and, for the first time, allowing these targeted therapies to be truly cancer-specific sparing the healthy cells.

The full poster (P1249) is available on the Vor Bio corporate website at <https://ir.vorbio.com/news-and-events/events-and-presentations>.

### 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](http://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 "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 Bio's statements regarding the potential of Vor Bio's multiplex editing approach for the treatment of AML and of Vor Bio's approach to editing HSCs more generally. 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, as well as the results of such studies and 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 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.

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