Vor Bio Demonstrates Potential of Base and Sequential Multiplex Editing of Hematopoietic Stem Cells for Next-generation AML Treatment

October 11, 2022

-Multiplex deletion of myeloid antigens CD33 and CLL-1 or CD33 and CD123 in human hematopoietic stem cells resulted in long-term engraftment and persistence of editing

-Data presented at European Society of Gene & Cell Therapy (ESGCT) Annual Congress

CAMBRIDGE, Mass., Oct. 11, 2022 (GLOBE NEWSWIRE) -- Vor Bio (Nasdaq: VOR), a clinical-stage cell and genome engineering company, today announced data demonstrating the potential of the Company's novel platform to use various base editing strategies to successfully delete multiple cell surface targets from hematopoietic stem cells (HSCs), potentially enabling next-generation transplants for the treatment of acute myeloid leukemia (AML). The Company is presenting the data at the European Society of Gene & Cell Therapy (ESGCT) Annual Congress in Edinburgh, UK in two separate poster presentations.

"Because most tumor antigens are also expressed on normal blood cells, traditional multi-targeted immunotherapy increases the risk of severe cytopenia," explained Tirtha Chakraborty, Ph.D., Vor Bio's Chief Scientific Officer. "These data show that we can potentially address this challenge by using our platform to efficiently remove one or multiple target antigens from HSCs. This approach to transplant, coupled with targeted therapies, may enable the delivery of more efficacious, safer and durable treatments for patients with blood cancers."

Poster Presentation Summaries

Poster Title: Efficient knockout of both CD33 and CLL-1 by multiplex genome editing of human hematopoietic stem cells enhances the potential of next-generation transplants for acute myeloid leukemia (AML) treatment

Poster Summary: The preclinical data demonstrate 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 and no counterselection when compared to unedited control cells. In addition, genetically modifying HSPCs to remove select cell surface targets, in order to improve safety and efficacy of next-gen AML treatments, does not impair their function and these dual edited cells showed significant protection from targeted immunotherapy *in vitro*.
Presenter: Michelle Lin, PhD, Head of Preclinical Sciences and HSC Biology, Vor Bio
Poster ID number: P150
Poster Session: Thursday, 13th October, 2022
Poster Title: Multiplex deletion of myeloid antigens by base editing in human hematopoietic stem and progenitor cells (HSPCs) enables potential for next-generation transplant for acute myeloid leukemia (AML) treatment

Poster Summary: The preclinical data demonstrate that multiplex deletion using several different base editing strategies to edit dual targets (cytosine base editors to edit CD33 and CLL-1 and adenine base editors to edit CD33 and CD123) from 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 no detectable genotoxic risk when compared to unedited control cells. In addition, genetically modifying HSPCs to remove these cell surface targets, in order to improve safety and efficacy, does not appear to impair function. Dual-engineered cells persisted long-term, and loss of multiple antigens was well-tolerated demonstrating that base editing provides an efficient and safe strategy for multi-gene disruptions in HSPCs and can enable next-generation AML treatments.
Presenter: John Lydeard, PhD, Head of Discovery and Molecular Engineering, Vor Bio
Poster ID number: P432
Poster Session: Thursday, 13th October, 2022

The posters presentations are available on the Vor corporate website at: https://www.vorbio.com/publications/

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 [leading] 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 allowing these targeted therapies to kill only the cancer cells while protecting the healthy cells.

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 “could,” “demonstrate,” “design,” “develop,” “enable,” “enhance,” “expect,” “intend,” “may,” “plan,” “potential,” “project,” “should,” “target,” 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, the potential efficacy, safety and durability of those treatments, the progress of Vor Bio’s ongoing clinical trials, and the potential of Vor Bio’s novel platform. 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; the success of Vor Bio’s approach to transplant, coupled with targeted therapies, to enable the delivery of more efficacious, safer and durable treatments for patients with blood cancers; 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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