Vor Biopharma Announces Issuance of Foundational U.S. Patent Broadly Covering Compositions and Methods to Treat Hematologic Malignancies

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The patent covers first-in-class novel hematopoietic stem cell technology for targeted immunotherapies in hematologic malignancies

BOSTON, Nov. 28, 2018 — Vor Biopharma, an immuno-oncology company pioneering hematopoietic stem cell (HSC) therapies for the treatment of hematological malignancies and affiliate of PureTech Health plc (LSE: PRTC), today announced that the United States Patent and Trademark Office (USPTO) issued U.S. Patent No. 10,137,155 related to the Company's technology platform. This foundational patent is the first of its kind in the immuno-oncology field and broadly covers compositions and therapeutic methods related to using novel modified HSCs to enable targeted immunotherapies. The platform technology underlies Vor's pipeline of immuno-oncology candidates, including lead candidate VOR33.

The now patented technology is designed to address the fundamental limitations of traditional targeted therapies, including antibody-drug conjugates, bispecific antibodies, and chimeric antigen receptor (CAR) T cells, for the treatment of hematologic malignancies. These existing therapies target antigens on malignant cells that also appear on healthy cells, which can result in mass depletion of critical lifesustaining cells. Vor's approach employs the use of antigenmodified hematopoietic stem cells (amHSCs), which are designed to repopulate a patient's blood with cells that have a benign genetic modification to a specific surface antigen that escapes targeting and protects the cells from depletion by targeted immunotherapies. By sparing healthy blood cells, this approach may potentially improve patient safety, enable maximal dosing of cancer-targeted therapies, increase the number of potential patient populations that could benefit from these therapies, and expand the reach of targeted immunotherapies beyond Bcell cancers to a broad range of hematological malignancies, including acute myeloid leukemia (AML).

"The USPTO's allowance of these broad claims provides validation that our approach and intellectual property are novel and first-in-class," said Aleks Radovic-Moreno, Ph.D., PureTech Health Vice President and Vor program lead. "The compositions and methods covered in the patent protect our lead product candidate as we continue development of our technology towards the clinic. This technology has the potential to provide a novel therapeutic approach for patients with aggressive blood cancers that otherwise have very few treatment options and poor prognoses."

The relevant intellectual property is exclusively licensed to Vor Biopharma and is based on technology developed by Siddhartha Mukherjee, M.D., D. Phil, Associate Professor of Medicine at Columbia University and a Staff Physician at Columbia University Medical Center, and his colleagues.

"This approach has the potential to broaden the use of targeted immunotherapies beyond Bcell cancers, and to help patients who have very limited treatment options," said Dr. Mukherjee. About VOR33 Vor's lead product candidate, VOR33, is designed to enable maximal CD33-targeted immunotherapy. CD33, a target that is present in the vast majority of acute myeloid leukemia (AML) patients, is also expressed in normal myeloid progenitor cells. Depletion of normal myeloid progenitor cells prevents the beneficial use of several CD33- targeted therapies at important stages throughout the treatment process, at higher doses, and for longer periods of time. By enabling new CD33-targeted therapies, VOR33 has the potential to overcome these challenges and improve treatment for AML. Vor anticipates initiating INDenabling studies for VOR33 in early 2019.

About VOR33

Vor's lead engineered hematopoietic stem cell (eHSC) product candidate, VOR33, is in development for acute myeloid leukemia (AML). VOR33 is designed to produce healthy cells that lack the receptor CD33, thus enabling the targeting of AML cells through the CD33 antigen, while avoiding toxicity to the bone marrow. Currently, targeted therapies for AML and other liquid tumors can be limited by on-target toxicity. By rendering healthy cells "invisible" to CD33-targeted therapies, VOR33 aims to significantly improve the therapeutic window, utility and effectiveness of these AML therapies, with the potential to broaden clinical benefit to different patient populations.

About Vor Biopharma

Vor Biopharma aims to transform the lives of cancer patients by pioneering engineered hematopoietic stem cell (eHSC) therapies. Vor's eHSCs are designed to generate healthy, fully functional cells with specific advantageous modifications, protecting healthy cells from the toxic effects of antigen-targeted therapies, while leaving tumor cells vulnerable.

Vor's platform could potentially be used to change the treatment paradigm of both hematopoietic stem cell transplants and antigen-targeted therapies, such as antibody drug conjugates, bispecific antibodies and CAR-T cell treatments. A proof-of-concept study for Vor's lead program has been published in *Proceedings of the National Academy of Sciences*.

Vor is based in Cambridge, Mass. and has a broad intellectual property base, including inlicenses from Columbia University, where foundational work was conducted by inventor and Vor Scientific Board Chair Siddhartha Mukherjee, MD, DPhil. Vor was founded by Dr. Mukherjee and PureTech Health and is supported by leading investors including 5AM Ventures and RA Capital Management, Johnson & Johnson Innovation — JJDC, Inc. (JJDC), Novartis Institutes for BioMedical Research and Osage University Partners.

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