



PureTech's Vor Granted Foundational US Patent Broadly Covering Compositions and Methods to Treat Haematologic Malignancies

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PureTech Health plc (LSE: PRTC) ("PureTech Health"), an advanced biopharmaceutical company developing novel medicines for dysfunctions of the Brain-Immune-Gut (BIG) Axis, today announced that its affiliate, Vor Biopharma, has been granted a first-in-class patent broadly covering its technology platform for the treatment of haematological malignancies. This foundational patent is the first of its kind in the immuno-oncology field and it broadly covers compositions and therapeutic methods related to using novel modified HSCs to enable targeted immunotherapies. Vor's technology platform is designed to address the fundamental limitations of traditional targeted therapies (e.g., antibody-drug conjugates, bispecific antibodies, and chimeric antigen receptor (CAR) T cells) with the goal of potentially expanding the reach of targeted immunotherapies beyond B-cell cancers to a broad range of haematological malignancies, including acute myeloid leukaemia (AML).

Joseph Bolen, PhD, Chief Scientific Officer at PureTech Health, said: "One of the major challenges that hinders clinical utility of anti-cancer cell-based and biologics-based therapeutics is an absence of cancer-specific surface targets. This is especially relevant for very potent modalities such as CAR-bearing T cells and drug-conjugated antibodies where target expression on normal tissues surfaces leads to unacceptable ancillary toxicities. The USPTO's patent allowance of Vor's technology platform adds important validation to this unique approach and provides a measure of optimism for solving one of the major limitations denying cancer patients potentially life-saving treatments options."

The relevant intellectual property is exclusively licensed to Vor Biopharma and is based on technology developed by Siddhartha Mukherjee, MD, D Phil, Associate Professor of Medicine at Columbia University and a Staff Physician at Columbia University Medical Center, and his colleagues. Dr Mukherjee is the author of *The Emperor of All Maladies: A Biography of Cancer* (winner of the 2011 Pulitzer Prize in general nonfiction), *The Laws of Medicine*, and *The Gene: An Intimate History*.

The full text announcement from Vor is as follows:

Vor Biopharma Announces Issuance of Foundational US Patent Broadly Covering Compositions and Methods to Treat Haematologic Malignancies

The patent covers first-in-class novel haematopoietic stem cell technology for targeted immunotherapies in haematologic malignancies

BOSTON, Nov. 28, 2018 — [Vor Biopharma](#), an immuno-oncology company pioneering haematopoietic stem cell (HSC) therapies for the treatment of haematological malignancies and affiliate of PureTech Health plc (LSE: PRTC), today announced that the United States Patent and Trademark Office (USPTO) issued US 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 haematologic malignancies. These existing therapies target antigens on malignant cells that also appear on healthy cells, which can result in mass depletion of critical life-sustaining cells. Vor's approach employs the use of antigen-modified haematopoietic 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 B-cell cancers to a broad range of haematological malignancies, including acute myeloid leukaemia (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, PhD, PureTech Health Vice President and Vor programme 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."

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"This approach has the potential to broaden the use of targeted immunotherapies beyond B-cell 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 leukaemia (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 IND-enabling studies for VOR33 in early 2019.

About Vor Biopharma

Vor Biopharma is an immuno-oncology company developing novel antibody and cell therapies with broad potential for treating cancer. Vor's key differentiation is a focus on technologies that can selectively target cancer cells without impacting normal cells. Engineered cells, such as chimeric antigen receptor (CAR) T cells, are now FDA-approved drugs for treating haematologic malignancies. However, these and similar technologies target both cancer and normal cells, causing substantial toxicities and limiting their potential. Vor is taking a fundamentally novel approach to targeting cancer selectively by developing antigen-modified haematopoietic stem cells (amHSCs). These amHSCs generate healthy, functional cells that are protected from depletion by cancer-targeted therapies.

Vor's platform is broad and can potentially be used to vastly improve the therapeutic window of several CAR-modified cells (such as CAR T cells, CAR NK cells, and others), expanding the reach of CAR-modified cells beyond B-cell malignancies to other myeloid leukaemias, such as acute myeloid leukaemia, as well as enhancing the effectiveness of other therapies such as antibody-drug conjugates or conventional antibodies targeted against leukaemias and solid tumours. When combined with targeted therapies, this technology could potentially enable transformative outcomes in patients with otherwise grim prognoses.

This technology platform is covered by broad intellectual property exclusively licensed from Columbia University, as well as Vor-owned intellectual property. Co-founded by PureTech Health (LSE: PRTC), Vor is working with some of the world's leading oncologists and immunologists to develop a pipeline of potentially life-altering immunotherapies that extend beyond what is possible with current treatment.

About PureTech Health

PureTech Health (LSE: PRTC) is an advanced biopharmaceutical company developing novel medicines for dysfunctions of the Brain-Immune-Gut (BIG) Axis. The Company has developed deep insights into the connection between the individual components of these systems and the resulting role in many chronic diseases, which have proven resistant to established therapeutic approaches. By harnessing this emerging field of human biology, PureTech Health is developing new categories of medicines with the potential to have great impact on people with serious diseases.

PureTech Health is advancing a rich pipeline of innovative therapies across two divisions: the Affiliates division and the Internal division. Its Affiliates division includes two product candidates that have been filed with the US Food and Drug Administration (FDA) for review and several other novel clinical and pre-clinical programmes. These affiliates are developing ground-breaking platforms and therapeutic candidates in collaboration with some of the world's leading experts.

PureTech's Internal division is advancing a pipeline fuelled by recent discoveries in lymphatics and immune cell trafficking to modulate disease in a tissue-specific manner. These programmes leverage the transport and biodistribution of various immune system components for the targeted treatment of diseases with major unmet needs, including cancers, autoimmune diseases, and neuroimmune disorders.

For more information, visit www.puretechhealth.com or connect with us on Twitter [@puretechh](https://twitter.com/puretechh).

Forward Looking Statement

This press release contains statements that are or may be forward-looking statements, including statements that relate to the company's future prospects, developments and strategies. The forward-looking statements are based on current expectations and are subject to known and unknown risks and uncertainties that could cause actual results, performance and achievements to differ materially from current expectations, including, but not limited to, those risks and uncertainties described in the risk factors included in the regulatory filings for PureTech Health plc. These forward-looking statements are based on assumptions regarding the present and future business strategies of the company and the environment in which it will operate in the future. Each forward-looking statement speaks only as at the date of this press release. Except as required by law and regulatory requirements, neither the company nor any other party intends to update or revise these forward-looking statements, whether as a result of new information, future events or otherwise.