# PureTech Founded Entity Vor Biopharma Announces Exclusive License of Clinical-Stage CD33 CAR-T from National Cancer Institute

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PureTech Health plc (LSE: PRTC) ("PureTech" or the "Company"), a clinical-stage biotherapeutics company dedicated to discovering, developing and commercialising highly differentiated medicines for devastating diseases, is pleased to note that its Founded Entity, Vor, today announced an exclusive licensing agreement with the National Cancer Institute (NCI), part of the National Institutes of Health (NIH), for intellectual property related to a clinical-stage anti-CD33 chimeric antigen receptor T-cell (CAR-T) therapy candidate. This CAR-T construct was devised by T-cell expert Dr Terry Fry during his tenure at the Pediatric Oncology Branch of the NCI, where he oversaw development of this therapeutic candidate from bench to bedside; it is currently being evaluated in a multi-site Phase 1/2 clinical trial in children and young adults with relapsed or refractory acute myeloid leukaemia (AML).

The full text of the announcement from Vor is as follows:

**Vor Biopharma Announces Exclusive License of Clinical-Stage CD33 CAR-T from National Cancer Institute**

CAMBRIDGE, Mass. - November 11, 2020 - Vor Biopharma, an oncology company pioneering engineered haematopoietic stem cells (eHSCs) for the treatment of cancer, today announced an exclusive licensing agreement with the National Cancer Institute (NCI), part of the National Institutes of Health (NIH), for intellectual property related to a clinical-stage anti-CD33 chimeric antigen receptor T-cell (CAR-T) therapy candidate. This CAR-T construct was devised by T-cell expert Dr Terry Fry during his tenure at the Pediatric Oncology Branch of the NCI, where he oversaw development of this therapeutic candidate from bench to bedside; it is currently being evaluated in a multi-site Phase 1/2 clinical trial in children and young adults with relapsed or

refractory acute myeloid leukaemia (AML).

"As a class, CAR-T cell therapies have had a major positive impact on the lives of certain patients with haematological malignancies. But because normal cells often express the same surface proteins as cancer cells, the utility and applicability of targeted therapies has been limited, in part, by on-target toxicity," said Christopher Slapak, MD, Vor's Chief Medical Officer. "This agreement provides Vor with access to a promising CD33 CAR-T that could potentially be administered either as bridge-to-transplant therapy for relapsed or refractory patients with AML, or following transplant with our lead developmental candidate VOR33, whereby the CAR-T may selectively target leukaemia cells while sparing normal myeloid cells."

Vor's lead programme VOR33, which is currently in pre-clinical development, consists of eHSCs that are engineered to provide AML patients with a donor-derived haematopoietic stem cell transplant that lacks the cell surface protein CD33, a clinically validated target for AML. The goal of removing this target is to make these eHSCs and their progeny treatment-resistant to anti-CD33 therapies. As such, Vor believes this CAR-T could be highly complementary to VOR33.

"This licensing agreement with the NCI is an important milestone for Vor, as it brings elements of a more complete AML treatment system under the same roof," said Hilary Eaton, PhD, Vor's Senior Director of Business Development. "The combination of our next-generation, treatment-resistant eHSCs with companion therapeutics such as this CD33 CAR-T is designed to provide a single company solution for some patients suffering from haematological malignancies, potentially transforming outcomes and shifting the standard of care."

More information about the Phase 1/2 study of this CD33 CAR-T can be found at clinicaltrials.gov.

Other terms of the agreement have not been disclosed.

**About VOR33**

Vor's lead product candidate, VOR33, consists of engineered haematopoietic stem cells (eHSCs) that lack the protein CD33. Once these cells are transplanted into a cancer patient, we believe that CD33 will become a far more cancer-specific target, potentially avoiding toxicity to the normal blood and bone marrow associated with CD33-targeted therapies. Vor aims to improve the therapeutic window and effectiveness of CD33-targeted therapies, thereby potentially broadening the clinical benefit to patients suffering from acute myeloid leukaemia.

**About Vor Biopharma**

Vor Biopharma aims to transform the lives of cancer patients by pioneering engineered haematopoietic stem cell (eHSC) therapies. By removing biologically redundant proteins from eHSCs, these cells become inherently invulnerable to complementary targeted therapies while tumour cells are left susceptible, thereby unleashing the potential of targeted therapies to benefit cancer patients in need.

Vor's platform could be used to potentially change the treatment paradigm of both haematopoietic stem cell transplants and targeted therapies, such as antibody drug conjugates, bispecific antibodies, and CAR-T cell treatments.

Vor is based in Cambridge, Mass. and has a broad intellectual property base, including in-licenses from Columbia University, where foundational work was conducted by inventor and Vor Scientific Board Chair Siddhartha Mukherjee, MD, DPhil.

**About PureTech Health**

PureTech is a clinical-stage biotherapeutics company dedicated to discovering, developing and commercialising highly differentiated medicines for devastating diseases, including intractable cancers, lymphatic and gastrointestinal diseases, central nervous system disorders and inflammatory and immunological diseases, among others. The Company has created a broad and deep pipeline through the expertise of its experienced research and development team and its extensive network of scientists, clinicians and industry leaders. This pipeline, which is being advanced both internally and through PureTech's Founded Entities, is comprised of 24 products and product candidates, including two that have received US Food and Drug Administration (FDA) clearance and European marketing authorisation. All of the underlying programmes and platforms that resulted in this pipeline of product candidates were initially identified or discovered and then advanced by the PureTech team through key validation points based on the Company's unique insights into the biology of the brain, immune and gut, or BIG, systems and the interface between those systems, referred to as the BIG Axis.

For more information, visit www.puretechhealth.com or connect with us on Twitter @puretechh.

**Forward Looking Statement**

This press release contains statements that are or may be forward-looking statements, including statements that relate to the Gelesis' 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, our expectations regarding the therapeutic potential of CD33 and the potential combination of CD33 with Vor's other product candidates as potential treatment options for AML and 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.

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