



## PureTech Founded Entity Vor Announces FDA Clearance of IND Application for VOR33

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*Phase 1/2a clinical trial expected to begin in first half of 2021*

**BOSTON, January 14, 2021** - [PureTech Health plc](#) (LSE: PRTC, NASDAQ: PRTC) ("PureTech" or the "Company") is pleased to note that its Founded Entity, [Vor Biopharma](#), a clinical-stage cell therapy company pioneering engineered hematopoietic stem cell (eHSC) therapies combined with targeted therapies for the treatment of cancer, today announced that the U.S. Food and Drug Administration (FDA) has cleared the company's Investigational New Drug (IND) application for VOR33, an eHSC therapy candidate being developed for the treatment of acute myeloid leukemia (AML). Vor plans to initiate a Phase 1/2a clinical trial for VOR33 in the first half of this year.

VOR33, consisting of hematopoietic stem cells that are engineered to lack the CD33 protein, is a cell therapy candidate intended to replace the standard of care in hematopoietic stem cell transplant settings for patients with AML who are at high-risk for relapse.

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

### Vor Announces FDA Clearance of IND Application for VOR33

*Phase 1/2a clinical trial expected to begin in first half of 2021*

**CAMBRIDGE, MA January 14, 2021** - [Vor Biopharma](#), a clinical-stage cell therapy company pioneering engineered hematopoietic stem cell (eHSC) therapies combined with targeted therapies for the treatment of cancer, today announced that the U.S. Food and Drug Administration (FDA) has cleared the company's Investigational New Drug (IND) application for VOR33, an eHSC therapy candidate being developed for the treatment of acute myeloid leukemia (AML). The company plans to initiate a Phase 1/2a clinical trial for VOR33 in the first half of this year.

VOR33, consisting of hematopoietic stem cells that are engineered to lack the CD33 protein, is a cell therapy candidate intended to replace the standard of care in hematopoietic stem cell transplant settings for patients with AML who are at high-risk for relapse.

"Though advances have been made in the treatment of AML and other myeloid malignancies, the median overall five-year survival rate for patients diagnosed with AML remains under 30 percent," said Christopher Slapak, MD, Vor's

Chief Medical Officer. "With the development of VOR33, we are seeking to change the treatment paradigm for AML and potentially other hematologic malignancies. We engineered VOR33 to provide patients with a hematopoietic stem cell transplant that we believe, upon hematopoietic reconstitution, will be treatment resistant to CD33 targeted therapies, potentially resulting in new treatment options and improved post-transplant outcomes."

"Clearance of this IND is the culmination of an incredible team effort at Vor and represents a key milestone for us," added Robert Ang, MBBS, MBA, Vor's President and Chief Executive Officer. "This brings us an important step closer to treating patients with our potentially transformative therapy."

The Phase 1/2a trial is expected to enroll patients with CD33-positive AML who are at high risk of relapse. The primary goals of the trial are to evaluate tolerability and feasibility of the VOR33 stem cell transplant, with a focus on confirming that VOR33 can engraft normally. Following engraftment, patients will be eligible to be treated with Mylotarg<sup>®</sup>, an FDA approved CD33-directed antibody drug conjugate (ADC) therapy owned by Pfizer, in order to potentially prolong leukemia-free survival and provide evidence that VOR33 protects against the myelosuppression that typically accompanies treatment with Mylotarg<sup>®</sup>.

### **About VOR33**

VOR33 is Vor's lead product candidate, consisting of eHSCs that we have engineered to lack the protein CD33, and is designed to replace the standard of care in transplant settings for patients suffering from AML and potentially other hematologic malignancies. Once the VOR33 cells have engrafted, we believe that patients can be treated with anti-CD33 therapies, such as Mylotarg<sup>®</sup> or, if approved by the FDA, Vor's in-licensed CD33 chimeric antigen receptor T-cell (CAR-T) therapy candidate, with limited on-target toxicity, leading to durable anti-tumor activity and potential cures. In preclinical studies, we have observed that the removal of CD33 provided robust protection of VOR33 eHSCs from the cytotoxic effects of CD33-directed therapies, yet had no deleterious effects on the differentiation or function of hematopoietic cells.

### **About Vor Biopharma**

[Vor Biopharma](#) is a clinical-stage cell therapy company that aims to transform the lives of cancer patients by pioneering eHSC therapies to create next-generation, treatment-resistant transplants that unlock the potential of targeted therapies. By removing biologically redundant proteins from eHSCs, we design these cells and their progeny to be treatment-resistant to complementary targeted therapies, thereby enabling these therapies to selectively destroy cancerous cells while sparing healthy cells.

Our platform could be used to potentially change the treatment paradigm of both hematopoietic stem cell transplants and targeted therapies, such as ADCs, bispecific antibodies and CAR-T cell treatments, including Vor's in-licensed CD33 CAR-T.

### **About PureTech Health**

PureTech is a clinical-stage biotherapeutics company dedicated to discovering, developing and commercializing 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, as of the date of PureTech's most recently filed Registration Statement on Form 20-F, was comprised of 24 products and product candidates, including two that have received FDA clearance and European marketing authorization. All of the underlying programs 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](http://www.puretechhealth.com) or connect with us on Twitter @puretechh.

#### **Cautionary Note Regarding Forward-Looking Statements**

This press release contains statements that are or may be forward-looking statements, including statements that relate to our product candidates and approach towards addressing major diseases, 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, expectations regarding the initiation of a Phase 1/2a clinical trial for VOR33 in the first half of this year, the potential therapeutic benefits of VOR33 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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