# PureTech Founded Entity Vor Bio Announces First AML Patient Successfully Transplanted with its Investigational Trem-cel (VOR33) and Tolerated Mylotarg™

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**PureTech Founded Entity Vor Bio Announces First AML Patient Successfully Transplanted with its Investigational Trem-cel (VOR33) and Tolerated Mylotarg™**

*Trem-cel (formerly VOR33) successfully manufactured and engrafted normally*

*Blood counts successfully maintained following post-transplant treatment with Mylotarg*

*Vor Bio also announced the pricing of an underwritten offering and a private placement, with combined gross proceeds of approximately $115.8 million*

PureTech Health plc (Nasdaq: PRTC, LSE: PRTC) ("PureTech" or the "Company"), a clinical-stage biotherapeutics company, noted that its Founded Entity, Vor Bio (Nasdaq: VOR), a clinical-stage cell and genome engineering company, today announced initial clinical data from VBP101, its Phase 1/2a multicenter, open-label, first-in-human study of tremtelectogene empogeditemcel or "trem-cel" (formerly VOR33) in patients with acute myeloid leukemia (AML). The data observed from the first treated patient support the potential of a trem-cel transplant to be successfully manufactured, to engraft normally, and to maintain blood counts following treatment with the CD33-targeted therapy Mylotarg. The clinical trial continues to enroll patients and additional data are expected in 2023.

Vor also separately announced today the pricing of an underwritten offering and a private placement, with combined gross proceeds of approximately $115.8 million. PureTech's percentage ownership of Vor Bio as at November 4, 2022 was approximately 8.2 percent on a beneficial ownership basis, prior to the transaction.

The full text of the announcement from Vor is below:

**First AML Patient Successfully Transplanted with Vor Bio's Investigational Trem-cel (VOR33) and Tolerated Mylotarg™**

- *Trem-cel (formerly VOR33) successfully manufactured and engrafted normally*
- *Blood counts successfully maintained following post-transplant treatment with Mylotarg*

- *Conference call scheduled for today, December 7 at 8:00am ET*

**CAMBRIDGE, Mass., Dec. 07, 2022 --** Vor Bio (Nasdaq: VOR), a clinical-stage cell and genome engineering company, today announced initial clinical data from VBP101, its Phase 1/2a multicenter, open-label, first-in-human study of tremtelectogene empogeditemcel or "trem-cel" (formerly VOR33) in patients with acute myeloid leukemia (AML). The data observed from the first treated patient support the potential of a trem-cel transplant to be successfully manufactured, to engraft normally, and to maintain blood counts following treatment with the CD33-targeted therapy Mylotarg. The clinical trial continues to enroll patients and additional data are expected in 2023.

"These early engraftment data represent the first time genome engineering has been used to genetically alter donor cells by removing an antigen present on blood cells, thereby allowing treatment using a CD33 targeted therapy while protecting normal blood cells," said Dr. Robert Ang, Vor Bio's President and Chief Executive Officer. "These encouraging data represent the first clinical validation of our platform to potentially enable next-generation transplants for patients with blood cancers. We look forward to sharing additional data updates in 2023."

**Trem-cel Displayed Normal Engraftment**

A product dose of 7.6 x$10^6$ $CD34^+$ viable cells/kg, with a CD33 editing efficiency of 88% was manufactured. Following myeloablative conditioning, trem-cel was infused with no infusion reactions. The patient achieved neutrophil engraftment 10 days post-transplant which was within expectations for CD34-enriched transplants. Platelet recovery was observed on Day 22. Hematopoietic cell sub-population reconstitution was robust with over 90% of peripheral blood cells negative for CD33 expression, and 100% donor chimerism was achieved. These data provide proof-of-concept that trem-cel can engraft as expected and that CD33 does not appear to be biologically necessary for engraftment and hematopoietic reconstitution.

**Mylotarg Tolerated at Initial Dose Level**

The patient received Mylotarg at a dose of 0.5 mg/$m^2$. At this dose, Mylotarg saturates CD33 antigen in patients with relapsed/refractory AML[1], and in the original Phase 1 trial of Mylotarg[2], neutropenia was observed across dose levels starting at 0.25mg/$m^2$ within 14 days of infusion. No treatment related adverse events and no liver enzyme changes were observed through day 20 following Mylotarg dosing. No negative impacts to neutrophil and platelet counts were observed through day 20, suggesting tolerability at this initial dose level.

"The unmet medical need for AML is significant and hematopoietic cell transplant is the best hope for these patients," said Brenda Cooper, M.D., Professor of Medicine in the Cellar Therapy Program at University Hospitals, Seidman Cancer Center, and an investigator in the VBP101 study. "Early treatment data in the first patient show that trem-cel can engraft normally and maintain normal hematopoiesis following Mylotarg dosing, which typically causes severe cytopenias. These data support the promise of this approach."

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[1]*Mylotarg ODAC 2017*

[2]*Sievers 1999 Blood 93:3678*

**Conference Call & Webcast Information**
Members of the Vor Bio management team, joined by Dr. Brenda Cooper, will conduct a live conference call and webcast today at 8:00 am Eastern Time.

Listeners can register for the webcast via this link.

Analysts wishing to participate in the Q&A session should use this link.

A replay of the webcast will be available via the investor section of the Company's website at www.vorbio.com approximately two hours after the call's conclusion.

**About AML**

AML is the most common type of acute leukemia in adults and one of the deadliest and most aggressive blood cancers, affecting 20,000 newly diagnosed patients each year in the United States. Approximately half of patients with AML who receive a hematopoietic cell transplant (HCT) suffer a relapse of their leukemia, with two-year survival rates

of less than 20%, and relapse rates are higher for patients with certain adverse risk features. The fragility of engrafted hematopoietic stem cells prevents treatment following transplant, giving the cancer a chance to return.

**About the VBP101 Clinical Trial**

VBP101 is a Phase 1/2a, multicenter, open-label, first-in-human study of trem-cel in participants with AML who are undergoing human leukocyte antigen (HLA)-matched allogeneic hematopoietic cell transplant (HCT). Trem-cel is an allogeneic CRISPR/Cas9 genome-edited hematopoietic stem and progenitor cell (HSPC) therapy product, lacking the CD33 protein. It is being investigated for participants with $CD33^+$ AML at high risk for relapse after HCT to allow post-HCT targeting of residual $CD33^+$ acute AML cells using Mylotarg without toxicity to engrafted cells. Participants undergo a myeloablative HCT with matched related or unrelated donor CD34-selected HSPCs engineered to remove CD33 expression (trem-cel drug product). Mylotarg is given after engraftment for up to four cycles. The primary endpoint is the incidence of successful engraftment, defined as the first day of 3 consecutive days of absolute neutrophil count (ANC) 500 cells/$mm^2$ by day 28. Part 1 of this study is evaluating the safety of escalating Mylotarg dose levels to determine the maximum tolerated dose (MTD) and recommended Phase 2 dose. Part 2 will expand the number of participants to evaluate the Mylotarg recommended Phase 2 dose. For more information, visit: https://clinicaltrials.gov/ct2/show/NCT04849910

**About Trem-cel**

Tremtelectogene empogeditemcel (trem-cel), formerly VOR33, is a genome-edited hematopoietic stem and progenitor allogeneic donor product candidate where CD33 has been deleted using genome engineering. Transplant with trem-cel is designed to replace standard of care transplants for patients suffering from AML and potentially other blood cancers. Trem-cel has the potential to enable powerful targeted therapies in the post-transplant setting including CD33-targeted CAR-T 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 "aim," "anticipate," "can," "continue," "could," "design," "enable," "expect," "initiate," "intend," "may," "on-track," "ongoing," "plan," "potential," "should," "target," "update," "will," "would," 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 feasibility of a trem-cel transplant to be successfully manufactured, to engraft normally, to maintain blood counts following treatment with Mylotarg following allogeneic hematopoietic cell transplant and to be well tolerated, the potential of Vor Bio's platform, and timing expectations for additional release of clinical data. 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; availability and timing of results from preclinical studies and clinical trials; whether interim results from a clinical trial will be predictive of the final results of the trial or the results of future trials; expectations for regulatory approvals to conduct trials or to market products; the success of Vor Bio's in-house manufacturing capabilities and efforts; and availability of funding sufficient for its foreseeable and unforeseeable operating expenses and capital expenditure requirements. The interim data presented in this press release is based on one patient and future results for this patient or additional patients may not produce the same or consistent results. 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.

**About PureTech Health**

PureTech is a biotherapeutics company dedicated to changing the treatment paradigm for devastating diseases. 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 28 therapeutics and therapeutic candidates, including two (Plenity® and EndeavorRx®) that have received both U.S. FDA clearance and European marketing authorization and a third (KarXT) that will soon be filed for FDA approval, as of the most recent update by the Company. All of the underlying programs and platforms that resulted in this pipeline of therapeutic candidates were initially identified or discovered and then advanced by the PureTech team through key validation points based on unique insights in immunology and drug development.

**PureTech Ownership**
PureTech's percentage ownership of Vor Bio as at November 4, 2022 was approximately 8.2 percent on a beneficial ownership basis, prior to the transaction. PureTech's ownership of Vor will be updated in due course in certain of its public materials, including its publicly-available investor deck, following completion of the transaction.

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

**Cautionary Note Regarding Forward-Looking Statements**

This press release contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995. All statements contained in this press release that do not relate to matters of historical fact should be considered forward-looking statements, including without limitation those related to Vor Bio's statements regarding the feasibility of a trem-cel transplant to be successfully manufactured, to engraft normally, to maintain blood counts following treatment with Mylotarg following allogeneic hematopoietic cell transplant and to be well tolerated, the potential of Vor Bio's platform, and timing expectations for additional release of clinical data, and Vor's future prospects, development plans, and strategies. The forward-looking statements are based on current expectations and are subject to known and unknown risks, uncertainties and other important factors that could cause actual results, performance and achievements to differ materially from current expectations, including, but not limited to, those risks, uncertainties and other important factors described under the caption "Risk Factors" in our Annual Report on Form 20-F for the year ended December 31, 2021 filed with the SEC and in our other regulatory filings. 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, we disclaim any obligation to update or revise these forward-looking statements, whether as a result of new information, future events or otherwise.

**Contact:**
**PureTech**
Public Relations
publicrelations@puretechhealth.com
Investor Relations
IR@puretechhealth.com

**EU Media**
Ben Atwell, Rob Winder
+44 (0) 20 3727 1000
ben.atwell@FTIconsulting.com

**U.S. Media**
Nichole Sarkis
+1 774 278 8273
nichole@tenbridgecommunications.com



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