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PureTech Founded Entity Vor Bio Announces New Clinical Data Validating Approach of Using Shielded Transplants to Deliver Targeted Therapies

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PureTech Founded Entity Vor Bio Announces New Clinical Data Validating Approach of Using Shielded Transplants to Deliver Targeted Therapies

Trem-cel + Mylotarg demonstrated engraftment, shielding, broadened therapeutic window, and patient benefit

VCAR33^{ALLO} demonstrates encouraging biomarker data at lowest dose

New asset VADC45 with significant potential opportunities across oncology, gene therapy, and autoimmune disorders

PureTech Health plc (Nasdaq: PRTC, LSE: PRTC) ("PureTech" or the "Company"), a clinical-stage biotherapeutics company dedicated to changing the lives of patients with devastating diseases, noted that its Founded Entity, Vor Bio (Nasdaq: VOR), a clinical-stage cell and genome engineering company, announced new clinical data from its ongoing Phase 1/2 VBP101 study of patients with relapsed/refractory AML receiving trem-cel followed by Mylotarg™. The data demonstrated reliable engraftment, shielding from Mylotarg on-target toxicity, a broadened Mylotarg therapeutic window, and early evidence of patient benefit.

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

New Clinical Data Validates Vor Bio's Approach of Using Shielded Transplants to Deliver Targeted Therapies

Trem-cel + Mylotarg demonstrated engraftment, shielding, broadened therapeutic window, and patient benefit

VCAR33^{ALLO} demonstrates encouraging biomarker data at lowest dose

New asset VADC45 with significant potential opportunities across oncology, gene therapy, and autoimmune disorders

CAMBRIDGE, Mass., Sept. 05, 2024 -- Vor Bio (Nasdaq: VOR), a clinical-stage cell and genome engineering company, today announced new clinical data from its ongoing Phase 1/2 VBP101 study of patients with relapsed/refractory AML receiving trem-cel followed by Mylotarg™. The data demonstrated reliable engraftment, shielding from Mylotarg on-target toxicity, a broadened Mylotarg therapeutic window, and early evidence of patient benefit.

"We are encouraged by this data and the potential benefit that trem-cel in combination with Mylotarg may offer to patients in a disease that has extremely poor outcomes even after transplant," said Dr. Eyal Attar, Vor Bio's Chief Medical Officer. "With this data, we plan to explore a registrational trial while we continue to pursue other synergistic opportunities for Vor Bio's platform such as VCAR33^{ALLO} and VADC45."

The data released today included 18 patients treated with trem-cel of which ten had received Mylotarg as of the data cut-off date of July 19, 2024. The data demonstrated:

- Reliable engraftment, with 100% of patients achieving primary neutrophil engraftment (median 9 days) and robust platelet recovery (median 16.5 days). High CD33 editing efficiency (median 89%, range 71-94%) and full myeloid chimerism at Day 28.
- Shielding of the blood system, with maintained neutrophil and platelet counts across multiple Mylotarg doses of 0.5, 1, and 2 mg/m^2.
- Broadened therapeutic index for Mylotarg with drug exposure represented by AUC which is related to efficacy, consistent with labeled Mylotarg doses, and with maximal concentrations, measured by C_{max} and related to veno-occlusive disease, well below known toxic range.
- Early evidence suggesting patient benefit as measured by relapse-free survival when compared to published high-risk AML comparators[1].

"All the hope I had in the safety of this approach has been supported by the data from this trial thus far," said Guenther Koehne, MD, PhD, an investigator on the VBP101 study and Deputy Director and Chief of Blood & Marrow Transplant and Hematologic Oncology at Miami Cancer Institute of Baptist Health South Florida. "I look forward to treating my next patients at high risk of relapse on this trial as their outcomes are otherwise limited with standard transplants."
Vor Bio plans to approach the U.S. Food & Drug Administration to discuss a pivotal trial design for trem-cel + Mylotarg by around year end.

Continued progress with VCAR33^{ALLO}

- VCAR33^{ALLO} represents another potentially significant synergistic treatment option after trem-cel.
- The VBP301 study continues enrolling patients with initial focus on relapsed/refractory AML post-transplant.
- Vor Bio is encouraged by *in vivo* CAR-T expansion data from three patients treated to date, all at the lowest dose of 1 x 10^6 CAR+ cells/kg.

Vor Bio announced today, a new preclinical asset, VADC45, which has a number of potential opportunities in oncology, gene therapy, and autoimmune disorders.

- VADC45 is an ADC that targets the CD45 protein. CD45 is a well-validated target for a wide variety of blood cancers with clinical proof of concept. The linker-payload used in VADC45 is also clinically validated.
- VADC45 has the potential to treat a number of diseases, including treatment of hematologic malignancies, as a targeted conditioning agent for gene therapies such as for sickle cell disease, holistic immune reset for autoimmune disorders, and for Vor Bio's approach of combining this asset with epitope modification of CD45 to shield healthy stem cells.
- Vor Bio already has robust preclinical data for VADC45 and is progressing IND-enabling studies to enable future Phase 1 studies.

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 potential of its product candidates to positively impact quality of life and alter the course of disease in the patients it seeks to treat, the timing of regulatory filings and initiation of clinical trials, the timing and pace of patient enrollment and dosing in clinical trials and the availability of data therefrom, the expected safety profile of its product candidates, its intentions to use $VCAR33^{ALLO}$ in combination with trem-cel as a Treatment System, the potential of trem-cel to enable targeted therapies in the post-transplant setting including Mylotarg and CD33-targeted CAR-Ts, and the ability of VADC45 to treat hematologic malignancies and to be used as a targeted conditioning agent for gene therapies, as a holistic immune reset for autoimmune disorders, and in combination with opitope modification of CD45 to shield healthy stem cells. 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; uncertainties regarding 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 and Vor Bio's ability to continue as a going concern. 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 clinical-stage biotherapeutics company dedicated to giving life to new classes of medicine to change the lives of patients with devastating diseases. The Company has created a broad and deep pipeline through its experienced research and development team and its extensive network of scientists, clinicians and industry leaders that is being advanced both internally and through its Founded Entities. PureTech's R&D engine has resulted in the development of 29 therapeutics and therapeutic candidates, including two that have received both U.S. FDA clearance and European marketing authorization and a third (KarXT) that has been filed for FDA approval. A number of these programs are being advanced by PureTech or its Founded Entities in various indications and stages of clinical development, including registration enabling studies. 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.

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

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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's Bio's statements regarding the potential of its product candidates to positively impact quality of life and alter the course of disease in the patients it seeks to treat, the timing of regulatory filings and initiation of clinical trials, the timing and pace of patient enrollment and dosing in clinical trials and the availability of data therefrom, the expected safety profile of its product candidates, its intentions to use VCAR33^{ALLO} in combination with trem-cel as a Treatment System, the potential of trem-cel to enable targeted therapies in the post-transplant setting including Mylotarg and CD33-targeted CAR-Ts, and the ability of VADC45 to treat hematologic malignancies and to be used as a targeted conditioning agent for gene therapies, as a holistic immune reset for autoimmune disorders, and in combination with opitope modification of CD45 to shield healthy stem cells. 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, 2023, 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.

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[1] Araki et al. JCO 2016; Jentzsch et al. Blood Cancer Journal 2022.

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