PureTech Affiliate Vor Biopharma Appoints Senior Cell and Gene Therapy Leaders

October 1, 2019

PureTech Health plc (LSE: PRTC) ("PureTech"), a clinical stage biotechnology company dedicated to discovering, developing and commercialising highly differentiated medicines for dysfunctions of the Brain-Immune-Gut (BIG) axis, is pleased to note that its affiliate Vor Biopharma has appointed Sadik Kassim, PhD, previously of Kite Pharma and Novartis, as chief technology officer, and Tirtha Chakraborty, PhD, previously of Sana Biotechnology and CRISPR Therapeutics, as vice president of research.

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

Vor Biopharma Hires Senior Cell and Gene Therapy Leaders as Chief Technology Officer and Vice President of Research

CAMBRIDGE, Mass., October 1, 2019 — Vor Biopharma, an oncology company pioneering engineered haematopoietic stem cells (eHSCs) for the treatment of cancer, today announced senior appointments to its leadership team. Sadik Kassim, PhD, a cell and gene therapy bioprocessing and translational research expert, joins Vor from Kite Pharma as Chief Technology Officer. Tirtha Chakraborty, PhD, a haematological and gene engineering research specialist with experience at Sana Biotechnology and CRISPR Therapeutics, joins as Vice President of Research. These new positions follow Vor's recent move into an integrated headquarters in Cambridge, Mass., the appointment of Robert Ang, MBBS, MBA, as President and Chief Executive Officer and a $42 million Series A financing directed at developing Vor's platform technology and advancing its pipeline of eHSC-based candidates.

"Vor is bringing a fundamentally novel approach to haematopoietic stem cells to empower targeted cancer therapies, and we are rapidly building an industry-leading team to realize the value in this scientific foundation," said Dr Ang. "Dr Kassim brings his substantial experience with the complex methods and processes that are required for manufacturing genetically-manipulated cell therapies, and Dr Chakraborty provides deep expertise in haematology and genetic engineering. Their complementary knowledge will aid Vor's expansion, platform development and the move towards our first Investigational New Drug filing for VOR33."

"I am impressed that compelling in vivo data already supports the potential of Vor's cellular engineering platform to protect healthy cells from antigen-directed therapies via antigen removal," said Dr Kassim. "This is especially noteworthy when therapeutic effectiveness is so often highly limited by co-location of target antigens on healthy immune cells, creating a huge opportunity for Vor to significantly broaden the applicability of these and future therapies."

"It's exciting to join the Vor team during this period of accelerated expansion," said Dr Chakraborty. "As a geneticist and cell biologist, I look forward to developing this new approach to treat a range of devastating cancers, beginning with VOR33 in acute myeloid leukaemia."

Dr Kassim is a former Executive Director at Kite Pharma where he led the development of manufacturing processes for autologous CAR- and TCR-based gene-modified cell therapies. Prior to Kite, he served as Chief Scientific Officer at Mustang Bio, where he was the first employee and oversaw the foundational build-out of the company's preclinical and manufacturing activities. Prior to Mustang, Dr Kassim was Head of Early Analytical Development for Novartis' Cell and Gene Therapies Unit, where he contributed to the BLA and MAA filings for Kymriah®. Earlier in his career, Dr Kassim was a research biologist at the National Cancer Institute, where he was involved in early research and CMC work that led to the development of several first-in-human TCR and CAR-T products, including Kite's Yescarta®. Dr Kassim has also conducted preclinical immunology research at Janssen and was a research fellow in the University of Pennsylvania Gene Therapy Program, where he led the initial discovery and preclinical studies for an AAV8 gene therapy for familial hypercholesterolaemia, a program that is now in the clinic. Dr Kassim earned his BS in Cell and Molecular Biology from Tulane University and received his PhD in Microbiology and Immunology from Louisiana State University.

Dr Chakraborty joins Vor from Sana Biotechnology, where he served as the Vice President of Cell Therapy Research. Prior to Sana, Dr Chakraborty was the Head of Haematology at CRISPR Therapeutics, where his team's work on haemoglobin disorders paved the way for the first clinical trial for the CRISPR industry. Before that, at Moderna Therapeutics, Dr Chakraborty led synthetic mRNA platform technology research. He was trained as an RNA biologist and an immunologist during his postdoctoral research at Harvard Medical School. Dr Chakraborty received his PhD from the Tata Institute of Fundamental Research in Mumbai, India.

About VOR33

Vor's lead engineered haematopoietic stem cell (eHSC) product candidate, VOR33, is in development for acute myeloid leukaemia (AML). VOR33 is designed to produce healthy cells that lack the receptor CD33, thus enabling the targeting of AML cells through the CD33 antigen, while avoiding toxicity to the bone marrow. Currently, targeted therapies for AML and other liquid tumours can be limited by on-target toxicity. By rendering healthy cells "invisible" to CD33-targeted therapies, VOR33 aims to significantly improve the therapeutic window, utility and effectiveness of these AML therapies, with the potential to broaden clinical benefit to different patient populations.

About Vor Biopharma

Vor Biopharma aims to transform the lives of cancer patients by pioneering engineered hematopoietic stem cell (eHSC) therapies. Vor's eHSCs are designed to generate healthy, fully functional cells with specific advantageous modifications, protecting healthy cells from the toxic effects of antigen-targeted therapies, while leaving tumour cells vulnerable.

Vor's platform could potentially be used to change the treatment paradigm of both hematopoietic stem cell transplants and antigen-targeted therapies, such as antibody drug conjugates, bispecific antibodies and CAR-T cell treatments. A proof-of-concept study for Vor's lead program has been published in Proceedings of the National Academy of Sciences.

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. Vor was founded by Dr Mukherjee and PureTech Health and is supported by leading investors including 5AM Ventures and RA Capital Management, Johnson & Johnson Innovation — JJDC, Inc. (JJDC), Novartis Institutes for BioMedical Research and Osage University Partners.

About PureTech Health

PureTech is a clinical stage biotechnology 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 affiliates, is comprised of 24 product candidates and one product that has been cleared by the US Food and Drug Administration (FDA). 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 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.