



Vor Biopharma Completes \$42 Million Series A Financing Round to Advance Lead Cell Therapy Product Candidate for the Treatment of Acute Myeloid Leukemia

- Financing led by 5AM Ventures and RA Capital Management
- Participation from Johnson & Johnson Innovation – JJDC, Novartis Institutes for BioMedical Research, Osage University Partners, and Vor Co-founder PureTech Health

BOSTON, Feb. 14, 2019 — [Vor Biopharma](#), an immuno-oncology company pioneering engineered hematopoietic stem cell (HSC) therapies for the treatment of hematological malignancies, today announced a \$42 million Series A financing round led by 5AM Ventures and RA Capital Management. Johnson & Johnson Innovation – JJDC, Inc. (JJDC), Novartis Institutes for BioMedical Research (NIBR), and Osage University Partners also participated in the round along with Vor Co-founder PureTech Health. Vor plans to use the proceeds from the financing to advance its lead HSC-based candidate for the treatment of acute myeloid leukemia (AML) towards the clinic, and to further build its pipeline to treat hematologic malignancies.

“Vor’s unique and patented technology platform for enabling targeted immunotherapies using engineered HSCs has the potential to disrupt the treatment landscape for hematologic malignancies,” said Kush Parmar, M.D., Ph.D., Managing Partner at 5AM Ventures who has joined the Vor Biopharma Board of Directors as Executive Chair. “We are excited to work with Vor to drive this next chapter of growth forward.”

“The need for new therapies for hematologic malignancies is dire. I am gratified that this discovery from my lab continues to advance towards the clinic. This new platform may enable more patients to benefit from the life-saving potential of targeted immunotherapies,” said Siddhartha Mukherjee, M.D., D.Phil, Vor Co-founder and Associate Professor of Medicine at Columbia University. “I look forward to further engaging with the scientific community when we publish our results in peer-reviewed journals.”

“We are excited about Vor’s bold and novel approach to potentially revolutionize the way stem cell transplants are used to treat severe hematologic cancers,” said Joshua Resnick, M.D., Managing Director at RA Capital who has also joined the Vor Biopharma Board of Directors.

Vor’s engineered HSC technology platform is designed to address fundamental limitations of today’s immunotherapies. Vor’s approach has the potential to expand the reach of targeted immunotherapies to a broad range of patient populations and hematological malignancies by

enabling new dosing paradigms for cancer-targeted immunotherapies, which can substantially improve the therapeutic window for efficacy and improve patient safety.

“We are delighted to welcome this terrific syndicate of investors, who share our passion, commitment, and vision for bringing Vor’s potentially life-saving new therapies to patients with acute myeloid leukemia and other hematologic malignancies,” said Bharatt Chowrira, J.D., Ph.D., Board of Directors at Vor Biopharma and President and Chief of Business & Strategy at PureTech Health.

About VOR33

Vor’s lead engineered hematopoietic stem cell (eHSC) product candidate, VOR33, is in development for acute myeloid leukemia (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 tumors 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 tumor 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 inlicenses 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.

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