



Vor Biopharma Announces Exclusive License of Clinical-Stage CD33 CAR-T from National Cancer Institute

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CAMBRIDGE, Mass. – November 11, 2020 – [Vor Biopharma](#), an oncology company pioneering engineered hematopoietic stem cells (eHSCs) for the treatment of cancer, today announced an exclusive licensing agreement with the National Cancer Institute (NCI), part of the National Institutes of Health (NIH), for intellectual property related to a clinical-stage anti-CD33 chimeric antigen receptor T-cell (CAR-T) therapy candidate. This CAR-T construct was devised by T-cell expert Dr. Terry Fry during his tenure at the Pediatric Oncology Branch of the NCI, where he oversaw development of this therapeutic candidate from bench to bedside; it is currently being evaluated in a multi-site Phase 1/2 clinical trial in children and young adults with relapsed or refractory acute myeloid leukemia (AML).

“As a class, CAR-T cell therapies have had a major positive impact on the lives of certain patients with hematological malignancies. But because normal cells often express the same surface proteins as cancer cells, the utility and applicability of targeted therapies has been limited, in part, by on-target toxicity,” said Christopher Slapak, MD, Vor’s Chief Medical Officer. “This agreement provides Vor with access to a promising CD33 CAR-T that could potentially be administered either as bridge-to-transplant therapy for relapsed or refractory patients with AML, or following transplant with our lead developmental candidate VOR33, whereby the CAR-T may selectively target leukemia cells while sparing normal myeloid cells.”

Vor’s lead program VOR33, which is currently in pre-clinical development, consists of eHSCs that are engineered to provide AML patients with a donor-derived hematopoietic stem cell transplant that lacks the cell surface protein CD33, a clinically validated target for AML. The goal of removing this target is to make these eHSCs and their progeny treatment-resistant to anti-CD33 therapies. As such, Vor believes this CAR-T could be highly complementary to VOR33.

“This licensing agreement with the NCI is an important milestone for Vor, as it brings elements of a more complete AML treatment system under the same roof,” said Hilary Eaton, PhD, Vor’s Senior Director of Business Development. “The combination of our next-generation, treatment-resistant eHSCs with companion therapeutics such as this CD33 CAR-T is designed to provide a single company solution for some patients suffering from hematological malignancies, potentially transforming outcomes and shifting the standard of care.”

More information about the Phase 1/2 study of this CD33 CAR-T can be found at clinicaltrials.gov.

Other terms of the agreement have not been disclosed.

About VOR33

Vor’s lead product candidate, VOR33, consists of engineered hematopoietic stem cells (eHSCs) that lack the protein CD33. Once these cells are transplanted into a cancer patient, we believe that CD33 will become a far more cancer-specific target, potentially avoiding toxicity to the normal blood and bone marrow associated with CD33-targeted therapies. Vor aims to improve the therapeutic window and effectiveness of CD33-targeted therapies, thereby potentially broadening the clinical benefit to patients suffering from acute myeloid leukemia.

About Vor Biopharma

Vor Biopharma aims to transform the lives of cancer patients by pioneering engineered hematopoietic stem cell (eHSC) therapies. By removing biologically redundant proteins from eHSCs, these cells become inherently invulnerable to complementary targeted therapies while tumor cells are left susceptible, thereby unleashing the potential of targeted therapies to benefit cancer patients in need.

Vor’s platform could be used to potentially change the treatment paradigm of both hematopoietic stem cell transplants and targeted therapies, such as antibody drug conjugates, bispecific antibodies and CAR-T cell treatments.

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.

Media Contact

Matthew Corcoran

+1 617-866-7350

mcorcoran@tenbridgecommunications.com