Vor Announces FDA Clearance of IND Application for VOR33

Phase 1/2a clinical trial expected to begin in first half of 2021

CAMBRIDGE, Jan. 14, 2021 — Vor Biopharma, a clinical-stage cell therapy company pioneering engineered hematopoietic stem cell (eHSC) therapies combined with targeted therapies for the treatment of cancer, today announced that the U.S. Food and Drug Administration (FDA) has cleared the company's Investigational New Drug (IND) application for VOR33, an eHSC therapy candidate being developed for the treatment of acute myeloid leukemia (AML). The company plans to initiate a Phase 1/2a clinical trial for VOR33 in the first half of this year.

VOR33, consisting of hematopoietic stem cells that are engineered to lack the CD33 protein, is a cell therapy candidate intended to replace the standard of care in hematopoietic stem cell transplant settings for patients with AML who are at high-risk for relapse.

"Though advances have been made in the treatment of AML and other myeloid malignancies, the median overall five-year survival rate for patients diagnosed with AML remains under 30 percent," said Christopher Slapak, MD, Vor's Chief Medical Officer. "With the development of VOR33, we are seeking to change the treatment paradigm for AML and potentially other hematologic malignancies. We engineered VOR33 to provide patients with a hematopoietic stem cell transplant that we believe, upon hematopoietic reconstitution, will be treatment resistant to CD33 targeted therapies, potentially resulting in new treatment options and improved post-transplant outcomes."

"Clearance of this IND is the culmination of an incredible team effort at Vor and represents a key milestone for us," added Robert Ang, MBBS, MBA, Vor's President and Chief Executive Officer. "This brings us an important step closer to treating patients with our potentially transformative therapy."

The Phase 1/2a trial is expected to enroll patients with CD33-positive AML who are at high risk of relapse. The primary goals of the trial are to evaluate tolerability and feasibility of the VOR33 stem cell transplant, with a focus on confirming that VOR33 can engraft normally. Following engraftment, patients will be eligible to be treated with Mylotarg®, an FDA approved CD33-directed antibody drug conjugate (ADC) therapy owned by Pfizer, in order to potentially prolong leukemia-free survival and provide evidence that VOR33 protects against the myelosuppression that typically accompanies treatment with Mylotarg®.

About VOR33

VOR33 is Vor's lead product candidate, consisting of eHSCs that we have engineered to lack the protein CD33, and is designed to replace the standard of care in transplant settings for patients suffering from AML and potentially other hematologic malignancies. Once the VOR33 cells have engrafted, we believe that patients can be treated with anti-CD33 therapies, such as Mylotarg® or, if approved by the FDA, Vor's in-licensed CD33 chimeric antigen receptor T-cell (CAR-T) therapy candidate, with limited on-target toxicity, leading to durable anti-tumor activity and potential cures. In preclinical studies, we have observed that the removal of CD33 provided robust protection of VOR33 eHSCs from the cytotoxic effects of CD33-directed therapies, yet had no deleterious effects on the differentiation or function of hematopoietic cells.

About Vor Biopharma

Vor Biopharma is a clinical-stage cell therapy company that aims to transform the lives of cancer patients by pioneering eHSC therapies to create next-generation, treatment-resistant transplants that unlock the potential of targeted therapies. By removing biologically redundant proteins from eHSCs, we design these cells and their progeny to be treatment-resistant to complementary targeted therapies, thereby enabling these therapies to selectively destroy cancerous cells while sparing healthy cells.

Our platform could be used to potentially change the treatment paradigm of both hematopoietic stem cell transplants and targeted therapies, such as ADCs, bispecific antibodies and CAR-T cell treatments, including Vor's in-licensed CD33 CAR-T.

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