Hemab Therapeutics Announces Start of Velora Pioneer, a Phase 1/2 Clinical Trial Investigating HMB-002 for the Treatment of Von Willebrand Disease

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First participant with Von Willebrand Disease dosed with HMB-002, a novel subcutaneous therapy designed to increase endogenous von Willebrand Factor and Factor VIII levels

COPENHAGEN, DENMARK AND CAMBRIDGE, MASS., US – February 27, 2025 – Hemab Therapeutics, a clinical-stage biotechnology company developing novel prophylactic therapeutics for serious, underserved bleeding and thrombotic disorders, today announced the first participant with Von Willebrand Disease (VWD) has been dosed in Velora Pioneer, a Phase 1/2 clinical trial investigating HMB-002, a potential first-in-class subcutaneous therapy for VWD.

“We are excited to advance the clinical development of HMB-002 for Von Willebrand Disease,” said Catherine Rea, MD, PhD, Vice President of Clinical Development at Hemab. “HMB-002 is the first fixed-dose subcutaneously administered antibody-based preventative therapy aiming to transform daily life for patients.”

HMB-002 is a monovalent antibody uniquely designed to increase levels of von Willebrand Factor (VWF) and Factor VIII and provide a long-acting, subcutaneous prophylactic treatment for individuals with all types of VWD. Preclinical data for this investigational therapy were first presented at the 18th Annual Congress of the European Association for Haemophilia and Allied Disorders (EAHAD), showcasing its potential to target the underlying patho-etiology of VWD by stabilizing VWF and promoting sustained hemostatic correction.

“Von Willebrand Disease is characterized by a deficiency in VWF activity and sometimes associated with lowered levels of FVIII – both essential to control severe bleeding,” said Dr. Priyanka Raheja, the Royal London Hospital, Barts Health NHS Trust. “HMB-002 functions by increasing VWF and FVIII and could offer a complete disease correction for some and substantial modification for bleeding tendency for others. I am excited to be a contributing investigator on the Phase 1/2 trial.”

The Phase 1/2 study is designed to evaluate the safety, tolerability, pharmacokinetics, pharmacodynamics, and efficacy of HMB-002 in individuals with VWD. The initial part of the study will evaluate single ascending doses of HMB-002 in a controlled setting. The first participant was dosed at Richmond Pharmacology in London under the supervision of Principal Investigator, Dr. Ulrike Lorch. The study is actively enrolling participants, with interim data anticipated later this year.

“The initiation of the HMB-002 clinical program establishes Hemab as a multi-asset clinical-stage biotech and underscores our unwavering commitment to reimagine blood clotting therapies for people living with a variety of underserved bleeding disorders,” said Benny Sorensen, MD, PhD, CEO of Hemab. “Von Willebrand Disease has been documented for about 100 years. However, the current standard of care is sparse, ancient, and inconvenient. It’s time to leapfrog treatment into the 21 st century, and we believe HMB-002 can become a new back-bone therapy for all types of Von Willebrand Disease.”

In addition to Velora Pioneer, Hemab is also conducting Velora Discover, a prospective natural history study collecting data on bleeds, treatments, and quality-of-life elements in people with VWD. Hemab is partnered with Haemnet, a specialist research and communications consultancy in the bleeding disorders community, to conduct VWD 360, the largest-ever natural history study in all types of VWD based on people’s detailed reported lived experience. Recent initial results were shared at EAHAD 2025.

About Von Willebrand Disease

Von Willebrand Disease (VWD) is the most common inherited bleeding disorder, characterized by quantitative or qualitative defects in Von Willebrand Factor (VWF), often resulting in frequent mucocutaneous bleeding events and heavy menstrual bleeding in women. The severity of bleeding ranges from low-volume events to potentially life-threatening hemorrhages. Chronic blood loss frequently leads to iron deficiency anemia, exacerbating the disease burden and reducing quality of life, particularly for those with clinically understated subtypes. Despite its prevalence, current treatment options for VWD primarily focus on managing symptoms rather than addressing the underlying defect in VWF production or function.

About HMB-002

HMB-002 is a monovalent human antibody developed as the first-in-class prophylactic treatment for Von Willebrand Disease targeting the underlying root cause of the disease, a condition driven by a deficiency or defect in Von Willebrand Factor (VWF), a key regulator of hemostasis. By specifically targeting the C-terminal CK domain of VWF, which is distinct from regions critical to its essential interactions, HMB-002 shields the protein from degradation, boosting endogenous levels without compromising its function. Preclinical data suggest strong potential for meaningful therapeutic benefit. For more information, please visit clinicaltrials.gov (NCT06610201).

About Hemab Therapeutics

Hemab is a multiple clinical-asset biotech company developing novel prophylactic therapeutics for serious, underserved bleeding and thrombotic disorders. Based in Cambridge, MA, and Copenhagen, Denmark, Hemab is progressing a pipeline of innovative therapeutic solutions, leveraging a variety of cutting-edge technologies and approaches to transform the treatment paradigm for patients with high unmet need. The company's strategic guidance, Hemab 1-2-5 TM, targets building a pipeline of multiple development programs to deliver long-awaited innovation for patients with high unmet need blood-clotting disorders like Glanzmann thrombasthenia, Factor VII Deficiency, Von Willebrand Disease, and others. Learn more at hemab.com. Follow us on LinkedIn, Facebook, Instagram, and X.

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