Hemab Therapeutics Presents Interim Data from Ongoing Phase 2 Study of HMB-001 as First Ever Prophylactic Treatment in Glanzmann Thrombasthenia and Preclinical Data from HMB-002 in Von Willebrand Disease at the 2025 EAHAD Annual Congress

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Interim results demonstrate treatment with HMB-001 resulted in >50% reduction in treated bleeds at all tested dose levels

In second oral presentation, Hemab presented first preclinical data for HMB-002, a potential novel treatment for Von Willebrand Disease

Results from lived experience of Glanzmann thrombasthenia (GT360) and Von Willebrand Disease (VWD 360) natural history studies were included in two additional oral presentations

COPENHAGEN, DENMARK AND CAMBRIDGE, MASS., US – February 7, 2025 – Hemab Therapeutics, a clinical-stage biotechnology company developing novel prophylactic therapeutics for serious, underserved bleeding and thrombotic disorders, today presented interim data from the ongoing evaluation of HMB-001, a novel bispecific antibody in development as first ever prophylactic treatment for the bleeding disorder Glanzmann thrombasthenia (GT). The Phase 2 study consists of a minimum 6-week prospective run-in where participants record bleeds via an electronic bleed diary, followed by 3 months of treatment with HMB-001. Interim efficacy data to date demonstrated >50% reduction in treated bleeds in all 3 tested dose cohorts. Underscoring its potential to address unmet medical needs and to expedite patient access, HMB-001 has been granted Orphan Drug Designation by the U.S. Food and Drug Administration and the UK Medicines and Healthcare products Regulatory Agency has awarded it designation under the Innovative Licensing and Access Pathway.

Hemab also unveiled compelling preclinical data for HMB-002, a potential novel treatment for Von Willebrand Disease (VWD).

These data were featured as oral presentations at the 18th Annual Congress of the European Association for Haemophilia and Allied Disorders (EAHAD) held this week in Milan, Italy.

“Glanzmann thrombasthenia is a serious and devastating bleeding disorder where patients often experience in excess of 60-80 treatment-requiring bleeds annually. These interim Phase 2 results signal the potential of a new reality for the Glanzmann thrombasthenia community, bringing us a step closer to a much-needed breakthrough in preventing bleeding,” said Kate Madigan, Chief Medical Officer at Hemab. “The preclinical data for HMB-002 highlights its potential to specifically address the underlying patho-etiology and revolutionize care for the Von Willebrand Disease community, offering hope to prevent life-threatening complications and provide patients with a critical new lifeline.”

Interim pharmacokinetic (PK) data from the Phase 1/2 study of HMB-001 supports the use of weekly or biweekly dosing regimens. Pharmacodynamic (PD) responses are aligned with the mechanism of action, demonstrating a dose-dependent accumulation of activated Factor VII (FVIIa) and enhanced potentiation through TLT-1 binding. Efficacy assessments indicate a reduction of over 50% in treated bleeding episodes at all tested dose levels.

In the single ascending dose (SAD) study, doses of 0.2, 0.5, and 1.25 mg/kg were evaluated, while the multiple ascending dose (MAD) study explored biweekly doses of 0.3, 0.6, and 0.9 mg/kg. The findings suggest a safe and effective dosing range of 0.3-0.6 mg/kg, while doses of 0.9 mg/kg and above were associated with elevated D-dimer levels and risk of venous thrombosis. These results provide valuable insights into optimizing dose and regimen for improved outcomes.

“Bleeding disorders such as Glanzmann thrombasthenia present significant hardships, including unpredictable and life-altering bleeds, physical pain, social stigma, and emotional isolation. Despite these burdens, preventive treatments have yet to be developed,” said Suthesh Sivapalaratnam, MD, PhD, Consultant Hematologist at Barts Health NHS Trust and Associate Professor at Queen Mary University of London. He adds: “With a bleed reduction of greater than 50%, the Phase 2 results highlight the transformative potential of HMB-001, offering people with Glanzmann thrombasthenia and providers a long-awaited breakthrough that could redefine care standards and bring renewed hope to a community in urgent need of innovation.”

“As a principal investigator in this study, I’ve seen firsthand how HMB-001 has the potential to significantly improve outcomes for people with Glanzmann thrombasthenia,” said Laurent Frenzel, MD, PhD, Head of the Hemophilia Treatment and Research Center at the Necker-Enfants Malades Hospital (Paris Cité). “The Phase 2 data demonstrate promising efficacy and a manageable safety profile, representing a significant advancement in the treatment landscape. I look forward to seeing these results translate into a meaningful impact for those who have long faced limited options and unmet medical needs.”

Hemab plans to complete Phase 2 study recruitment in the first half of 2025.

Hemab presented compelling preclinical data for HMB-002, a monovalent antibody uniquely designed to address VWD by increasing endogenous Von Willebrand Factor (VWF) levels and improving overall hemostasis. Unlike current treatments that often require frequent intravenous infusions or focus on symptom management, HMB-002 has the potential to provide a long-lasting, subcutaneous prophylactic solution that targets the root cause of VWD. Data demonstrate a promising safety and efficacy profile with high-affinity target-selective binding and preservation of key physiological functions of VWF when HMB-002 is bound. Administration of HMB-002 to cynomolgus monkeys resulted in a time-dependent accumulation of VWF, a corresponding rise in VWF activity, and a rise in the amount of another important blood clotting protein called Factor VIII (FVIII). The distribution of different sizes of VWF molecules (multimers) remained largely the same, suggesting that the activity levels of VWF were consistent. These results indicate that HMB-002, by boosting the levels of naturally occurring VWF, has the potential to be an effective, convenient and long-lasting preventive treatment option for people with VWD. The company is actively enrolling participants in **VELORA-Discover**, a prospective observational study for individuals with Type 1 VWD, to further understand the disease's natural history and support future clinical trials.

Hemab's partner Haemnet, a specialist research and communications consultancy in the bleeding disorders community, also presented results from two natural history studies. The Glanzmann's 360 and Von Willebrand Disease 360 studies showcased the unmet needs and burdens the patients face every day.

About Glanzmann Thrombasthenia

Glanzmann thrombasthenia (GT) is a rare and severe bleeding disorder marked by debilitating, sometimes life-threatening bleeding episodes. Results from an international Glanzmann's 360 (GT360) natural history study revealed the substantial burden of this disease: 88% of the 117 participants reported at least one bleed in the previous week, with 34% of those bleeds requiring medical treatment. These bleeding episodes significantly impact patients' mental health and quality of life, with 67% reporting low mood, 52% reporting emotional problems, and 46% experiencing social isolation. Additionally, 81% of participants reported missing school or work due to bruising or bleeding. To date, there are no effective prophylactic treatment options for GT.

About HMB-001

HMB-001 is a subcutaneously administered bispecific antibody that binds and stabilizes endogenous Factor VIIa with one antibody arm and binds to TLT-1 on activated platelets with the other arm. This allows for the accumulation of endogenous Factor VIIa in the body, recruitment of Factor VIIa directly to the surface of the activated platelets, where it is known to facilitate hemostatic plug formation. HMB-001 is designed to be a first-in-class prophylactic treatment for Glanzmann thrombasthenia (GT) with the potential to treat other debilitating rare bleeding disorders. The U.S. Food and Drug Administration granted Fast Track Designation and Orphan Drug Designation to HMB-001 for the treatment of GT while the UK The Medicines and Healthcare products Regulatory Agency has awarded it designation under the Innovative Licensing and Access Pathway (ILAP). For more information, please visit clinicaltrials.gov (NCT06211634).

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. 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 clinical-stage 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, and X.

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