



Hemab Therapeutics Announces First Patient Dosed in Phase 2 Clinical Study Investigating HMB-001 for the Treatment of Glanzmann Thrombasthenia

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HMB-001 is a novel bispecific antibody designed to be the first prophylactic treatment for Glanzmann Thrombasthenia (GT) and other debilitating bleeding disorders

Phase 1 was successfully completed in the UK; Hemab plans additional sites in Europe and the U.S. for Phase 2

The U.S. FDA has cleared the Investigational New Drug (IND) application and granted Fast Track Designation to HMB-001 for the treatment of GT

COPENHAGEN, DENMARK AND CAMBRIDGE, MASS., US – December 11, 2023 – Hemab Therapeutics, a clinical-stage biotechnology company developing novel prophylactic therapeutics for serious, underserved bleeding and thrombotic disorders, announced today that it has completed Phase 1, the single ascending dose part, and transitioned to Phase 2, the multiple ascending dose part, of its Phase 1/2 clinical study of HMB-001 in Glanzmann Thrombasthenia (GT), a platelet disorder that causes severe, potentially life-threatening bleeding episodes.

The company also announced that the U.S. Food and Drug Administration (FDA) has cleared Hemab's investigational new drug application (IND) for HMB-001 in GT, enabling enrollment in the U.S. Phase 1 of the clinical study was completed in the UK, and Phase 2 will include additional sites in Europe as well as the U.S.

In addition, the FDA granted Fast Track designation to HMB-001, emphasizing the seriousness and high unmet need for treatments for GT. The Fast Track program enables Hemab to have more frequent interactions with the FDA to facilitate the development of HMB-001.

"People living with Glanzmann Thrombasthenia can experience frequent, sometimes severe bleeds that can be life-threatening and compromise their quality of life. Currently, there are no prophylactic treatment options that would reduce or prevent these bleeding episodes," said Benny Sorensen, MD, PhD, CEO of Hemab. "The completion of Phase 1 on time and transitioning to Phase 2 is an important milestone for HMB-001 and Hemab. Furthermore, expanding our clinical study into the U.S., following clearance of the IND, and Fast Track designation are a testament to the importance of advancing prophylactic treatment for people living with Glanzmann Thrombasthenia."

The Phase 1/2 clinical study evaluates the safety, tolerability, pharmacodynamics, and pharmacokinetics of HMB-001. Initial efficacy signals based on an assessment of changes in bleeding frequency will also be measured. The study is composed of three parts: Part A, single ascending dose, Part B, multiple ascending dose, and Part C, extended dosing. Hemab plans to report data from the Phase 1, single ascending dose portion, at an international scientific conference in early 2024.

The Phase 1/2 study design was detailed in the company's poster presentation (number 1225), "A Phase 1/2, First-in-Human, Study to Investigate the Safety, Tolerability, Pharmacokinetics, Pharmacodynamics, and Efficacy of HMB-001 in Participants with Glanzmann Thrombasthenia," at the 65th American Society of Hematology Annual Meeting and Exposition.

About Glanzmann Thrombasthenia

Glanzmann Thrombasthenia (GT) is a rare and severe bleeding disorder associated with debilitating and sometimes life-threatening bleeding episodes. Initial findings from the international Glanzmann's 360 (GT360) natural history study, Hemab's research initiative in partnership with UK specialist research consultancy Haemnet, found that 87% of the 104 respondents reported experiencing at least one bleed in the previous week, and 37% of those bleeds required medical treatment.

These bleeding episodes have a significant impact on the mental health and quality of life of people living with GT. Low mood, emotional problems, and social isolation were reported by participants (66%, 50%, and 44% respectively) and 80% reported that they missed school or work due to bruising or bleeding. To date, there are no effective prophylactic treatment options for people living with GT.

About HMB-001

HMB-001 is bispecific antibody that binds and stabilizes endogenous factor VIIa (FVIIa) with one antibody arm and binds to TLT-1 on activated platelets with the other arm. This allows for accumulation of endogenous FVIIa in the body, recruitment of FVIIa directly to the surface of the activated platelets where it is known to facilitate clotting, and avoidance of clotting activity in the absence of tissue damage. HMB-001 is designed to be a first-in-class prophylactic treatment for Glanzmann Thrombasthenia with the potential to treat other debilitating rare bleeding disorders.

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 monoclonal and bispecific antibody-based therapeutics to transform the treatment paradigm for patients with high unmet need. The company's strategic guidance, Hemab 1-2-5™, targets the development of 5 independent assets by 2025 to deliver long-awaited innovation for patients with high unmet need blood-clotting disorders like Glanzmann Thrombasthenia, Factor VII Deficiency, Bernard Soulier Syndrome, Von Willebrand Disease, and other serious disorders. Learn more at hemab.com.

Media Contact

Lia Dangelico
lia.dangelico@vergescientific.com
540-303-0180