



Hemab Therapeutics Presents New Preclinical Research Demonstrating Effects of Its Bispecific Antibody HMB-001 in Factor VII Deficiency

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HMB-001 recognizes and binds to factor VII (FVII) protein variants associated with moderate/severe FVII deficiency and drives accumulation of endogenous FVII/FVIIa to normal ranges in in-vivo models

COPENHAGEN, DENMARK AND BOSTON, MASS., US – June 24, 2023 – [Hemab Therapeutics](#), a clinical-stage biotechnology company developing the first prophylactic therapeutics for serious, underserved bleeding and thrombotic disorders, announced results today from preclinical research of HMB-001 in models of factor VII (FVII) deficiency at the International Society on Thrombosis and Haemostasis (ISTH) 2023 Congress in Montreal.

"We believe that HMB-001 has the potential to transform treatment in several serious bleeding disorders and have already initiated a Phase 1/2 study in Glanzmann Thrombasthenia," said Benny Sorensen, MD, PhD, CEO and President of Hemab. "The new preclinical data presented today show HMB-001 successfully targeted and accumulated endogenous FVIIa to levels that would be expected to provide clinical benefit in FVII deficiency, supporting the potential for HMB-001 in an additional underserved bleeding disorder."

FVII is a protein necessary in the formation of hemostatic plugs to control bleeding. FVII deficiency can cause spontaneous or excessive and prolonged bleeding after injury or surgery; heavy or prolonged menstrual bleeding in women; and in very severe cases, life-threatening bleeding inside the skull or digestive tract.

HMB-001, Hemab's lead candidate, is a bispecific antibody that binds to and stabilizes endogenous activated FVII (FVIIa) with one antibody arm and localizes FVIIa to the surface of activated platelets by binding to TLT-1 with the other arm. This allows for accumulation of FVIIa in the body and recruitment of FVIIa directly to the surface of activated platelets at the site of vascular injury where FVIIa is known to facilitate the formation of protective hemostatic plugs to stop bleeding.

The preclinical research presented at ISTH, "HMB-001, a Bispecific anti-FVIIa/anti-TLT-1 Antibody Demonstrates Effect in Models of FVII Deficiency," assessed key requirements for HMB-001 to function in FVII deficiency, specifically its ability to bind with FVII protein variants associated with moderate/severe deficiency and the potential of HMB-001 to accumulate FVIIa in an *in-vivo* non-human primate model of FVII deficiency.

A panel of 12 FVII variants from the European Association for Haemophilia and Allied Disorders (EAHAD) database was produced based on high prevalence and association with moderate/severe FVII deficiency phenotype as well as proximity to the HMB-001 binding site. In subsequent binding studies, HMB-001 was shown to bind to all FVII variants at clinically relevant concentrations.

The ability of HMB-001 to accumulate endogenous FVIIa in FVII deficiency was assessed using small interfering RNA (siRNA) to knock down FVII/FVIIa to levels between 10 to 30 percent of normal in animal models (n=3). After continuous, stable knock down, HMB-001 (5 mg/kg) was administered. The total accumulation of FVIIa observed with HMB-001 was comparable to the normal range seen in healthy animals. These initial results suggest HMB-001 may have potential application as a treatment for FVII deficiency.

About HMB-001

HMB-001 is bispecific antibody that binds and stabilizes endogenous factor VIIa (FVIIa) with one antibody arm and TLT-1 on activated platelets with the other arm. This allows for accumulation of 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 was designed to be a first-in-class prophylactic treatment for Glanzmann Thrombasthenia (GT) with potential for other debilitating rare bleeding disorders, including factor VII deficiency.

About Hemab Therapeutics

Hemab is a clinical-stage biotech company developing the first prophylactic therapeutics for serious, underserved bleeding and thrombotic disorders. Based in the US and 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 clinical 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](#).

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