Hemab Therapeutics Presents Positive Clinical and Preclinical Data Across Bleeding Disorder Pipeline at ISTH 2025 Congress

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Sutacimig Phase 2 interim results demonstrate >50% reduction in treated bleeding events and prophylactic treatment potential in Glanzmann thrombasthenia
HMB-002 clinical proof-of-mechanism data in Von Willebrand disease support its potential as a first-in-class prophylactic therapy by elevating VWF levels and extending half-life
Natural history insights illuminate true patient burden and prophylactic opportunity to inform transformative, preventative solutions for underserved bleeding disorders

COPENHAGEN, DENMARK AND CAMBRIDGE, MASS., US – June 24, 2025 – Hemab Therapeutics, a clinical-stage biotechnology company developing novel prophylactic therapeutics for serious, underserved bleeding and thrombotic disorders, today announced clinical and preclinical results at the International Society on Thrombosis and Haemostasis (ISTH) 2025 Congress in Washington, DC. The company's 11 abstracts present breakthrough data for sutacimig (formerly HMB-001) and HMB-002, its lead therapeutic candidates, along with crucial insights from natural history studies.

"The data presented at ISTH underscore the potential for Hemab's impact in addressing the severe unmet needs of people with bleeding disorders," said **Kate Madigan**, Chief Medical Officer at Hemab. "Our sutacimig and HMB-002 data reveal important progress towards transformative, preventative solutions for these devastating diseases. We're demonstrating the possibilities to meaningfully reduce bleeding and elevate care for people living with Glanzmann thrombasthenia and Von Willebrand disease."

Highlights of Clinical Presentations

Sutacimig shows promising safety and efficacy in Glanzmann thrombasthenia (GT). The Phase 2 study is fully enrolled (N=34 in Part B), and data show a clinically meaningful reduction in treated bleeding events.

- **Clinically meaningful bleeding reduction:** Sutacimig treatment in the efficacy population (n=33) demonstrated a >50% reduction in median Annualized Treated Bleeding Rate (ATBR), with the median ATBR decreasing from 21.2 to 4.61. Select patient reports indicate a reduced severity of bleeds and decreased IV rFVIIa use.
- **Safety profile:** Sutacimig demonstrated a favorable safety profile, with most adverse events (AEs) being mild to moderate in severity, and no reported thromboses or discontinuations due to AEs in ongoing dose levels of 0.3 and 0.6 mg/kg.
- **PK/PD insights:** At ongoing dose levels of 0.3 and 0.6 mg/kg, Factor VIIa (FVIIa) accumulation reached 2–4× above baseline. Exploratory thrombin generation assays suggested hemostatic activity, with observed improvements comparable to those seen with clinically relevant concentrations of rFVIIa.

Late Breaking Abstract presentation of HMB-002 demonstrates positive proof of mechanism in Von Willebrand disease (VWD), with the first-in-human VELORA Pioneer study in Type 1 VWD patients.

- **Favorable safety profile:** No treatment-emergent AEs, injection-site reactions, hypersensitivity, or serious AEs were reported in the VELORA Pioneer Cohort A1.
- **Encouraging endogenous VWF and FVIII accumulation:** The VELORA Pioneer study demonstrated that the initial single 20 mg subcutaneous dose of HMB-002 induced consistent and sustained increases in VWF and Factor VIII levels. Within 14 days, mean VWF rose >1.5× from baseline.
- **Normalized APTT and improved thrombin generation:** Parallel to the increases in VWF, levels of Factor VIII were corrected, resulting in normalization in APTT and thrombin generation.

Preclinical Data Highlights

- **Sutacimig enhances rFVIIa efficacy in vitro**, enabling potentially lower doses for breakthrough bleeds. Studies also show sutacimig preserves the function of stored platelet concentrates (PCs), supporting its mechanism that could reduce both rFVIIa doses and PC volumes needed for bleeding control.
- **Nonclinical safety evaluations of HMB-002** in cynomolgus monkeys and in vitro/ex vivo assays showed no adverse effects, immunotoxicity (including complement or platelet activation or cytokine release), or off-target binding, indicating a favorable safety profile.
- **Extended half-life and infrequent dosing:** Pharmacokinetic modeling estimated a long HMB-002 half-life, supporting infrequent subcutaneous dosing. In monkey studies, HMB-002 also extended recombinant VWF's half-life ~3×, driving endogenous VWF and FVIII accumulation.

Natural History Highlights

- **GT:** ATHN Transcends GT study reports a mean Annualized Bleeding Rate (ABR) of 72.0 (ATBR 51.9); 56% of participants experienced severe disease burden. Real-world data highlight a cycle of blood loss → iron deficiency anemia, disproportionately impacting women (75% hemorrhage; 23% anemia) and men (36% GI bleeds; 30% hematomas). Care is often limited to on-demand treatments outside specialized centers, underscoring the need for prophylaxis.
- **VWD:** VWD360 study reveals 1.33 bleeds/week on average; 71% miss work; 73% report low mood. New data challenge assumptions: Type 1 VWD bleeding rates are comparable or higher than other subtypes, revealing a major care gap—especially for women with heavy menstrual bleeding.

"Our multiple ISTH presentations underscore Hemab's commitment to leveraging the biotechnological revolution and deep hemostasis expertise to develop innovative prophylactic therapies for patients with bleeding disorders. Listening to people living with these diseases has generated extensive natural history data revealing substantially greater unmet medical need than previously recognized, driving our continued clinical advancement."
—**Benny Sorensen, MD, PhD**, CEO of Hemab

About Glanzmann Thrombasthenia

Glanzmann thrombasthenia (GT) is a severe bleeding disorder marked by debilitating, sometimes life-threatening bleeding episodes. In the GT360 natural history study (117 participants), 88% reported at least one bleed in the previous week; 34% required treatment. Disease burden includes low mood (67%), emotional problems (52%), social isolation (46%), and missed school/work (81%). No prophylactic treatments currently exist.

About Sutacimig (formerly HMB-001)

Sutacimig is a subcutaneously administered bispecific antibody: one arm stabilizes endogenous FVIIa; the other recruits it to activated platelets via TLT-1, facilitating hemostatic plug formation. It is designed as a first-in-class GT prophylactic. Designations: FDA Fast Track & Orphan Drug; UK MHRA ILAP. See ClinicalTrials.gov NCT06211634.

About Von Willebrand Disease

VWD is the most common inherited bleeding disorder, caused by quantitative/qualitative VWF defects. Patients suffer mucocutaneous bleeds and heavy menstrual bleeding, leading to chronic blood loss and iron deficiency anemia. Current therapies focus on symptom management, not underlying VWF dysfunction.

About HMB-002

HMB-002 is a monovalent human antibody targeting VWF's C-terminal CK domain to shield it from degradation and boost endogenous VWF without compromising function. Nonclinical and clinical data suggest strong therapeutic potential. See ClinicalTrials.gov NCT06610201 & NCT06754852.

About Hemab Therapeutics

Hemab is a multi-asset biotech developing prophylactic therapies for bleeding and thrombotic disorders. Based in Cambridge, MA and Copenhagen, Denmark, Hemab's Hemab 1-2-5™ strategy drives a pipeline targeting high-need diseases (GT, Factor VII Deficiency, VWD, etc.). Learn more at hemab.com and follow us on LinkedIn, Facebook, Instagram, and X.

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ClinicalTrials.gov Links:
https://clinicaltrials.gov/study/NCT06211634?term=NCT06211634&rank=1
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