Hemab Therapeutics Announces Positive Complete Phase 2 Data for Sutacimig in Glanzmann Thrombasthenia at ASH 2025; Plans to Advance to Pivotal Phase 3 Study

December 8, 2025

Consistent and robust efficacy demonstrated across bleed types, locations, and dose cohorts

The weekly dosing cohort achieved an estimated 87% reduction in annualized treated bleeding rate (ATBR)

Results validate potential as the first prophylactic therapy to address the heavy physical and psychosocial burden of Glanzmann thrombasthenia; Phase 3 registration study planned for 2026

CAMBRIDGE, MA, USA & COPENHAGEN, Denmark – December 8, 2025 – Hemab Therapeutics, a clinical-stage biotechnology company developing novel prophylactic therapeutics for serious, underserved bleeding and thrombotic disorders, today announced positive results from its completed Phase 2 multiple ascending dose (MAD) portion of the CL-101 study of sutacimig for the prophylactic treatment of Glanzmann thrombasthenia (GT).

The data, presented today in an oral session at the 67th American Society of Hematology (ASH) Annual Meeting in Orlando, demonstrate clinically meaningful efficacy that was consistent across bleed locations, bleed types (spontaneous and traumatic), and dose cohorts evaluated. Based on these results, Hemab plans to **advance sutacimig into a pivotal Phase 3 registration study in 2026**.

"These Phase 2 results represent transformational potential for people living with Glanzmann thrombasthenia, who have waited a lifetime for a modern prophylactic treatment," said **Benny Sorensen, MD, PhD, Chief Executive Officer of Hemab**. "The clinically meaningful reductions in bleeding demonstrated across this study provide compelling evidence that sutacimig could shift the treatment paradigm from reactive crisis management to prevention. We are moving forward with urgency to bring this therapy to patients who have been overlooked for far too long."

"What stands out in these results is the reduction of the most severe bleeding events requiring intensive interventions," said **Paul Saultier, MD, PhD, Head of the French Platelet Reference Center, APHM Hospital de la Timone**. "These are the bleeds that bring patients to the hospital and create the greatest burden. Combined with the reductions we saw across different bleed types and anatomical locations, these data suggest sutacimig could provide meaningful benefit for GT patients."

Phase 2 Clinical Data Highlights: Hemab's Phase 2 study of sutacimig (N=34) is intended to address a profound gap in care for GT as there are currently no effective prophylactic treatment options. Sutacimig was assessed at varying doses to determine the optimal regimen for Phase 3. Key findings include:

- **Consistent and clinically meaningful reductions in bleeding:** Sutacimig demonstrated robust and clinically meaningful reductions in ATBR across dose cohorts, with an approximate 50% reduction in mean ATBR in the overall efficacy population (N=31). The weekly dosing cohort achieved an estimated 87% reduction in ATBR (95% CI: 80%, 92%). Importantly, efficacy was consistent across all major bleed locations including nose, gum/mouth, and gastrointestinal sites, and demonstrated robust activity against both traumatic and spontaneous bleeding events.
- **Reduction of bleeds requiring high intensity treatment:** Participants experienced a 100% reduction in mean ATBR of bleeding events requiring high intensity treatment (defined as those requiring recombinant factor VIIa, platelet transfusions, plasma, cryoprecipitate, or medical procedures) during the treatment period. This represents a meaningful reduction of the most clinically consequential acute bleeding events.
- **Dosing schedule optimization:** Analyses indicate that weekly dosing provides consistent exposure across the dosing interval, resulting in optimal clinical response.
- **Safety and tolerability:** Overall sutacimig was well tolerated. Adverse events were primarily mild to moderate and either non-specific or typical for patients with GT, with a single related serious adverse event (grade 2 DVT) occurring at the highest dose level (0.9 mg/kg). Anti-drug antibodies impacting PK/PD were observed in five participants; however, titers resolved in one participant with continued dosing, and no associated safety events were reported.
- **Retention:** Underscoring the perceived benefit, **82%** of participants elected to enter the ongoing long-term extension study.

Presentation Details

- **Title:** Sutacimig, a Novel Bispecific Antibody for Prophylactic Treatment of Glanzmann Thrombasthenia: Analysis of a Phase 2 Study
- **Session:** OCCC - W304EFGH
- **Presenter:** Paul Saultier, MD, PhD, APHM Hospital de la Timone, France

About Glanzmann Thrombasthenia
Glanzmann thrombasthenia (GT) is a 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 Sutacimig (formerly HMB-001)
Sutacimig 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 mechanism allows for the accumulation of endogenous Factor VIIa in the body and recruitment of Factor VIIa directly to the surface of the activated platelets, where it facilitates hemostatic plug formation. Sutacimig is designed to be a first-in-class prophylactic treatment for Glanzmann thrombasthenia (GT) with the potential to treat other debilitating bleeding disorders. The U.S. Food and Drug Administration granted Fast Track Designation and Orphan Drug Designation to sutacimig for the treatment of GT while the UK 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 Hemab Therapeutics
Hemab is a multiple clinical-asset biotechnology 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 ™, targets building a pipeline of development programs to deliver long-awaited innovation for people with high unmet need diseases like Glanzmann thrombasthenia, Factor VII Deficiency, Von Willebrand Disease, and others. Learn more at hemab.com. Follow us on LinkedIn, Facebook, Instagram, and X.

Media:
Deerfield
Peg Rusconi
peg.rusconi@deerfieldgroup.com

Investor Contact:
Hemab Therapeutics
Mads Behrndt
mads@hemab.com