Hemab Therapeutics Announces First Drug Candidate: HMB-001, a Novel Bispecific Antibody with Potential for Treatment of Rare Bleeding Disorders

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Preclinical data demonstrate HMB-001's promise as a preventative treatment for patients with underserved bleeding disorders, including Glanzmann Thrombasthenia

Data presented at 2022 European Association for Haemophilia and Allied Disorders Virtual Congress

Copenhagen, Denmark and Boston, Mass., February 4, 2022

Hemab Therapeutics, a biotechnology company developing next-generation therapeutics for serious, underserved bleeding and thrombosis disorders, today unveiled its lead candidate, HMB-001, a novel bispecific antibody with potential for the treatment of Glanzmann Thrombasthenia (GT) and other rare bleeding disorders. The company presented promising preclinical data on HMB-001 during the 2022 European Association for Haemophilia and Allied Disorders Virtual Congress, held February 2-4, 2022.

"Patients suffering with rare bleeding and clotting disorders deserve the same advancements in care that hemophilia patients have experienced for decades," said Benny Sorensen, MD, PhD, President and CEO of Hemab. "We aim to reach every patient suffering from these often-unmanageable diseases and offer innovative, state-of-the-art prophylactic solutions, like HMB-001, to restore proper clotting function and help them live more healthy, active lives."

At the conference, Hemab highlighted data from studies exploring the mechanism of action for HMB-001 in cynomolgus monkeys and experiments with platelets from GT patients. The in vivo subcutaneously administered single dosages of HMB-001 showed dose-dependent accumulation of endogenous factor VIIa (FVIIa), with durability of effect supporting weekly to once monthly dosing. TLT-1, a protein found on the surface of activated platelets at the site of injury, was confirmed to be present on GT platelets and HMB-001 was found to substantially improve FVIIa facilitated ex vivo GT platelet aggregation in a TLT-1-dependent manner.

Combining accumulation and activity potentiation, HMB-001 brings the activity of endogenous FVIIa to levels that are considered therapeutically effective based on clinical experience with recombinant factor VIIa (rFVIIa). HMB-001 is a bispecific antibody, with one arm binding FVIIa already present in the patient's bloodstream (endogenous) and the other arm binding TLT-1 present on the surface of the activated platelet; this effectively, and specifically, recruits FVIIa to the surface of the activated platelet. The arm that binds FVIIa prevents the body from breaking it down, increasing the available concentration in the bloodstream to a new higher steady state level over the course of a few days. The neutral binding of FVIIa means it is still active and can play a role in forming hemostatic plugs. By targeting activated platelets at the site of vascular damage with a high concentration of FVIIa (via TLT-1), the body generates enough structurally sound building blocks to form healthy hemostatic plugs.

"HMB-001 is a pioneering approach that binds, amplifies, and recruits endogenous FVIIa to the site of vascular injury via binding to TLT-1 expressed on activated platelets to overcome defects in patients' ability to form healthy clots," said Dr. Roger Schutgens, Professor and Consulting Hematologist at University Medical Centre Utrecht, and Head of the Van Creveldkliniek, a Netherlands-based center for excellence in rare disorders of thrombosis and hemostasis. "The data show it may have broad applicability across multiple bleeding disorders, enabling subcutaneous and long-term prophylactic treatment."

In partnership with Haemnet, which works with healthcare professionals, patients, communities, and other stakeholders to improve the lives of people with bleeding disorders through research, education, and communication, Hemab is commencing natural history studies of patients with GT through early 2022 to better understand the frequency and types of bleeds, number of individuals affected, disease variability, and impacts on life and lifespan.

Hemab will begin a Phase 1/2 clinical trial in patients with GT in late 2022 to assess HMB-001's safety and efficacy, initially in collaboration with leading UK Phase 1 clinical research firm, Richmond Pharmacology Ltd.

About Hemab Therapeutics

Hemab is a biotech company developing next generation therapeutics for serious, underserved bleeding and thrombosis disorders. The company announced an oversubscribed $55 million Series A led by RA Capital Management, Novo Holdings and HealthCap in July 2021. Based in Denmark and the US, Hemab aims to progress its pipeline of monoclonal and bispecific antibody-based therapeutics with the vision of transforming the treatment paradigm for blood disease patients—from orphan disorders to broad indications with high unmet need.
Learn more at hemab.com.

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