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TIP1. TRIALS IN PROGRESS: CLASSICAL HEMATOLOGY

A Phase 2 study to evaluate the safety, tolerability, pharmacokinetics, pharmacodynamics, and efficacy of sutacimig for prophylaxis in glanzmann thrombasthenia: A trial in progress

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Abstract Background: Glanzmann thrombasthenia (GT) is a severe genetic bleeding disorder characterized by impaired platelet aggregation preventing normal clot formation. Variants in *ITGA2B* and *ITGB3* genes render the αIIbβ3 integrin, which is essential for platelet aggregation, non-functional. This impairment in clotting leads to frequent bleeding events ranging from low-volume mucocutaneous bleeds to life-threatening hemorrhages, contributing to, among others, iron deficiency anemia and, most importantly, diminished quality of life.

People with GT experience frequent bleeding episodes requiring treatment, with numerous untreated bleeds driving a debilitating cycle of heavy menstrual bleeding and iron deficiency anemia that can worsen the bleeding tendency. Current standard of care is reactive, with no approved therapies for prophylaxis. Available treatments, such as intravenous recombinant FVIIa and platelet transfusions, have critical limitations and necessity of hospitalizations, and in the case of platelet transfusions, risk of anti-platelet immunization.

Sutacimig (HMB-001), an investigational bispecific antibody, is currently being evaluated to prevent and reduce bleeding events. Sutacimig administered subcutaneously binds to endogenous FVIIa with one arm, increasing its half-life and accumulation in bloodstream. The other arm binds to TLT-1 expressed only on activated platelets, thereby localizing endogenous FVIIa on activated platelets and potentiating FVIIa activity. The dual mechanism of action of sutacimig allows potentiation of downstream thrombin generation and fibrin formation at the site of platelet activation, fostering the development of stable fibrin-platelet mesh essential for hemostasis.

Objectives: To investigate the pharmacokinetics (PK), pharmacodynamics (PD), safety, tolerability, and preliminary efficacy of sutacimig in participants with GT.

Methods:

This open-label study (NCT06211634) is designed to assess sutacimig in GT across three parts:

- Part A (Completed): single ascending dose study.
- Part B (Completed): multiple ascending dose study assessing effects on bleeding events.
- Part C (Ongoing): long-term extension study (over 9 months) evaluating safety and impact on bleeding frequency and severity.

Participants aged 18-67 years with GT, experiencing an average ≥2 bleeding events per week, and at least one spontaneous or traumatic bleed event within the last 12 months requiring prescribed treatment, medical or surgical procedure were eligible for Part B. This study is ongoing at sites in Belgium, France, Italy, The Netherlands, United Kingdom, and United States. Key outcomes include annualized bleeding rates and annualized treated bleeding rates, and safety and tolerability measures.

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