



The 65th ASH Annual Meeting Abstracts

ONLINE PUBLICATION ONLY**311.DISORDERS OF PLATELET NUMBER OR FUNCTION: CLINICAL AND EPIDEMIOLOGICAL****A Prospective, Observational Study of Bleeding and Quality of Life in Patients with Glanzmann Thrombasthenia in the United Kingdom: Updated Results**

Catherine Rea, MD PhD¹, Thomas York, MD², Ulrike Lorch, MD FRCA, FFPM², Kate Khair, PhD³, Luke Pembroke³, Jigar Amin, PharmD⁴

¹Hemab Therapeutics, Copenhagen, Denmark

²Richmond Pharmacology, London, United Kingdom

³Haemnet, London, United Kingdom

⁴Hemab Therapeutics, Colleyville, TX

Glanzmann thrombasthenia (GT) is a rare bleeding disorder characterized by impaired platelet function. Recent studies suggest that patients with GT experience high-frequency bleeds, often access medical care, and have a significant burden of disease. There are no detailed, prospective studies characterizing bleeds and associated patient-reported impacts in GT.

We conducted a prospective diary study characterizing bleeds and associated patient-reported impacts in 30 patients with GT over 3 months. Patients record bleed events daily in a custom-designed electronic bleed diary. At approximately the same time each day, they answer the question, "Have you had a bleed?" If the answer is yes, they also include the anatomical site of the bleed. Female patients are asked to record menstrual bleeding. For each bleed reported, patients can document symptoms, treatments received, and the location where the treatment was received (e.g., at home or in the hospital). They are also asked to record the socioeconomic impacts of individual bleeds, such as missed social or leisure activities, missed work, and missed education.

The updated results confirm the high frequency of bleeds in patients with GT. Key variables will include diary completion compliance, mean monthly bleed rate (excluding menstrual bleeding), as well as detailed recordings of types and anatomical locations of bleed events. Further details will include patient reported, bleed-associated symptoms and socioeconomic impacts. In addition, a thorough description of menstrual bleeds and the impacts reported by women will be presented.

This study provides further evidence of the high frequency and significant impact of bleeds in patients with GT. These prospective data establish a direct link between bleed events and the corresponding impact on QOL (quality of life) in patients with GT. In addition, the findings of this study suggest that there is a need for new treatments for GT that can reduce the frequency of bleeds and improve the quality of life for patients.

Disclosures No relevant conflicts of interest to declare.

<https://doi.org/10.1182/blood-2023-187558>