ISRCTN15050281 https://doi.org/10.1186/ISRCTN15050281

Prospective bleeding study in Glanzmann's thrombasthenia

Submission date	Recruitment status	[_] Prospectively registered
15/08/2023	No longer recruiting	[_] Protocol
Registration date	**Overall study status**	[_] Statistical analysis plan
28/09/2023	Completed	[X] Results
Last Edited	**Condition category**	[_] Individual participant data
15/04/2024	Haematological Disorders	

Plain English summary of protocol

Background and study aims

Glanzmann's thrombasthenia (GT) is a rare, inherited bleeding disorder affecting both males and females. There are about 150 patients with GT in the UK. In normal blood clotting, platelet cells attach at the site of injury and group together via proteins on the platelet cell surface, forming a plug to stop bleeding. In GT a protein is missing or reduced (glycoprotein IIb/IIIa). The platelets don't plug injuries efficiently resulting in increased bleeding in patients with GT. Due to the rare nature of the condition, there has been limited knowledge regarding the types and frequency of bleeds in GT. Little is known about the impact of bleeding on patient symptoms and quality of life.

Hemab is a company developing HMB-001, a bispecific antibody, for the prophylactic treatment of GT. Understanding bleeding symptoms and impact will provide Hemab valuable information as it moves forward in developing HMB-001. The aim of this study is to collect information regarding bleeding episodes, quality of life, and the social and clinical impact of bleeds in participants with GT.

Who can participate?

Patients aged 16 years and over with GT

What does the study involve?

Participants undertake quality-of-life assessments to explore the effects of their symptoms. They will be asked to complete a detailed daily diary for 3 months to document all bleeding symptoms. This will help us learn about the types and frequency of bleeding that people experience. Participants will also be asked about the effects of bleeds. For example, participants will be asked to report pain, missed activities caused by bleeds or visits to hospital required. After 85 days, participants will be given the option to continue to complete the diary card and receive monthly follow-up calls for up to a further 3 months.

What are the possible benefits and risks of participating?

There are no direct benefits for participants. However, the study may help others in the future. Completing the questionnaires and the diary will require giving up some time every day for 12 weeks. Although the researchers do not think that answering the surveys or the diary completion will be distressing, weekly calls from will be undertaken to support the participants.

Psychological support can be accessed via a GP or through the treatment centre if the participant feels this is required.

Where is the study run from?
Richmond Pharmacology Ltd (UK)

When is the study starting and how long is it expected to run for?
May 2022 to September 2023

Who is funding the study?
Hemab Therapeutics ApS (Denmark)

Who is the main contact?
Dr Catherine Rea, catherine@hemab.com

Contact information

Type(s)
Principal investigator

Contact name
Dr Ulriche Lorch

ORCID ID
https://orcid.org/0000-0002-6316-5860

Contact details
Richmond Pharmacology Ltd
1A Newcomen Street
London Bridge
London
United Kingdom
SE1 1YR
+44 (0)2070425800
u.lorch@richmondpharmacology.com

Type(s)
Scientific

Contact name
Dr Joao Almeida Melo

ORCID ID
https://orcid.org/0000-0002-6364-9822

Contact details
Richmond Pharmacology Ltd
1A Newcomen Street
London Bridge
London
United Kingdom

SE1 1YR
+44 (0)2070425800
j.melo@richmondpharmacology.com

Additional identifiers

Integrated Research Application System (IRAS)
313531

Protocol serial number
HMB-001-NH01

Study information

Scientific Title
A prospective, observational study of bleeding and quality of life in subjects with Glanzmann thrombasthenia in the United Kingdom

Study objectives
Glanzmann's thrombasthenia (GT) is a rare, inherited bleeding disorder affecting both males and females. There are approximately 150 patients with GT in the UK. In normal blood clotting, platelet cells attach at the site of injury and group together via proteins on the platelet cell surface, forming a plug to stop bleeding. In GT a protein is missing or reduced (glycoprotein IIb /IIIa). The platelets don't plug injuries efficiently resulting in increased bleeding in patients with GT. Due to the rare nature of the condition, there has been limited knowledge regarding the types and frequency of bleeds in GT. Little is known about the impact of bleeding on patient symptoms and quality of life. In order to develop new treatments and ways of managing patients, it is important to first understand the types of symptoms and their impact on patients. The Glanzmann 360 project is being undertaken to improve understanding of GT and the effects that it has on individuals.

Ethics approval required
Ethics approval required

Ethics approval(s)
Approved 10/05/2022, London - Surrey Research Ethics Committee (The Old Chapel, Royal Standard Place, Nottingham, NG1 6FS, United Kingdom; +44 (0)2071048388; surrey.rec@hra.nhs.uk), ref: 22/PR/0374

Primary study design
Observational

Study design
Observational cohort study

Study type(s)
Diagnostic, Quality of life, Screening

Health condition(s) or problem(s) studied
Glanzmann's thrombasthenia

Interventions
The researchers will ask participants to undertake quality-of-life assessments to explore the effects of their symptoms. They will then be asked to complete a detailed daily diary for 3 months to document all bleeding symptoms. This will help us learn about the types and frequency of bleeding that people experience. Participants will also be asked about the effects of bleeds. For example, participants will be asked to report pain, missed activities caused by bleeds or visits to the hospital required.

Intervention Type
Other

Primary outcome(s)
1. Quantitative assessment of bleeding using the International Society on Thrombosis and Haemostasis Bleeding Assessment Tool (ISTH-BAT) at baseline
2. Clinical outcomes over 3 months of observation, including:
2.1. Number of bleeding events measured using daily electronic bleed diary
2.2. Sites of bleeding events measured using daily electronic bleed diary
2.3. Treatments administered for bleeding events measured using daily electronic bleed diary
2.4 Number of hospitalizations due to bleeding events measured using daily electronic bleed diary
4. Quality of life measured at baseline using:
4.1. Patient-Reported Outcomes Measurement Information System (PROMIS)-29
4.2. Menstrual Impact Questionnaire (MIQ)
5. Social outcomes, including missed work, school, and activities, measured using [daily electronic bleed diary] over 3 months of observation

Key secondary outcome(s)
There are no secondary outcome measures

Completion date
01/09/2023

Eligibility

Key inclusion criteria
Each participant must meet all of the following inclusion criteria to be eligible for enrollment in the study:
1. Participant has signed the informed consent form
2. ≥16 years of age
3. Participant has confirmed GT (documented diagnosis on patient held registry document or clinical record)
4. Participant has sufficient English language skills to complete the consent process, electronic questionnaires, and diary

Participant type(s)
Patient

Healthy volunteers allowed
No

Age group
Mixed

Lower age limit
16 Years

Upper age limit
99 Years

Sex
All

Total final enrolment
30

Key exclusion criteria
Each participant must not meet any of the following exclusion criteria to be eligible for enrolment in the study:
1. Participant is currently pregnant
2. Participant has a cancer diagnosis (excluding benign or localized disease eg. basal cell carcinoma of the skin) and is currently undergoing systemic treatment
3. Participant has renal impairment currently requiring renal replacement therapy
4. Participant has liver disease currently requiring treatment
5. Participant has heart disease currently requiring treatment
6. Participant is receiving current treatment with anticoagulation or anti-platelet therapies

Date of first enrolment
10/05/2022

Date of final enrolment
01/05/2023

Locations

Countries of recruitment
United Kingdom

England

Study participating centre
Richmond Pharmacology Ltd
1A Newcomen Street
London Bridge
London
United Kingdom
SE1 1YR

Sponsor information

Organisation
Hemab Therapeutics

Funder(s)

Funder type
Industry

Funder Name
Hemab Therapeutics

Results and Publications

Individual participant data (IPD) sharing plan
The data-sharing plans for the current study are unknown and will be made available at a later date.

IPD sharing plan summary
Data sharing statement to be made available at a later date

Study outputs

Output type	Details	Date created	Date added	Peer reviewed?	Patient-facing?
Results article		02/11/2023	15/04/2024	Yes	No