ISRCTN63686338 https://doi.org/10.1186/ISRCTN63686338

The lived experience of people with Glanzmann's thrombasthenia

Submission date
06/04/2022

Recruitment status
No longer recruiting

Registration date
07/04/2022

Overall study status
Completed

Last Edited
10/10/2024

Condition category
Haematological Disorders

[X] Prospectively registered

[X] Protocol

[_] Statistical analysis plan

[X] Results

[_] Individual participant data

Plain English summary of protocol

Background and study aims
Glanzmann’s thrombasthenia (GT) is a rare inherited blood clotting (coagulation) disorder where platelets (small cells in the blood) do not stick together properly. This leads to bruising and abnormal bleeding, which may be severe and may be life-threatening. Treatment is given at specialist hospitals which often means a delay in access to treatment and is burdensome to the patient and family. Males and females are affected equally, but women have many more symptoms because of menstruation and childbirth, often resulting in anaemia and severe limitations on lifestyle. This study aims to investigate the impact of living with GT on the affected person and their family - this work has never been done before.

Who can participate?
Adults aged over 16 years and parents of children aged under 16 years with GT via UK hospitals and social media. This will allow non-UK participants.

What does the study involve?
Part one of the study is in two parts - the first is the completion of the online survey using questionnaires that are recognised as useful in measuring quality of life, mental health, impact of bleeding and self-management. From these survey respondents the researchers will invite 30 people with GT (10 women, 10 men and 10 parents of affected children) to take part in an in-depth interview about the impact of living with GT on their daily lives.

What are the possible benefits and risks of participating?
There are no immediate benefits of taking part. The risk is that discussing sensitive issues may cause emotional distress. The researchers recognise this and will give participants a 'debrief sheet' with details of where to get support. Most treatment centres have access to psychological support services for patients with bleeding disorders.

Where is the study run from?
Haemnet (UK)

When is the study starting and how long is it expected to run for?
October 2021 to May 2023

Who is funding the study?
Hemab (Denmark)

Who is the main contact?
Dr Kate Khair
Kate@haemnet.com

Contact information

Type(s)
Scientific

Contact name
Dr Kate Khair

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Additional identifiers

EudraCT/CTIS number
Nil known

IRAS number
308011

ClinicalTrials.gov number
NCT05315232

Secondary identifying numbers
IRAS 308011, CPMS 52408

Study information

Scientific Title
Glanzmann's 360. The lived experience of people with Glanzmann's thrombasthenia: a mixed-methods observational study

Acronym
Glanzmann's 360

Study objectives
Glanzmann’s thrombasthenia (GT) is a rare inherited platelet disorder characterized by impaired platelet function due to absent or reduced glycoprotein IIb/IIIa complex which is instrumental in platelet aggregation. The bleeding phenotype varies but is usually severe with most people being diagnosed in early childhood. Patients and families experience considerable psychosocial impact and treatment burden. There remains a need for a comprehensive understanding of the experience of people with GT in order to identify:
1. The nature and range of symptoms that people present with to services
2. The variability in pathways through which patients progress to access appropriate care
3. The impact of living with GT on the individual’s quality of life and that of their family

Ethics approval required
Old ethics approval format

Ethics approval(s)
Approved 29/03/2022, South Central - Oxford B Research Ethics Committee (Ground Floor, Temple Quay House, 2 The Square, Bristol, BS1 6NP, UK; +44 (0)207 104 8178, +44 (0)207104 8360, +44 (0)207 104 8270; oxfordbrec@hra.nhs.uk), ref: 22/SC/0095

Study design
Mixed-methods observational study

Primary study design
Observational

Secondary study design
Cohort study

Study setting(s)
Internet/virtual

Study type(s)
Quality of life

Participant information sheet
See study outputs table

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

Interventions
Participants will be identified via their treatment centres and will be sent a postcard inviting them to undertake an online survey using validated quality of life, impact of bleeding, psychological health and well-being instruments. They will then be able to opt in to an in-depth interview which will be offered either face to face or via an online platform, at a time that is convenient to them.

Intervention Type
Other

Primary outcome measure

The impact of Glanzmann's thrombasthenia on affected individuals and their families, measured using an array of validated questionnaires (EQ5D, Minnesota Importance Questionnaire [MIQ], Patient Health Questionnaire-9 [PHQ9], Patient-Reported Outcomes Measurement Information System [PROMIS], Rosenberg's self-efficacy scale) via an online survey at baseline

Secondary outcome measures
1. Satisfaction with current treatments and management approaches
2. Identification of areas of unmet need among people with Glanzmann's thrombasthenia

Both will be measured using one in depth (up to 1-hour duration) qualitative interview per participant at the end of the study

Overall study start date
01/10/2021

Completion date
01/05/2023

Eligibility

Key inclusion criteria
1. Confirmed diagnosis of inherited Glanzmann's thrombasthenia
2. Adults aged >16 years
3. Parents of children aged <16 years
4. Ability to read/write/speak English for questionnaire and interview completion
5. Give informed consent

Participant type(s)
Patient

Age group
Adult

Lower age limit
16 Years

Sex
Both

Target number of participants
150

Total final enrolment
104

Key exclusion criteria
1. Acquired Glanzmann's thrombasthenia
2. Participants unable to read/write/speak English
3. Those who do not consent

Date of first enrolment
28/05/2022

Date of final enrolment
01/05/2023

Locations

Countries of recruitment
England

United Kingdom

Study participating centre
Churchill Hospital
Churchill Hospital
Old Road
Headington
Oxford
United Kingdom
OX3 7LE

Study participating centre
Birmingham Childrens Hospital
Steelhouse Lane
Birmingham
United Kingdom
B4 6NH

Study participating centre
Basingstoke and North Hampshire Hospital
Aldermaston Rd,
Basingstoke
United Kingdom
RG24 9NA

Study participating centre
Queen Alexandras Hospital
Southwick Hill Road
Cosham
Portsmouth
United Kingdom
PO6 3LY

Study participating centre
St. Bartholomews Hospital
West Smithfield
London
United Kingdom
EC1A 7BE

Study participating centre
Royal Free Hospital
Pond Street
London
United Kingdom
NW3 2QG

Study participating centre
Bristol Childrens Hospital
Upper Maudlin Street
Bristol
United Kingdom
BS2 8BJ

Sponsor information

Organisation
Haemnet

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Sponsor type
Research organisation

Funder(s)

Funder type
Industry

Funder Name
Hemab

Results and Publications

Publication and dissemination plan
Planned publications in high-impact peer-reviewed journals.

Intention to publish date
31/10/2024

Individual participant data (IPD) sharing plan
The datasets generated during and/or analysed during the current study will be available upon request from Dr Kate Khair, Director of Research at Haemnet (kate@haemnet.com). The data will become available at study end (December 2023) and will be available for 5 years. Reasonable requests for access to anonymised data will be reviewed by Haemnet as long as the data is to be used for non-commercial analyses. Participant consent to share anonymised data will have been granted. Data will be anonymised to study participant number only.

IPD sharing plan summary
Available on request

Study outputs

Output type	Details	Date created	Date added	Peer reviewed?	Patient-facing?
Participant information sheet	version 1.1	24/03/2022	07/04/2022	No	Yes
Protocol file	version 1.0	28/02/2022	07/04/2022	No	No
HRA research summary			28/06/2023	No	No
Results article		09/10/2024	10/10/2024	Yes	No