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The lived experience of people with von Willebrand disease

Submission date	**Recruitment status**	☒ Prospectively registered
21/06/2023	No longer recruiting	☒ Protocol
Registration date	**Overall study status**	☐ Statistical analysis plan
24/08/2023	Completed	☐ Results
Last Edited	**Condition category**	☐ Individual participant data
18/12/2024	Haematological Disorders	☒ Record updated in last year

Plain English summary of protocol

Background and study aims
von Willebrand Disease (vWD) is an inherited blood clotting disorder that causes prolonged or spontaneous bleeding from birth. Affected individuals tend to bruise easily, may have frequent nosebleeds, bleeding from the gums, joints and sometimes stomach and intestinal bleeding (more common later in life). vWD also causes prolonged bleeding following injury, trauma, or surgery (including dental work). Women with vWD can have prolonged and heavy periods, they may also have an increased risk of excessive bleeding during pregnancy and childbirth. The severity and frequency of the bleeding episodes in vWD can vary greatly among affected individuals, even within the same family.

Treatment varies based on the diagnosis and rate and type of any bleeding experienced though is usually ‘on-demand’ (given after bleeding occurs) with some patients prophylaxis (treatment given to prevent bleeding from occurring) may be needed.
The lack of routine prophylaxis means that most patients are reliant on hospital-delivered care, which may involve frequent clinic appointments, causing prolonged bleeding due to a lack of timely administration of treatment. This can result in concurrent illnesses such as iron deficiency anaemia, which further impacts on the quality of life of affected individuals.
There remains a need for a comprehensive understanding of the experience of people with vWD in order to identify:

- The nature and range of symptoms that people experience and how these vary with the different disease subtypes.
- The variability in pathways through which people with vWD progress to access appropriate care.
- The impact of living with vWD on the individual’s quality of life.

Who can participate?
Adults over 16 years in the UK and Ireland & over 18 years in the US with a confirmed diagnosis of vWD.

What does the study involve?
Every participant will be asked to complete an online survey
Some will be asked if they would be willing to do an interview
some will be asked to complete a 30-day bleed diary

What are the benefits and risks of participating?
There are no risks in taking part and there are no direct medical benefits to you. However, participation may help others with the condition in the future.

Where is the study run from?
Oxford University Hospitals NHS Foundation Trust (UK)

When is the study starting and how long is it expected to run for?
June 2023 to December 2024

Who is funding the study?
The study is being funded by Hemab, a biotechnology company based in Denmark.

Who is the Main contact for the study?
Simon Fletcher, simon@haemnet.com

Contact information

Type(s)
Principal Investigator

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Mr Simon Fletcher

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Additional identifiers

EudraCT/CTIS number
Nil known

IRAS number
328463

ClinicalTrials.gov number
NCT06064643

Secondary identifying numbers
IRAS 328463, CPMS 57207

Study information

Scientific Title
The lived experience of people with von Willebrand disease: a mixed methods study

Acronym
vWD360

Study objectives
To identify the lived experience of people with von Willebrand Disease (vWD) and including:
1. Experiences of bleeding
2. Impact on quality of life
3. Access to therapeutic options
4. Satisfaction with current treatments and management
5. Areas of unmet need

Ethics approval required
Old ethics approval format

Ethics approval(s)
Approved 27/10/2023, London - Brighton and Sussex Research Ethics Committee (Health Research Authority, 2 Redman Place, London, E20 1JQ, United Kingdom; +44 (0)20 7104 8202; approvals@hra.nhs.uk), ref: 23/PR/1013

Study design
Qualitative mixed methods research project

Primary study design
Observational

Secondary study design
Cohort study

Study setting(s)
Home, Hospital

Study type(s)
Quality of life

Participant information sheet
Not available in web format, please use contact details to request a participant information sheet

Health condition(s) or problem(s) studied
von Willebrand Disease (any known subgroup)

Interventions

All participants will be asked to complete an online survey. 30 participants will also be asked to take part in a single semi-structured qualitative interview. 50 participants will be asked to complete a 30-day bleed diary

Intervention Type
Other

Primary outcome measure
Bleeding rates, daily activities, pain/discomfort and anxiety/depression will be measured using a retrospective, self-reported, validated survey (EQ-5D, Menstrual Impact Questionaire (for women) PHQ8 and GAD 7). This data will also be recorded prospectively using a 30 day bleed dairy.

Secondary outcome measures
Treatment satisfaction and assessment of unmet needs will be measured through analysis of the descriptive interview narratives of the lives of people with vWD collected at a single time point

Overall study start date
22/06/2023

Completion date
31/12/2024

Eligibility

Key inclusion criteria
1. Adults aged 16 years and above (UK and Ireland) and adults aged over 18 years (in USA) with a confirmed diagnosis of inherited vWD of known diagnostic subtype and vWF level.
2. For the qualitative interview-based substudy, 30 adults who have completed the survey and who wish to be interviewed will be purposively selected for a broad range of ages and diagnostic subtype.
3. For the bleed diary substudy, 50 adults who have completed the survey and who wish to take part will be purposively selected for a broad range of ages and diagnostic subtype.

Participant type(s)
Patient

Age group
Adult

Lower age limit
16 Years

Sex
Both

Target number of participants
Survey - 450, Interviews - 30, Bleed Diary - 50.

Key exclusion criteria

1. Have acquired vWD
2. Have other inherited bleeding disorders
3. Do not wish to participate in or to consent to the study.
4. Are under 16 years old (UK & Ireland) or 18 years old (US).
5. Those for whom written/spoken English would prohibit participation will also be excluded.

Date of first enrolment
18/12/2023

Date of final enrolment
31/12/2024

Locations

Countries of recruitment
England

Ireland

United Kingdom

United States of America

Study participating centre
Oxford University Hospitals NHS Foundation Trust
John Radcliffe Hospital
Headley Way
Headington
Oxford
United Kingdom
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Sponsor information

Organisation
Haemnet

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Sponsor type
Research organisation

Website
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Funder(s)

Funder type
Industry

Funder Name
Hemab

Results and Publications

Publication and dissemination plan
The results of the study will be published in peer-reviewed journals as well as at conferences as either presentations or posters. All participants will be sent a copy of all of the publications.

Intention to publish date
31/12/2025

Individual participant data (IPD) sharing plan
The datasets generated durang and/or analysed during the current study are/will be available upon reasonable request from Simon Fletcher (Principal Investigator) simon@haemnet.com

IPD sharing plan summary
Available on request

Study outputs

Output type	Details	Date created	Date added	Peer reviewed?	Patient-facing?
Protocol file	version 1.5		27/10/2023	No	No