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The lived experience of people with von Willebrand disease

Submission date 21/06/2023	**Recruitment status** No longer recruiting	[X] Prospectively registered [X] Protocol [_] Statistical analysis plan [_] Results [_] Individual participant data [X] Record updated in last year
Registration date 24/08/2023	**Overall study status** Completed	
Last Edited 23/01/2026	**Condition category** Haematological Disorders	

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

Contact name
Mr Simon Fletcher

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Additional identifiers

Integrated Research Application System (IRAS)
328463

ClinicalTrials.gov (NCT)
NCT06064643

Central Portfolio Management System (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

Study type(s)
Quality of life

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(s)
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.

Key secondary outcome(s))
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

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

Healthy volunteers allowed
No

Age group
Mixed

Lower age limit
16 years

Upper age limit
100 years

Sex
All

Total final enrolment
645

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
United Kingdom

England

Ireland

United States of America

Study participating centre
Oxford University Hospitals NHS Foundation Trust
John Radcliffe Hospital
Headley Way
Headington
Oxford
England
OX3 9DU

Sponsor information

Organisation
Haemnet

Funder(s)

Funder type
Industry

Funder Name
Hemab

Results and Publications

Individual participant data (IPD) sharing plan
The datasets generated during 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