Abstract Submission Number: AR022

PROBE®
Patient Reported Outcomes Burdens and Experiences Study

Quality of Life Related to Access and Barriers to Care for People with von Willebrand Disease (VWD): A Thematic Analysis as Part of the Expansion of the Patient Reported Outcomes Burdens and Experiences (PROBE) Study

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Background & Objective

VWD is the most common bleeding disorder, yet is considered a rare disease with prevalence estimates ranging from 108.9 to 2,200 per 100,000 in population-based studies.[1] Multi-stakeholder consensus from the coreVWD Initiative included quality of life (QoL) as a core outcome to be measured and reported in studies on VWD.[2] The PROBE Study is an established QoL survey currently available only for people with hemophilia, carriers, and healthy controls.

The PROBE Investigator group has initiated a two-year project to adapt PROBE for use by people with VWD (PwVWD) so that their QoL can be measured and benchmarked against the ongoing PROBE Study database. PROBE-VWD is being developed by conducting qualitative work to create a VWD-specific module and update current items to reflect the experience of living with and managing VWD.

Materials & Methods

A background and evidence review was completed to establish a draft conceptual framework to organize elements pertaining to QoL for PwVWD. For the concept elicitation phase of the patient reported outcome (PRO) development, a series of 60-minute semi-structured interviews were held with eligible PwVWD. Interviews were recorded and transcribed and thematic analysis was performed using NVivo15 software. Relevant quotes were coded by two researchers to sections of the draft conceptual framework; additional nodes were created for novel concepts.

Results & Next Steps

Twenty-eight participants consented to be interviewed for the study (Figure 1). The final conceptual framework developed from the thematic analysis contains five domains which contribute to QoL for PwVWD: Physiological/Clinical, Functioning/ Well-Being, Access/Barriers to Care, Women and Girls' Health, and Complications.

Within the Access and Barriers to Care domain, 534 quotes were coded across three sub-topics: Diagnosis, Treatment, and Quality of Care. New nodes were added to the draft framework in these sub-topics after repeated discussion by the participants (Figure 2). Key themes identified by in the domain focused on uncertainty in normal bleeding, missed diagnosis vs. misdiagnosis, access to specialists and prophylaxis, and diagnostic delay.

Key Themes

1. Uncertainty in what is normal bleeding

"I have to be questioning, is this something I should be calling about? I'm never quite sure what constitutes serious enough." *(female, 52)*

"I kind of always thought it was normal. I felt like, because everybody's different, everybody can bleed different amounts." *(female, 38)*

2. Misdiagnosis

"They didn't run the type 2 test. They ran it for the type 1…But then… when they did further, more deeper testing, they found the 2N." *(female, 34)*

"My original diagnosis was type 2M… Then later on in life, they realized it was actually type 2A." *(male, 35)*

3. Missed Diagnosis

"There were always signs, just nothing… significant enough that anybody really paid attention to." *(female, 52)*

"Because when I had the diagnosis, I realized that there were many incidents which were not normal growing up. When they had to remove my back teeth, when I was a child, I bled for days. I had a joint bleed in my right ankle joint, I think one year before I was diagnosed." *(female, 38)*

4. Specialist Referral Access

"But I would say in terms of my VWD care, until I reached a clinic, which I was 38. So, I was diagnosed at 18, didn't go to a clinic until I was 38." *(female, 42)*

"Then I think he was nervous and said, 'Maybe it's time you need to see a clinic.'" *(female, 33)*

5. Access to Prophylaxis

"Then you've got to go through the governments then saying, "Yes, you can use it prophylactically." Then you have to fight them for it, and they're not gonna want it because there's cheaper products out there." *(male, 42)*

"Yeah, they've advised me to go on prophylactics many, many, many times. And it's just not something that I want to do." *(male, 40)*

6. Diagnostic Delay

"I thought it was just normal… I thought I was just too stupid to use a tampon." *(female, 38)*

"I was not diagnosed till I was 38 years old…and I already had all my kids and lost several… But my symptoms started when I was little." *(female, 56)*

Figure 3. *Six concepts included in the framework in the Access/Barriers to Care Domain*



Figure 2. *Final Conceptual Framework*

Acknowledgements: Our sincere thanks to the patient organizations that supported the project by assisting with recruitment: The National Bleeding Disorders Foundation (NBDF), the Canadian Hemophilia Society (CHS), the World Federation of Hemophilia (WFH) and VWD Connect. We also sincerely thank the HTCs and healthcare professionals who supported the study and shared the interview opportunity. This work is sponsored by Takeda, Roche, and Genentech.

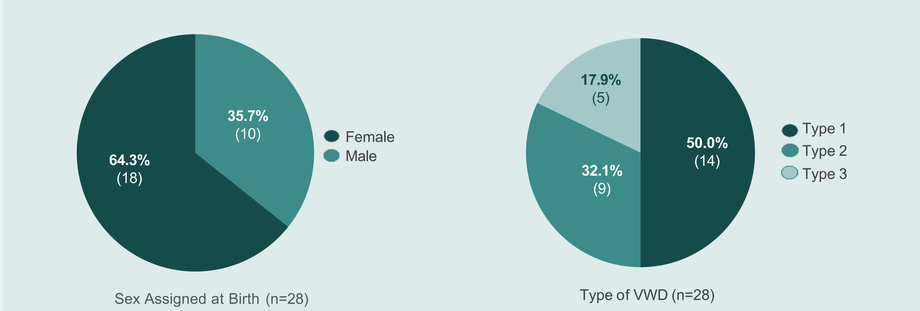


Figure 1. *Participant Demographics*

Conclusion

The concept elicitation phase of the PRO development is complete. Next steps include a mapping exercise to the current PROBE instrument,[3] development and programming on the online platform of the new VWD-specific module, and cognitive debriefing exercises. The content-validated module will be available for cross-cultural validation and psychometric testing in 2027.

References

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