

Development of a WGPPM-Focused PROBE Survey: Addressing Underrepresentation in Patient-Reported Outcomes and Quality of Life Research

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OBJECTIVE

Women, Girls, and People with the Potential to Menstruate (WGPPM), including those with von Willebrand disease (VWD), platelet function disorders, rare coagulation factor deficiencies, and other rare bleeding disorders, remain significantly underrepresented in research related to patient-reported outcomes (PROs) and health-related quality of life (HRQoL). Existing tools such as the Patient Reported Outcomes, Burdens, and Experiences (PROBE) survey—originally developed in 2012—have primarily focused on individuals with hemophilia A or B and carriers, with data also collected from individuals without a bleeding disorder (NoBD) serving as a control group. However, the unique experiences, burdens, and health impacts specific to WGPPM have not been fully captured in existing survey instruments.

SUMMARY

The updated framework expands the PROBE instrument with an additional 13 questions which include several WGPPM-specific concepts, such as access to healthcare (including specialty care), emergency department utilization, and bleeding episodes unique to this population, including menstruation and gynecological complications. Additional domains address reproductive decision-making, including considerations around family planning, as well as the social and emotional impact of living with a bleeding disorder—particularly as it pertains to leisure activities and interpersonal relationships. Furthermore, the revised tool allows for improved representation of individuals with rare and often overlooked bleeding disorders.

Updated bleeding disorders

Von Willebrand Disease (VWD), platelet disorder, rare factor deficiency

Additional treatments

factor concentrates, recombinant factor, platelet transfusions, iron treatments, and other

Diagnoses

age of diagnoses, abnormal bleeding episodes

Health care professionals

access to medical professionals, missing access to medical professionals, urgent care visits

Bleeding events

nosebleeds, easy bruising, heavy menstrual bleeding

Gynecological

menopause, changes in treatment/access to treatment, gynecological issues

Quality of Life

decision to have children, impact on social/leisure life

METHODS

The group identified von Willebrand Disease (VWD), platelet disorders, rare factor deficiencies, and other rare bleeding disorders for inclusion. New concepts:

- Access to healthcare (including specialties)
- Visits to emergency room
- Bleeding episodes unique to WGPPM (e.g., menstrual bleeding; gynecological problems)
- Decision-making around having children
- Impact on social and leisure life



CONCLUSIONS

The WGPPM-specific PROBE exploratory pilot was launched in early 2025 through an online platform. It is anticipated that this pilot will generate comprehensive and representative data to better understand the lived experiences of WGPPM. These findings will inform future efforts in research, advocacy, clinical care, and health policy, while also serving as the basis for subsequent updates to the PROBE questionnaire to ensure its continued inclusivity and relevance.

CONTACT US

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At our website you can learn about PROBE's worldwide reach, access our e-platform, find answers to frequently asked questions, and click through our published research to see the types of analyses that can be done using PROBE data.

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