WFH COMPREHENSIVE CARE SUMMIT

New Developments in Bleeding Disorders and MSK

Development of Women, Girls, and People Who Have or Had the Potential to Menstruate (WGPPM) questionnaire for PROBE study

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PROBE®
Patient Reported Outcomes Burdens and Experiences Study

INTRODUCTION

The Patient Reported Outcomes, Burdens, and Experiences (PROBE) survey was first developed in 2012. PROBE currently collects data from people with hemophilia A or B, including carriers, and people without a bleeding disorder (NoBD) who serve as a control group. To date, there has been limited global comparative patient reported data on health outcomes and quality of life (HRQoL) about WGPPM.

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RESULTS

The group identified von Willebrand Disease (VWD), platelet disorders, rare factor deficiencies, and other rare bleeding disorders for inclusion. New concepts include 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, and impact on social and leisure life.

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

METHOD

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

To advance management and treatment for this underserved population it is crucial to collect and report comprehensive data on WGPPM health outcomes and quality of life. The WGPPM PROBE exploratory pilot is scheduled to initiate data collection in early 2025. Through this pilot we anticipate more accurate and comprehensive data will be available to support future advocacy, research, management and treatment. Insights from the pilot will be applied to future PROBE updates.

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- Association française des hémophiles, Paris, France
- The European Haemophilia Consortium (EHC), Brussels, Belgium
- The World Federation of Hemophilia (WFH), Montreal, Canada
- Canadian Hemophilia Society (CHS), Montreal, Canada
- McMaster University, Hamilton, Canada

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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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