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The Patient Reported Outcomes Burdens and Experiences (PROBE) Study Questionnaire Development and Validation

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Abstract

Objectives: The health status of people living with hemophilia (PWH) has not been systematically investigated globally. There is a substantial need to improve capacity to collect and interpret relevant patient-reported outcomes (PRO) data to support patient-centered research and optimal care of PWH. PROBE aimed to: 1) implement a structured data collection of PRO across countries to build a robust evidence base for comparative effectiveness research, evidence-based decision making, and advocacy, 2) explore the measurement properties of the PROBE questionnaire and 3) assess the feasibility of PROBE for assessing health status among PWH and participants without bleeding disorders across regions. Three intermediate objectives were identified: develop a patient-led research network; develop a standardized questionnaire to gather PRO; and perform a feasibility study of implementing the PROBE questionnaire. Methods: Data collection from April 2015 to February 2017. 2,101 surveys were collected through all study phases across 24 countries. 1,541 met study criteria for analysis. Clinical Trial registration: NCT02439710.Summary: The PROBE questionnaire consists of four major sections: demographic data, general health problems, hemophilia-related health problems and health-related quality of life. Outcomes of importance to PWH and metrics to consider for measurement were determined. Domains for outcomes of importance to measure reduced burden of living with hemophilia include (metrics): Life and Family (family life, marital status, children, current health status); Education/School and Employment (attendance, educational attainment, employment duration, underemployment) and Activities (impact on activities of daily living, mobility impairment, assistance required). Domains for reduced complications associated with hemophilia and treatment (metrics): Joint Disease (joint status); Pain, Depression/Anxiety (chronic/acute pain, pain interference, pain occurrence, pain medication, depression); and Other Comorbidities (HIV/HCV, obesity, resource utilization, mortality, longevity). PROBE questionnaire validation studies established face validity, relevance, clarity and completeness (Skinner, Pilot and Feasibility Studies 2018); test-retest reliability (reproducibility) (Chai-Adisaksopha, Haemophilia 2019); a core analytic framework (psychometric properties) (Chai-Adisaksopha BMJ Open 2018); and cross-cultural validation (Chai-Adisaksopha, Haemophilia 2019). Conclusions: The PROBE questionnaire established and assessed patient-important outcomes in PWH and control participants, with a demonstrated short completion time using both paper and electronic versions. PROBE proved the feasibility to engage diverse patient communities in the structured generation of real-world outcome research at all stages. Results demonstrate that the PROBE questionnaire is valid for assessing PROs and health status among PWH and participants without bleeding disorders across regions. The known group property of PROBE will allow its

use in future clinical trials, longitudinal studies, health technology assessment studies, routine clinical care or registries. Longitudinal PRO data collection using an instrument such as PROBE will be useful within clinical development programs, clinical management settings and to support access to care initiatives.