Validation of the Patient Reported Outcomes Burdens and Experiences (PROBE) Study Questionnaire

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Background

A substantial need exists to improve capacity to collect and interpret relevant Patient Reported Outcome (PRO) data to support patient-centered research and optimal care in People With Hemophilia (PWH).

Conclusions

- The PROBE questionnaire assesses 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 to implement 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.

Aims

- Implement structured data collection of PRO across countries to build a robust evidence base for comparative effectiveness research, evidence-based decision making and advocacy. (Fig 1)
- Explore the measurement properties of the PROBE questionnaire and validity to implement PROBE for assessing health status among PWH and participants without bleeding disorders across regions. (Fig 2)

References

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Methods

- Data collection April 2015 to February 2017
- 2,101 surveys collected through all study phases across 24 countries
- Clinical Trial registration: NCT02439710

Results

- Outcomes of importance to PWH and metrics to consider for measurement were determined. (Table 1)
- The PROBE questionnaire consists of four major sections (demographic data, general health problems, hemophilia-related health problems and health-related quality of life).

Table 1

Patient Important Outcomes for PWH and Measurement Metrics

Outcomes of Importance	Metrics
Reduced burden of living with hemophilia	
Life, Family	Family life, Marital status, Children, Current health status (HRQoL)
Education/school, Employment	Attendance, Educational attainment, Employment duration, Underemployment
Activities	Impact on daily living, Activities of daily living, Mobility impairment, Assistance required
Reduced complications associated with hemophilia and treatment	
Joint disease	Joint status
Pain, Depression/Anxiety	Pain (chronic, acute, interference with activity, timing, medication), Depression
Other comorbidities, HIV/HCV, Obesity	Resource utilization, Mortality, Longevity

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Figure 1

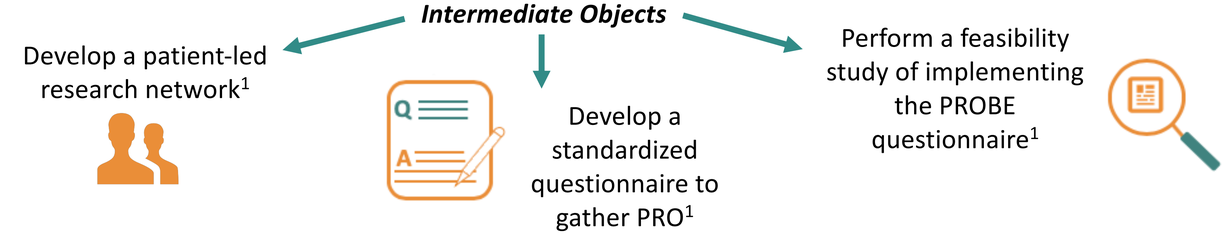
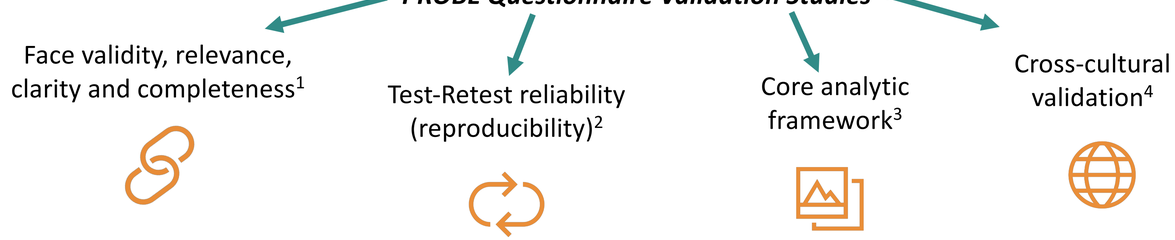


Figure 2



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