The Patient Reported Outcomes, Burdens and Experiences (PROBE) Study Phase 1 Methodology and Feasibility

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OBJECTIVE

- PROBE aims to develop and validate a standardized survey to gather experiential data reported by patients and collectable by patient organizations
- PROBE will enhance the direct patient-voice in the delivery of care and move advocacy efforts to sustain and expand care beyond emotion to arguments grounded in evidence and data
- PROBE will address healthcare payers' desire to better understand health outcomes important to patients
- PROBE will illustrate how patients' knowledge, perspectives and experience can contribute to defining and measuring key health outcomes
- General objective of Phase 1 was to prove feasibility

METHODS

- The PROBE questionnaire was developed, refined and tested for face validity, relevance, clarity and completeness (with the same and different groups) until no more questions about the meaning and scope of the questions were raised. Local language versions were produced.
- Subsequently, patient organizations from 17 mid to highly developed countries participated in the Phase 1 feasibility assessment (Fig 1)
- The questionnaire incorporates EQ-5D-5L VAS with additional domains identified as important by patients: pain, independence, educational attainment, employment, relationships, and activities of daily living
- Relevant patient characteristics (e.g., treatment, bleeding history and joint status) are also collected
- Comparator data is being collected from those not personally effected with a bleeding disorder
- Phase 1 countries participated in a workshop to test the utility of PROBE data to build patient-centric evidence-based advocacy arguments
- For Phase 2 a web-based version is being implemented. Emerging and developing countries are included
- Future phases of research will validate the proof of concept, assess reproducibility, discrimination and responsiveness of PROBE by comparing different treatment delivery modalities and regimens, and compare outcomes utilizing both pooled cross-sectional and longitudinal analyses

PROBE
Patient Reported Outcomes Burdens and Experiences Study

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Figure 1. Country Participation

Argentina (Cordoba Chapter), Australia, Brazil, Canada, France, Germany, Hungary, Ireland, Italy, Japan, Mexico, The Netherlands, New Zealand, Spain, United Kingdom, United States, and Venezuela

Figure 3. Individual Time to Survey Completion

Time to Completion in Minutes	0-15 min.	16-20 min.	21-25 min.	26-30 min.	>30 min.
Number of Respondents (N=665)	474	115	42	18	16
Percentage	71.28%	17.29%	6.32%	2.71%	2.41%

Figure 2. Survey Respondents by Category

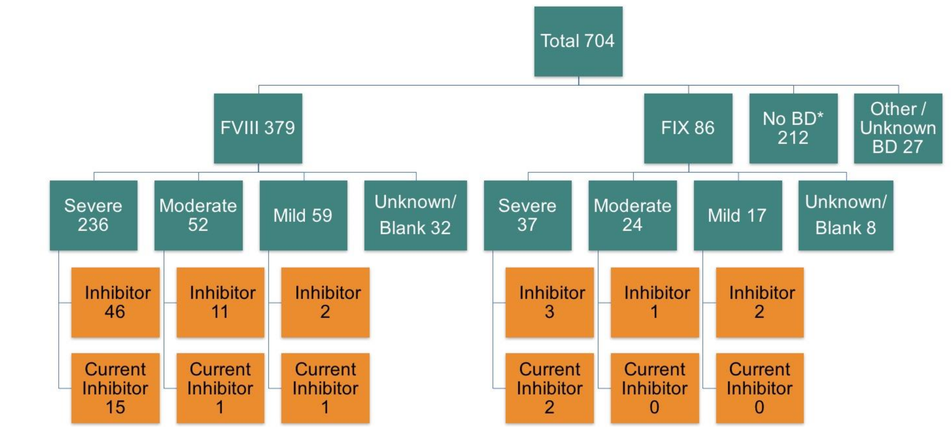


Figure 4. "Target Joint"

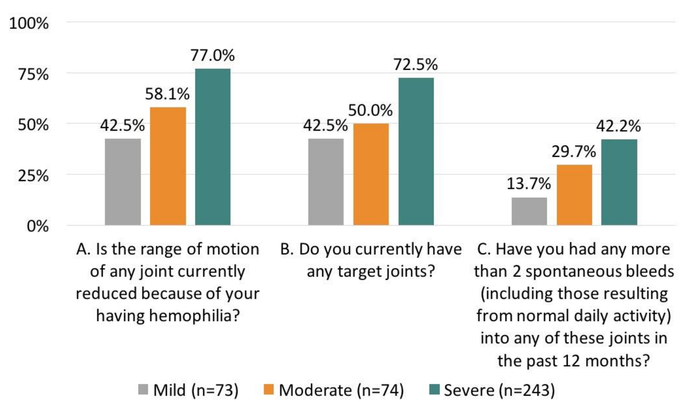
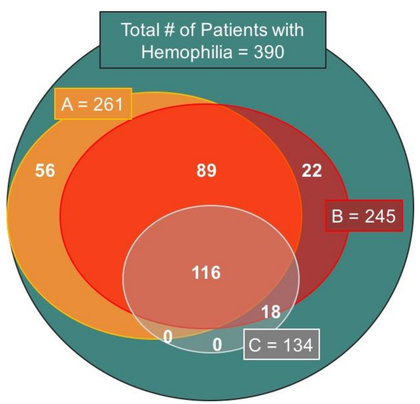


Figure 5. Agreement of "Yes" Responses



RESULTS

- 704 responses (117.33% of study objective) (Fig 2)
- Analysis indicates study methodology is feasible and individual time to completion met study objective of 0-15 minutes (Fig 3)
- Countries reported minimal to acceptable volunteer/paid staff time required to carry out study (2 - 40 hours; median 9 hours)
- Preliminary analysis indicates patient-reported outcomes and clinical endpoints may differ. E.g., "Target joint" results (Fig 4):
  - A. 77% of severe patients reported range of motion reduction in at least one joint
  - B. 73% reported at least one "target joint" when asked a generic question "Do you currently have any 'target joints'?"
  - C. Only 42% reporting a "target joint" also answered "yes" using the definition for a target joint of 2 or more spontaneous bleeds into a joint in the past 12 months
- Agreement of "Yes" responses for 390 PWH Age ≥10 with known severity (Fig 5)
- The variation highlights the importance of continued research to assess whether clinical trial end-points are the appropriate measures to evaluate real-world patient outcome.

CONCLUSIONS

- Feasibility of patient-centered generation of health outcome data demonstrated
- Robust and relevant patient reported data will improve advocacy efforts to build comprehensive care programs, promote home treatment and implement preventative treatment regimens
- Defining and measuring health outcomes with greater direct patient engagement could improve their relevance
- Future phases of PROBE will provide valuable global perspectives through patient-reported health outcomes and experiences

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