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

#141

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INTRODUCTION

- There is a significant need to improve our ability to collect, collate, and interpret relevant patient-centered data to support the implementation of comprehensive care, home treatment and preventative treatment regimens (e.g., prophylaxis) for persons living with hemophilia.
- The PROBE study is intended to fill this gap and 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.

OBJECTIVE

PROBE aims to develop and validate an inventory able to recode experiential data contributed directly by patients through their patient organization to a valid foundation for evidence-based decision making suitable for submission to entities responsible for reimbursement and coverage decisions.

METHODS

- Phase 1 includes developing and testing a paper questionnaire for content, relevance, clarity and completeness, as well as assessing methodology and feasibility. The pilot questionnaire, generated by the investigator team, included a review of available tools.
- It was tested in small groups of patients and normal subjects, and questions were refined (with the same and different groups) until no more questions about the meaning and scope of the questions was raised. Face validity was assessed on the English version only. Local language versions were produced.
- It incorporates the EQ-5D-5L instrument with additional questions covering domains identified as important by patients (e.g., pain, independence, educational attainment, employment, relationships, and activities of daily living).
- Comparator data is being collected from those not personally effected with a bleeding disorder.
- For Phase 2 open text questions have been reduced or converted to pick-lists and a web-based version with logic-based skipping rules is being implemented. Emerging and developing countries are included.
- Future phases of research will validate the proof of concept, assess reproducibility, compare different treatment delivery modalities and regimens on patient outcomes, and compare outcomes utilizing cross-sectional pooling methodology.

PROBE
Patient Reported Outcomes Burdens and Experiences Study

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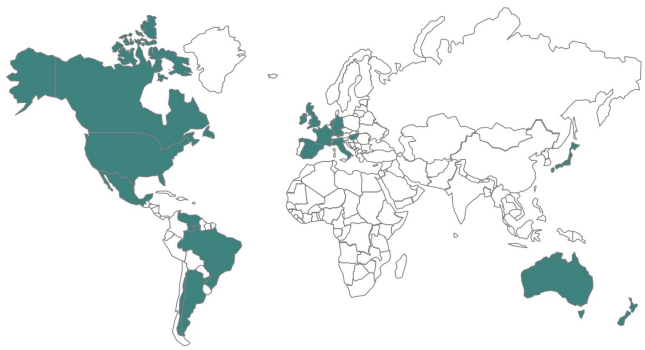
Figure 1. Country Participation



Phase 1 Participating Countries: 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 2. Respondents per Country

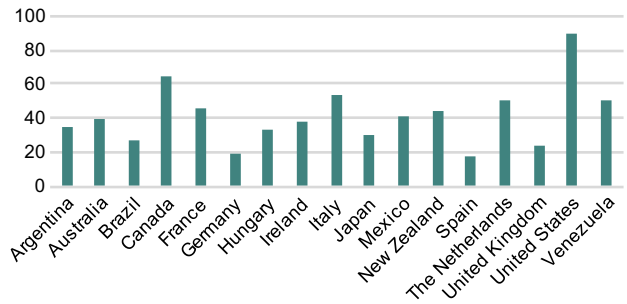
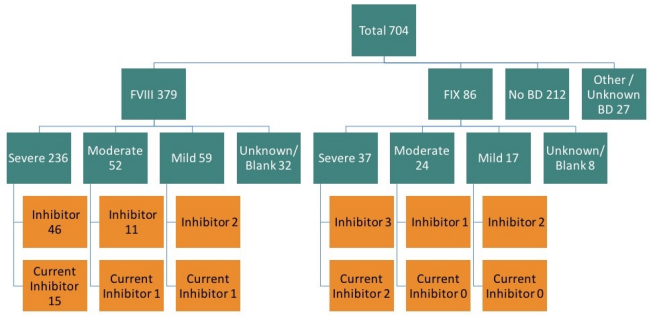


Figure 3. Survey Respondents by Category



No BD = Not personally effected with a bleeding disorder (comparator data)

Figure 4. Individual Time to Completion

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

RESULTS

- Patient organizations from 17 mid to highly developed countries participated in the Phase 1 feasibility assessment (Fig 1).
- Phase 1 field work is complete with 704 responses recorded (117.33% of study objective) (Fig 2) including 379 FVIII PWH, 86 FIX PWH, 212 not personally effected with a bleeding disorder and 27 other / unknown bleeding disorder (Fig 3).
- Preliminary analysis indicates the study methodology is feasible and individual time to completion has met study objectives of 0-15 minutes (Fig 4).
- Countries reported minimal to acceptable volunteer or paid staff time was required to carry out the survey (2 - 40 hours; with a median of 9 hours).
- Preliminary data analysis also indicates variable correlation between patient responses to PROBE study questions using standardized clinical definitions when compared with responses to questions using patient developed outcomes. The variation highlights the importance of continued research to assess whether clinical trial end-points are the appropriate measures to evaluate real-world patient outcomes.

CONCLUSIONS

- It is feasible to engage patient societies in outcomes research through the collection of experiential data directly from patients.
- Robust and relevant patient reported data will improve advocacy efforts to build comprehensive care programs, promote home treatment and implement preventative treatment regimens.
- Clinical outcome assessment augmented with greater direct patient engagement in defining and measuring outcomes could improve their relevance.

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- Deutsche Hämophiliegesellsch aft (Germany)
- Magyar Hemofilia Egyesulet (Hungary)
- Irish Haemophilia Society
- Federazione delle Associazioni Emofilici (Italy)
- National Hemophilia Network of Japan
- Federación de Hemofilia de la República Mexicana (Mexico)
- Nederlandse Vereniging van Hemofilie- Patiënten (The Netherlands)
- Haemophilia Foundation of New Zealand
- Federación Española de Hemofilia (Spain)
- The Haemophilia Society (United Kingdom)
- National Hemophilia Foundation (United States)
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