The Patient Reported Outcomes, Burdens and Experiences (PROBE) Study - Phase 1 Results Show PROBE Study Methodology Feasible

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OBJECTIVES

• The PROBE study aims to develop and validate a standardized survey to gather experiential data reported by patients and collectable by patient organizations to support evidence-based decision making
• Address healthcare payers desire to better understand health outcomes.
• General objective was to prove feasibility
• Patient-reported outcomes and clinical endpoints may differ
• Specific objective of this analysis is to verify the performance in a patient reported outcome framework of the construct “target joint”
• Patients’ knowledge, perspectives and experience can contribute to defining and measuring key health outcomes

RESULTS

• Phase 1 fieldwork completed. 704 responses received from 17 participating countries (117% of study objective)
• Completion time shorter than 15 minutes for >70% of participants
• These included 77 mild, 77 moderate and 276 severe hemophilia patients, as well as 274 controls with no bleeding disorder
• “Target joint” results are reported in the figure:
  o 72% of severe patients reported at least one “target joint” when asked a generic question, “Do you currently have any ‘target joints?’”
  o 77% reported reduction in range of motion in at least one joint
  o 44% answered yes to having a “target joint” according to the ISTH definition of 2 or more spontaneous bleeds into a joint in the past 12 mo.

METHODS

• The PROBE questionnaire was developed, refined and tested for face validity, relevance, clarity and completeness
• The PROBE questionnaire incorporates EQ-SD-5L VAS with additional domains identified important by patients: pain, independence, schooling, employment, relationships and activities of daily living
• Relevant patient characteristics (e.g., treatment, bleeding history and joint status) are also collected

CONCLUSIONS

• Feasibility of a patient-centered generation of health outcome data demonstrated
• Limitation in range of motion is a common and important patient reported outcome and closely correlates to patients’ concept of a “target joint”
• Use of a clinical definition of target joint might led to different results, possibly explaining important variation between observations performed in clinical trials and real world settings
• Defining and measuring health outcomes with greater direct patient engagement could improve their relevance
• 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
• Future phases of PROBE will provide valuable global perspectives through patient-reported health outcomes and experiences

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