Test-Retest Reliability Analysis of the Patient Reported Outcomes Burdens and Experiences Study Questionnaire

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• Introduction: PROBE is a 29-item questionnaire developed with direct patient involvement in questionnaire design, conduct and analysis to directly assess health status using outcomes patients with hemophilia (PWH) identify as important to their care.
  - Phase 1 confirmed robustness of methodology.
  - Phase 2 assesses test-retest reliability of the questionnaire.
  - The core section covers general health (acute and chronic pain, mobility and function, school attainment, days lost to school and work, concomitant health conditions or comorbidities).
  - The PWH only section covers patient demographics and hemophilia treatment (disease severity, number of bleeds, presence of target joints, and treatment history).

• Methods: 63 individuals - PWH (47.6%) and individuals without a bleeding disorder (52.5%) from 21 countries participated.
  - Paper-based questionnaires were completed on day 1 and 2, participants were instructed to log into the website within 2 weeks to complete a 3rd time.
  - Test-retest reliability was analyzed using percentage agreement and Kappa statistics (κ).

• Results:
  - For core questions common to PWH and individuals without a bleeding disorder Kappa ranged 0.69 to 0.92 indicating substantial to almost perfect agreement for all items. Lowest item was “acute pain” (κ=0.69). Web-based version showed moderate to substantial agreement for all except acute pain.
  - For PWH only questions, Kappa ranged 0.5-1.0. Perfect agreement (κ=1.0) for 5 of 11 items. Web-based version showed substantial to almost perfect agreement.
  - EQ-5D-5L Kappa ranged 0.63 to 0.85. Lowest domain was “pain and discomfort” (κ=0.63). EQ VAS Correlation coefficient 0.90 (95% CI; 0.83-0.94).
  - Test-retest comparable between PWH and individuals without a bleeding disorder.

• Conclusions: Results suggest PROBE is a reliable tool to assess patient reported outcomes for PWH and benchmark data in participants without a bleeding disorder. The web-based questionnaire and paper-based version can be used interchangeably.