Development And Preliminary Testing Of The Idiopathic Pulmonary Fibrosis Patient Reported Outcome Measure (ipf-Prom): Uk And Ireland Multi-Centre Study

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Introduction

Developing a PRoM for a rare condition such as IPF has inherent challenges. While there is no definitive methodological gold standard, FDA guidelines[1] provide a framework to optimise PRoM design and strengthen the endpoint model. We set out to develop a patient-centred FDA concordant IPF PRoM.

Methodology:

A literature review identified 26 outcome measures used in IPF studies. 14 meeting the inclusion criteria were deconstructed. Items (n=1240) underwent duplicity screening; 410 items were submitted to consensus rounds.

Five focus groups across the UK, stratified for disease severity, generated patient descriptors of life with IPF. Transcripts underwent content and thematic analysis using NVIVO software.

A Nominal Group (NG) of IPF clinical experts rank-ordered domains identified in both the literature and focus groups using voting methods.

350 items were included in a Delphi Survey. Items were reduced in two rounds according to criteria in the Table.

106 items were retained in the final Delphi completed by participants (n=510) recruited through UK and Ireland IPF charities. Complete responses (n=281) were factor analysed (FA) (varimax rotation). Factors with an eigen value ≥1 and questions with a factor loading ≥ 0.5 were retained. Cronbach's alpha statistic assessed internal reliability and consistency of the scale.

Results

Top 3 domains ranked by the NG were mortality, breathlessness and emotional well-being. Delphi rounds 1 and 2 were completed by patients (n=79) caregivers (n=19) and clinical experts (n=32) with response rates ≥93% across all groups. Delphi round 3: participants accessing survey n=510; completing in full n=281; male n=181 (65%); FA identified 15 factors accounting for 74% of the variance. 6 items were removed due to ambiguity. Systematic application of cronbach's alpha and FA yielded an 8 item IPF-PRoM.

Discussion

The IPF PRoM evolved through an iterative process concordant with FDA guidelines. Its construct validity and test-retest reliability are being measured against MRC; EQ-5D and SGRQ.

Recruitment through IPF charities is an efficient way to generate large data in a short time frame. Our methodology offers one approach for generating rich data in rare conditions

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Reference: [1]U.S. Department of Health and Human Services Food and Drug Administration (FDA). Guidance for Industry: Patient-Reported Outcome Measures: Use in Medical Product Development to Support Labeling Claims. FDA: Maryland; 2009.

Table

Statement result	Threshold to apply
Definitely include	>=70% of participants rate statement as >=6 OR median rating of >=5
Maybe include	>=70% of participants rate statement as >=5 OR median rating of >=5

Definitely exclude	<70% of participants rate statement as <=4 AND 100% participants understand statement OR median <=4 AND 100% panel understand statement
Review	<70% of panel rate statement as >=6 AND <100% panel understand statement

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