



THE FORUM
For Collaborative ResearchSM

PSC PRO Working Group Update

Douglas Thorburn

Royal Free Hospital
April 10, 2018

PSC PRO Working Group:

- Goal: Review the landscape of PRO tools that have been considered or used in previous PSC clinical trials
- Considered a short term working group until goal is accomplished

Working Group Members:

Patient Groups

- Ricky Safer (PSC Partners)
- Martine Walmsley (PSC Support)

Academia

Doug Thorburn* (Royal Free)

Industry

- Rob Myers (Gilead)
- Rich Pencek (Intercept)
- Steve Rossi* (NGM Bio)
- David Shapiro (Intercept)

Regulatory Authorities

- Elmer Schabel (EMA)
- Johannes Taminiau (EMA)
- Lara Dimick-Santos (FDA)

Working Group Discussion:

- Martine Walmsley (PSC Support)- Patient perspective, the importance of PSC PROs
- Michelle Campbell (FDA)- Regulatory perspectives on PROs used in PSC clinical trials
- Steve Rossi (NGM Bio)- PRO on pruritus and fatigue in PSC patients
- Rob Myers (Gilead)- discussion of the recent publication “Development and Validation of a Primary Sclerosing Cholangitis- Specific Patient-Reported Outcomes Instrument: The PSC PRO”

Martine Walmsley

- Patient perspective
- Aspects of a PSC patient's experience (i.e. living with pain, fatigue, itch and uncertainty about the future) must be taken into consideration
- A PSC PRO is needed to track the quality of life in PSC patient's over time

Michelle Campbell

- Regulatory perspective (FDA Clinical Outcomes Assessment staff)
- Encouraged individuals and organizations to work with regulators at the beginning of PRO development and throughout the research process
- Provided a roadmap to create a Patient-Focused Outcome Measurement in Clinical Trials and gave a high level overview of the process

Stephen Rossi

- Industry perspective
- Highlighted his experience in developing a PRO tool for pruritus (VAS and 5-D Itch measure)
- Noted the importance of working with outside groups when needed (i.e. statistics and outcomes groups)
- Evaluated using fatigue in a PRO but was considered too challenging as an endpoint

Rob Myers:

- Industry perspective
- Discussed the “*Development and Validation of a Primary Sclerosing Cholangitis- Specific Patient-Reported Outcomes Instrument: The PSC PRO*” article
- Reviewed the process needed to develop the PSC PRO and described the instrument

Key Discussion Points

- Need for validated qualified PSC PRO
- Content validity (appropriate for the study population, captures what is important to the patient, makes sense) then reliability, validity, interpretability
- Importance of clinically v statistically meaningful change
- Must be easy to use/applicable to clinical trials
- Dichotomy of measuring specific issues according to mechanism of drug action v global measure of experience



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UK PSC Quality of Life Tool Update

Douglas Thorburn

Royal Free Hospital
April 10, 2018

UK PSC QoL Tool: Questionnaire development process

STAGE 1

Generate a list of quality of life issues which may affect people with PSC

Extract issues from existing QoL tools

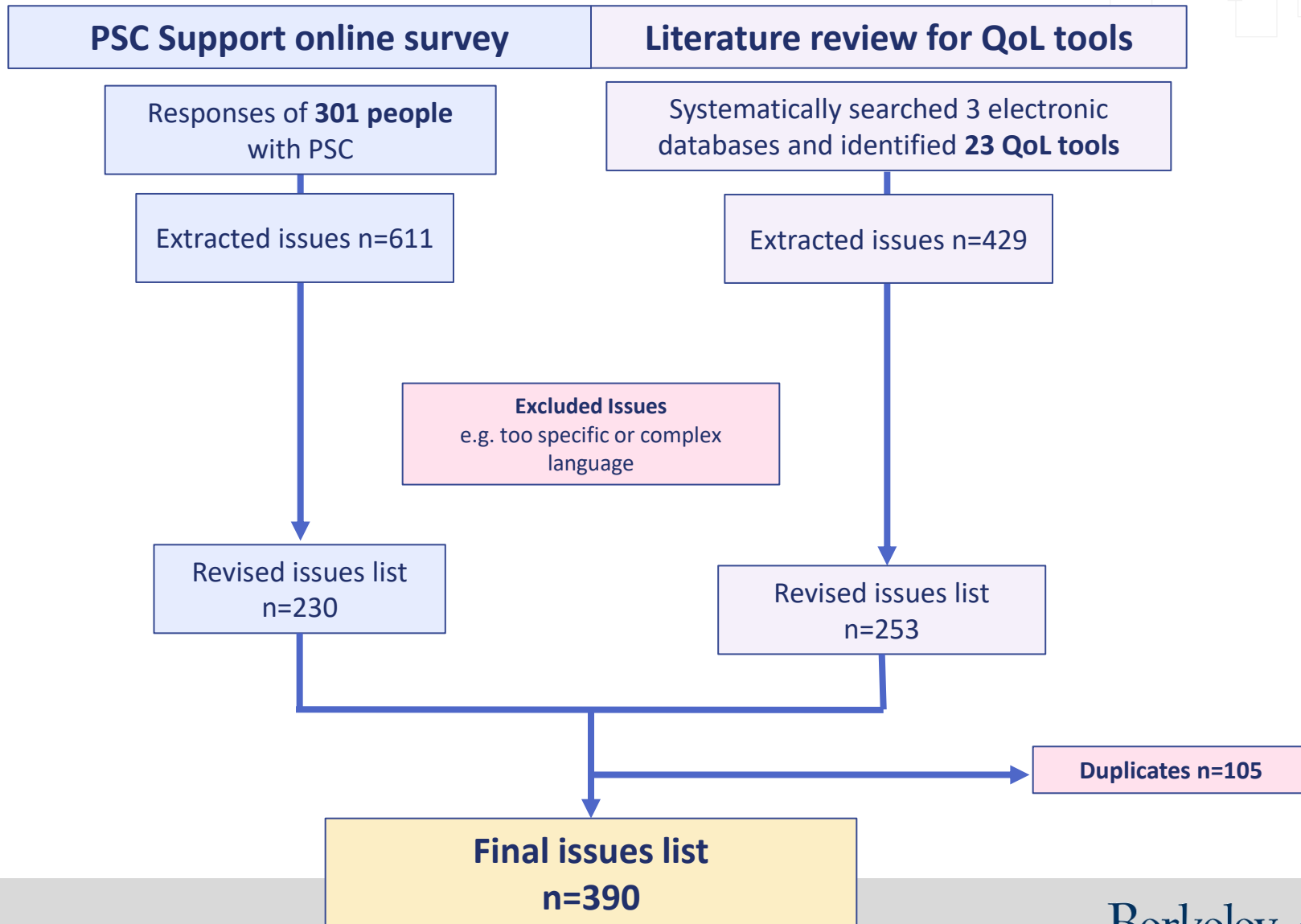
Extract issues from the PSC Support Survey (2014)

Run focus groups with people with PSC and clinicians

STAGE 2

Construct and pre-test provisional questionnaire

UK PSC QoL Tool



Themes

Examples of issues included



1. Uncertainty, knowledge and information

(a) Uncertainty about how my condition will develop,
 (b) lack of knowledge from general medical professionals



2. Thoughts and feelings

(a) Ability to enjoy life, (b) feeling controlled by my illness,
 (c) concerned about condition in the future



3. Social activities

(a) Impact of PSC on social life, (b) being unable to plan ahead, (c) having to cancel events



4. Relationships

(a) Ability to interact with family, (b) feeling close to friends,
 (c) people not understanding I'm ill



5. Work, education and money

(a) Impact of condition on education, (b) needing to change job due to condition, (c) lack of income



6. Physical health

(a) Feeling ill, (b) feeling exhausted, (c) pain in abdomen,
 (d) problems with maintaining ideal weight



7. Experience of medical care and treatment

(a) Being unable to see a PSC specialist, (b) unplanned hospital visits, (c) troubled by treatment side effects

Included participants

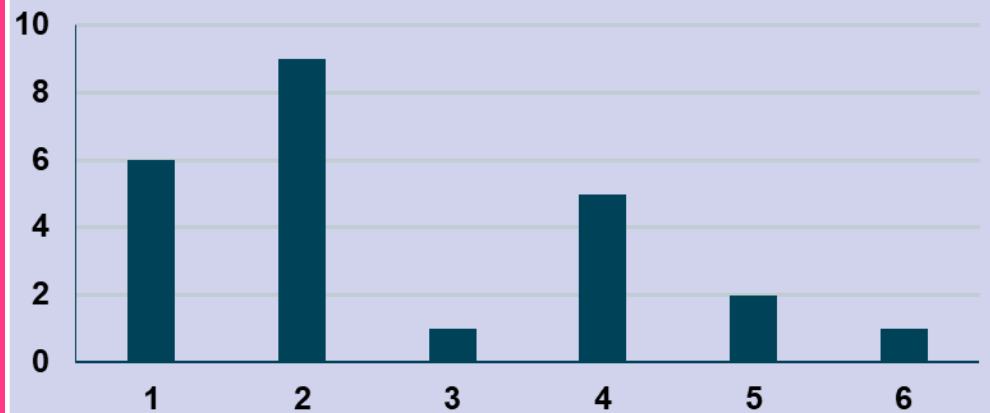
1. People with PSC only
2. People with PSC and IBD
3. People with PSC who have been assessed/waiting for a liver transplant
4. People who have had a liver transplant but do not currently have PSC
5. People who have had a liver transplant and have recurrent PSC
6. People with PSC who also have a related cancer

Characteristics

Age: 22-70 years (mean= 54)

Gender: 13/24 male (54%)

Number of participants per category



Feedback from participants

- Issues list is comprehensive
- Found session interesting
- Found it reassuring to hear that other people shared a similar experience

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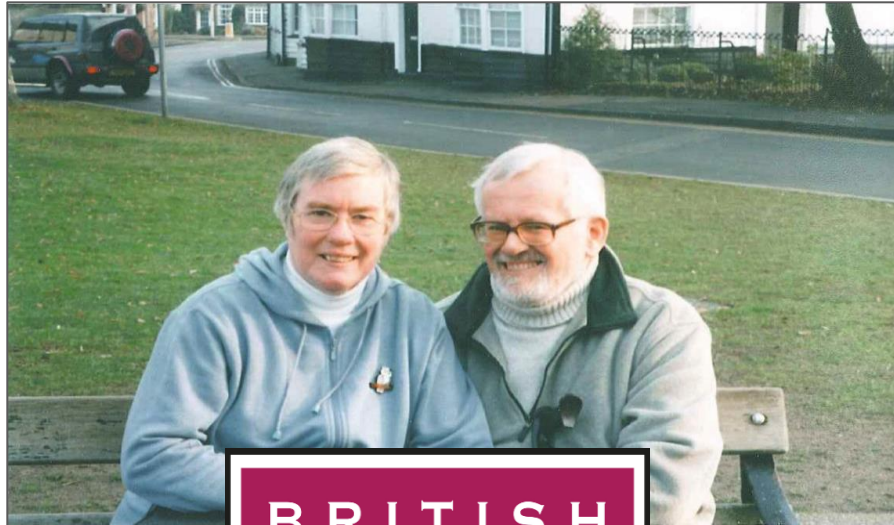
Turn issues into questionnaire items and construct provisional questionnaire

Test questionnaire with people with PSC

Refine questionnaire to produce a final version



Funders



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