

Quality of life (QoL) for people with rare diseases: Recruitment challenges and consequences in a study pilot-testing a provisional QoL tool for people with primary sclerosing cholangitis in the UK

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BACKGROUND

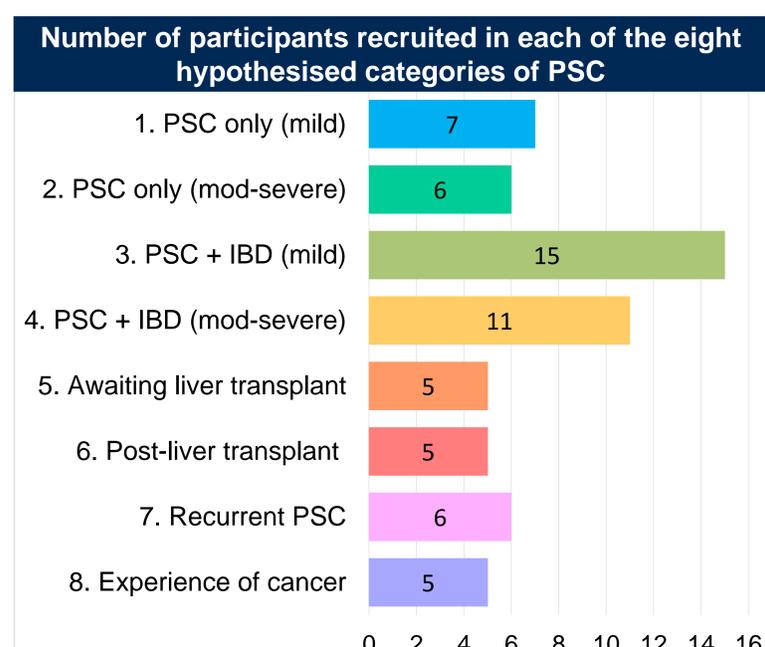
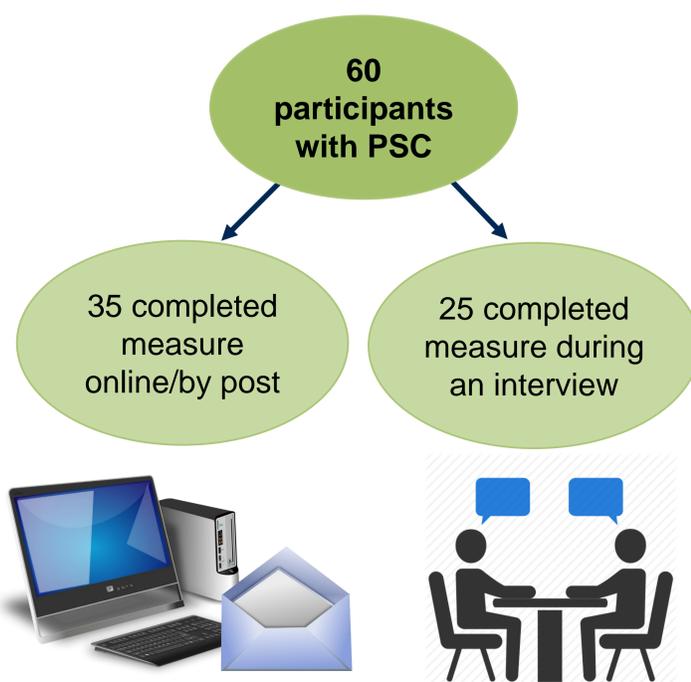
- Primary sclerosing cholangitis (PSC) is a rare incurable bile duct and liver disease which can considerably impact quality of life (QoL).
- PSC is more common in men (2:1) and median age at diagnosis is 41 years. Some people with PSC may eventually require liver transplantation or develop a PSC-related cancer (e.g. liver cancer or bile duct cancer).
- This ongoing project is developing the UK-PSC-QoL, a new measure of QoL, currently focusing on people living with PSC in the UK.

METHODS

- Issue generation and reduction → 83 issues constructed as items in six domains.
- Pilot-testing with people with PSC in the UK, grouped in one of eight hypothesised categories for severity of PSC, and co-morbidities (e.g. inflammatory bowel disease [IBD], awaiting liver transplant, recurrent PSC, PSC-related cancers).
- Participants across all categories completed the provisional measure.
- Participants with more severe PSC, plus a sub-group with less severe disease, interviewed to explore item comprehensibility, acceptability, relevance, and redundancy.
- Mean scores, prevalence ratios, and response distributions for each item explored.

RESULTS

Participant characteristics (N=60)	
Age (median, range)	52 (23-75)
Sex (male, female)	34 (57%), 26 (43%)
Ethnicity	
White (British/other)	54 (90%)
Asian British	4 (7%)
Black British	2 (3%)
PSC severity (self-report)	
Mild	35 (58%)
Moderate	19 (32%)
Severe	6 (10%)
Time since first symptoms	
Asymptomatic	9 (15%)
<1 year	7 (12%)
1-5 years	11 (18%)
6-10 years	11 (18%)
>10 years	22 (37%)



Decisions made for the provisional UK-PSC-QoL (83 items)

- **Retained, no problems identified: 27 items**
- **Retained despite minor problems: 27 items**
 - e.g. one participant described one item as irrelevant/annoying
 - e.g. one participants stated that one item was caused by factors other than PSC
- **Modified to address ambiguity or lack of clarity: 24 items**
 - e.g. 'My PSC has had a negative impact on my financial situation' simplified to 'My PSC has affected my financial situation (for the worse)'
 - e.g. 'I have been able to travel' re-phrased as 'I have been able to travel locally for my day-to-day activities'
- **Deleted: 5 items**
 - e.g. 'I have worried that my PSC will get worse' deleted because described as very similar to existing item: 'I have been concerned about how my PSC will develop'
 - e.g. 'I have felt non-specialist healthcare professionals have enough knowledge about PSC' deleted due to evidence of a ceiling effect
- **New items: 1 item** → 'I have had enough support from my employer'

CONCLUSIONS & NEXT STEPS

- Initial analysis of pilot-testing data → 54 items retained; 24 items re-phrased; 5 items deleted.
- Recruitment challenges for six of the eight categories of PSC, particularly more severe categories (e.g. awaiting liver transplant), prevented definitive decisions about other items.
- Future research, broadened internationally, would enable the recruitment of more people in under-represented groups, and thereby assist decisions on Item selection.

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