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.

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 then 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, recurrent PSC, PSC-related cancers), 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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