Background

- Primary biliary cholangitis (PBC) is an autoimmune cholestatic disease affecting the liver that has a significant impact on an individual’s health and quality of life.
- PBC can progress to cirrhosis and eventually liver failure (Poupon 2010).
- As a rare disease, PBC prevalence is uncertain (Dahlqvist et al. 2017). However, it has been estimated between 6.7 and 940 cases per million population (depending on age and sex) (Kumar 2008).
- itch (pruritus) has been one of the most common complaints amongst PBC patients (Sehmi et al. 2011). Occurring in 70% of PBC patients (Sehmi et al. 2011), pruritus can impact sleep and even lead to suicide ideation (Touraine et al. 2000).

Methods

- A cross-sectional, qualitative study involving individual, in-person qualitative interviews was conducted with PBC patients experiencing itch.
- The study objectives were:
  - To understand PBC patients’ itch experience and its impact
  - To examine the construct validity of three existing pruritus patient-reported outcome (PRO) measures: Itch Numeric Rating Scale (NRS), 0-10 itch Scale, and the Itch Visual Analog Scale (VAS)
  - To inform the endpoint selection for a Phase III clinical trial focusing on itch.

Participants

- Participants were identified through PBC patient advocacy groups and had a score of ≥4 on the itch NRS.
- A standardized semi-structured interview guide was used to conduct the interviews. Participants completed three PRO measures:
  - The NRS itch is a 10-cm visual analog scale designed to capture patient itch on a visual scale from 0 to 10.
  - The 0-10 itch Scale is a brief and multidimensional questionnaire designed to be a measure of itch with five dimensions: degree, duration, direction, disability, and distribution. It has a 2-week recall period.
  - The Itch VAS is a 10-cm line (0 no itch to 10 worst possible itch) which patients mark to describe the severity of their pruritus. It has a 24-hour recall period.

All participants reported itch impacting their lives. The most commonly reported itch sites were:
- front and back
- inner arms and thighs
- fingers and toes
- back of neck

Participants were engaged in one-on-one, semi-structured concept elicitation and cognitive interviews conducted during a PBC patient conference (as well as at locations convenient to participants).

During the concept elicitation portion, participants described their PBC itch experience.

- Itching is an involuntary reaction to scratch, or a tingling or creepy sensation that often wasn’t relieved with scratching (Table 2).
- Participants reported a meaningful change on the VAS ranging from a 5- to 55-point (average 30-point) reduction.

Results

- Patient-reported itch impacted their lives. The most commonly reported impacts from itch were being unable to sleep, daily activities, and social functioning from scratching in public or having sores on their skin (Figure 1).

Conclusions

- This study identified itch as an important symptom for PBC patients with multiple impacts.
- Study results were used to inform the selection of PROs for a Phase III clinical trial.
- Participants considered itch NRS, 0-10 itch, and Itch VAS relevant instruments for assessing itch.
- Participants found that the following would reflect a meaningful change in treatment effectiveness:
  - a 3- to 5-point reduction in the itch NRS score (≤50%)
  - a 5- to 55-point (average 30-point) reduction.

References