Introduction

Primary Biliary Cholangitis (PBC) is a chronic liver disease with debilitating and under-recognized symptoms.

The aim of the optional patient reported outcome (PRO) portion of TARGET-PBC is to evaluate patient reported disease symptoms in a large and diverse PBC population.

Methods

TARGET-PBC is a longitudinal observational study of PBC patients at 35 U.S. sites (20 academic/9 community).

Patients are asked to electronically complete symptom and health status assessments via the Symptom-5 (S-5), PBC-40, and PROMIS Fatigue questionnaires at baseline, and every 6-months thereafter.

Baseline assessments are completed at study entry and are not baseline disease assessments.

The S-5 itch questionnaire asks patients to report pruritus duration, degree, direction, disability and distribution over the last 2 weeks.

The PBC-40 questionnaire is a disease-specific quality of life questionnaire evaluating patient reported symptoms over the last 4 weeks.

The PROMIS fatigue questionnaire evaluates patient reported fatigue symptoms within the last 7 days.

Respondents are defined as participants who completed at least 1 questionnaire and not participants who have responded to treatment.

Participant Characteristics

Total enrollment for TARGET-PBC: 510.

Completed at least 1 PRO: 94.

Participants who have responded to treatment.

Method

- History of depression or anxiety was associated with higher social impairment PRO scores.

- In this real-world cohort, PBC patients commonly reported pruritus and fatigue.

- Suboptimal response to UDCA defined by IFL initially recognized as a predictor of survival, also affects PROs, leading further clinical significance to the criterion.

- History of depression or anxiety was associated with higher social impairment PRO scores.

- The concordance rate between PRO reported itch symptoms and medication usage is lower for moderate/severe complaints compared to severe/near severe complaints.

- There was no correlation between advanced liver disease and patient-reported severity of pruritus and fatigue.

Conclusion

- In this real-world cohort, PBC patients commonly reported pruritus and fatigue.

- Suboptimal response to UDCA defined by IFL initially recognized as a predictor of survival, also affects PROs, leading further clinical significance to the criterion.

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