

Investigating the Burden of Illness in Primary Sclerosing Cholangitis (PSC): A Multinational Study

Poster
SAT-301

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Introduction

- Primary sclerosing cholangitis (PSC) is a progressive, autoimmune, cholestatic liver disease characterised by scarring and destruction of intrahepatic and extrahepatic bile ducts that can lead to fibrosis, cirrhosis, hepatocellular carcinoma, or bile duct cancer.¹⁻³
 - Key clinical manifestations include fatigue, pruritus, difficulty sleeping, brain fog, anxiety, pain, and jaundice.^{1,2,4,5}
- Pruritus is experienced by up to 91% of people with PSC and can be severe and debilitating, leading to reduced health-related quality of life (HRQoL).^{1,4,6}
- There is a paucity of data characterising the burden of illness experienced by people with symptomatic PSC.

Objective

- To present the results of a cross-sectional, multinational survey assessing the burden of illness in people with symptomatic PSC matched to a general population sample.

Methods

Web-based surveys were distributed to adults aged ≥18 years^a:

People with PSC currently experiencing pruritus

Recruited via advocacy groups in:


PSC Partners


PSC Support


ALBI


Deutsche Leberhilfe


AMAF

General population

Recruited via third-party vendor and matched to the cohort of people with PSC based on:

 Age

 Gender

 Employment status

Participants completed 133-question surveys assessing quantitative elements associated with PSC,^a including validated outcome measures (Adult ItchRO, PROMIS Fatigue, PROMIS Cognitive Function, SF-36) covering physical, emotional, and financial burdens

September 2025

Data were collected and analysed using descriptive statistics; t-statistics and linear regression analyses were performed to examine the relationship between pruritus severity and HRQoL

January 2026

Semi-structured interviews with people with PSC were conducted to build on survey topics

- Pruritus severity was measured using Adult ItchRO, an 11-point (0-10) scale, where 0 = no itch and 10 = worst possible itch.^b
- PROMIS Fatigue assesses the impact/experience of fatigue and consists of a 7-item questionnaire.^b

^aThe general population did not answer PSC-specific questions. ^bResults were from the web-based survey only.

Abbreviations

HRQoL, health-related quality of life; ItchRO, Itch Reported Outcome; PROMIS, Patient-Reported Outcomes Measurement Information System; PSC, primary sclerosing cholangitis; r, Pearson correlation coefficient; SD, standard deviation; SF-36, 36-Item Short Form Health Survey.

Disclosures

DJ reports serving as a medical consultant and/or receiving honoraria from Advanz Pharma, Boston Scientific, Cook Medical, Dr Falk, Gilead Sciences, Ipsen Pharma, Q3 Medical, and Mirum Pharmaceuticals, Inc. (via Initiate Consultancy Ltd); receiving research grants from PSC Support and Q3 Medical; and giving sponsored lectures for Gilead, Ipsen, and Mirum Pharmaceuticals. GL, JTR, MP, LQ, and RH are employees of and shareholders in Mirum Pharmaceuticals, Inc. JM, OW, and SS are employees of Initiate Consultancy Ltd, who received funding from Mirum Pharmaceuticals, Inc. for conducting this research. DR and PAP are/were employees of University of Sheffield, which received funding from Mirum Pharmaceuticals, Inc. (via Initiate Consultancy Ltd) for conducting this research. MW is a consultant for (all consulting fees received given to PSC Support) and has received sponsorship for charitable activities from Dr Falk UK, Mirum Pharmaceuticals, Inc., Ipsen, and Chemomab. MJM is a consultant for CymaBay, Gilead, Intercept, Ironwood, Ipsen, Kezar, and Mirum Pharmaceuticals, Inc. and receiving research funding from CymaBay, Gilead, GSK, Ipsen, and Mirum Pharmaceuticals, Inc. BT, AL, and FS report no conflicts of interest to disclose.

Results

Table 1. Participant Characteristics

Parameter	People with PSC N=165	General population N=165
Country, n		
United States	50	50
United Kingdom	50	50
France	28	28
Germany	23	23
Italy	14	14
Age, years, mean (SD)	44.2 (13.4)	43.8 (13.1)
Gender, female, n (%)	98 (59)	98 (59)
Employment status, n (%)		
Paid employment	70 (42)	84 (51)
Self-employed	32 (19)	13 (8)

- Ethnicity representation was similar between people with PSC and the general population.
- Respondents in the general population reported a range of underlying health conditions, including mental health conditions (29%), skin conditions (26%), chronic fatigue/low energy (22%), joint or musculoskeletal issues (20%), and cardiovascular conditions (20%).

96% of People With PSC Were Taking Off-Label Medications for Their PSC

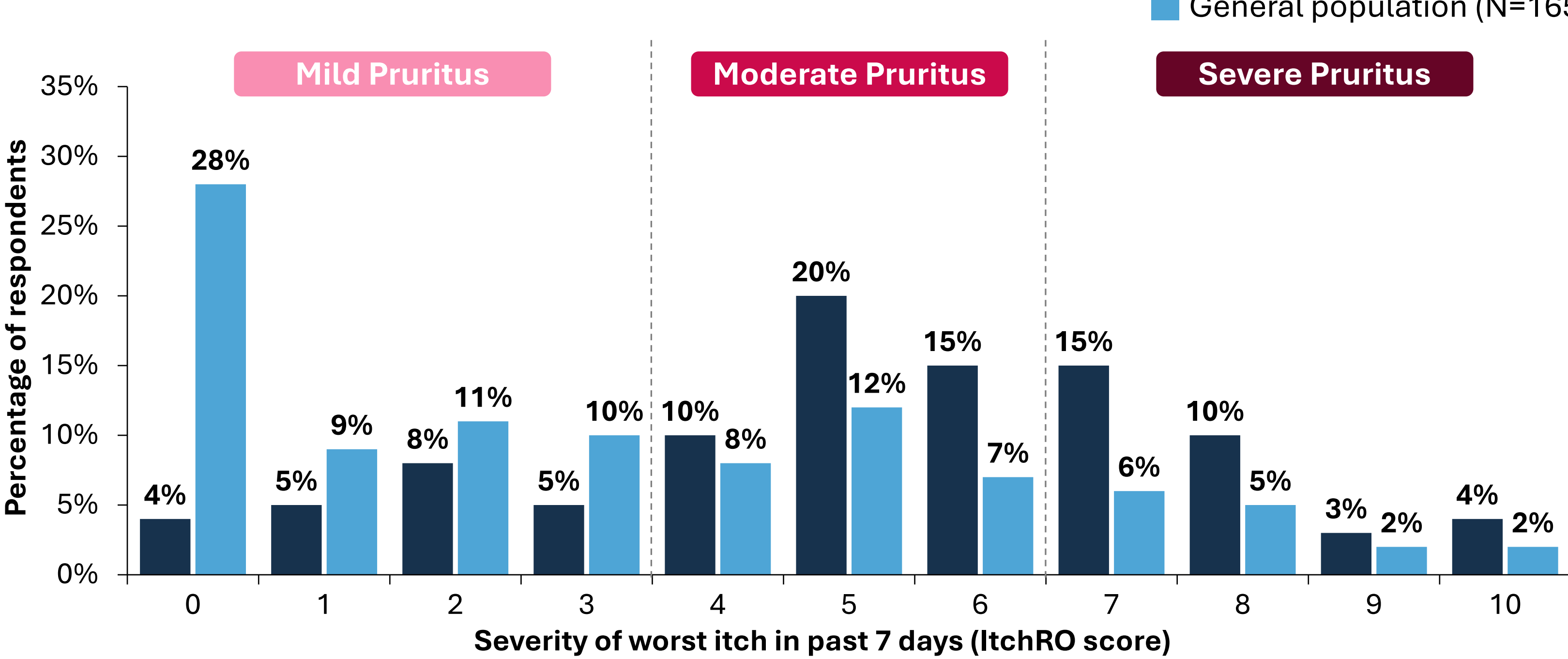
Table 2. Current Treatment Usage in People With PSC

Treatment, n (%)	People with PSC N=165
Ursodeoxycholic acid	144 (87)
Antihistamines	55 (33)
Naltrexone/sertraline	37 (22)
Rifampicin	36 (22)
Cholestyramine	32 (19)
Bezafibrate	24 (15)
Other ^a	75 (45)

^aIncluded vitamin D and vitamin B12 supplements, beta-blockers, probiotics, and azathioprine.

78% of People With PSC Had Moderate to Severe Pruritus Compared With 42% of the General Population

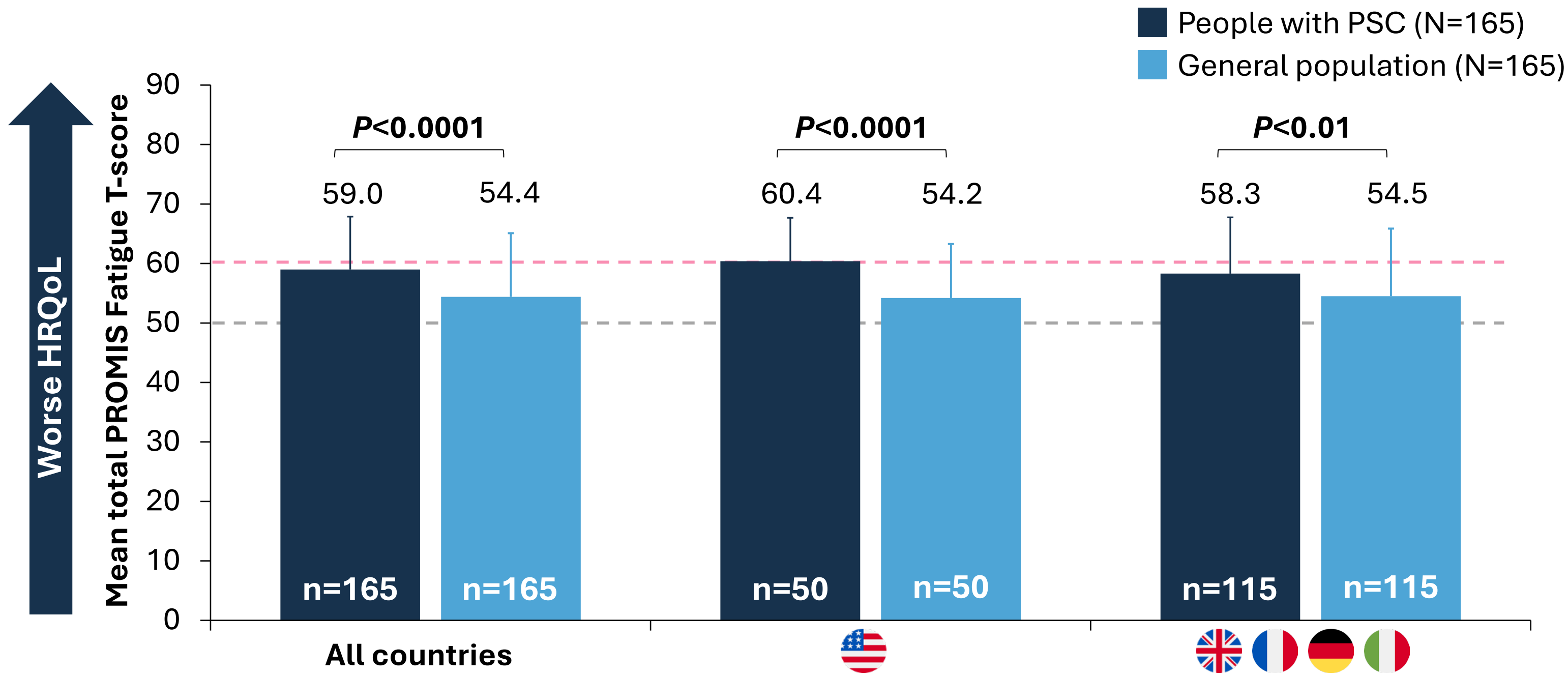
Figure 1. ItchRO Score Distribution



- The mean (SD) ItchRO score for people with PSC was 5.2 (2.5).

61% of People with PSC Reported Having to Make Lifestyle Adjustments Due to PSC-Related Fatigue

Figure 2. PROMIS Fatigue Scores by Respondent Location^{a,b}

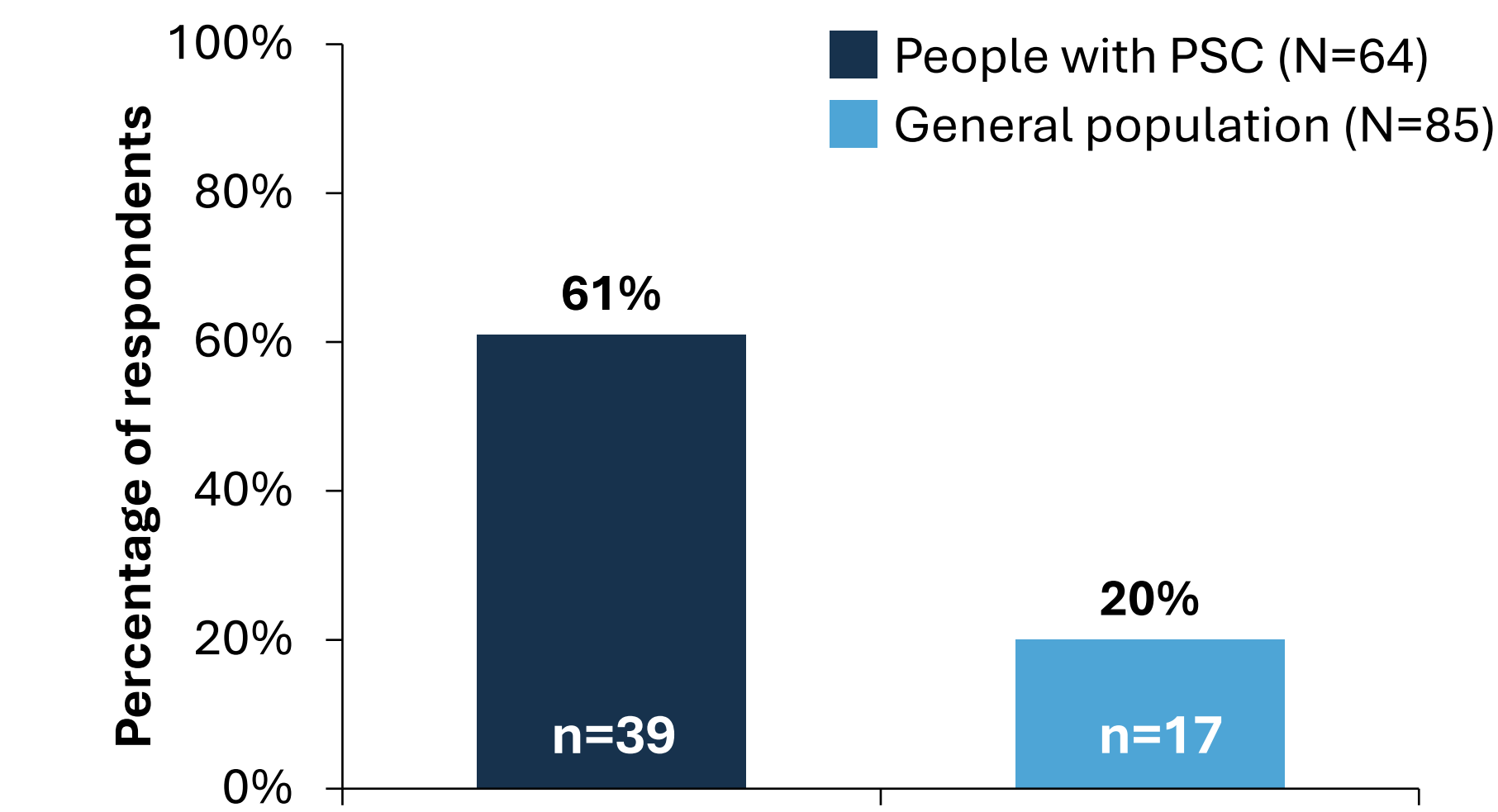


^aError bars indicate standard deviation. ^bThe grey and pink dotted lines represent the average and one standard deviation worse than the average PROMIS Fatigue T-score of the general population calibration set, respectively.⁷

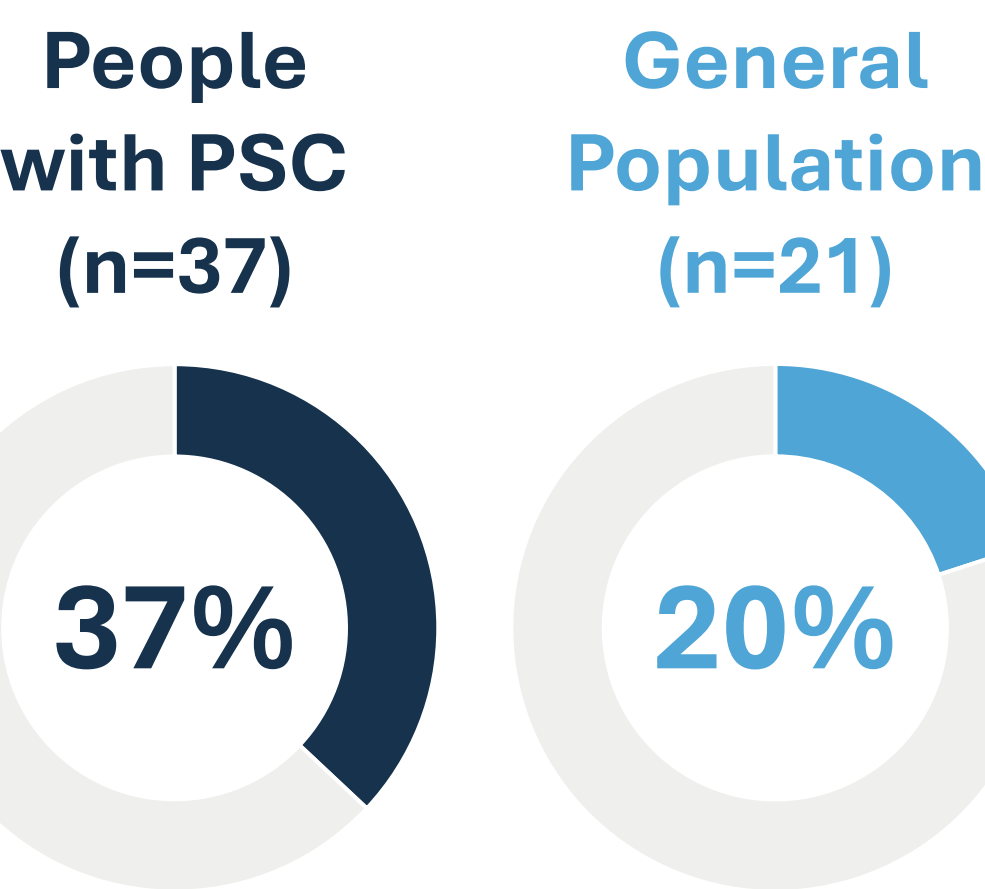
- Total PROMIS Fatigue scores were scaled to a general population calibration set to generate T-scores.
 - A T-score of 50 represents the average of the general population calibration set, and T-scores above 55 are considered outside of normal limits.^{7,8}
- The general population cohort in this survey had a mean T-score of 54.4, indicating worse fatigue scores than the general population calibration set.
- There is a modest but significant correlation between ItchRO and PROMIS Fatigue score in people with PSC (r=0.16; **P<0.01**).

46% of People With PSC Reported Making Changes to Their Employment Due to Their PSC

Figure 3. Respondents Who Experienced Physical or Psychological Problems at Work During the Past 4 Weeks



Reported absence from work in the previous 4 weeks:



“It’s very difficult to reconcile the fatigue inherent in the illness with professional activity. You have to be resourceful, take micro-naps, work remotely to more easily adjust your work schedule if the fatigue becomes too overwhelming.”

“Become self-employed so that I can choose not to work if feeling unwell or appointments/find it difficult to do things two days in a row.”

- Longer-term sickness absence (lasting more than four weeks) was more common among people with PSC (43%; n=16) compared with the general population (19%; n=4).

Conclusions

- Initial findings demonstrate that clinically significant pruritus and fatigue were present and that people with PSC experience tangible impacts on work participation, productivity, and daily living.
- These symptoms continue to be present and inadequately managed despite multiple medications used, underscoring an unmet need in the treatment of PSC.

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