

Self-reported experiences of patients living with primary biliary cholangitis (PBC): Are we treating the liver but not the patient?

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INTRODUCTION

In 2015, primary biliary cholangitis (PBC) patient support groups from Europe and North America pushed to change the name of their disease from primary biliary cirrhosis to *primary biliary cholangitis*. Their main complaints were that cirrhosis was not only a misnomer for the 50% of patients with early F1–F3 disease, but also that the word *cirrhosis* was felt to stigmatize patients as suffering from alcoholic abuse (1). This action of the support groups was all the more notable because PBC is rare, with a stable incidence rate of 3.4 in 100,000 people (2).

PBC is a progressive hepatobiliary disease. Reports suggest that major symptoms of fatigue are observed in 65%–78% and pruritus in a range of 20%–70% of patients, accompanied by sicca syndrome, arthralgia with myalgia, and right upper quadrant pain, among other symptoms (3–5).

The first-line of therapy for PBC, ursodeoxycholic acid (UDCA), has been linked to a significant reduction in mortality (2). The second-line therapy for UDCA non-responders, obeticholic acid, is a potent farnesoid X receptor agonist undergoing continued evaluation. Neither treatment has been reported to positively impact symptoms. A 2017 Cochrane Review concluded that current medications were ineffective for treating PBC patients' symptoms (6), and therefore therapy for fatigue and pruritus remain an unmet need.

Our aim was to capture the experiences of patients with PBC treated with UDCA and to understand their views regarding further treatment.

METHODS

Members of the Canadian PBC Society ($n = 253$) were invited to participate in an online survey about the previous 12 months by addressing the question



“How has PBC affected your life?” Anonymous responses were analyzed using percentages (binary and Likert-scale) while free-text responses to an open-ended question were manually coded by authors KI and BA into themes and sub-themes determined during content analysis. Differences between coding by KI and BA were identified and discussed or author AM resolved coding disagreements.

RESULTS

The completed survey response rate was 47% ($n = 119$). All participants were either on UDCA treatment or had completed therapy following liver transplantation (7%). Notably two-thirds of patients had required assistance with activities of daily living, including cleaning (58%), grocery shopping (48%), meal preparation (46%), and driving (39%). Fewer than 10% required assistance with bathing or dressing.

Four recurrent themes were identified following response to the question of how PBC affected their life (Figure 1). Two-thirds of respondents reported PBC-related symptoms, including fatigue (61%), aches or pain (16%), pruritus (13%), digestive issues (12%), and dry eyes or mouth (4%). Notably, 29% reported increasing levels of debilitation. Within the socioeconomic theme, 45% of respondents have had to decrease their social interactions with family and friends to accommodate their symptoms, primarily fatigue and pain. Current and future financial security was a concern for a subset of 19%, as they were forced to take early retirement, reduce their work hours, or go on disability leave. Patients who were still working reported that it was an everyday struggle and that they worried about their performance. Reducing work hours did not appear to ameliorate fatigue or to increase work productivity.

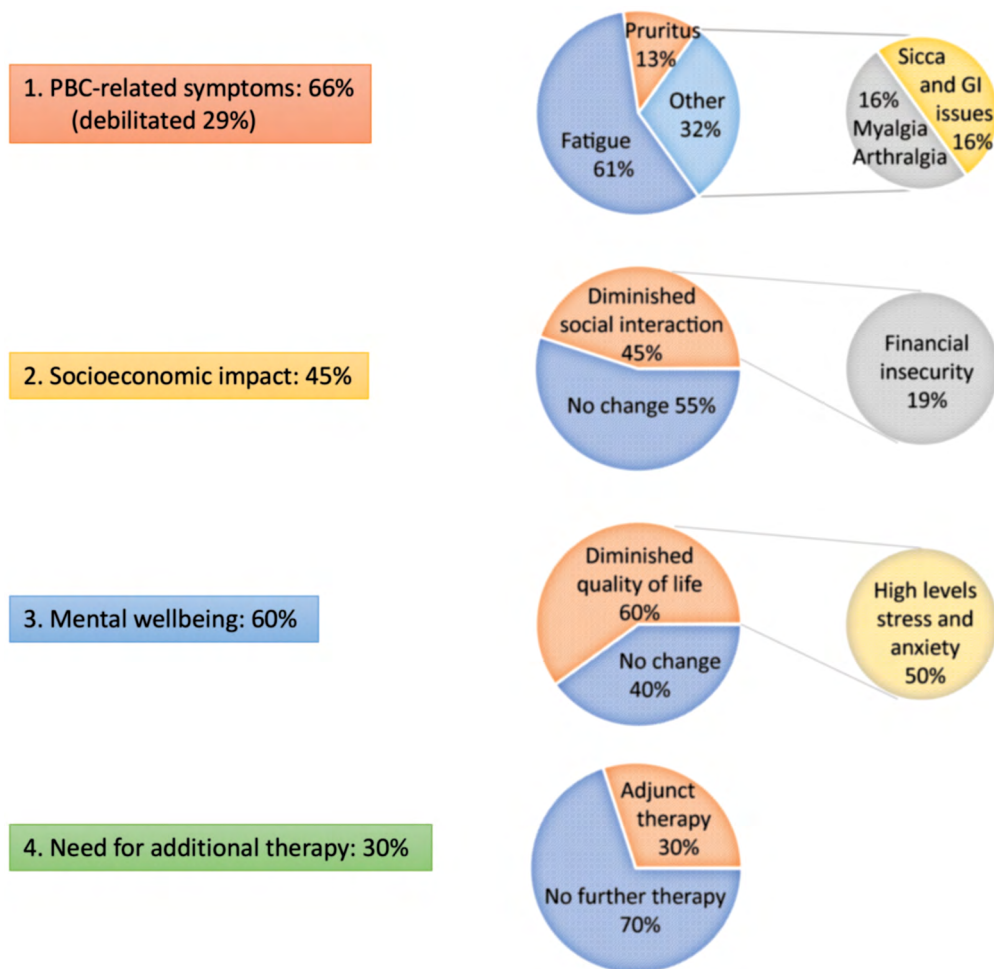


Figure 1: Unstructured, self-reported symptoms and concerns from primary biliary cholangitis patients asked how their disease process had affected their lives over a 12-month period

The mental health burden of living with PBC was indicated by 50% of patients reporting high levels of self-perceived stress and anxiety, described as “lassitude,” “chronic fatigue,” or simply “exhaustion.” Overall, 60% of respondents were unhappy with their current lifestyle, behaviours, and goals as they had to be modified to accommodate their symptoms. Those with high symptom burdens commented that these changes were unrelated to aging. Newly diagnosed patients or those with minimal symptoms were worried about their future symptom burden, but not necessarily about dying prematurely.

Nearly one-third of respondents either directly mentioned or inferred the need for additional PBC-specific medical treatments other than UDCA and liver transplantation. Some individuals were skeptical about the benefits of UDCA, while others expressed frustration at the medical community’s perceived reluctance or inability to integrate PBC care with that of their comorbidities.

CONCLUSION

Consistent with findings from other studies (7,8), we found that patients with PBC experienced high levels of disease-related symptoms despite ongoing treatment with UDCA. Fatigue clearly orchestrated a complex interplay on activities of daily living, mental health, productivity, and socializing that in turn impacted both financial security and quality of life. That 30% of PBC patients in this study suffered from debilitation provides a clear therapeutic dissonance whereby available and new therapies in the pipeline to improve hepatic biochemistry and life expectancy do so without addressing symptomatic needs. Additional emphasis is required to tackle the root cause of the disease to have an impact on symptoms and make PBC patients’ lives worth living.

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