



Liver, Pancreas and Biliary Tract

A short version of a HRQoL questionnaire for Italian and Japanese patients with Primary Biliary Cirrhosis[☆]

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ABSTRACT

Background: The available self-report questionnaire for the quality of life in patients with primary biliary cirrhosis (PBC-40) is currently validated only in the British population but it lacks an evaluation of its dimensionality.

Aims: To validate the Italian and Japanese versions of PBC-40 and to assess the dimensionality of the original structure of PBC-40 by a confirmatory factor analysis. PBC-40 was translated to Italian and Japanese using the forward-backward method and then reviewed in focus groups in the framework of a large multicentric study.

Methods: A sample of 290 patients with PBC (125 Italian and 165 Japanese) was administered two questionnaires previously validated for PBC-specific (PBC-40) and general quality of life (SF-36).

Results: The confirmatory model failed to fit adequately the original hypothesized structure. A principal component analysis led to a seven-factor structure, with exclusion of 13 items characterized by lower load; PBC-27 questionnaire was the final instrument. The validity of the PBC-27 was supported by its strong correlation with the SF-36 scores.

Conclusion: We here propose an alternative structure of the quality of life questionnaire for PBC, namely PBC-27, which appears to be effective in detecting the impact of PBC on quality of life in Italian and Japanese patients.

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1. Introduction

Primary biliary cirrhosis (PBC) is a progressive, chronic liver disease characterized by the immune-mediated damage to the biliary epithelial cells lining the small intrahepatic bile ducts [1]. The

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course of the disease is generally slow and often asymptomatic [2]. However, most patients suffer from elusive symptoms, such as fatigue and pruritus, known to reduce their individual health related quality of life (HRQoL) and well-being [3,4] particularly at early stages of liver disease (i.e. before the appearance of liver cirrhosis and its complications).

In the past two decades HRQoL has become an important read-out in clinical research evaluating secondary treatment outcomes. The understanding of factors related to HRQoL in chronic disorders is becoming increasingly relevant in clinical practice, with the recent emphasis on the comprehensive management of patients. The World Health Organization states that quality of life is a complex concept resulting from the individual physical health, psychological state, level of independence, social relationship, and

salient environmental features [5]. HRQoL is a subset relating only to the health domain of that existence.

It is widely recognized that evaluation of the HRQoL is a particularly complex issue, since it is influenced by several social and possibly geographical factors. HRQoL in specific patient population can be measured by generic and/or disease-specific questionnaires [6]. Generic questionnaires, such as the SF-36, are designed to be applicable to populations with a wide variety of conditions. Their major advantage is that they have been validated and widely used to measure the HRQoL in various conditions so that they provide a global assessment and allow comparisons with other conditions [7]. However, such questionnaires have less sensitivity to small, but clinically relevant, changes in patient HRQoL over time, a major problem in case of rare diseases due to floor or ceiling effects [8]. On the contrary, domain-specific and disease-specific questionnaires are more sensitive based on their “custom-design” to focus on disease-specific issues, and are ultimately more reliable in assessing the patient subjective well-being, effectiveness of interventions, or extent of disease progression [9].

To date, few studies have examined HRQoL in patients with PBC and such assessment has not routinely entered clinical trial use or normal clinical practice, despite the numerous studies suggesting the impact of the disease symptoms [3]. A group from UK has recently addressed this limitation and developed the first disease-specific HRQoL measure for patients with PBC, named PBC-40 [10], covering six hypothesized domains (Cognitive, Itch, Fatigue, Social, Emotional and other Symptoms), later reduced to a five-domain structure with the collapse of the social and emotional ones [11]. Nevertheless, the PBC-40 has been validated only in English with British patients. Moreover we could not find in the literature a statistical analysis of the PBC factor structure, which represents the only way to assess the dimensionality of a scale [12,13].

Based on the suggested population and cultural variations in symptom relevance and impact for PBC [14], we herein validated an Italian and Japanese version of a PBC-specific HRQoL questionnaire. By applying a confirmatory factor analysis (CFA) to evaluate its psychometric properties, we also propose a shortened PBC-40 version, namely PBC-27, which provides a better fit in Italian and Japanese patients with PBC.

2. Materials and methods

2.1. Study population and design

290 patients affected by PBC were consecutively enrolled at one liver unit in Milan, Italy and six liver units in Japan between June 2007 and June 2008. The diagnosis of PBC was based on the presence of two out of three internationally accepted criteria, i.e. detectable serum anti-mitochondrial antibodies (titre >1:40), increased enzymes indicating cholestasis (i.e. alkaline phosphatase) for more than six months, and a compatible or diagnostic liver histology [1]. One hundred and twenty-five patients were Italian (116 females; mean age 62 years, range 39–84) and 165 Japanese (143 females; mean age 61 years, range 30–83). Serum biochemical tests including aminotransferases, gamma-glutamyltransferase, alkaline phosphatase, albumin, total bilirubin, lipids, immunoglobulins, hepatitis B surface antigen, antibody to hepatitis B core antigen, and antibody to hepatitis C virus were assessed by routine laboratory methods in all patients upon enrolment. Similarly, anti-mitochondrial, anti-nuclear, and anti-smooth muscle antibodies were available in all patients using indirect immunofluorescence and/or ELISA methods [15]. The presence of symptoms was defined as the occurrence of pruritus, jaundice, or major complications of portal hypertension: i.e. ascites, gastrointestinal bleeding, portal-systemic encephalopathy. The Mayo Score

was used as an overall measure of disease severity [16]. Disease duration was calculated as the time between the date of the earliest suspected evidence of liver disease and the date of enrolment in the study. The histological picture of PBC was classified according to Ludwig et al. [17]. Table 1 illustrates the characteristics of this PBC population. Ursodeoxycholic acid was being administered to 156 (95%) of the Japanese and 83 (66%) of the Italian patients as the only treatment for liver disease at the time of enrolment.

We designed a three-phases study which included (i) the development of the Italian and Japanese versions of PBC-40; (ii) the evaluation of the psychometric properties of the Italian and Japanese versions of PBC-40; and (iii) the correlation of the PBC-specific HRQoL questionnaire with SF-36. The study protocol conforms to the ethical guidelines of the 1975 Declaration of Helsinki (6th revision, 2008) as reflected in a priori approval by the institution's human research committee. This project received ethical approval from the local IRB in each involved hospital and all subjects entering the protocol provided written informed consent after receiving a complete description of the study and having the opportunity to ask questions.

2.2. Questionnaires

The PBC-40 is a disease-specific HRQoL measure derived and validated for self-completion use in PBC [10]. The PBC-40 has six hypothesized domains: Fatigue (11 items), Cognitive (6 items), Social (10 items), Emotional (3 items), Itch (3 items) and other Symptoms (7 items). Items are rated on an ordinal scale ranging from 1 to 5 (with high scores denoting the greater symptom impact and the worse HRQoL). The total score is obtained by averaging the 40 items.

The SF-36 is a widely used and validated generic questionnaire adopted to measure the HRQoL of various conditions. It includes 36 items divided into eight domains, which can be aggregated into two summary scores: a “mental component summary” and a “physical component summary”. These indices include Physical Functioning, Role Physical (role limitations as a result of physical health), Bodily Pain, General Health, Vitality, Social Functioning, Role-Emotional (role limitations as a result of mental problems) and Mental Health. SF-36 scores on the individual scales range between 0 and 100. SF-36 was found to have the best performance in terms of internal consistency and test–retest reliability as the generic measures of HRQoL for PBC patients.

Both the Italian and the Japanese version of PBC-40 were developed by translating and then back-translating the questionnaire to determine possible discrepancies with the English original version. The resulting questionnaires were reviewed by a team of physicians who usually provide care to patients with PBC.

2.3. Questionnaire administration

All patients with PBC attending a regular outpatient visit were asked to fill out two self-report questionnaires, the PBC-40 and the SF-36. Eight patients (3 Italians and 5 Japanese) declined to take part in the study (7 claiming ‘lack of time’, 1 for ‘excessive stress’). Demographic questions were also included in the forms and all questionnaires were self-administered in the presence of an instructed psychologist or physician in quiet rooms within the liver unit facilities. On average, the completion of the questionnaire took about 20 min.

2.4. Statistical analyses

All the analyses were performed in all subjects and subsequently for each language separately. Cronbach's α and CFA were first utilized on the six-domain model of PBC-40 documented in the

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