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Development, Reliability, and Validity of a New Preference and Satisfaction Questionnaire

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A B S T R A C T

Objectives: Existing questionnaires that assess preference and/or satisfaction with postmenopausal bone loss treatments were reviewed and determined to be inadequate for the assessment of an oral pill versus a subcutaneous injection. The Preference and Satisfaction Questionnaire (PSQ) was developed to assess preference, satisfaction, and bother with a weekly oral tablet versus a once every 6 months subcutaneous injection for treatment of postmenopausal bone loss. **Methods:** Questions were developed based on literature review and expert input. Content validity of the PSQ in this patient population was assessed among current or previous bisphosphonate users in group interviews, and item comprehension and readability were also evaluated. Reliability, validity, and structure of the questionnaire were assessed in two phase 3 randomized clinical trials. **Results:** Twenty-four women participated in cognitive interviews and found the PSQ understandable and acceptable. Subsequently, 1583 trial participants took the PSQ. Interitem correlations, ranging from 0.50 to 0.97 for preference items, 0.85 to 0.94 for pill-satisfaction items, and 0.84 to 0.92 for injection-satisfaction items, and a well-fitting confirmatory factor analysis (root mean square error of approximation 0.04, nonnormed fit index 0.99, and root mean square residual 0.08) supported the structure of the instrument. Cronbach's alpha reliability values for pill satisfaction, injection satisfaction, pill bother, and injection bother were 0.93, 0.89, 0.82, and 0.61, respectively. Discriminative validity was indicated with better satisfaction and bother scores being related to adherence and the absence of adverse events. **Conclusions:** The PSQ is a valid and reliable measure and may be a valuable tool to assess patient preference and satisfaction with a weekly oral tablet and 6-month subcutaneous injection for postmenopausal bone loss.

Keywords: preference, questionnaire development, reliability, satisfaction, validation.

Introduction

Osteoporosis is a major public health concern, affecting an estimated 200 million people worldwide [1]. In the United States and Europe approximately one-third of postmenopausal women have osteoporosis [2]. Osteoporosis is characterized by increased bone resorption and decreased bone mass, resulting in microarchitectural deterioration of the skeleton and increased fracture risk. Osteoporosis is initially an asymptomatic disease, with few clinical symptoms before fracture. Studies have shown that compliance and persistence with pharmaceutical treatments for osteoporosis are suboptimal, with many patients discontinuing therapy within the first year [3–5], and likely not receiving the full therapeutic benefit [5,6]. Reasons for nonadherence are multifactorial and include convenience and frequency of the dosing regimen, perceived efficacy, and side effects [7,8]. Results from a large, longitudinal study of postmenopausal women who were prescribed treatments for osteoporosis found that women who were less satisfied with their osteoporosis treatment were more likely to discontinue treatment within the first year than women who were more satisfied with their osteoporosis treatment [9].

Bisphosphonates are the therapeutic agents most frequently used to treat postmenopausal bone loss. These agents are available as oral tablets to be taken daily, weekly, or monthly, and intravenous infusions given quarterly or annually. Denosumab (Prolia®) is a fully human monoclonal antibody to RANK ligand, a key mediator of osteoclast formation, function, and survival. Denosumab is administered as a twice-yearly subcutaneous injection. Two randomized phase 3 trials directly compared the efficacy of denosumab with branded alendronate (Fosamax) in postmenopausal women with low bone mass [10,11]. In both studies, denosumab treatment significantly increased bone mineral density at the total hip compared with alendronate treatment. In a separate blinded, randomized, placebo-controlled, phase 3 trial, denosumab was shown to significantly reduce the incidence of new vertebral, nonvertebral, and hip fractures in postmenopausal women with osteoporosis [12]. The less frequent administration of denosumab could promote greater adherence with treatment, resulting in better therapeutic outcomes for patients.

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Patient preference and satisfaction are important determinants of adherence to therapies for chronic conditions, including osteoporosis [4,13–15]. Preference is a relative measure of desirability, and has a strong theoretical basis in economics and psychology. It can be measured as a choice between alternatives or scaled (degree of desirability) [16]. Treatment satisfaction measures the degree to which patient expectations with different attributes of their treatment are met [17,18]. Attributes of treatment may include perceived efficacy, presence and/or severity of side effects, convenience, and the bother with treatment. Further understanding of the factors that influence patient perception of osteoporosis treatments may result in improved educational efforts to increase adherence.

To date, no existing questionnaire adequately captures patient preference for and satisfaction with a subcutaneous injection versus oral pill for the treatment of postmenopausal bone loss. Thus, we developed the Preference and Satisfaction Questionnaire (PSQ) to compare patient preference for and satisfaction with two agents for the treatment of postmenopausal osteoporosis: a subcutaneous injection given every 6 months and an oral pill taken weekly. The objective of this article is to describe the development of and establish evidence for the reliability and validity of the PSQ in evaluating patient preference for and satisfaction with treatment for low bone mass and osteoporosis.

Methods

PSQ development

A literature review using Medline was conducted to assess the existing evidence on patient preference for and satisfaction with medical therapies. The review focused on English language articles published from 1990 to 2006 that reported results from preference and satisfaction studies in patients with osteoporosis, preference and satisfaction studies in other disease settings—especially in primary care—and patient preference and satisfaction related to injections. A total of 348 articles were identified; 49 were retrieved. Subsequent to a more detailed evaluation, 32 publications were selected for data abstraction and detailed review. An additional five studies were identified from the reference lists, for a total of 37 studies. These publications covered a broad range of medical conditions, including osteoporosis, pain, migraine, diabetes, chronic obstructive pulmonary disease, and asthma. Injection was discussed in 13 studies (intramuscular, subcutaneous, or intravenous). The majority of studies (n = 28) assessed preference once, generally at the end of study, whereas the balance (n = 9) assessed preference at more than one time point during the study. Preference was assessed using a single item in 18 studies and multiple items in 11 studies; eight studies did not indicate the number of items used to assess preference. In 34 studies preference was measured along with other items such as utility, satisfaction, or symptoms. Many studies (n = 23) did not provide details on the validity of the instruments and only seven studies provided a published source for the instrument (we did not retrieve the source to determine validity).

Existing questionnaires that assess preference and/or satisfaction with treatment also were reviewed to create an initial pool of topics. We identified nine studies in osteoporosis that evaluated preferences [19–27]. These studies evaluated daily versus daily [22,24], weekly versus daily [21,23,25,26], or weekly versus monthly oral treatment regimens [20,27]; none evaluated injections. Only four studies were identified that compared treatment with oral tablets to an injection. In two of the studies the oral and subcutaneous injection were given with the same dosing frequency for treatment of migraines [28,29]. A third study evaluated oral versus intravenous versus intramuscular dosing regimens for treatment of acute pain [30]. The fourth study evaluated monthly intramuscular injection versus oral dosing for contraception [31].

Three experts in addition to the study's authors reviewed the initial item pool generated from the literature to identify relevant concepts, ensure item clarity, and eliminate redundancy to generate an initial set of items. The draft PSQ contained 34 questions relating to preference, satisfaction, bother, convenience, long-term use, and lifestyle fit. For nine items (1–3, 6, and 9–13) patients were asked to choose in terms of preference, bother, or satisfaction, one of the following: the pill, the injection, or neither (indicating any difference in preference or satisfaction between the two treatments). For 20 items (4a–4f, 5a–5f, 7a–7d, and 8a–8d) patients used a five-point response scale to specify the degree of bother (scored from not bothered at all to severely bothered) or satisfaction (scored from not satisfied at all to very satisfied) directed at each of the treatments. For five items (14a–14e) patients selected the degree to which she agreed or disagreed with the question or statement on a five-point response scale (strongly agree to strongly disagree). In these items, patients compared treatment regimens with one treatment being favored over the other.

PSQ evaluation

In-depth interviews were conducted by experienced facilitators with two different focus groups in two cities in the United States (n = 12/group). Participants (n = 24) were postmenopausal women who currently used or had used a bisphosphonate within the past three years. The semistructured interviews included open-ended questions addressing the effects of the disease and disease treatment on social, psychological, physical functioning, and perceived well-being. Patients completed the draft questionnaire and were debriefed on the PSQ item relevance, interpretation of content, clarity of wording, format and length, and concepts. Revisions were made to question wording and the layout of the survey based on feedback from the first focus group. The revised version of the PSQ was administered to the second focus group and additional revisions were made based on feedback. Subjects' comments were recorded during the survey and the cognitive debrief in addition to the detailed notes generated by the interviewer. To evaluate if the order of response options influenced the choices made by the subject, participants in the first focus group were given a version of the questionnaire where injection preceded oral for all response options whereas participants in the second focus group were given a version of the questionnaire where oral preceded injection for all response options.

The pilot English questionnaire was then culturally adapted into 17 languages for use in 14 countries in accordance with the Translation and Cultural Adaptation group for linguistic validation of quality of life questionnaires [32]. The linguistic validation process involves forward and backward or harmonized translations of the original American English questionnaire, followed by cognitive debriefing of patients from each country to ensure items are understood across cultures consistently. The culturally adapted questionnaires were included in two randomized, double-blind, double-dummy phase 3 trials for psychometric validation.

The PSQ was administered to participants enrolled in the phase 3 DECIDE and STAND trials. Details on the design of the DECIDE and STAND studies are published elsewhere [10,11]. In brief, both studies compared the efficacy and safety of twice-yearly subcutaneous injection of denosumab (60 mg) with weekly oral alendronate (70 mg) in postmenopausal women with low bone mass. Subjects received both an oral tablet weekly and a subcutaneous injection once every 6 months. In the DECIDE study (N = 1189), patients had no or very limited prior exposure to oral bisphosphonates, whereas in the STAND study (N = 504) patients had received bisphosphonate therapy for a minimum of 6 months before study enrollment. In both studies, patients took the PSQ at