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Development of the Shortness of Breath with Daily Activities Questionnaire (SOBDA)

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ABSTRACT

Objectives: Based on qualitative research of patients with chronic obstructive pulmonary disease (COPD), the Shortness of Breath (SOB) with Daily Activities (SOBDA) questionnaire was developed as a patient-reported outcome instrument to evaluate the impact of therapy on SOB and assess how SOB affects daily activities. **Methods:** Development of the SOBDA questionnaire consisted of three components. First, focus groups of patients with COPD were asked to describe their experiences of SOB with daily activities. A pool of items was drafted on the basis of information from the focus groups and literature reviews, and then discussed among instrument development and clinical experts. Cognitive debriefing interviews of patients were conducted to assess the draft item pool, and their feedback was used to develop newer versions of the questionnaire. Input was also sought from the Food and Drug Administration, patients, and clinicians. **Results:** Forty patients participated in seven focus groups. The terms most often used to describe SOB were "short of breath" or "difficulty breathing." Patients were clearly able to distinguish SOB from chest congestion and wheezing, other common symptoms associated with COPD. The resulting item pool contained 37 items to assess SOB associated with everyday activities, and concept saturation was reached. Thirty-seven patients participated in the subsequent cognitive debriefing interviews. Patients found the items clear and easy to understand with relevance to their everyday experiences, and easy to use in an electronic format. **Conclusions:** Instructions and response options to the SOBDA questionnaire were well understood by patients with COPD, and item relevance was confirmed. Prospective validation and item reduction studies are highly anticipated.

Keywords: COPD, patient-reported outcomes, qualitative research, quality of life.

Introduction

Chronic obstructive pulmonary disease (COPD) is a preventable and treatable disease characterized by progressive airflow limitation that is not fully reversible [1]. It is associated with an abnormal inflammatory response in the lung to noxious particles or gases.

The principal marker for the physiologic changes in airflow limitation, which is characteristic of the disease, is lung function, measured as forced expiratory volume in 1 second (FEV1). This marker correlates poorly with the severity of dyspnea (usually described by patients as shortness of breath [SOB]) and other symptoms of COPD [1,2]. Therefore, changes in FEV1 may not always reflect symptomatic changes that are clinically meaningful for patients. A variety of biologic, physiologic, and symptomatic markers are currently being explored as alternative methods for assessing disease severity, response to therapy, and disease progression [3–5].

Dyspnea is one of the most common and disabling symptoms in COPD [3,6,7]. It is frequently associated with decreases in functional status, physical activity, and quality of life [8–10]. The therapeutic goals for patients with COPD include relief from symptoms such as dyspnea, improving health status, preventing and treating exacerbations, slowing the progression of disease, and reducing mortality [1,11]. Licensed indications for most current COPD treatments are limited to improving airflow obstruction, and yet no US Food and Drug Administration (FDA)-approved pharmacologic therapy currently has information on dyspnea in its US label. As dyspnea is so important to the lives of patients with COPD and it affects many of their daily activities, the relationship between the two is important to properly evaluate.

The relationship between physical activity and breathlessness in COPD is complex, and various models have been developed to help facilitate an understanding of this association. Jolley and Moxham [9] described a physiologic model of patient-reported breathlessness based on the relationship between ventilatory load, respiratory muscle capacity, neural respiratory drive, and neuromechanical dissociation during daily activities. Conversely,

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Victorson et al. [12] developed a conceptual model to inform patient-reported outcome (PRO) instrument development using patient descriptions of dyspnea and functional limitations in COPD. On the basis of qualitative research, Victorson's group concluded that five primary components make up the patient's experience of dyspnea: breathlessness, fatigue, activity modification, activity limitation, and emotional response. Their model describes how dyspnea symptoms impair function and are mediated by personal and environmental factors. Both the physiologic and conceptual models provided a structure on which to base Shortness of Breath with Daily Activities (SOBDA) questionnaire development for measuring the severity of breathlessness during daily activities. With the understanding gained from these models, we attempted to assess qualitative outcomes in COPD relating to dyspnea.

Qualitative studies are increasingly recognized to be as important to our understanding of the patient experience of dyspnea as studies focusing on other physical aspects of COPD. The results of such studies explain, at least in part, why two people with the same physiologic markers of COPD severity often experience and describe different levels of dyspnea. To develop an instrument that accurately captures how patients perceive dyspnea, a patient-centered approach using their words to describe symptoms is necessary. Such an instrument needs to be valid, reliable, and responsive to change, meeting the criteria outlined in the FDA PRO Guidance document [13], if the intent is to support a label claim for a medicinal product in the United States. No instruments for assessing COPD-related dyspnea have been qualified for the target population to achieve an indication of a medicinal product by the FDA for inclusion into product labels at the time of writing. We developed the SOBDA questionnaire to assess the impact of daily activities on dyspnea in patients with COPD. The goal of this phase of development was to construct an instrument for assessing SOB during patient-identified daily activities that is based on patient feedback on specific terminology and patient experiences with SOB.

Methods

The process for developing the SOBDA questionnaire involved multiple steps and review processes. Focus groups of patients with COPD were conducted in clinic offices and meeting rooms in San Diego, CA, San Antonio, TX, New Brunswick, NJ, and Miami, FL, and each session lasted for approximately 1.5 to 2 hours. The moderator's discussion guide for the focus groups was developed on the basis of current relevant literature, learnings from previous models such as those developed by Jolley and Moxham [9] and Victorson et al. [12], and input from clinical experts, and was used to facilitate discussions on patients' experiences of SOB with daily activities. A pool of items was drafted on the basis of information gathered from the focus groups and literature reviews, and these items were then discussed among instrument development and pulmonary experts. In addition, four translation experts and a lexibility expert reviewed the questionnaire to ensure cross-cultural equivalence and translational feasibility, as well as clarity of wording. Cognitive debriefing interviews of patients were subsequently conducted to evaluate the draft item pool, and feedback from these interviews was used to develop newer versions of the questionnaire.

Patients

For both the focus group discussions (phase 1) and cognitive debriefings (phase 2), efforts were made to recruit from pulmonary clinics in the United States participants with a variety of educational, sociodemographic, and ethnic backgrounds, as well as diverse disease experiences. The demography and clinical characteristics of the recruited participants were intentionally chosen to include and expand beyond that of a typical COPD clinical trial population in order for the instrument to be able to be used in a broader trial population. Economic diversity was addressed by using zip codes as a surrogate for socioeconomic status [14]. Clinics from across the United States were instructed to enroll participants with different disease severities to achieve the following target population: 15% Global Initiative for Chronic Obstructive Lung Disease (GOLD) stage I, 35% GOLD stage II, 35% GOLD stage III, and 15% GOLD stage IV. The target number of desired participants for this study was 40; however, the total number could be modified on the basis of whether concept saturation (i.e., no new concepts or information emerging from subsequent focus groups) was reached [15–18]. Saturation was expected to be reached during focus group discussions by approximately 30 patients. If saturation was not reached, additional participants could be added. Protocols were approved by an institutional review board, and patient consent was obtained prior to the discussion of study-related materials. Clinicians completed an enrollment form, confirming each patient's eligibility and disease severity.

Inclusion criteria were as follows: 40 to 80 years of age; current or former smokers with a history of at least 10 pack-years; current diagnosis of COPD and/or chronic bronchitis as defined by the GOLD initiative [1]; willing and able to provide written informed consent; able to participate in a group discussion; and able to speak and read English.

Exclusion criteria were as follows: respiratory disorders other than COPD (e.g., asthma); organic heart disease with resultant left ventricular failure and New York Heart Association class II to IV; clinically relevant bronchiectasis; recent COPD exacerbation (within previous 60 days); neuromuscular disease; possible causes of significant dyspnea/fatigue other than COPD, including severe anemia; and concurrent medical or psychiatric condition or cognitive impairment potentially affecting participation in the study.

Measures

Upon completion of both the focus group discussions and cognitive debriefings, all patients completed a brief sociodemographic questionnaire that provided reviewers with additional information on the patient population. In addition, patients were assessed by using the following validated measures: the modified Medical Research Council dyspnea scale [19], the St. George's Respiratory Questionnaire for COPD patients [20,21], and the Chronic Respiratory Questionnaire – Self-Administered Standardized [22–24].

Focus Groups

Moderators used a standardized discussion guide to solicit terminology used by patients to describe the sensation of dyspnea and to explore the circumstances in which participants experienced the sensation. Patients were initially asked to "tell me about your breathing," which prompted them to explain their experience with dyspnea and the differences in sensations of dyspnea compared with chest congestion, chest tightness, and wheezing. Patients were then asked to describe the general activities they conducted on a daily basis, as well as their level of dyspnea as they conducted these activities. Moderators probed on specific dyspnea-inducing aspects of the activities, and patients were asked to describe any body movements or positions that impact dyspnea. All discussion probes were phrased as open-ended questions, using only the terminology that patients provided. The verbatim terms that patients used to describe their dyspnea were coded for the frequency of occurrence. As each concept reached saturation, final sessions were focused on supplementing missing information relating to activities, but an open discussion of the other topics was still encouraged by the moderators.