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ORIGINAL RESEARCH

Economic Evaluation

The Use of Qualitative Methods in Developing the Descriptive Systems of Preference-Based Measures of Health-Related Quality of Life for Use in Economic Evaluation

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ABSTRACT

Objectives: To describe how qualitative methods can be used in the development of descriptive systems of preference-based measures (PBMs) of health-related quality of life. **Methods:** The requirements of the National Institute for Health and Clinical Excellence and other agencies together with the increasing use of patient-reported outcome measures has led to an increase in the demand for PBMs. Recently, interest has grown in developing new PBMs and while previous research on PBMs has mainly focused on the methods of valuation, research into the methods of developing descriptive systems is an emerging field. **Results:** Traditionally, descriptive systems of PBMs were developed by using top-down methods, where content was derived from existing measures, the literature, or health surveys. A contrasting approach is a bottom-up methodology, which takes the views of patients or laypeople on how their life is affected by their health. This approach generally requires the use of qualitative methods. Qualitative methods lend themselves well to the development of PBMs. They also ensure that the measure has appropriate language, content validity, and responsiveness to change. While the use of qualitative methods in the development of non-PBMs is fairly standard, their use in developing PBMs was until recently nonexistent. **Conclusions:** In this article, we illustrate the use of qualitative methods by presenting two case studies of recently developed PBMs, one generic and one condition specific. We outline the stages involved, discuss the strengths and weaknesses of the approach, and compare with the top-down approach used in the majority of PBMs to date.

Keywords: outcomes, preference-based measures, QALYs, qualitative methods.

Introduction

There are a large number of health-related quality-of-life (HRQOL) questionnaires, most of which define the health status of those completing them in terms of various dimensions such as mobility or pain. Each of these dimensions typically has a number of levels that indicate different degrees of severity. These questionnaires seek to obtain information directly from the patient and are often referred to as patient-reported outcome measures or more widely patient-reported outcomes (PROs). PROs can be used as an umbrella term to cover a range of potential types of questionnaires that gather self-reported information from the patient and include symptom burden, quality of life (QOL), and HRQOL. In the context of this article, the focus is only on those aspects of HRQOL that are influenced by health care interventions and treatments.

The majority of existing HRQOL questionnaires cannot be used in economic evaluation because they are not preference based and do not take account of the relative importance of the different dimensions [1]. The need to have HRQOL instruments that can be used for effectively describing the impact of health care interventions on patients has been driven by two factors. The first is the desire to collect data on the quality of care from the patient's rather than the clinician's perspective. The second is the need to compare interventions, through economic evaluation, to use limited health care resources more efficiently. This has been formalized in many countries through agencies such as the National Institute for Health and Clinical Excellence (NICE) in the United Kingdom [2] and others around the world [3–5]. These decision-making bodies require evidence on the cost-effectiveness of health care interventions under consideration as part of the decision-making process. The majority of these agencies have formal guidelines for the methods of economic evaluation and while in the past these guidelines have not stipulated the measure of benefit for cost-effectiveness analysis, more recently they have explicitly stated that health effects be measured in quality-adjusted life-years (QALYs) [2,3,5]. In 2004, NICE introduced its reference case (the set of methods considered

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most appropriate by NICE) and stated QALYs as the measure of benefit. All submissions to NICE now require a cost-effectiveness analysis based on these methods, with health effects measured in QALYs [2]. The QALY combines length of life and QOL into a single summary measure. As well as being able to take account of changes in HRQOL, quantity of life, or both, it is useful in health care resource allocation decision making as it allows comparison across clinical areas because of the use of a common measure of benefit [1]. QALYs are calculated as the product of the time spent in a particular health state multiplied by the utility or preference weight associated with that health state.

There has been an increasing use of data obtained from non–preference-based PROs within the UK National Health Service (NHS) stimulated by a key recommendation of the Darzi report, "High Quality Care for All" [6], published under the last Labor government. This recommended that the impact of treatments on HRQOL should be measured through the routine use of PROs. As a result, since 2009, NHS providers have been required to ask patients to complete a PRO before and after four surgical procedures (hip replacements, knee replacements, hernia repair, and varicose veins). These data are now published on a monthly basis.

One problem associated with the PROs now routinely used within the NHS is that they are non–preference-based and scores from these are calculated by summing the responses for any domains. However, simply adding up the scores does not provide information on the relative weights or importance of the different questions or the different domains because more often than not each domain is allocated an equal weight [7]. Preference-based measures (PBMs) are an attempt to take into account the relative importance of the domains and questions and give an overall index score that corresponds with the preference-weight component of the QALY for a specific health state described by the PRO. These are based on a scale where 1 is full health and 0 is equivalent to being dead (with negative values for health states judged to be worse than dead). These preference weights for health states can be obtained in a number of different ways, including the use of expert opinion, literature, direct valuation from the patient, or the use of PBMs [1]. A PBM is a type of PRO that typically consists of a health state classification system (HSCS) and a set of preference weights for each of the health states defined by the HSCS. Usually, patients complete the HSCS, which defines their current health state, and then the preference weight assigned to that health state can be used to calculate QALYs.

Preference-Based Measures

There are two main types of PBMs, generic and condition specific. A generic PBM is intended to cover all areas of health and should be applicable to any clinical condition. An example is the EuroQol five-dimensional (EQ-5D) questionnaire, which has been widely used in numerous clinical conditions [8]. A condition-specific PBM is concerned only with a particular condition, for example, asthma or diabetes. It may be used when there are concerns that a generic PBM may not be valid or reliable, for example, by not being sensitive enough. Examples include the Sexual Quality of Life-3D for sexual QOL [9] and the Asthma Quality of Life-5D for asthma [10]. An alternative is to develop extra dimensions or "bolt-ons" to fill any important gaps identified in the coverage of a generic measure [10].

Generic PBMs such as the EQ-5D questionnaire, six-dimensional health state short form (derived from short form 36 health survey), health utilities index mark 2 (HUI2), and HUI3 have sometimes been found to be inappropriate or insensitive for some conditions [1]. Generic measures can sometimes be insensitive to changes in HRQOL because they do not contain dimensions important for a specific condition. In these cases, condition-specific questionnaires may be more appropriate because they aim to fill in the "gaps" not covered by generic instruments. The most common method to date to do this has been to modify an existing condition-specific non-PBM [11]; however, an alternative is to develop a new measure from scratch where existing measures have been shown to have poor measurement properties or have issues with their validation.

In order for non-PBMs to be converted into a PBM, they need to demonstrate specific properties in terms of their practicality and ordering of their item scales [1]. The main constraint, however, of developing a PBM is that the health states defined by the HSCS should be amenable to valuation. Health state valuation is the way in which the preference weights for the health states are determined. It can be undertaken by using a variety of methods, including standard gamble, time trade-off, or ordinal methods such as ranking and discrete choice experiments [1]. To be amenable to health state valuation, HSCS should have dimensions with ordinal levels and ideally one item per dimension. In addition, there is a limit to the number of dimensions that it can contain. Typically, people can value seven (plus or minus two) pieces of information at any one time [12], and so the number of dimensions is typically no more than nine. The most widely used generic descriptive systems range from five to nine dimensions [1]. This is a practical constraint on the number of dimensions within a descriptive system because it is unlikely that respondents would be able to handle a larger number when undertaking valuation exercises. Non-PBMs of HRQOL do not have to operate within these constraints and hence can have much larger descriptive systems.

One example of the limitations of converting existing non-PBMs has been highlighted in the area of venous leg ulcers. A review found that there were no existing PBMs, generic PBMs showed limited sensitivity, and none of the seven condition-specific non-PBMs were amenable for conversion to PBM [13]. The reasons for this included that the instruments had limitations in terms of their practicality, validation, and development.

The requirements of NICE and other agencies together with the increasing use of PROs in the NHS has led to an increase in the use of PBMs in health care research and as a consequence, an increase in the demand for PBMs. In the past, attention has been focused on the methods of health state valuation and less on the methods of developing the HSCS. Recently, however, there has been increasing interest in developing new PBMs, particularly condition-specific ones [11], and so there is increasing interest in the methods of development. The objective of this article was to describe how qualitative methods can be used in the development of descriptive systems of PBMs of HRQOL.

Methods of Development for PBMs

The main existing generic PBMs for adults have all used a top-down approach in the development of their descriptive systems; that is, the content has been derived from existing literature, instruments, and health surveys. The Measurement and Valuation of Health Survey, which was used to develop the EQ-5D questionnaire, used 196 members of the general population to validate five existing descriptive systems by surveying lay concepts [14]. The Quality of Well Being drew its items mainly from an existing US Health Interview Survey and Social Security Administration Survey [15], the short form 36 health survey (from which the six-dimensional health state short form is derived) used data from existing instruments [16], and the Assessment of Quality of Life (AQOL) was developed from a literature review from 1970 and interviews and focus groups with 24 clinicians [17]. The HUI2 was developed from a review of epidemiological surveys and a review of the literature, which generated a large pool of potential attributes. A sample of child and parent pairs then rated these items to select attributes for inclusion. The HUI3