

Estimating a preference-based single index from the Overactive Bladder Questionnaire

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Background and Objective

Background: The reimbursement of new interventions is becoming increasingly dependent upon being able to show that the intervention is both clinically efficacious and cost effective. To accomplish this, the quality-adjusted life year (QALY) is typically used, which relies on some preference-based measures of health. However, for Overactive Bladder (OAB), existing generic preference-based measures of health (e.g. EQ-5D) have been shown to be insensitive in OAB patients, whereas none of the existing disease-specific measures is preference-based.

Objective: The objective of this study is to estimate a preference-based single index for estimating QALYs for patients with OAB, based on a valuation survey of the UK general population using the validated Overactive Bladder Questionnaire (OAB-q).

The Overactive Bladder Questionnaire (OABq): The Overactive Bladder Questionnaire (OAB-q) is a 33-item questionnaire that consists of an 8-item "symptom bother scale" and a 25-item "health related quality of life (HRQL) scale" that has 4 sub-scales: coping, concern/worry, sleep and social interaction (Coyne et al, 2002). The questionnaire is used in both continent and incontinent overactive bladder (OAB) patients. Each of the 33 items is assessed on a scale (or level) of 1-6, with 1 for no limitation and 6 for maximal limitation, leading potentially to $6^{33} = 4.7 \times 10^{25}$ different combinations.

Methods

The study applied the methods originally developed in the SF-6D study (Brazier et al, 2002):

Stage 1: Derivation of a health state classification

The objective was to revise the OAB-q into a health state classification amenable to valuation by identifying one or two items from each of the 5 OAB-q domains and reducing levels. Rasch analysis (Rasch 1960; Duncan et al, 2003) provided the main rationale for the selection, supported by a set of conventional psychometric analyses. In addition, factor analysis and cluster analysis were used to identify dimensions. This process resulted in a reduced instrument based on the existing OAB-q, which we refer to as OAB-5D (see table 1).

Stage 2: Valuation survey

The aim of the valuation survey is to elicit preference values from the general public for a sample of health states defined by the OAB-5D. Ninety-eight health states were selected on the basis of a balanced design for valuation. A representative sample of the UK general public were asked to value eight health states each against full health and death using the Time Trade-Off technique. The eight health states included seven intermediate OAB-q health states plus the worst possible state defined by the OAB-5D, or the 'pits' state which was valued by all respondents.

Stage 3: Modelling

The overall aim of modelling is to use regression analysis to predict health state valuations for all health states defined by the OAB-5D from the observations of the valuation survey. The primary model will be an additive specification with the dis_TTO (1-TTO) value as the dependent variable and levels of each dimension entered as a dummy variable using OLS estimation. The modelling will be examined at both individual level and mean level. One-way error components random effects modelling with maximum likelihood estimation (MLE) was also undertaken to allow for between respondent variations. Models were compared and selected using a set of criteria: overall fit of the model, expected sign of the regression coefficients, appropriate ranking of coefficients within a dimension, statistical significance of coefficients, and predictive ability.

Table 1 OAB-5D

URGE

- 1 Not at all bothered by an uncomfortable urge to urinate
- 2 Bothered by an uncomfortable urge to urinate a little bit or somewhat
- 3 Bothered by an uncomfortable urge to urinate quite a bit
- 4 Bothered by an uncomfortable urge to urinate a great deal
- 5 Bothered by an uncomfortable urge to urinate a very great deal

URINE LOSS

- 1 Not at all bothered by urine loss associated with a strong desire to urinate
- 2 Bothered by urine loss associated with a strong desire to urinate a little bit or somewhat
- 3 Bothered by urine loss associated with a strong desire to urinate quite a bit
- 4 Bothered by urine loss associated with a strong desire to urinate a great deal
- 5 Bothered by urine loss associated with a strong desire to urinate a very great deal

SLEEP

- 1 Bladder symptoms interfered with your ability to get a good night's rest none of the time
- 2 Bladder symptoms interfered with your ability to get a good night's rest a little of the time
- 3 Bladder symptoms interfered with your ability to get a good night's rest some of the time
- 4 Bladder symptoms interfered with your ability to get a good night's rest a good bit or most of the time
- 5 Bladder symptoms interfered with your ability to get a good night's rest all of the time

COPING

- 1 Bladder symptoms caused you to plan 'escape routes' to restrooms in public places none of the time
- 2 Bladder symptoms caused you to plan 'escape routes' to restrooms in public places a little of the time
- 3 Bladder symptoms caused you to plan 'escape routes' to restrooms in public places some of the time
- 4 Bladder symptoms caused you to plan 'escape routes' to restrooms in public places a good bit or most of the time
- 5 Bladder symptoms caused you to plan 'escape routes' to restrooms in public places all of the time

CONCERN

- 1 Bladder symptoms caused you embarrassment none of the time
- 2 Bladder symptoms caused you embarrassment a little of the time
- 3 Bladder symptoms caused you embarrassment some of the time
- 4 Bladder symptoms caused you embarrassment a good bit or most of the time
- 5 Bladder symptoms caused you embarrassment all of the time

Results

A sample of 312 members of the public in South Yorkshire UK was interviewed (response rate 26.7%). One respondent was excluded due to ambiguities in responses. In all there were 2487 health state valuations generated by 311 responders. Mean health state values range from -0.88 to 1.00 with standard deviation around 0.2 to 0.4. Distributions of valuations for different health states at the individual respondent level reveal considerable negative skew, with most health state values above 0.6.

The basic mean model and random effects (RE) model for predicting health state values from the OAB-q are shown in Table 2. The coefficients on the dimension levels broadly support the ordinality of the OAB-q health state classification. The RE model performs better than the mean model in terms of inconsistency of coefficients within levels in OAB dimensions. On the other hand, the mean model does better than the RE model in prediction with smaller MAE (0.044), less number of errors greater than 0.05 (39) and less number of errors greater than 0.10 (8). Given that the primary purpose of modelling is prediction, the mean model has been chosen as the best model.

Conclusion

On the basis of the best model chosen, a preference-based scoring algorithm can be established for calculating QALYs using OAB-q data. This will permit the cost-effectiveness of new interventions to be assessed in patients with OAB using a condition-specific instrument.

Table 2 Regression models

	Mean model	Random effects model
Urge2	0.034	0.034
Urge3	0.068	0.031
Urge4	0.081	0.068
Urge5	0.076	0.084
Urineloss2	0.032	0.020
Urineloss3	0.036	0.049
Urineloss4	0.047	0.031
Urineloss5	0.044	0.047
Sleep2	0.028	0.028
Sleep3	0.020	0.019
Sleep4	0.049	0.056
Sleep5	0.050	0.052
Coping2	0.032	0.008
Coping3	0.073	0.021
Coping4	0.046	0.022
Coping5	0.081	0.066
Concern2	0.022	0.033
Concern3	0.047	0.045
Concern4	0.110	0.088
Concern5	0.139	0.136
N	99	2487
Inconsistency	7	4
MAE	0.044	0.076
No> 0.05	39	57
No> 0.10	8	27

Independent variable: $dis_TTO = 1 - TTO$
 Estimates shown in **bold** are significant at the 0.05 level
 MAE: Mean Absolute Error
 No>|0.05|: number of errors greater than 0.05 in absolute value
 No>|0.10|: number of errors greater than 0.10 in absolute value

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