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Development of the EXACT-U: A Preference-Based Measure to Report COPD Exacerbation Utilities

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A B S T R A C T

Background: The exacerbations of chronic obstructive pulmonary disease tool (EXACT) is a condition-specific daily diary recently developed to evaluate the frequency, severity, and duration of chronic obstructive pulmonary disease (COPD) exacerbations. A preference-based algorithm for the EXACT would allow utilities to be reported from patients during an exacerbation when EQ-5D data are not available. **Objective:** To develop the exacerbations of chronic obstructive pulmonary disease tool-utility (EXACT-U), a condition-specific preference-based measure to report utilities from the EXACT for use in cost-effectiveness studies. **Methods:** Five items with three to five levels comprise the EXACT-U. Two groups of health states and respondents were constructed to allow for model development (Development group) and predictive validity testing (Validation group) using independent samples. Members of the UK general public each valued 11 randomized health states using time trade-offs (TTOs) scaled from full health/dead with 10-year durations. Regression models estimated from the Development group using individual data, mean data, and panel designs. Models assessed by number of inconsistent coefficients estimated and R^2 and tested against observed utilities from the Validation group using mean absolute error (MAE) and root mean squared error (RMSE). **Results:** A total of 55 health states, including the best and worst states, were valued in TTO interviews conducted with 400 respondents. Ten models were developed. The final preferred model contained no logical inconsistencies and found MAE = 0.04 and RMSE = 0.05 with a predicted utility range from 0.09 to 0.95. **Conclusions:** The EXACT-U is a condition-specific preference-based measure with strong predictive validity to report daily utilities during an exacerbation.
Keywords: chronic bronchitis, condition-specific, COPD, emphysema, preference, time-trade-off, utility.

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Introduction

Generic preference-based measures, also called multi-attribute utility measures (MAUs) for their multi-dimensional composition, evaluate health-related quality of life (HRQL) across a number of dimensions [1]. These instruments are designed to apply to a wide range of conditions. In order to standardize utility measurement and reporting for health technology assessments, the EuroQol five-dimensional (EQ-5D) questionnaire (EQ-5D) was selected as the preferred utility instrument for UK cost-effectiveness studies [2].

The extent to which an MAU reflects the full experience of a condition, such as chronic obstructive pulmonary disease (COPD), depends on the accuracy of the health state descriptions and the range of dimensions addressed. Changes in health states may not be accurately measured if the condition affects dimensions not captured by the MAU. The sensitivity of the EQ-5D to change associated with effective interventions has been questioned in several disease areas, including urinary incontinence, visual acuity loss, and heart failure, and, more relevantly, in measurement of COPD exacerbations [3–7].

COPD exacerbations have been documented to involve symptoms such as cough, sputum production, chest discomfort/tightness, and breathlessness, as well as systemic manifestations including disruption of sleep, psychological distress, fatigue, and activity limitation [8]. Generic MAUs may not adequately capture the impact on a patient's health-related quality of life. A number of studies have documented the limited responsiveness of the EQ-5D in this population following pharmacologic treatment [6,7,9,10].

If the EQ-5D is unable to detect clinically relevant treatment change for COPD exacerbations, or when EQ-5D data are not collected during an exacerbation, it would be advantageous to have valid, alternate patient-reported utility data for exacerbations. Utilities reported from a condition-specific measure (CSM) have the potential to maintain sensitivity to patient change and differences in severity levels, by evaluating domains that are relevant to the condition.

CSMs can be used to report utilities once an appropriate preference-based scoring algorithm is developed [11,12]. Generally, a subset of items from the CSM is identified using traditional psychometric methods such as factor analysis and item response theory for best performance and good scale coverage. The selected items comprise a reduced version for valuation [13–16]. In order to encourage comparisons with generic MAUs such as the EQ-5D, the Measurement and Valuation of Health protocol has been followed for standardized utility valuations [14–17]. In turn, these CSMs then have the ability to

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report utilities, possibly with stronger performance properties, for economic evaluations [18].

The exacerbation of chronic obstructive pulmonary disease tool (EXACT) is a validated daily diary that measures the frequency, severity, and duration of an exacerbation [19,20]. To date, there is no other standardized measure for assessing the severity of COPD exacerbation symptoms and quantifying the magnitude of change over time. COPD-specific measures such as the St George's Respiratory Questionnaire and the Chronic Respiratory Questionnaire are not designed as daily measures and have demonstrated limited sensitivity to changes experienced during an exacerbation [9,21]. The EXACT diary has demonstrated good reliability, validity, and responsiveness to COPD exacerbations and is now included in a number of intervention trials [22]. A preference-based algorithm derived from the EXACT could report utilities for each day during the onset, height, and recovery stage of an exacerbation. These values could be used instead of EQ-5D values as a more sensitive measure of COPD exacerbations or where the EQ-5D was not administered.

The objective of this study was to develop a condition-specific preference-based algorithm, the exacerbations of chronic obstructive pulmonary disease tool-utility (EXACT-U), to report utilities from the EXACT for use in cost-effectiveness studies.

Methods

This study was conducted in five stages, using methods previously established in the conversion of the SF-36 to the SF-6D [23]. The first stage was to identify a subset of items and levels from the original instrument that maintains the factor structure and severity range for the preference-based instrument. The second stage was to develop a set of EXACT-U health states to be valued using a preference elicitation technique. The third stage was a valuation survey with the UK general public. The fourth stage was to develop a range of econometric models to predict utilities for health states defined by the new classification system. The fifth stage was to test the predictive properties of the resulting models for final selection. Methodologies for each stage are detailed below.

Item identification

EXACT comprises respiratory and systemic attributes of an exacerbation, including chest discomfort (three items), cough and sputum (two items), shortness of breath (five items), difficulty with mucus, sleep disturbance, psychological state, and weak/tired (copyright restrictions prevent showing the full measure). Change in total score on the EXACT is used to evaluate frequency, severity, and duration of exacerbation events. Items are scored from 1 (not at all) to 5 (extremely), where higher values represent greater exacerbation severity.

With 14 items and five levels for each item, the EXACT is capable of identifying over six billion health states. Identification of a subset of EXACT items that maintain the instrument's severity range would decrease the number of health states that need to be valued thus facilitating data collection [23]. To identify the subset of items to include in the EXACT-U, a mixed-method approach was used involving classical test theory and Rasch analysis [16,24,25]. Patients, clinical experts, and the developers reviewed the item selection for content and face validity prior to finalization. Item identification and content validation stages are reported separately; this article focuses on the methods for health state valuation and model development [26].

The EXACT-U comprises five items: chest discomfort; cough, shortness of breath with activity; psychological state; and weak/tired. Response options range from not at all to extremely, with three to five levels each. An example of EXACT-U Health State 44331 includes the following:

- Frequently coughing
- Extreme chest discomfort
- Breathless during light activity
- Moderately weak or tired
- Not scared or worried at all about your lung problems

Health state development

Health states are identified by stating the attribute levels in the order they appear, for example 44331 for the state describing level 4 on the first two attributes, level 3 on the next two attributes, and level 1 on the last attribute.

Two sets of health states were assembled: the Development group to derive the algorithm; and the Validation group to test predictive validity. The use of different health states and different respondents provides a more robust evaluation of predictive validity [27]. Both groups included the best and worst states for comparative purposes.

There is currently no consensus regarding the best method to identify health states or sample sizes for valuation [14]. For this study, health states were created using empirically based methods where possible, based on published research and available data. The number was limited by the number of observations needed for each and the funding available for total interviewees. A minimum of 50 observations per health state were sought for the Development group, based on previous research supporting this threshold for adequate health state differentiation [23,27]. For the Validation group, 40 observations per health states was deemed adequate since the group will be used to compare against predicted utilities rather than for development.

Development group health states were derived using two methods for maximum coverage and diversity. The first set included health states derived using the method common to most other statistical inference studies, which employs the orthogonal plan to estimate main effects. The orthogonal array of health states identified dimension levels that have an equal chance of being combined with all levels of the other dimensions using a statistical program. This resulted in 24 health states being developed for valuation. It was believed that more than 24 health states could be included for model development; therefore some health states reported by patients were also included. The second method conducted secondary analyses on previously collected patient data that included the EXACT [22]. A patient data set for the EXACT was examined and patients were stratified by stable COPD and exacerbation severity. Fifteen health states were selected on the basis of the frequency of occurrence. Without patient data, health states may not represent the actual experiences of patients and only non-occurring health states would be valued, potentially resulting in high error between predicted and actual utilities [28]. The total number of 39 states, not including the best and worst states, was limited by resources and a need to ensure an adequate number of observations per health state.

Validation group health states were derived using the Rasch item threshold map which demonstrates the severity range of the instrument as a whole, with increasing severity levels corresponding with item level changes (e.g., 11111 to 12111, for the next health state as severity increases). This method of health state selection has been used previously, with results suggesting there is a good match to patient-reported health states that are based on natural occurrences of COPD exacerbation states [16]. The map provides a graphical representation of the response option ordering for each item, and the most likely corresponding location on the severity scale. By moving from left to right across the item threshold map, health states are identified for valuation. This method also provides health states that are ordered by increasing severity, allowing for inconsistent utilities to be more easily detected. The number of health states was limited by the Rasch out-

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