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Valuing Treatments for Parkinson Disease Incorporating Process Utility: Performance of Best-Worst Scaling, Time Trade-Off, and Visual Analogue Scales

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ABSTRACT

Objective: The objective of this study was to compare treatment profiles including both health outcomes and process characteristics in Parkinson disease using best-worst scaling (BWS), time trade-off (TTO), and visual analogue scales (VAS). **Methods:** From the model comprising of seven attributes with three levels, six unique profiles were selected representing process-related factors and health outcomes in Parkinson disease. A Web-based survey (N = 613) was conducted in a general population to estimate process-related utilities using profile-based BWS (case 2), multiprofile-based BWS (case 3), TTO, and VAS. The rank order of the six profiles was compared, convergent validity among methods was assessed, and individual analysis focused on the differentiation between pairs of profiles with methods used. **Results:** The aggregated health-state utilities for the six treatment profiles were highly comparable for all methods and no rank reversals were identified. On the individual level, the convergent validity between all methods was strong; however, respondents differentiated less in the utility of closely related treatment profiles with a VAS or TTO than with BWS. For TTO and VAS, this resulted in nonsignificant differences in mean utilities for closely related treatment profiles. **Conclusions:** This study suggests that all methods are equally able to measure process-related utility when the aim is to estimate the overall value of treatments. On an individual level, such as in shared decision making, BWS allows for better prioritization of treatment alternatives, especially if they are closely related. The decision-making problem and the need for explicit trade-off between attributes should determine the choice for a method.

Keywords: health-state utility, Parkinson disease, preference, process utility.

Introduction

Patients with chronic diseases are confronted with reduced quality of life and decreased length of life while the management of chronic disease results in high costs for society [1]. Management of chronic disease is generally aimed at symptom control and improving daily functioning, thus limiting the effects of living with a chronic disease and preventing further functional loss [2].

It is generally accepted that optimizing the process of care in patients with chronic conditions is of paramount importance [3,4]. However, because of the chronicity, current approaches to valuing outcomes may not be sensitive nor do they capture all value components. First, effective disease management requires adherence to guidelines and treatments prescribed. However, treatment adherence is reported to be moderate in many patient groups suffering from chronic diseases [5]. This can be explained because the treatment primarily has a preventive purpose, whereas negative consequences such as adverse events are experienced immediately. Second, the ease of use or convenience while participating in a disease management program generally is not captured, although these elements constitute a value relevant to patients. Therefore, recent studies recommend that the valuation space should include a broader range of patients' experiences such as process of care factors and factors that relate to enabling individuals to the best they can be [6,7].

Even though many people advocate widening the evaluation space, the traditional health economist view does not consider that factors such as the process of receiving care and valuation are restricted to health outcomes alone [8,9]. In this view, the process of care has value only because it is a commodity that can be exchanged to derive health gains [10]. Thus, lack of treatment adherence or a less desirable process of care will be reflected in the health outcomes achieved and do not require explicit valuation.

Despite these different views, the present article assumes that process-related attributes do affect the value of a treatment independent from health outcomes achieved. Hence, to determine the value of a treatment in chronic disease it is important to go beyond health outcomes and include attributes such as the ease of use and process of care.

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Standard gamble (SG), time trade-off (TTO), and visual analogue scales (VAS) are common methods to value health outcomes. SG is mostly used in economic evaluation studies and clinical decision analyses. SG is considered to be the "criterion standard" for utility measurement because it involves making choices under conditions of uncertainty and has rigorous foundations in expected utility theory [11]. Alternatively, TTO forces trade-offs between length of life and quality of life under conditions of certainty. TTO is well known for being the standard approach to derive health utility weights for EuroQol five-dimensional questionnaire (EQ-5D) health states (EQ-5D tariff) [12,13]. A VAS asks respondents to rate health states on a scale anchored at "worst imaginable health state" and "best imaginable health state" under conditions of certainty. They are easy and are often used in combination with the EQ-5D [14,15].

Although TTO, SG, and VAS can be used to capture process-related utility in addition to health outcomes, they are not frequently used for that purpose. A review by Brennan and Dixon [4] concluded that direct valuation methods SG and TTO have been used in only 13 studies to assess process-related utility.

Another approach being used to estimate process utility is discrete choice experiment (DCE) and best-worst scaling (BWS) methods [16,17]. In BWS, respondents are asked to choose the best and the worst from a number of options. Essentially, respondents are asked to choose the pair that maximizes the difference in value (on the latent utility scale) between them. BWS case 3 (BWS-3)—which is comparable to a standard DCE choice set—asks respondents to choose the best and the worst from a number of alternative treatment options, which can be composed from multiple criteria. Alternatively, BWS case 2 (BWS-2) lets respondents consider one treatment at a time, asking for the best and worst characteristics within a profile. The indirect valuations are used to estimate the utility of multiple criteria.

DCE and BWS are more often used to explicitly value process factors and nonhealth outcomes than are methods such as VAS and TTO [4,6,18]. Their popularity might be explained by the lower degree of abstract reasoning required to answer a task of DCE and BWS and its methodological rooting in random utility theory [16,18–22]. However, DCE and BWS have outcomes on a latent scale, which means that no reference can be made to the health utility scale (0–1), which is favored in health technology assessment. The potential of BWS and DCE to estimate health utilities from part-worth utilities using anchoring and mapping techniques is currently being studied [20,23–25].

One study was found that compared VAS, TTO, and DCE using vignettes depicting moderate-risk pregnancy at term (including process factors). The authors concluded that DCE was superior to TTO and performed equal to VAS with regard to validity and reliability and that DCE had slightly higher user feasibility [26]. However, because of a lack of head-to-head comparisons, it is unclear which methods differentiate best between health states.

The objective of this article was to compare the health-state utilities incorporating a process-related attribute for treatment profiles in Parkinson disease (PD) using TTO, VAS, and BWS-2 and BWS-3. Several unique treatment profiles were identified in which the process and outcomes of the treatment differed. A head-to-head comparison of aggregated utility scores and the comparison of individual scores were included. Because individuals used all the methods to evaluate treatment profiles, we had the possibility to conduct a within-person comparison analysis to study whether individuals were able to differentiate between the values of treatment profiles with all methods. PD was chosen because of the specific problems in managing the disease over a longer time and the different treatment modalities that are used during this time. Patients on drug treatment eventually may undergo neurosurgery or will receive their medication through pump infusion. Such treatments differ from pharmacological therapy in its process because they require surgery, daily cleaning routines, and use of mechanical equipment.

Methods

BWS Experiments

Relevant attributes of care were identified through literature review and qualitative interviews with 15 patients with PD. Seven attributes were selected: process of care, resting tremor, posture and balance problems, slowness of movement, dizziness, drowsiness, and dyskinesia. Symptoms and adverse effects were assigned the same level scale values (from "seldom to never" to "sometimes" or "often suffer from"). The "process of care" attribute was described as the oral intake of tablets, continuous pump infusion of medication, and neurosurgery. In the BWS-2 experiment, respondents were asked to select the aspects of treatment that they perceived as the most and the least preferable within one single treatment profile. For the BWS-3 experiment, respondents were asked to select the most and the least desirable treatments from three treatment profiles. It was not feasible to provide respondents with a full factorial design: 2187 (3^7) possible profiles. Experimental-design software from Sawtooth Software was used to generate a D-efficient design and four blocks [27,28]. In total, each respondent answered nine profile tasks for BWS-2 and 10 choice tasks for BWS-3. Appendix 1 in Supplemental Materials found at http://dx.doi.org/10.1016/j.jval.2015.11.011 displays examples of a BWS-2 task and a BWS-3 task.

Treatment Profile Valuations

Six hypothetical core treatment profiles were defined: both extreme profiles and four intermediate (closely related) treatment profiles (Table 1). A full variation of the process attribute was not included in the six core scenarios because we also wanted to test the effect of small changes in health outcomes on utility scores. All respondents valued both the best and worst treatment profiles and were randomly assigned to value two of the four intermediate profiles with both a TTO and a VAS. The end points of the VAS were labeled as "best imaginable treatment" and "worst imaginable treatment."

A computer-based TTO was executed to determine how many years of life lived in perfect health followed by death would be equivalent to 10 years of life lived in the particular PD treatment profile followed by death.

Respondents were asked to put themselves in the hypothetical scenarios and were asked whether they were willing to give up any life-years to return to full health again. If they did, a second question determined whether the respondent was willing to give up 5 life-years. This was followed by an iteration procedure in which the number of life-years was increased or decreased until the point of indifference was reached.

Sampling

A Web-based survey was conducted in June 2013 to measure treatment preferences for PD management in the United Kingdom and the Netherlands. The respondent sample consisted of members of the general population. Survey Sampling International (Rotterdam, the Netherlands) recruited 613 respondents (aged 18–65 years) from large panels, which have gone through rigorous quality controls. The sample size was based on a rule of thumb for conjoint analysis: estimate precision increases quickly at sample sizes of less than 150 and flattens out at ±300 observations [27]. Sample size calculation for TTO and VAS was not applicable, because no expectations were set regarding the desired difference in utility scores between treatment profiles.