Mind the gap : explanations for the differences in utilities between respondent groups

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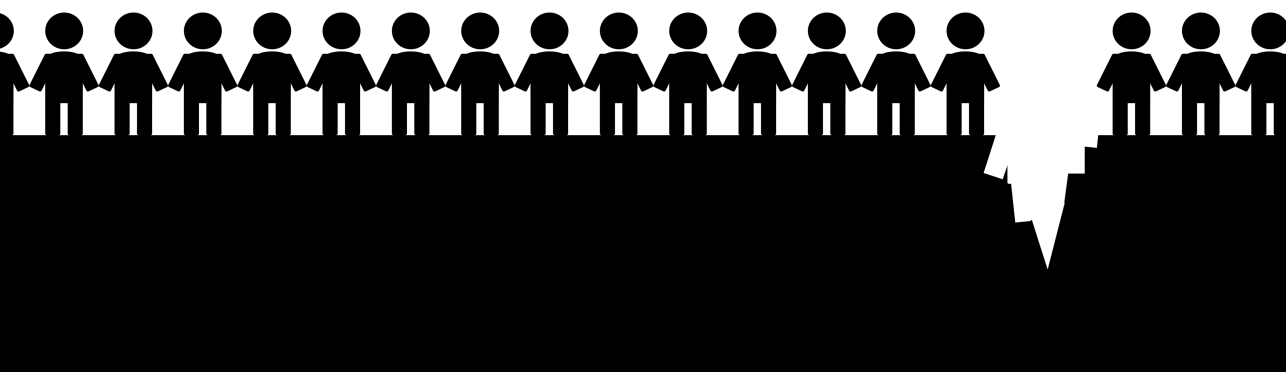
General Introduction



1.1 Cost-Utility analyses

In medical care resources are scarce and choices have to be made about how these resources are to be distributed. For example, should we vaccinate all Dutch students against mumps[1] or should we introduce cartilage transplant for patients with rheumatoid arthritis,[2] or maybe both? To judge the optimal allocation of medical resources, systematic economic evaluations are needed, comparing costs with the benefits of health services.[3] For these economic evaluations different techniques can be used, among which cost-utility analysis. Cost-utility analysis compares the costs of treatment to the outcomes obtained.

In cost-utility analysis preferences for a certain set of outcomes are measured using health state utilities. Health state utilities are strongly related to health related quality of life (HRQL) but they differ in that health state utilities measure both quality of life and the valuation of this quality of life compared to perfect health and death.[4] Health state utilities are values between 0 and 1 that represent individuals' preferences for health states. Preferences are elicited using different methods such as the Standard Gamble (SG), the Time-Trade-Off (TTO), and the Visual Analogue Scale (VAS).[5]

In the SG participants are asked to choose between a certain outcome, the health state to be valued, or a gamble with a probability (p) of receiving the best possible outcome, perfect health, and a probability $(1-p)$ of receiving the worst possible outcome, usually death. By varying p, the indifference point is searched, the probability at which the participant is not able to choose between the gamble and the sure outcome. The value obtained is the utility for the health state under valuation ($\mu = ((p \cdot 1) + ((1-p) \cdot 0) = p)$). In the TTO participants are asked to choose between a number of years living in the health state to be valued or living a shorter period of time in perfect health. The time in perfect health is varied to obtain an indifference point, the number of years in perfect health equal to a higher number of years in the health state to be valued. The health state utility is calculated by $\frac{\sharp \textit{ years in perfect health}}{\sharp \textit{ years in health state to be valued}}$[a]. In the VAS, participants are asked to give a valuation for the health state to be valued by placing a mark on a 100 mm. horizontal line ranging from perfect health to the worst possible outcome, usually death. The health state utility is the number of mm. from the death anchor divided by 100.

Which method should be used when eliciting health state utilities has been

[a] For states worse than death slightly different procedure is used. But in this thesis only the described procedure is used.

topic of debate.[4] Initially the SG had a reputation of being the gold standard since it meets the axioms, as described by Neumann and Morgenstern, of expected utility theory.[6] Nowadays the feasibility and validity of the SG is questioned. Participants experience the SG as a complex method,[7] and answers elicited by the SG are vulnerable to probability weighting.[8,9] The TTO on the other hand, is simpler to elicit, is not vulnerable to probability weighting, and appears to have good face validity.[4,7] Nevertheless the TTO is vulnerable to other biases, but these biases probably cancel one another out. The bias of the utility curvature which is downwards makes up for the upward bias cause by loss aversion and scale compatibility.[9] Furthermore, the time-line used in the TTO gives a good representation of decisions made in clinical settings.[10] The VAS is often used because of its feasibility, it is easy to elicit but its construct validity has been questioned.[7] Given the above reasons the TTO is now the most often used method to elicit health state utilities.

Health state utilities can be elicited directly, by asking patients or members of the public to give valuations to health states, or with indirect instruments. In studies using indirect utility instruments, health state utilities of members of the general public are based on patients' answers to a short descriptive questionnaire. These answers are fed into a model estimated from an earlier study,[11] which generates the utility values of the general public. The EQ-5D-tariff is such an indirect instrument that is widely used. The EQ–5D consists of five dimensions: mobility, self-care, usual activities, pain/discomfort, and anxiety/depression. Each dimension is described according to one of three levels of severity: no problems(1), some problems (2) and extreme problems (3). In total the EQ-5D can thus create 243(3^5) theoretically possible health state descriptions. A selection of these health state descriptions has been valued by a large sample of members of the public, and based on these valuations a model has been estimated from which utilities for each of the 243 descriptions can be generated.[11]

Quality-Adjusted Life-Years (QALY) can be computed based on these elicited health state utilities. QALYs define the overall utility for a certain time path or life expectancy. To explain the concept of QALY, I revert to the cartilage transplant as illustration. When patients with RA receive cartilage transplant their utility might deteriorate initially (due to surgery) but will reestablish over time (assuming that this treatment will give no long term side effects). For example, a patient with a life expectancy of 30 years might initially have a health state utility of 0.6. Due to surgery this utility will deteriorate to 0.5 for a year, however after this year it will increase to a health state utility of 0.8 which will remain for the next 29 years.

The QALY is then $(1 \cdot 0.5) + (29 \cdot 0.8) = 23.7$. The utility of patients who do not receive the transplant will not deteriorate initially (they do not have to recover from surgery), but over a longer period of time these patients will continue having pain complaints affecting their utility. For example a patient with a life expectancy of 30 years will continue to have a health state utility of 0.6 for 30 years long; leading to a QALY of $(30 \cdot 0.6) = 18$. Gain in QALYs from transplant is computed by comparing the QALYs of patients with transplant compared to the QALYs of patients without transplant. In cost-utility analyses this gain in QALYs will be compared with the costs that have to be made, resulting in cost per QALY gained.

1.2 Public or patients' preferences

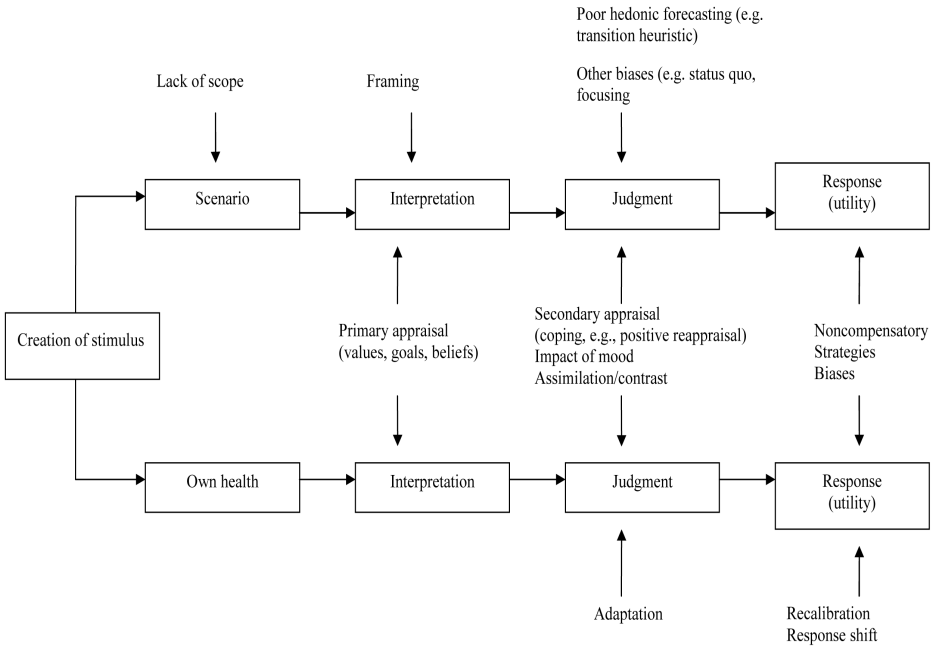
Cost-utility analyses for allocation decisions are recommended to be made from the societal perspective. This implies that health state utilities should be elicited from members of the public. Since cost-utility analyses should lead to a just allocation of resources these analyses should not only be based on the opinion of those who gain health but also on that of those who pay for it.[12] Organizations involved in developing guidelines on the use of new and existing treatments, such as the National Institute for Health and Clinical Excellence (NICE), the panel of the U.S. Public Health Service, and the Dutch Health Care Insurance Board (CvZ), advise the use of the societal perspective, in which health state utilities elicited from a fully informed representative sample of members of the public are preferred.[12–14] However it might be challenging to fully inform members of the public. Instead, health state utilities of patients might be informative given that certainly patients experiencing an illness are well-informed.[12,15] The panel of the U.S. Public Health Service already suggested that in cost-utility analyses in which alternative interventions are compared patient preferences might be the better choice.[12]

Whose' preferences are used in cost-utility analyses does matter. Preferences of members of the public are often found to be lower than patients' preferences.[16] Several explanations for this gap in health state valuations between patients and public have been provided by research from different fields.[17–19] To make more evidence-justified suggestions about whose preferences to use, the mechanisms underlying this gap in health state utilities have to be understood.[15] Whose valuations are most valid depends on the explanations for this gap. Is this gap caused by errors in the method used, or rather due to cognitive mechanisms?[18]

1.3 Mechanisms underlying the gap between patients and public

Stiggelbout and de Vogel-Voogt[15] systematically described mechanisms that might cause the gap between utilities given by patients and members of the public, by using stimulus response models (Figure 1.1). This resulted in a framework in

Figure 1.1 Framework of mechanisms underlying the gap by Stiggelbout & Vogel-Voogt[15]



which the different valuation processes of patients and members of the public are presented simultaneously. A short description of the mechanisms provided in their framework, and of mechanisms mentioned by other researchers is provided below. The outline of the mechanisms described here is not intended to be conclusive. By combining information from different research fields one can always come up with additional mechanisms that are more or less related to the ones described below.

Lack of Scope When eliciting health state utilities patients are generally asked to value their own health of the previous week, whereas members of the public have

to value a health state based on a description. Health state descriptions can be developed by researches based on experience of physicians or patients,[20] or they can be based on classification systems, such as the Health Utility Index (HUI)[21] or the EQ-5D.[22] A lack of correspondence between health state descriptions and the actual experience of a health state might cause the gap in health state valuations between patients and public.[23] Insinga and Fryback[23] found that participants gave different health state valuations for their own experienced health than for an EQ-5D health state description of their own health. Possibly the EQ-5D health state description is too sparse. Jansen et al.[20] found similar results in a sample of patients undergoing radiation therapy. The own experienced health, while being treated with radiation therapy, was valued higher than the health state description of this radiation therapy.

Framing Framing of the health state description influences how a health state is interpreted. Most health state descriptions tend to include only limitations and handicaps caused by the health state. Due to this negative framing, members of the public focus on the limitations of a health state whereas patients might also think about positive aspects in their lives.[24]

Focusing illusion The fact that members of the public focuses on limitations is probably not only caused by the negative framing of health state descriptions. People have a natural tendency to focus on the difference between their current situation and an imaginary situation; they will overestimate the differences and overlook the similarities.[25] This focusing illusion has e.g. been demonstrated among assistant professors who were asked to imagine that they would not achieve tenure[26] and among football fans.[27] However, among members of the public imagining a disability no evidence was found for this focusing illusion.[24]

Status Quo Bias Status quo bias indicates that people value goods more highly when they own them. Evidence for status quo bias has been shown previously. Participants who were randomly assigned to a car would not part with this car, even if they were given the opportunity to choose a different car without penalty.[28] In medical decision making evidence for this status quo bias has also been found. Salkeld et al.[29] studied preferences for a bowel cancer prevention test. On average patients were willing to pay more for a test that they had used before (status quo) instead of starting to use a new test. Both tests were equal on all attributes. Regarding the gap in health state valuations status quo bias might cause patients to give higher valuations. Patients valuing their own life are probably less willing to trade-off own life years than members of the public valuing a hypothetical health state.[15]

Loss Aversion Related to status quo bias is loss aversion. People evaluate

outcomes as gains and losses and are more sensitive to losses than to gains. For instance people value the loss of €10,- worse than the gain of the same amount of money. The difference between losses and gains in health state valuations depends on the reference point.[30] In patients whose reference point is their own health, the loss of life years that a patient has to trade will get more weight than the health that is gained leading to an upward bias.[9,15] Further, for patients trading of life years or increasing risk of dying, it might feel as tempting fate. Members of the public are probably less concerned about trading life years or increasing risk of dying since the situation remains hypothetical.

Adaptation Adaptation can be defined as a response that diminishes or remains constant over time despite and increase in the stimulus.[31] When confronted with adverse circumstances such as an illness people tend to adapt peculiarly well.[31] Therefore adaptation is often suggested to explain the patients' relative high reported quality of life.[32–36] Adaptation takes place on physical and psychological level.[31] Physically patients learn to handle handicaps and mentally they learn to deal with the illness. Different processes are suggested to initiate psychological adaptation, among which coping strategies and benefit finding. When providing health state valuations patients will include their ability to adapt whereas members of the public may fail to anticipate on this ability to adapt.[37] Members of the public have the tendency to overpredict the duration of emotional reactions to future events[27] and underestimate their cognitive mechanisms which alleviate this reaction.[26] Tentative support has been found for this failure to anticipate on adaptation. Members of the public who were made aware of their ability to adapt gave higher valuations on a person trade-off (PTO) and on a VAS measuring quality of life,[24,38] but not on the TTO and SG.[39]

Valuation shift Dolan[40] suggested that experiencing poor health might result in higher valuations of other hypothetical health states, a process they called valuation shift. Dolan showed that participants in poor health assigned higher valuations to various EQ-5D scenarios than did participants in good health. Scale recalibration To measure health state utilities, subjective scales are used, which are susceptible to different interpretations between people, but more importantly within people.[37] When people experience illness they might change their internal standards, leading to a change in interpretation of these scales.[41] For instance a patient with RA who first valued her joint pains as 8 on a VAS scale ranging from 0 (no pain) to 10 (major pain), recalibrated her pain to a 5 after experiencing kidney stones. The pain she experienced due to kidney stones was significantly more intense than any pain she

had ever experienced before, resulting in a recalibration of her valuation of major pain.

Implicit theories of stability and change Implicit theories of stability and change are heuristics that people use to recall emotions. To recall emotions, people first note their present status and then decide if their status has changed over time. This reconstruction of emotions is guided by theories that include specific beliefs regarding the inherent stability of an attribute.[42] For instance people have the implicit belief that they will become happier over time. When people are asked to give an estimation of their previous happiness, they assume that they had been less happy than they are now.[43] Depending on the method used to investigate health state utilities, implicit theories might cause bias. Such may be the case in the increasingly popular method of asking patients to recall how their health state has changed over time.[37]

1.4 Objective and outline of the thesis

Several mechanisms have been suggested to cause the gap between valuations given by patients and members of the public, of which a number have been examined empirically. However still no conclusive suggestions can be made, and more research is necessary to enhance our knowledge. Although adaptation is often mentioned it has never been tested empirically. Other mechanisms have only been studied among members of the public and not among patients, such as focusing illusion, or the reverse, lack of scope. The overall objective of this thesis is to further examine some of the mechanisms proposed to cause the gap between health state valuations, in order to gain insight in the relative validity of health state utilities of patients and of members of the public.

In Chapter 2 first a meta-analytical comparison of health state valuations of patients and members of the public is presented. Previously, studies described contrasting findings[16,44] about the difference in health state valuations between patients and members of the public. The aim of our study was to investigate the influence of respondent group on health state valuations. Post hoc, other design-effects were tentatively studied using moderator analyses.

In Chapters 3 through 8 mechanisms potentially underlying the difference between patients and members of the public were studied. Chapter 3 starts with the influence of lack of scope and framing of a health state description. Patients with RA valued their own experienced health, an EQ-5D description of their own health, and

an enriched EQ-5D description of their own health. These valuations were compared to investigate the influence of differences in health state descriptions. Next, in Chapter 4 the effects of focusing illusion and adaptation were examined, as well as the sparseness of the EQ-5D description (lack of scope). In this study open-ended questions were used to assess aspects important to patients with RA and to members of the public imagining having RA. In Chapter 5 the effect of lack of scope and framing was investigated further. Here the effect of a health state description was not only investigated among patients, but also among partners of patients and among members of the public. All participants valued their own imagined/experienced health state, a standard EQ-5D description of this health state, and an enriched EQ-5D description of this health state. By comparing the valuations given by partners of patients to the valuations of patients and of members of the public the effect of vicarious experience could also be examined. Chapter 6 describes a cross-sectional study among patients with RA in which the effect of adaptive abilities on health state valuations is examined. Adaptive abilities were based on Cognitive Adaptation Theory (CAT) as suggested by Taylor.[32,45] Chapter 7 further describes adaptation and valuation shift, investigated in a longitudinal study among patients with Spinal Cord Injury (SCI). Health state valuations of patients with recent onset acute SCI were assessed at three points in time. In Chapter 8 the effect of adaptation was also examined. Here the ability to anticipate on adaptation by patients experiencing new adversities as well as the effect of implicit theories of stability and change were studied.

While examining the mechanisms suggested to cause the difference in health state valuations between patients and members of the public we were challenged by often ambiguous descriptions of these mechanisms. Among others we felt that the language used by "response shift" gathers together different terms already existing in the scientific literature. In Chapter 9 the conceptual confusions related to the language of response shift is described.