

Cost–utility analysis and otolaryngology

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Abstract

As providers of health care, we face increasing demand on our limited, indeed diminishing, resources. Economic appraisal of our interventions means assessing the trade-off between effectiveness, efficiency and equity. When rationing becomes inevitable, calculation of utility values is a valuable decision-making tool. This paper reviews objective measures of patient benefit, such as quality of life, and focuses on their application within otolaryngology.

Key words: Utility Theory; Quality Of Life; Cost Analysis; Otolaryngology

Introduction

Within the context of health and health-care provision, ‘utility value’ is used as a measure of the strength of an individual’s preference for a particular health state. It consists of a number between zero and one; zero rates the health state as being equal to death whilst one represents full health.¹ Utility values provide a single, ordinal measure of quality of life which may be used for assessment of health-care interventions.

Utility values are employed in economic evaluation, which is used in decision-making within the health-care sector. The focus of this review is economic evaluation and specifically one method of economic evaluation – cost–utility analysis – which, as its name suggests, employs utility values. Cost–utility analysis is recommended by the National Institute for Health and Care Excellence (NICE) as the optimum method of assessing cost-effectiveness.² The review also explains methods of calculating utility values, and of deriving Quality-Adjusted Life Years (also termed ‘QALYs’) using these utility values.

Pressure on resources, coupled with a desire to maximise the health gains from those resources, means that the requirement to demonstrate the quality of clinical treatments has never been greater. In this review, we outline the importance of utility values in evaluating treatments, and we discuss how they can be applied to clinical ENT practice.

Economic evaluation using utility values

Recent developments in the structure and management of the National Health Service (NHS), combined with financial limitations, mean that, now more than ever,

ENT surgeons need to demonstrate the benefits and quality of their treatments. This is especially true as many treatments do not increase the patient’s life span but, rather, may affect their quality of life. There are three main measures of economic evaluation: cost–benefit analysis, cost-effectiveness analysis and cost–utility analysis.³

Cost–benefit analysis

The first measure, cost–benefit analysis, is concerned with both the benefits arising from an intervention and the cost associated with its provision. It is likely to include longer-term costs and benefits. Both are given a monetary value; if an intervention’s monetary value outweighs its cost, then that intervention is deemed to be worth doing. Cost–benefit analysis can be used to guide budget allocation for different and competing interventions; for example, bone-anchored hearing aids (BAHAs) versus conventional hearing aids.

Cost-effectiveness analysis

Unlike cost–benefit analysis, which gauges the worth of an intervention in monetary terms, cost-effectiveness analysis uses a clinical indicator or validated outcome measure. It enables comparative analysis of the costs and outcomes (i.e. the effectiveness) of competing interventions. Different analyses use a single, uni-dimensional and thus comparable indicator of clinical outcome (for example, a quantified improvement in hearing, enabling comparison of different types of digital hearing aid). Thus, cost-effectiveness analysis is concerned predominantly with technical efficiency.

Cost-utility analysis

This last type of comparative analysis also assesses the costs and effectiveness of competing interventions; however, rather than using a clinical outcome indicator, cost-utility analysis employs Quality-Adjusted Life Years as the outcome of interest. This latter measure reflects both the quantity and quality of life, basing the latter on predetermined utility values. The utility value is multiplied by the number of expected life years to calculate the Quality-Adjusted Life Years value.

Calculation of utility values

In order to calculate Quality-Adjusted Life Years, it is necessary to first determine a utility value reflecting the degree of quality of life associated with a particular health state. In order to place a value on a particular health state, one must define that health state, specify who will assess the health state and determine its utility value, and choose an appropriate method of determination.

Define health state

The health state in question is defined by preparing a vignette describing the typical experience of individuals who have received a particular treatment. Vignettes usually contain five to nine descriptors detailing physical, psychological and/or social effects.⁴ It is essential that vignettes are accurate representations of the health states in question; for this reason, they are prepared both from the research literature and also from the accounts of patients and health-care professionals. The extent to which potential participants are informed about the health state has an effect on the utility value that they assign to it.⁵

Select assessors

The next step is to choose the most appropriate group to assess the health state. This is a contentious area. Members of the public can be recruited in large numbers, and generate utility values which may be true at the population level; however, it is not always valid to apply utility values generated in this way to individual patients.⁶ However, this is the preferred method for valuation of health states by NICE.² Patients who have experienced the health state in question, on the other hand, are experts in that health state and tend to give the most accurate representation. However, people who have experienced a health state tend to rate it higher than do others.⁷ There is therefore disagreement about which population of assessors provides the most accurate assessment of a given health state. The sample number required depends on the expertise of the group and whether they have experienced the health state in question.⁸

Select method

There are various methods for calculating the utility value of a health state, and the method chosen is likely to affect the utility value assigned. Torrance was at the heart of the development of measures of utility in the 1970s.⁹ He collaborated in his early work with Sackett, who became one of the major proponents of evidence-based medicine. Torrance describes three methods for calculating utility: linear health utility scale (i.e. visual analogue scale (VAS)); time trade-off; and standard gamble technique.

The standard gamble tends to calculate higher utility values than the time trade-off method, which in turn calculates higher values than the visual analogue scale method.^{7,10} Models have been derived which attempt to predict how the utility values derived from each method are related, but results are variable.¹¹⁻¹³

Torrance's three methods are described below, together with a fourth option, indirect methods.

Visual analogue scale. The basic format of the VAS is a single line, usually on paper but possibly formatted electronically. The scale usually runs from 0 to either 1, 10 or 100. The higher number represents perfect health whilst 0 represents the worst health state imaginable (death in some scales). The line may have marked units; whether the line truly has interval properties, such that point 50 is twice the quality of point 25, is controversial.¹⁴ Visual analogue scales have been used to rate the improvement associated with cochlear implantation¹⁵ and treatment for benign paroxysmal positional vertigo.¹⁶

Use of the VAS has several advantages. Patients have little difficulty with the concept of a scale, saving cost and research time. The VAS can be effectively completed by postal survey, avoiding the need for face to face training of participants. Studies have shown good reliability when patients who had filled in postal forms were retested in person.¹⁷

However, use of the visual analogue scale requires no 'trade-off' or choice. The rater does not have to justify their line scale scoring based on monetary, time or health factors; rather, they just (possibly arbitrarily) pick a scale point.¹⁸ Context bias may occur when a rater faces several potential conditions to rate. For example, on a scale from 0 to 10, a rater may score quinsy as 7 out of 10 for severity; however, if quinsy is scored in the context of both tonsillitis and (more life-threatening) deep space neck infections, it is likely that the rater will downgrade their quinsy scoring. In addition, when scoring conditions on a linear scale raters may display end-scale aversion, preferring not to use the minimum or maximum scores. Mathematical transformation can correct for both context bias and end-state aversion.¹⁴

Time trade-off. Time trade-off is the commonest method of utility valuation used in the calculation of Quality-Adjusted Life Years.¹⁹ The participant expresses a

preference for one of two alternative scenarios: a specified, diminished health state for a specified time, versus full health for a shorter life span. Using the time trade-off method, a portion of life span is sacrificed in exchange for a specified time of improved health. Mooney³ cites an example: a participant considers the scenario of 20 years of living with the loss of use of both legs, followed by immediate death; they then specify how many years of life they would sacrifice in order to retain full use of both legs. The health state is ascribed a value by dividing the number of years of chosen life span reduction in order to be free of the health state, by the number of proposed years with the health state. Thus, in the example given, if participants would reduce their life expectancy by 15 years in order to avoid living for 20 years with the loss of use of both legs, the value of the health state of loss of use of both legs is valued as $15/20$, i.e. 0.75. If the health state was survival in extreme pain, then the participant would probably sacrifice more years of life span, resulting in a lower health state valuation.¹⁹ In a landmark 1981 study, McNeill *et al.* compared laryngectomy and radiotherapy for advanced laryngeal cancer. Based on expected utility theory, participants were asked how many survival years they would trade-off in order to maintain a near-normal voice with non-surgical management. Results suggested that 20 per cent of healthy controls would opt for non-surgical management.²⁰ In another study, investigators at the Nottingham Medical Research Council used healthy volunteers to estimate the utility of unilateral and bilateral cochlear implants, using the time trade-off method; a greater utility was estimated for bilateral implants.²¹

The time trade-off method has a number of advantages. It involves choice: there is an 'opportunity cost' of extra years of life which the participant states they are willing to sacrifice in order to gain a specified period of improved health. Participants are required to weigh health states against life expectancy, in contrast to the VAS method. The concepts of time and health are usually intellectually accessible to participants. The time trade-off method also balances both quality and quantity, whilst the VAS method is confined to measuring quality.

However, there are a number of limitations to the time trade-off method. Some participants may be unwilling to consider the idea of trading time for health.¹⁹ This may be due to personal, philosophical or religious beliefs, or because they have difficulty with the concept. Others may exhibit a strong time preference and value extra life span over everything else.

In addition, some have challenged whether considering a trade-off between longevity and quality of life reveals a participant's true preference. The time trade-off method is based on consumer theory, which states that items have no true value but are valued depending on what people are prepared to pay.¹⁹ Time trade-off scenarios are generally hypothetical, as there are few

situations where people can actually trade time for health.

It is important to consider that the 'currency' of life years is not consistent through the ageing process. The value individuals place on a year of their life can vary depending on whether this survival is short-term or long-term. Participants may perceive that their health will deteriorate generally with age and may be more prepared to trade years of later life. In a US time trade-off comparison study, head and neck cancer patients and students were asked how much time they would trade-off for relief of the health states which could result from head and neck cancer treatment.²² The younger students were prepared to trade-off significantly more time than the older patients.

Standard gamble. The standard gamble method is generally considered to be the economic 'gold standard' for valuing utilities, as individuals make decisions under conditions of uncertainty but still express preferences.²³ The participant faces a choice between the certainty of staying in a chronic health state for the rest of their life, or the uncertainty of a gamble. This offers them the chance of being healthy for the rest of their life, with a probability of p , or immediate death, with a probability of $1 - p$ (Figure 1). The value of p is varied until a point of indifference is reached. For example, in a British oesophageal cancer study²⁴ participants were offered the choice of remaining with their terminal cancer symptoms, or taking a gamble involving p chance of being symptom-free then dying or $1 - p$ chance of immediate death. The amount of p was varied from 1.0 (i.e. a 100 per cent probability of being symptom-free, with a 0 per cent chance of dying) to 0.1 (i.e. a 10 per cent chance of being symptom-free, with a 90 per cent chance of immediate death) until a point of indifference was met. The p value, derived in this way, represents the utility weighting.

The strength of the standard gamble method is that it combines time, risk and quality; however, this is also a potential weakness. As with the time trade-off method, people who are very time-sensitive may not wish to take a gamble. Risk-averse individuals may be disinclined to take a gamble, leading to a distortion of the result if they dominate the study group. There is an analogy between the risk-averse, for whom the standard gamble method does not work, and the 'zero traders' who cannot participate in the time trade-off method.

Using the standard gamble method is more time-intensive and thus more costly than the VAS method because, like time trade-off, it requires interviews. This necessity is illustrated by Hammerschmidt and colleagues' comparative study of use of the standard gamble by a group of diabetic patients, rating diabetic complications, performed both at interview and by post.²⁵ While the results showed good consistency,

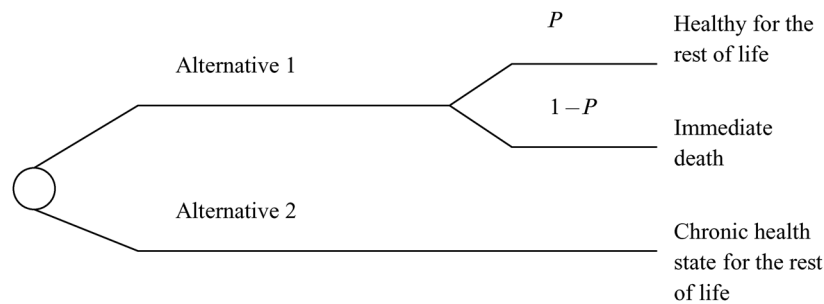


FIG. 1

Diagrammatic representation of a standard gamble for a chronic health state.²⁴

only about two-thirds of the completed postal forms were usable.

Not all standard gambles involve terminal illness (as seen above), so times and outcomes must be very specific. In a study of rhinoconjunctivitis, patients faced a choice of remaining in their current state for 10 years or gambling for a chance of complete symptom relief versus death.²⁶ The standard gamble for this condition did not correlate well with quality of life indices: unsurprisingly, patients were unwilling to accept anything greater than a minimal risk of instant death, for such a transient and seasonal condition. This suggests that the standard gamble method is inappropriate for minor ENT conditions.

Indirect methods. The VAS, standard gamble and time trade-off techniques are considered to be direct methods of obtaining a utility score. Other methods are available which produce an indirect utility rating by using a questionnaire to assess an individual's health condition. These methods are referred to as multi-attribute utility measures, and may be generic or condition-specific. One such measure is the EuroQol 5 Dimension (also termed 'EQ-5D') questionnaire, a generic health-related quality of life measurement tool. The EuroQol 5 Dimension questionnaire is a self-reported questionnaire with five dimensions or domains: mobility, self-care, usual activities, pain and discomfort, and anxiety and depression.²⁷ Each domain has three potential answers, resulting in 243 possible health states (see Appendix 1). Each of these 243 health states carries a utility weighting.²⁸ These weightings or valuations of health states were originally based on 3395 interviews conducted with members of the general public, which used the time trade-off method.

The EuroQol 5 Dimension questionnaire is simple and easy to administer and to complete. It asks respondents to rate their own level of functioning or perceived functioning, choosing one of three descriptors, for each of the five domains. A pre-defined algorithm then assigns a utility value based on the combination of answers given.

However, concerns about the EuroQol 5 Dimension questionnaire have been raised.²⁹ It was developed using a large general population, and the utility values derived were estimated means; smaller groups

of individuals sharing a specific health condition (e.g. tinnitus) may well generate different utility values. It has been calculated that only 60 per cent of the general public would agree with the ordering of some values.²⁹ This has important implications for the health service organisations which use the EuroQol 5 Dimension questionnaire as the basis of their utility value calculations. There are also concerns over whether the questionnaire can accurately assess subtle health benefits from otolaryngological interventions. In a paper which used the EuroQol 5 Dimension questionnaire to evaluate quality of life improvement after BAHA placement, patients' questionnaire scores failed to improve despite significant improvement in their Hearing Handicap and Disability Inventory scores.³⁰

A number of other multi-attribute utility measures have been used in ENT studies. The cochlear implant study discussed above used the Mark II Health Utilities Index, a generic utility measure, to calculate patients' perceived utility from unilateral or bilateral implantation.²¹

Multi-attribute utility measures differ from the quality of life measures most commonly used in ENT,³¹⁻³³ such as the Glasgow Benefit Inventory and the Obstructive Sleep Apnoea 18 and SinoNasal Outcome Test 22 questionnaires.³⁴ These questionnaires were developed using psychometric methods, and produce a quality of life score specific to that questionnaire. Whilst many of the multi-attribute utility measures were also developed using psychometric methods, their fundamental difference is that their scoring is based on preferences (utilities) which facilitate calculation of Quality-Adjusted Life Years. A review of NICE technology appraisal found that measures of utility were rarely included in studies, making it difficult to derive Quality-Adjusted Life Years.³⁵ However, there is now a large body of ongoing research exploring how clinical practice measures can be 'mapped onto' preference-based utility scores to derive Quality-Adjusted Life Years.^{36,37}

Quality-Adjusted Life Years

The Quality-Adjusted Life Year (QALY) is considered the gold standard of economic evaluation measurement

in the UK, as reflected by NICE recommendations for technical appraisal.² As stated above, Quality-Adjusted Life Years reflect both morbidity and mortality, and are calculated by multiplying the utility rating of a health state by the number of years a person is expected to spend in that health state. For example, a procedure which results in a 0.1 gain in utility for a patient with a life expectancy of a further 50 years would produce a gain of 5 Quality-Adjusted Life Years. However, each Quality-Adjusted Life Year may not have the same value.¹⁵ Across a person's lifespan, it is unlikely that they will experience a consistent utility value, for example due to comorbidity or to diminished effectiveness of a procedure over time (e.g. hip replacements that require further surgery).

The Quality-Adjusted Life Year is a popular, standardised and easily quantifiable measurement used to assess the value of an intervention. Quality-Adjusted Life Years can reliably be used to compare one treatment with another for a certain condition, for example, radiotherapy versus surgery for tumour stage one laryngeal cancers. They allow comparisons of services and interventions across health care programmes. At NICE, judgements on allocative efficiency typically include assessment of cost per Quality-Adjusted Life Year. The EuroQol 5 Dimension questionnaire is recommended by NICE as their preferred method of utility rating, in order to derive Quality-Adjusted Life Years.²

Cost–utility analysis and Quality-Adjusted Life Years calculation are already in use in a variety of ENT settings. For example, BAHA placement has been calculated to cost £17 610 per Quality-Adjusted Life Year.³⁸ When comparing treatments for early laryngeal cancer, laser excision is suggested to have a slightly higher cost utility due to the decreased cost of salvage.³⁹ Utility theory has also been used to assess the cost utility of screening for oral cancer⁴⁰ and for coagulation disorders prior to paediatric tonsillectomy.⁴¹ Cost–utility analysis is also a useful tool for facilitating treatment comparisons within a randomised, controlled trial (RCT), for example, surgical versus conservative management for recurrent sore throat.⁴² It has also been used to guide decision-making for patients with acoustic neuroma⁴³ and for elderly patients with hearing loss.⁴⁴

Conclusion

This review has summarised the uses of utility values, and the most common methods used to derive them. The VAS method has many advantages, due to its simplicity, and is widely used in otolaryngological research; examples include RCT assessment of tonsillectomy pain control,⁴⁵ comparison of topical anaesthetic agents for transoral rigid laryngoscopy,⁴⁶ and validation of self-assessment of smell impairment.⁴⁷ The time trade-off and standard gamble methods are more complex to administer but have a more robust grounding in economic theory, incorporating as they

do the concepts of opportunity cost and probability. The EuroQol 5 Dimension questionnaire is used by many organisations, including the NHS, as an indirect, multi-attribute utility measure.

Cost–utility analysis and patient-centred decision-making are an increasingly important part of modern health care, reflecting the need to assess value for money and quality of life when considering treatment choices. However, there are currently many ways of measuring quality of life. A recent systematic review of quality of life changes associated with tonsillectomy found no fewer than eight quality of life measures in use (although most studies used the Glasgow Benefit Plot or the Short Form Questionnaire).⁴⁸ A similar review of otitis media found that audiometric measures of hearing loss, used alone, ignored the health burden of aural discharge, and also that disease-specific measures could demonstrate benefit from reconstructive surgery.⁴⁹ Otolaryngologists should consider the use of utility values when assessing and choosing treatments, in order to take account of quality of life effects and to facilitate robust cost–utility analysis.

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Appendix 1. The EuroQol 5 Dimension questionnaire

Tick one answer in each group below to indicate your health state today.

Mobility

- I have no problems in walking around
 I have some problems in walking around
 I am confined to bed

Self-care

- I have no problems with self-care
 I have some problems washing and dressing myself
 I am unable to wash or dress myself

Usual activities

- (e.g. work, study, housework, family or leisure activities)
 I have no problems with performing my usual activities
 I have some problems with performing my usual activities
 I am unable to perform my usual activities

Pain/discomfort

- I have no pain or discomfort

I have moderate pain or discomfort

I have extreme pain or discomfort

Anxiety/depression

I am not anxious or depressed

I am moderately anxious or depressed

I am extremely anxious or depressed

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