

## Health Economics Learning Tool

### Health measurement and QALYs

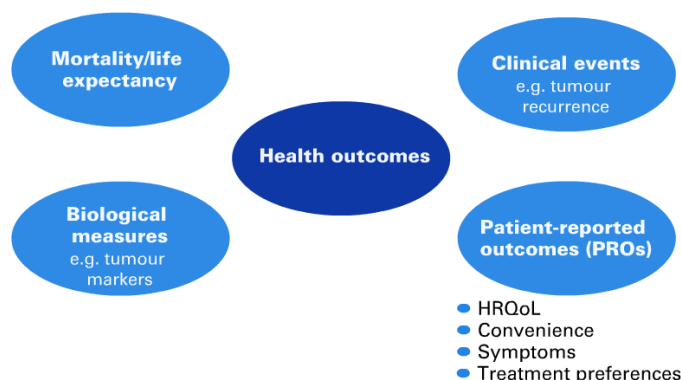
Measuring health is important in assessing the impact of diseases and healthcare interventions on the population. But in order to measure it, we must first think about what we mean by 'health'.

There are many definitions of health – one of the first, which is often quoted, is from the World Health Organisation's 1948 Constitution. This defines health as 'a state of complete physical, mental and social well-being and not merely the absence of disease or infirmity'.

#### World Health Organisation definition of health



Standard clinical measures of outcome may not fully capture all aspects of a patient's health – they look at the physical aspects of a condition but do not consider the patient's perspective. Reports about a health condition or intervention coming directly from the patient – or in certain cases a clinician or carer proxy – are called patient-reported outcomes. These include measures such as health-related quality of life, convenience, symptoms and treatment preferences. Patient-reported outcomes – in particular health-related quality of life – are increasingly measured in clinical trials and have become important from both a clinical and economic perspective.

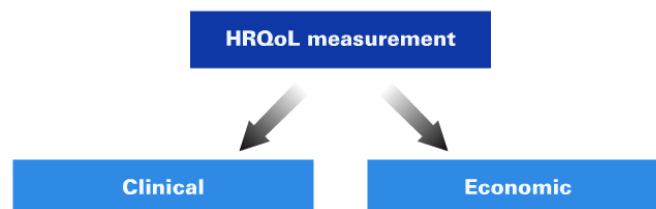


Quality of life is a broad concept describing an individual's general well-being – it is affected by numerous aspects of life, both health and non-health related. Health-related quality of life is a narrower concept that focuses only on aspects of well-being related to health. This is what we are interested in when examining the effect of healthcare interventions.

Measuring health-related quality of life is important in many situations. For example, when investigating chronic diseases, when improvements in function are expected, or when aiming to demonstrate that a serious adverse event does not affect long-term quality of life. The information can be used in many ways, for example, to support licensing applications – regulatory bodies will accept it as supportive evidence in certain diseases; as part of pricing and reimbursement arguments – either as evidence of effectiveness or incorporated into economic evaluation; and to support marketing activities.

Health-related quality of life is often divided into three domains which correspond to the World Health Organisation's definition of health, and these may be combined to provide an overall measure of health. It is assessed using standardised questionnaires which are usually self-administered. There are a large number of instruments available.

These instruments may be divided into two broad categories – those used to measure health-related quality of life as a clinical endpoint; and those used to generate data that can be incorporated in economic evaluation.



Let us now examine these in more detail.

Instruments used to measure health-related quality of life as a clinical endpoint are known as profile-based instruments. These are developed based on psychometric theory and can be categorised as either generic or specific.



Generic instruments can be used in any disease or population. There are many available that are widely used and accepted, and these produce results comparable across different populations and diseases. However, they may contain elements that are irrelevant to the condition being investigated and may not be sensitive enough to detect small but clinically relevant changes.

**Examples:**

- SF36 (Short Form 36)
- NHP (Nottingham Health Profile)
- SIP (Sickness Impact Profile)

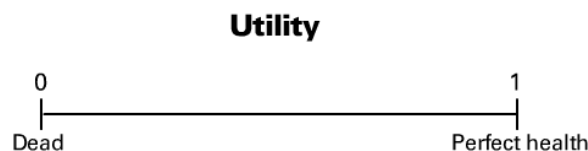
**Examples:**

- EORTC QLQ-C30 (European Organisation for Research and Treatment of Cancer core Quality of Life Questionnaire)
- LCSS (Lung Cancer Symptom Scale)

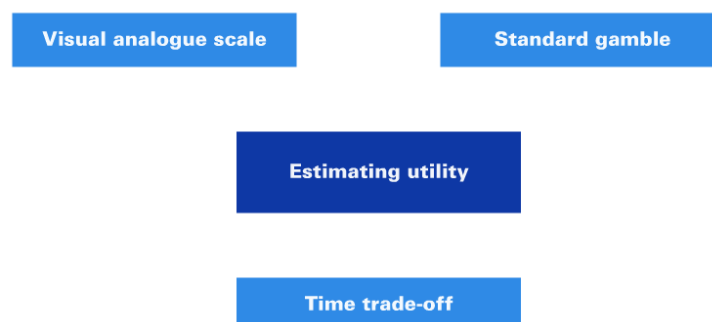
Specific instruments relate to a particular disease, population, intervention or health domain. They have obvious relevance to the population of interest and are more sensitive so may be able to detect small but clinically relevant changes. However, they are less widely available than generic instruments and cannot be compared across populations and diseases.

Now let's turn to incorporating health-related quality of life data in economic evaluation.

For this, a measure known as utility must be obtained. Utility is the strength of preference for an outcome or health state. This is usually measured on a scale of 0 to 1 where 0 is dead and 1 is perfect health.

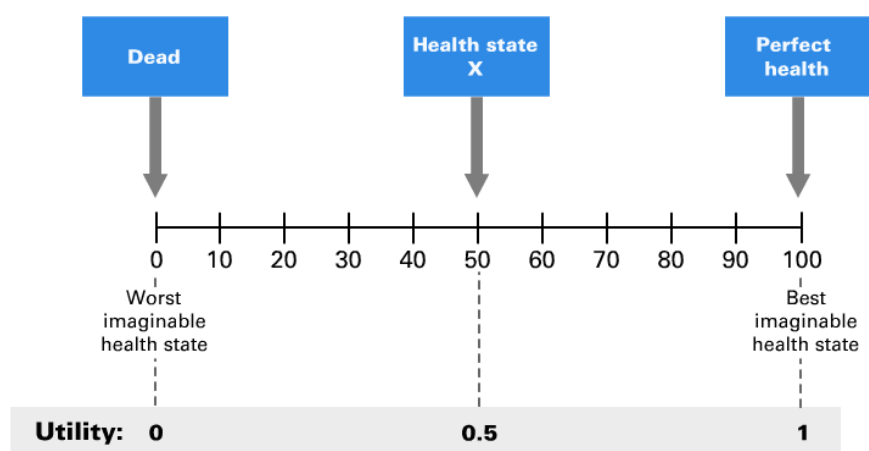


Utilities can be estimated using a range of techniques developed based on economic theory – the most widely used are visual analogue scales, which measure utility directly; and standard gamble and time tradeoff, which are indirect techniques.



Visual analogue scales are used to value health states using a single line on a page with verbal and numerical descriptors at each end. Scale markers are often added to the line, and these may be numbered.

Respondents are presented with a set of health states including two reference states – usually ‘dead’ and ‘perfect health’. They are first asked to rank the states in order of preference, and then to rate the desirability of each by marking a point on the line on or between the two endpoints, so that the intervals between states correspond to the strength of their preference. The reference states of dead and perfect health are defined as having utilities of 0 and 1 respectively, so the utility of the other health states can be calculated based on these.

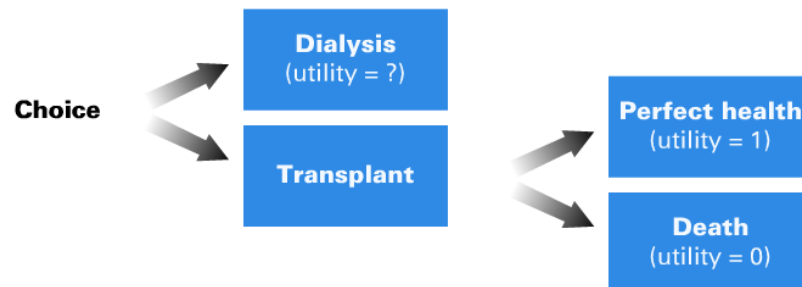


Of course, in some cases, respondents may not place the reference states at the ends of the scale – for example, they may view a health state as worse than dead, in which case the utility would be negative.

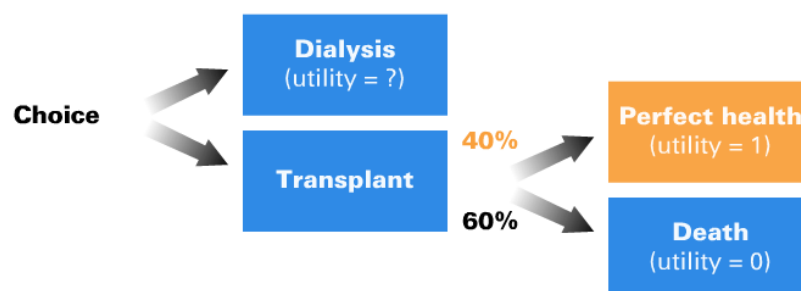
The main advantage of visual analogue scales is that they are quick for respondents to undertake and relatively easy to explain and administer. However, respondents may find the scaling task difficult, and the technique is subject to end-of-scale bias – where respondents are reluctant to score health states close to 100 or 0 – and spacing out bias – where respondents tend to spread the outcomes over the scale regardless of the actual size of their preference.

Standard gamble and time trade-off are generally considered superior valuation techniques.

The standard gamble technique can be used to establish the utility of a described health state by asking a respondent to choose between living in that state with certainty or 'gambling' on an uncertain outcome where they could be better or worse off – for example, living on dialysis or undergoing a risky transplant operation that could lead to either cure or death.



The probabilities in the gamble are varied until the respondent is indifferent between the gamble and living in the described health state – for more severe health states, they will be more willing to accept a higher probability of death in order to try and gain full health. At this point the utility can be calculated; when the respondent is choosing between a gamble of full health and death, this is equal to the probability of full health.

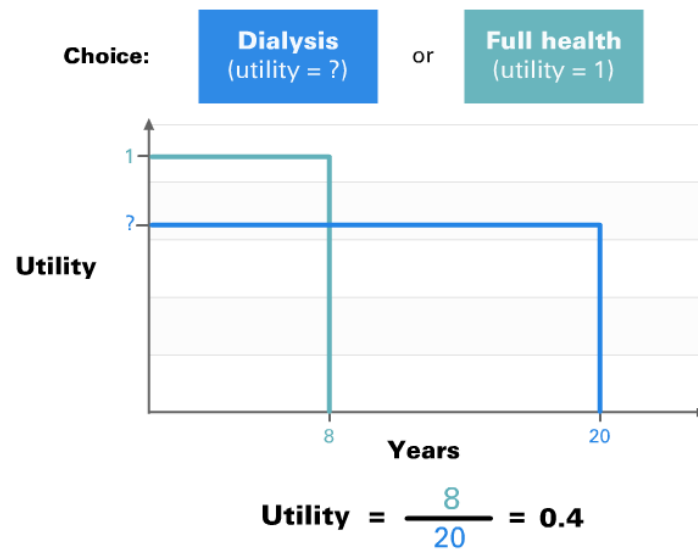


**Utility of dialysis = 0.4**

The standard gamble technique has a strong basis in economic theory and, unlike scaling, choosing between alternatives is a natural task at which people have considerable experience. However, it does require time-consuming experiments, and a key problem is that many respondents will not relate to probabilities.

The time trade-off technique can be used to establish the utility of a described health state by asking a respondent to choose between two 'lives' – for example, living on dialysis for 20 years or living with perfect health for a shorter lifespan.

The length of life in perfect health is varied until the respondent is indifferent between the two options – for more severe health states, they will be willing to accept a shorter lifespan in order to live in perfect health. At this point, the utility can be calculated; in this example, the utility of dialysis is the time spent in perfect health divided by the time spent on dialysis.



Like standard gamble, time trade-off has the advantage of being a choice-based technique. It also has the added advantage that probabilities are not involved, so it is easier for respondents to understand. However, it is still time-consuming to carry out and does not have such a strong theoretical basis.

Instruments used in clinical trials to estimate utility are known as utility- – or preference- – based instruments. They are most commonly generic.

**Examples:**

- EQ5D (Euroqol 5 Dimensions)
- HUI (Health Utilities Index)
- Quality of Well-Being Scale (QWBS)

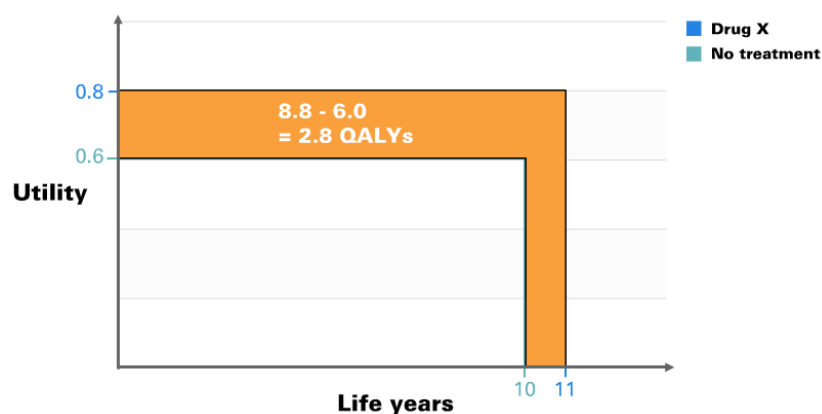
Utility-based instruments either directly measure a patient's preference for a health state – for example, using a visual analogue scale – or measure a patient's health-related quality of life and then infer the utility using a predefined 'tariff'. The tariff lists the utility associated with each possible health-related quality of life score from the instrument, obtained from a previous experiment – for example time trade-off – in the general population. It is generally considered that the preferences of society should be used in economic evaluation because the aim is to inform decisions that affect the whole society, so the tariff approach is often preferred.

Health-related quality of life is most-commonly incorporated into economic evaluation through quality-adjusted life years – or QALYs – in cost-utility analysis.

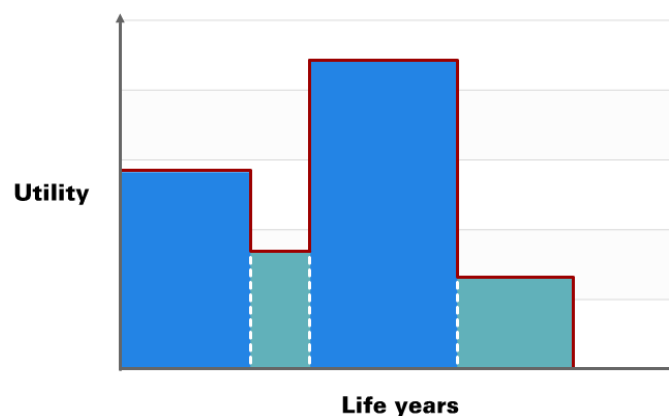


QALYs aim to comprehensively capture health outcomes by combining duration and quality of life. They are calculated by multiplying the utility of a health state by the number of years spent in it.

Let's consider a simple example where drug X prolongs life and improves health-related quality of life – a patient lives for 10 years with a utility of 0.6 if they do not receive any treatment, or for 11 years with a utility of 0.8 when treated with drug X. The outcome with no treatment is 6 QALYs and the outcome with drug X is 8.8 QALYs. Treatment with drug X therefore leads to an increase of 2.8 QALYs.



QALYs can also be calculated for more complicated scenarios – for example if a number of health states with different utilities are experienced over time. This might be the case in a chronic disease such as rheumatoid arthritis in which patients experience periodic exacerbations and remissions.



QALYs are by far the most commonly-used outcome measure for cost-utility analysis. However, their use is widely debated in the economic and medical literature and obtaining the required utility data can be practically difficult.

## Summary

This module covers the following topics:

### **Measuring HRQoL as a clinical endpoint:**

- Profile-based HRQoL instruments
  - generic
  - specific

### **Measuring HRQoL as an economic endpoint:**

- Estimating utility
  - visual analogue scale
  - standard gamble
  - time trade-off
- Utility-based HRQoL instruments
- QALYs