6 Estimating Health Benefits

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In 1948, the World Health Organization defined health as being not only the absence of disease and infirmity, but also the presence of physical, mental and social wellbeing (43 (https://www.pharmac.govt.nz/medicines/how-medicines-are-funded/economic-analysis/pfpa/references/#ref43)). Quality of life issues have become increasingly recognised as important in health care, particularly with the treatment of chronic conditions with long-term effects on quality of life.

6.1 Measures of Health-Related Benefit

Key Recommendation: Health benefits should be measured using quality-adjusted life years (QALYs). QALYs take into account patients' health-related quality of life as well as duration of survival. Only the QALYs of the individual patient being treated should be included in the base-case analysis.

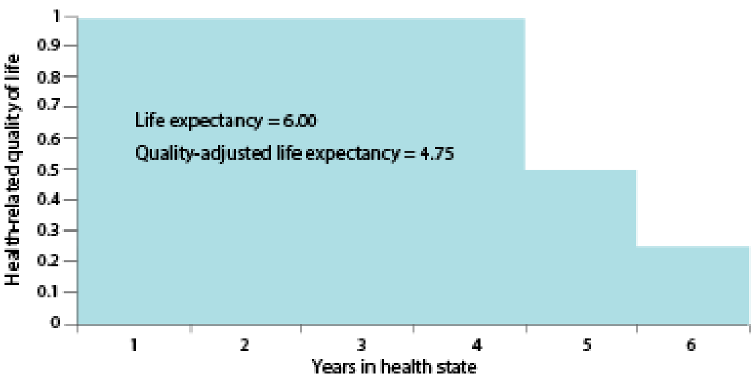
Health measures that incorporate both the quality and the length of life into a common currency include quality-adjusted life years (QALYs), disability-adjusted life years (DALYs)[14] (ftn14) and healthy year equivalents (HYEs)[15] (/medicines/how-medicines-are-funded/economic-analysis/pfpa/6-estimating-health-benefits/downloadpdf#ftn15).

6.1.1 Quality-Adjusted Life Years

QALYs have been used since the 1960s and remain the most widely used measure for integrating effects of treatments on length and quality of life.

Under the QALY framework, one QALY is equivalent to living one year in perfect health, or two years at half of perfect health, and so on. This is illustrated in the following figure. Here, life expectancy (the number of years left before death) is 6.00. Quality-adjusted life expectancy (the number of QALYs left before death) is 4.75. This is calculated by multiplying each life year by the average quality of life experienced in that year ((4×1)+(1×0.5)+(1×0.25)). This is equivalent to the area under the curve.

Figure 2: QALYs combine quality of life and life expectancy



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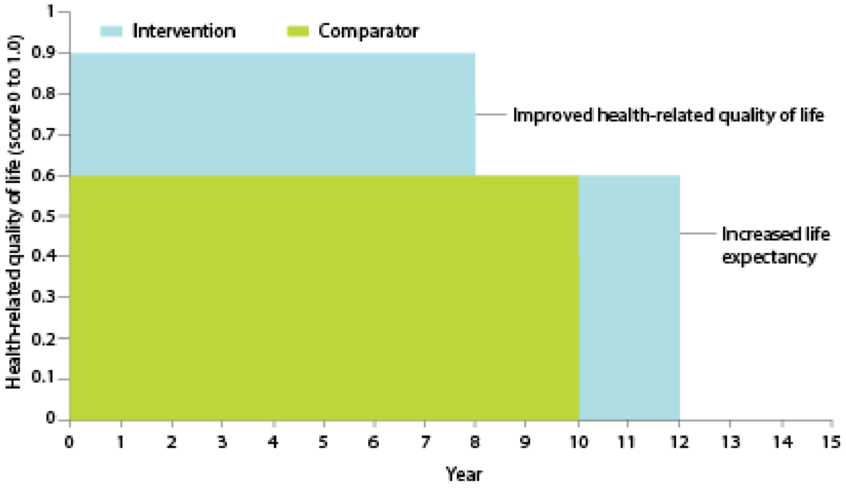
Text description of QALYs combine quality of life and life expectancy

(file:///sites/pharmac/releases/20170726104300/assets/_generated_pdfs/6-estimating-health-benefits-2062_pdf.html#text-description-of-qalys-combine-quality-of-life-and-life-expectancy)

This graph shows an example of changing quality of life over time. In the graph, the person is shown to have a normal quality of life (1.0) for the first four years. It then falls to a quality of life of 0.5 for the fifth year, then to a quality of life of 0.2 in the sixth year, then the person dies. As the person has lived six years in this example their life expectancy is 6.00, but their quality-adjusted life expectancy is the area of the graph which is 4.75.

The figure below illustrates how a theoretical intervention may gain QALYs through improving both patient quality of life and life extension.

Figure 3: Incremental gains in both quality and length of life



Text description of incremental gains in both quality and length of life

(file:///sites/pharmac/releases/20170726104300/assets/_generated_pdfs/6-estimating-health-benefits-2062_pdf.html#text-description-of-incremental-gains-in-both-quality-and-length-of-life)

This graph shows how improvements in quality and length of life are valued. The graph shows both the outcomes for the comparator treatment and the theoretical intervention. The comparator is shown to lead to a quality of life of 0.6 and a length of life of 10 years. The intervention is shown to lead to a quality of life of 0.9 for 8 years, then a quality of life of 0.6 for another 4 years. The difference between the intervention and comparator occurs in two places. Firstly, the first 8 years have improved health-related quality of life. Secondly, the patient has an increased life expectancy of another 2 years.

PHARMAC recommends using QALYs in assessments as they are simple to calculate, have face validity and enable cost-utility analysis; and because substantial empirical data are available on the preferences people place on various combinations of suffering and limits on activities.

6.1.2 Health Benefit to Family, Whānau and Society

When estimating QALYs, only the impact on health-related quality of life is measured, as opposed to taking into account all factors that may affect a person's general quality of life. Other aspects affecting health decisions can be considered through PHARMAC's other Factors For Consideration.

It is recommended that only the health-related quality of life (HR-QoL) of the patient being treated should be included in the base-case analysis. If the treatment might have a measurable but indirect impact on the HR-QoL of others, such as family and caregivers, this could be estimated and discussed in the report as a scenario.

Some treatments have direct effects on others that could be counted. Examples include vaccines, perinatal treatments, and treatments that aim to reduce vertical transmission. To ensure comparability of different CUAs, reporting of such results should be very clear as to what is and is not included in each incremental utility-cost ratio reported.

6.1.3 Value-Judgement Weightings

The health-related benefits included in a cost-utility analysis should not be weighted to account for other aspects such as health need or disease severity. It is considered important to keep the IUCRs as value free as possible. Consequently, it is recommended that additional weightings not be applied when calculating QALYs. In other words, the HR-QoL weights used to calculate QALYS should not be adjusted or weighted for value judgements on issues such as distributive justice, respect for autonomy, or health need. PHARMAC's Factors for Consideration provide a framework to ensure that all relevant aspects and issues are taken into account in an overall decision.

6.2 Health-Related Quality of Life Instruments

Key Recommendations: The New Zealand EQ-5D Tariff 2 should be referred to first when measuring health-related quality of life, and should be used to describe the health states. The Global Burden of Disease disability weights and published literature should be used to check for consistency with the estimated EQ-5D values.

A number of instruments have been developed to measure health state preferences (44 (https://www.pharmac.govt.nz/medicines/how-medicines-are-funded/economic-analysis/pfpa/references/#ref44)). These instruments provide a utility rating in the form of a single number representing the net aggregate impact of physical, emotional and social functioning on quality of life.

There has been much debate in the literature about the most appropriate tool for measuring preferences in health gains. Given the multidimensional nature of HR-QoL, it seems that no single measure has been (or is likely to be) accepted as the gold standard (45 (https://www.pharmac.govt.nz/medicines/how-medicines-are-funded/economic-analysis/pfpa/references/#ref45)). The Washington Panel on Cost-Effectiveness in Health and Medicine reviewed these instruments in 1996, and chose not to endorse one instrument above another (14 (https://www.pharmac.govt.nz/medicines/how-medicines-are-funded/economic-analysis/pfpa/references/#ref14)). They note that each instrument has different properties, and each member of the Panel valued these properties differently. However, it is recommended that decision makers such as PHARMAC adopt a reference case instrument.

Instruments available include (but are not limited to) the Health Utility Index (HUI); the EuroQol 5D (EQ-5D); 15D; the Assessment of Quality of Life instrument (AQoL); the Short-Form 6D (SF-6D); and the Quality of Well Being index (QWB) (2, Chapter 6 (https://www.pharmac.govt.nz/medicines/how-medicines-are-funded/economic-analysis/pfpa/references/#ref2)).

6.2.1 EQ-5D

The recommended Health-Related Quality of Life instrument is the EuroQol 5D with the New Zealand Tariff 2.

The EQ-5D is one of the most widely used and adapted instruments internationally. It consists of five dimensions (mobility, self-care, usual activities, pain/discomfort, and anxiety/depression) and three levels (no problems, some problems and extreme problems), resulting in 245 unique health states (including Unconscious and Dead).

In order to derive generic utility weights specific to the New Zealand population, Devlin et al undertook a survey of the New Zealand population in 1999 using the EuroQol Group's EQ-5D questionnaire (46 (https://www.pharmac.govt.nz/medicines/how-medicines-are-funded/economic-analysis/pfpa/references/#ref46)). The survey was mailed to 3000 randomly selected New Zealanders, and was completed by 1360, giving an approximately 45% response rate. Each respondent rated their health on the five EQ-5D dimensions and assigned a global score to their profile. Valuations for a subset of the 245 EQ-5D states were collected from respondents using the Visual Analogue Scale (VAS). Regression analysis was used to interpolate values over the 245 possible EQ-5D states (46 (https://www.pharmac.govt.nz/medicines/how-medicines-are-funded/economic-analysis/pfpa/references/#ref46))[16] (PFPA%202.2%20z%20compiled%20chapters%20and%20Appendices.docx#ftn16).

Almost two-thirds of the survey responses had to be rejected due to missing, implausible or otherwise unusable valuations (46 (https://www.pharmac.govt.nz/medicines/how-medicines-are-funded/economic-analysis/pfpa/references/#ref46)). This resulted in two tariffs being produced – one (Tariff 1) that included the 'logical inconsistencies'[17] (PFPA%202.2%20z%20compiled%20chapters%20and%20Appendices.docx#ftn17) and so may be more representative of the population's views, and the other (Tariff 2) that excluded these inconsistencies may more accurately reflect underlying preferences) (46 (https://www.pharmac.govt.nz/medicines/how-medicines-are-funded/economic-analysis/pfpa/references/#ref46)).

The validity and reliability of the EQ-5D health state valuations have also been examined in the Māori population. Perkins et al (49 (https://www.pharmac.govt.nz/medicines/how-medicines-are-funded/economic-analysis/pfpa/references/#ref49)) surveyed 66 Māori people to investigate the content validity and reliability of the EQ-5D in this population. They reported that approximately three-quarters of respondents considered the EQ-5D representation of health to be adequate, suggesting the instrument has content validity. However, a high prevalence of missing valuations (particularly for the health state 'Dead') and logical inconsistencies suggests that it lacks construct validity in this population.

The EQ-5D is widely used internationally and utility weights have been derived from the New Zealand population. Therefore, PHARMAC recommends referring to the EQ-5D Tariff 2 first and using it to describe the health states. Other instruments can be used, however, their use should be well justified.

6.2.2 Obtaining Utility Values

Key Recommendation: If subjective judgement is used to map health states, these health states should be validated through either published literature or expert clinical input. The report should provide a detailed description of the health state and impact on HR-QoL.

Utility values can be obtained directly or indirectly. Obtaining direct health utilities may require face-to-face interviews where people are asked to assign value to specific health states. Indirect health utilities use population-assigned weights to calculate utility scores for particular health states from health status instruments (eg the EQ-5D) (50 (https://www.pharmac.govt.nz/medicines/how-medicines-are-funded/economic-analysis/pfpa/references/#ref50)).

Three common methods used to evaluate health states are the standard gamble (SG), time trade-off (TTO) or visual analogue scale (VAS).

6.2.3 Mapping

Mapping health states to health status classification instruments requires subjective judgements; however, the estimates can be further validated by input from clinicians and the literature.

Mapping can involve both relating the baseline characteristics of the target patient population to relevant generic health states in the quality of life instrument(s) used for the CUA, and then estimating the extent to which treatment alters baseline health status.

It is essential that the symptoms patients experience in each of the health states are described in detail in the report. This will assist with the mapping process.

Further information on mapping can be found in *Appendix 6: Utility valuation of health outcomes* of the PBAC Guidelines (22 (https://www.pharmac.govt.nz/medicines/how-medicines-are-funded/economic-analysis/pfpa/references/#ref22)).

6.2.4 Literature

Existing utility values available in the literature can be used to check for consistency with the EQ-5D weights, providing similar health states and patients are used and the measurement instrument is credible.

Existing utility values can be sourced from published cost-utility analyses (refer to section 4.2 for website links) or studies that estimate HR-QoL scores, such as the Global Burden of Disease (GBD) study discussed in the next section.

6.2.5 Disability Weights – the Global Burden of Disease Study (GBDS)

The Global Burden of Disease Study (GBDS) (51 (https://www.pharmac.govt.nz/medicines/how-medicines-are-funded/economic-analysis/pfpa/references/#ref51), 52 (https://www.pharmac.govt.nz/medicines/how-medicines-are-funded/economic-analysis/pfpa/references/#ref52)) estimated the burden of 1160 separate sequelae of 220 health states. DALYs were used to measure the impact of mortality and non-fatal health outcomes for a wide range of diseases and illnesses. In order to estimate DALYs, a multicountry household survey and an open-access web-based survey were conducted. There was a significant degree of concordance between countries.

PHARMAC recommends using the GBDS weights to check for consistency and face validity with the EQ-5D weights, but they should not be used as the main source of utility values.

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[14] (/medicines/how-medicines-are-funded/economic-analysis/pfpa/6-estimating-health-benefits/downloadpdf#ftnref14) DALYs are expressed in terms of years of life lost due to premature death and years lived with a disability of specific severity and duration.

[15] (/medicines/how-medicines-are-funded/economic-analysis/pfpa/6-estimating-health-benefits/downloadpdf#ftnref15) HYEs incorporate individual preference structures over a complete path of health states (rather than discrete health states).

[16] (/medicines/how-medicines-are-funded/economic-analysis/pfpa/6-estimating-health-benefits/downloadpdf#ftnref16) This included negative values for health states considered to be worse than death (47 (https://www.pharmac.govt.nz/medicines/how-medicines-are-funded/economic-analysis/pfpa/references/#ref47)). Survey results indicated that respondents can and do evaluate some

health states as worse than death, and the study authors recommended the systematic inclusion of these states to describe a more complete range of preference values (48 (https://www.pharmac.govt.nz/medicines/how-medicines-are-funded/economic-analysis/pfpa/references/#ref48)).

[17] (/medicines/how-medicines-are-funded/economic-analysis/pfpa/6-estimating-health-benefits/downloadpdf#ftnref17) Logical inconsistency was defined as “when a state that ‘in logical terms’ is unambiguously less severe than another is assigned a lower value” (46 (https://www.pharmac.govt.nz/medicines/how-medicines-are-funded/economic-analysis/pfpa/references/#ref46)).

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Last updated: 10 August 2017