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2 Health Economic Analysis at PHARMAC

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2.1 What is Economic Analysis?

Economic analysis is the explicit consideration of the costs and benefits of a proposed course of action. Economics is based on three fundamental concepts that summarise the issues PHARMAC faces daily:

- **Scarcity** – resources will always be insufficient to support all possible activities.
- **Choices** – due to scarce resources, decisions must be made about how best to use them.
- **Opportunity cost** – by choosing to use resources one way, we forgo other opportunities to use the same resources.

Based on these concepts, resources are only used efficiently if the value of what is gained from their use is greater than the value of alternative options that could have been funded.

For further information on the purpose of, and techniques for, undertaking economic analysis in health care, please refer to standard health economics texts (eg, 2 (<https://www.pharmac.govt.nz/medicines/how-medicines-are-funded/economic-analysis/pfpa/references/#ref02>)).

2.2 Why Does PHARMAC Use Economic Analysis?

PHARMAC's objective is to secure the best health outcomes reasonably achievable from within the funding provided. As PHARMAC must work within a fixed budget, it is impossible to fund every new pharmaceutical that may potentially benefit someone. The demand for pharmaceuticals will always exceed our ability to pay for these pharmaceuticals. In short, choices are inevitable.

Economic analysis provides information on the health gains and costs associated with various funding options. It is a valid, replicable and scientific tool for PHARMAC to use to help identify proposals that would provide the best health outcomes if funded.

Economic analysis is not a technical fix for complex decisions, but merely a tool designed to bring greater objectivity and consistency to often complex decisions, and shed light on the logic behind choices. It is used to inform decision making rather than replace it.

2.3 Types of Economic Analysis

Key Recommendations: Most analyses undertaken by PHARMAC staff are in the form of cost-utility analysis (CUA), as it is practical and enables comparisons across different pharmaceuticals and opportunities for health funding. Cost-utility analysis help PHARMAC prioritise pharmaceuticals for investment decisions.

Several forms of economic analysis are available including:

- **Cost-minimisation analysis (CMA)**
CMA assumes that there is no net health change involved in moving from one treatment to another; hence the decision can be made on the basis of the difference in total cost alone. CMA is appropriate when the clinical outcomes of the drug and the comparator are equivalent.
- **Cost-effectiveness analysis (CEA)**
In CEA, the incremental costs are compared with the incremental outcomes, as measured in physical units such as life years saved or heart attacks prevented. A disadvantage of CEA is that it does not enable direct comparison of interventions treating different conditions.
- **Cost-utility analysis (CUA)**
CUA is a variation of CEA in which outcomes are weighted in common currency, usually quality-adjusted life years (QALYs). QALYs combine changes in quantity and quality of life (mortality and morbidity) into one composite measure. CUA enables comparison between the cost-effectiveness of interventions treating different conditions, and also takes into account benefits resulting from both decreases in mortality and decreases in morbidity.
- **Cost-benefit analysis (CBA)**
In CBA, incremental outcomes are expressed in monetary terms, usually using the willingness-to-pay approach. The results of CBA are expressed as one figure, representing the difference between benefits and costs ($B-C > 0$), or as a ratio (B/C). Disadvantages of CBA include the difficulty in comparing treatments that improve quality of life with those that save lives, and the difficulty associated with placing a dollar value on health benefits. There are also ethical objections to placing a monetary value on health, particularly with respect to valuing a human life.

Table 1 summarises the differences between the forms of economic analysis.

Table 1: Differences between Types of Economic Analysis

Type of Analysis	Measurement of Benefits
Cost-minimisation	Benefits found to be equivalent
Cost-effectiveness	Physical units (eg life years gained)
Cost-utility	Healthy years (eg quality-adjusted life years)
Cost-benefit	Monetary terms

2.4 What is the Process for Undertaking and Reviewing Cost-Utility Analyses at PHARMAC?

PHARMAC invites, reviews, and comments on analyses submitted by pharmaceutical suppliers. PHARMAC staff also implement their own models to better understand the value offered by new pharmaceuticals. The intention of this *Prescription for Pharmacoeconomic Analysis* is to offer guidance on consistent methods and standards to apply to all analyses, regardless of who they are created by.

2.4.1 PHARMAC Process for Undertaking Cost-Utility Analysis

Most CUAs are undertaken internally as analyses are required within short timeframes. The process also ensures continuity of methods and quality control. In addition, analyses often need to be updated at short notice following the receipt of further clinical advice or proposed price reductions – thus the process has to be flexible. PHARMAC analyses are based on the methods outlined in this document.

PHARMAC must reach practical funding decisions despite finite analytical capacity. Inevitably, there are trade-offs between the precision and timeliness of CUAs. Assessments can therefore be conducted at four levels: rapid, preliminary, indicative, and detailed. A summary of what may be included at each of the levels of analysis is given in Table 2. Any given analysis may include or exclude any of the criteria listed.

Table 2: Levels of PHARMAC Analyses

Type	General Description	FTE Required
Rapid	<p>Basic economic model constructed, largely based on opportunistic data. The analysis is undertaken over a time horizon that sufficiently captures the majority of incremental costs and benefits.</p> <p>Testing undertaken to ensure extent of analysis is sufficient.</p> <p>Brief documentation of CUA (but still detailed enough to allow reproduction of the CUA by others).</p> <p>Reviewed internally.</p> <p>May include reviews and basic amendments to external analyses.</p>	<2 weeks
Preliminary	<p>Assessment largely conducted using opportunistic data. Rapid systematic review of evidence undertaken.</p> <p>May require further modelling compared with a rapid CUA (due to disease complexity, risk, or uncertainty of results).</p> <p>Reviewed internally.</p>	2-4 weeks
Indicative	<p>An interim assessment using some opportunistic data, but more detailed than a preliminary analysis. Evidence critically appraised.</p> <p>Often involves more complex economic modelling. Full assessment undertaken on whether statistically insignificant events are likely to be clinically significant, or vice-versa.</p> <p>Further investigation into health-related quality of life scores, including a systematic review of the literature.</p> <p>Full multivariate sensitivity analysis may be undertaken, with detailed discussion of results.</p> <p>Detailed documentation of critical appraisal and economic analysis.</p> <p>Reviewed internally and by the Pharmacology and Therapeutic Advisory Committee (PTAC).</p>	1-2 months
Detailed	<p>Includes a detailed and systematic identification and synthesis of relative clinical effectiveness, prognosis, health-related quality of life, and cost data. Evidence critically appraised using the Graphic Appraisal Tool for Epidemiology (GATE) framework (or other similar tools).</p> <p>Detailed Markov model. All potential health states and clinical events included. The use of probability distributions considered.</p> <p>Detailed extrapolation of the clinical evidence, and statistically non-significant events tested.</p> <p>Further validation of utility mapping exercise, including obtaining expert clinical input.</p> <p>Probabilistic sensitivity analysis may be undertaken.</p> <p>Reviewed internally and externally (clinical assumptions reviewed by PTAC).</p>	>2 months

FTE = Full-Time Equivalent. Note that these are indicative timeframes. Actual timeframes vary depending on experience and workload.

Very few proposals receive a detailed assessment as these take between two and six months to complete, which can be too slow and resource intensive for a purchasing environment. While detailed analysis may improve the academic rigour of the assessment, we have found that increased levels of complexity do not often further inform the funding decision or impact the relative cost-effectiveness of the pharmaceutical. Undertaking detailed CUAs when not strictly needed also ties up resources, limiting the ability to undertake other analyses or funding work generally. In addition, at PHARMAC the CUA result is not critical to the setting of a subsidy level, so perfecting the CUA is seldom necessary. What is most important is that the CUA is sufficient to help inform PHARMAC of where the pharmaceutical should be placed on the priority list and to inform any funding decisions.

The assessment process is usually iterative. Further analysis will be undertaken if a rapid assessment indicates there is very large uncertainty in the result of the analysis, to the extent that the relative priority of the pharmaceutical is uncertain. The level of analysis generally aims to be sufficient to prioritise a proposal with enough certainty. Assessments can be updated as more information becomes available, or as the proposal changes during assessment and negotiation.

The level of analysis undertaken depends on the factors outlined in Table 3.

Table 3: Determinants of Level of Analysis Undertaken by PHARMAC

Determinants of level of analysis	Details
Timeframes	In some cases a CUA result may be required within a week; hence a more detailed analysis cannot be undertaken.
Impact on pharmaceutical budget	A proposal with high expenditure or a large plausible range of costs is more likely to require a more detailed CUA, especially if the pharmaceutical is highly effective.
Reliability of results	If the results of a CUA are very sensitive to key assumptions, a higher level of analysis may be required.
Extent of information available for analysis	Pharmaceuticals for rare conditions are more likely to undergo rapid analysis due to unavailability of data. More detailed analysis may not resolve key uncertainties.
Impact of CUA on funding decision	In some cases the main reasons for funding a pharmaceutical may be due to Factors for Consideration that do not fall within Health Benefits and Cost and Savings dimensions, and therefore a detailed CUA may not be required.
Availability of analyst resources	Given limited analyst resources, it may not be cost-effective to undertake a detailed analysis when a number of other CUAs are also required.

Most CUAs are written up as Technology Assessment Reports following a set template. CUAs are then peer-reviewed by colleagues, who examine the economic methodology. Analyses may also be clinically reviewed by the Pharmacology and Therapeutic Advisory Committee (PTAC) (3 (<https://www.pharmac.govt.nz/medicines/how-medicines-are-funded/economic-analysis/pfpa/references/#ref03>)), a specialist PTAC subcommittee, or clinical experts.

Appendix 1 includes guidelines for reviewing a cost-utility analysis.

2.4.2 PHARMAC Process for Reviewing Supplier Cost-Utility Analyses

The *Guidelines for Funding Applications to PHARMAC*, available on PHARMAC's website, specify all the information that PHARMAC requests in support of a funding proposal. The information that PHARMAC requests to support the economic analysis of a proposal is summarised at www.pharmac.govt.nz/medicines/how-medicines-are-funded/new-funding-applications/ (<https://www.pharmac.govt.nz/medicines/how-medicines-are-funded/new-funding-applications/>).

PHARMAC encourages pharmaceutical suppliers to provide an economic analysis when submitting a significant funding proposal. The provision of a good-quality analysis, following the methods outlined in the PFFPA, helps PHARMAC assess and prioritise a proposal more swiftly.

Economic analyses should be in the form of a cost-utility analysis, with benefits measured in terms of QALYs. In cases where the clinical outcomes of the drug and the comparator have been shown to be equivalent, a cost-minimisation analysis may be appropriate. Other forms of cost-effectiveness or cost-benefit analyses should not be provided.

PHARMAC's preferred software packages are TreeAge⁴ (<https://www.pharmac.govt.nz/medicines/how-medicines-are-funded/economic-analysis/pfpa/references/#ref04>) and Microsoft Excel. Models provided in other software packages will not be assessed unless by prior agreement. Excel models should minimise the use of Visual Basic code and similar complex features.

Economic models should not be unnecessarily complex, and should always be transparent, well described and reproducible. The structure, data and process of building the model should be detailed enough to enable competent analysts who are not familiar with the model to vary key parameters, and to reproduce it in other software.

If a CUA has been submitted to Australia's Pharmaceutical Benefits Advisory Committee (PBAC) or Medical Services Advisory Committee (MSAC), PHARMAC will accept the same CUA in the application to PHARMAC, providing an electronic copy of the TreeAge model and/or Excel spreadsheet are included. This ensures that PHARMAC can amend the costs and any other relevant inputs so the model is applicable to the New Zealand clinical and funding environment. A copy of any reviews undertaken by PBAC-contracted reviewers should also be provided.

When PHARMAC receives an economic model and assessment from an applicant, our health economists review it and amend it if required. The guidelines PHARMAC uses to review analyses are attached in Appendix 1.

If PHARMAC staff amend the analysis supplied, PTAC will usually review both the supplier CUA and PHARMAC's amended version, with any differences clearly explained.

2.5 When is a Pharmaceutical Considered to be 'Cost-Effective'?

A proposal to invest in a pharmaceutical can be considered 'cost-effective' only in comparison with another proposal. At PHARMAC, there is no threshold for when a pharmaceutical is considered 'cost-effective'. Proposals are only considered in relation to other funding proposals at the time. Also, PHARMAC's Factors for Consideration are broader than cost-effectiveness alone. One proposal may be more cost-effective than another but rate less well on other factors and therefore may not be funded.

Another reason for not having a threshold value is that the spending on pharmaceuticals is required to be kept within a fixed budget. Given the binding nature of this constraint, and all things being equal, what is and is not considered 'cost-effective' will vary with the amount of funding available. This is in terms of not just the total budget each year, but also the available budget that we anticipate in the future.

What may be considered 'cost-effective' therefore changes over time, with wide variations both in any year and between years⁵ (<https://www.pharmac.govt.nz/medicines/how-medicines-are-funded/economic-analysis/pfpa/references/#ref05>). For example, between the 1998 and 2015 financial years,

individual new investments made by PHARMAC varied between 25 QALYs gained for every \$1 million saved by the NZ health system (ie decisions that both reduced costs and improved health) and less than 5 QALYs gained for every \$1 million spent. Expressed as costs per QALY gained, investments varied between saving \$40,000 per QALY gained (\$-40,000/QALY) and spending over \$+200,000 per QALY. Investments varied widely each year, reflecting the mix of investment opportunities, the funding available at the time, and the impacts of other Decision Criteria.(6 (<https://www.pharmac.govt.nz/medicines/how-medicines-are-funded/economic-analysis/pfpa/references/#ref06>))

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