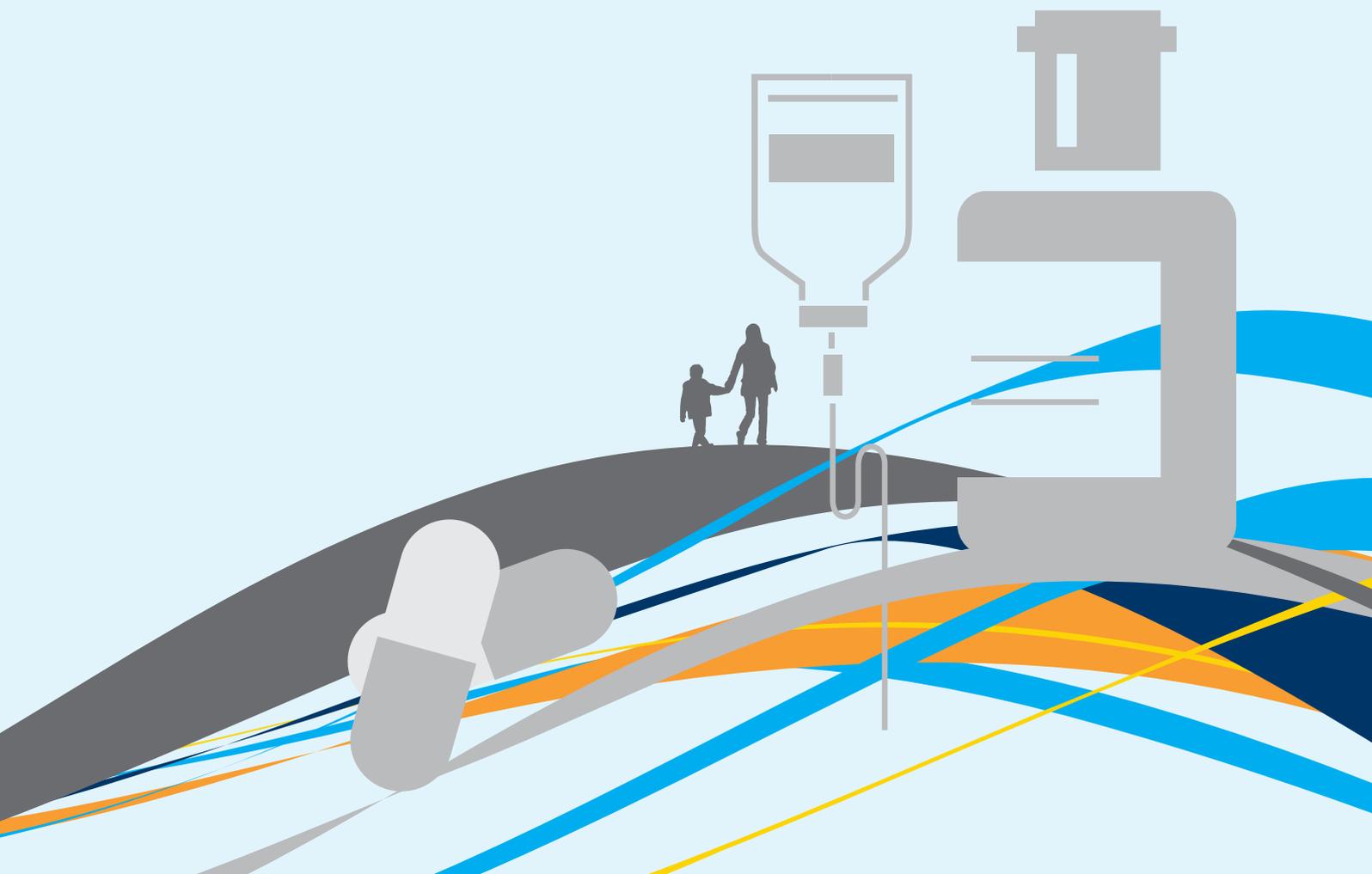


Pharmaceutical Management Agency

Cost-Utility Analysis (CUA) Explained



Cost-Utility Analysis (CUA) at PHARMAC Questions and Answers go to page 8 >>

The process described in this document is intended to be indicative of the process that PHARMAC may use when undertaking a cost-utility analysis (CUA). Note that PHARMAC may, at its discretion, adopt a different process or variations of the process.

This document is a simplified explanation of general CUA concepts. For detailed information on the process for CUA at PHARMAC, please refer to the Prescription for Pharmacoeconomic Analysis (PFPA):

www.pharmac.govt.nz/EconomicAnalysis/pharmacoeconomics

The PFPA is intended to be the definitive guide to PHARMAC's approach to CUA, and in the event of any inconsistency between this document and the PFPA (whether as a result of simplification of concepts and explanations or otherwise) the PFPA is to prevail.

For further information about PHARMAC, the things we do, and our place in the health system, please see PHARMAC Information Sheets:

www.pharmac.govt.nz/patients/AboutPHARMAC/infosheets

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Introduction

PHARMAC is the government agency charged with deciding which medicines will be publicly funded. Our objective is to, in effect, get the best health outcomes we can for people who need pharmaceutical treatment from within the amount of taxpayer funding provided. Because funding is always limited, this involves making some tough choices. PHARMAC undertakes cost-utility analysis (CUA) to inform our decisions on which medicines to fund. This guide explains the CUA process.

Why use CUA?

- How much better is this new medicine than those we already fund?
- What is the 'value' of the new medicine? Is any additional benefit worth it?
- Will funding this medicine make the best contribution to New Zealand's health?

CUA helps answer these questions. It's a well established analytical technique used all over the world.

The type of questions we face might sound familiar to anyone on a limited budget. Should we go out for dinner, or eat in and take the kids to the movies instead? Clearly every choice has a cost – not just financial, but also the benefits that could come from the other choice you could have made ('opportunity cost'). We each try to judge how to get the best value from our choices.

PHARMAC's job is essentially the same. Money spent on a particular medicine has an 'opportunity cost' because there is less money for other medicines that could have been funded. We want to get the best health outcomes from available funding. Like anyone we have to make careful choices. We make these choices with the help of CUA.

It can be hard to talk about health and money together. Our work, however, requires us to negotiate with pharmaceutical companies – who are understandably keen to maximise their commercial interests. We want to be fair to companies, without paying too much. Paying higher prices stops us from funding other medicines that could improve the lives of New Zealanders.

CUA: a quick overview

Medicines treat a wide range of medical conditions, from minor to fatal, and with very different benefits and costs associated with their use. CUA is the assessment of the additional benefits and costs associated with treatments.

When assessing a new medicine, we want to know how much better it is, and how much more it costs, than the medicines or treatments we already have. This comparison is made using standard clinical practice in New Zealand.

Assessing benefits

The benefits of a medicine in CUA are estimated using 'quality-adjusted life years' (QALYs). QALYs are a measurement that can be used to compare – in a consistent and standardised way – benefits of different treatments. In measuring QALYs, we look at the combination of two major things: a treatment's effects on how much longer we live, and also on how much better we live.

Assessing costs

Costs are also carefully considered in CUA. This includes the cost of the treatment itself and any other costs to the health sector that may occur as a result of funding the new treatment. It is also possible for medicines to save costs elsewhere, such as avoiding the need for people to go to hospital. We call these cost offsets, and include these as well.

Combining benefits and costs

The results of a CUA tell us how many QALYs we gain for every dollar we spend. This allows us to compare how much better a medicine is than other medicines. We can compare the results of assessments for different medicines – this is called 'relative cost-effectiveness'.

CUA helps us apply our decision criteria – the standard set of factors we judge medicines against (see below). CUA is particularly relevant to the 5th criterion (cost-effectiveness), but CUA can also include information related to other criteria.

Although we have focussed above on assessing new medicines, CUA is also helpful for assessing whether access to existing medicines should be widened, to allow for different uses of the same medicine.

How we decide PHARMAC's Decision Criteria

1. Health needs of eligible people
2. Health needs of Māori and Pacific peoples
3. Availability and suitability of existing medicines
4. Clinical benefits and risks
5. Cost-effectiveness
6. Overall budgetary impact
7. Direct cost to health service users
8. Government's priorities for health funding
9. Other criteria that PHARMAC thinks are relevant (with appropriate consultation).

Clinical evidence

– the key building block

Clinical evidence is core and is the fundamental building block for all CUAs. We always want to conduct a robust and fair assessment of all relevant clinical evidence.

Clinical evidence is used to find out how effective new medicines are compared with currently funded alternatives. Clinical evidence comes in different forms and levels of quality. PHARMAC considers both the strengths and weaknesses of the evidence. The highest quality evidence for assessing the health benefits and risks of a medicine is usually in the form of well conducted randomised controlled trials.

We take advice from our expert clinical advisors on PTAC - the Pharmacology and Therapeutics Advisory Committee - and also clinical advisors on its specialist subcommittees. These committees provide us with access to advice from over 50 senior practising clinicians.

Members of PTAC have broad experience and knowledge of medicines and the conditions they treat, and are specialists in the critical appraisal of evidence. PTAC discusses the ins and outs of clinical trials (and other evidence) and advises PHARMAC on the relevant clinical inputs to use for the CUA. It also considers PHARMAC's decision criteria, before recommending to PHARMAC whether a medicine should be funded, and at what priority. PTAC's objective advice is one of the factors that PHARMAC takes into account when making decisions.

A key challenge in assessing evidence is that clinical trials often follow patients for only short periods of time. It is often not known, therefore, whether a treatment shown to provide benefits for a short period of time (e.g. 3 years) also provides life-long benefits. As part of CUA we can test different future predictions and scenarios.



Assessing benefits

– Quality-Adjusted Life Years (QALYs)

We estimate the benefits of medicines in CUA by calculating the change in QALYs - Quality-Adjusted Life Years. In measuring QALYs, we combine a treatment's effects on how much longer we live (quantity of life) with how much better we live (quality of life).

A good way to think of QALYs is as a common currency. This allows us to fairly compare the health outcomes associated with different medicines, such as something for treating cardiovascular disease and something for treating bowel cancer.

The quality-adjusted life year (QALY) - captures a treatment's effect on the quantity (**living longer**) and health-related quality of life (**living better**).

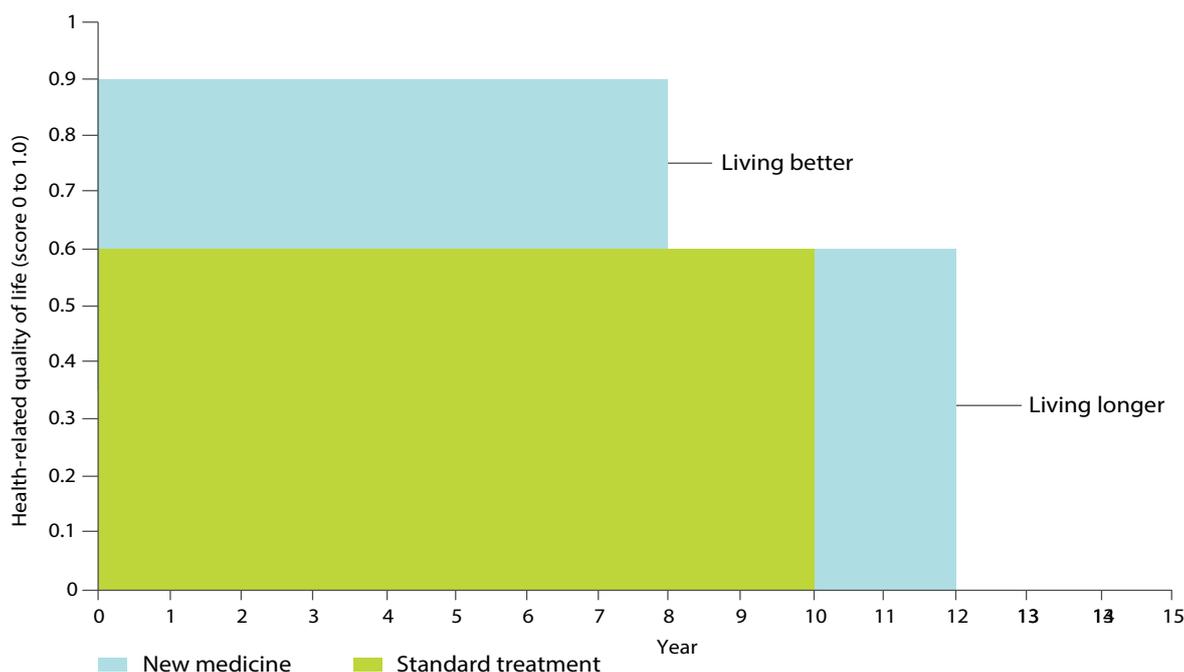
There are well-established ways of measuring the effect of a treatment on quality of life. These include looking at factors such as impact on mobility; ability to self care; ability to undertake usual activities (e.g. work, study, or leisure); levels of pain and discomfort; and anxiety and depression.

There is extensive international information about these factors, which allow people to 'score' illnesses - and score the improvement in health from taking a medicine. We also have information from a survey of New Zealanders about their perspectives on illness and the impact of the illness on their quality of life.

The overall benefits offered by a treatment are the additional QALYs it offers - health gains from living longer and/or better.

QALY = change in health-related quality of life x change in quantity of life
= (living better) x (living longer)

Increasing QALYs through improving health-related quality of life and living longer



We sometimes hear that PHARMAC should include 'return to work' benefits where a medicine allows someone to resume employment. We do include these benefits. Undertaking 'usual activity' (such as paid work) is part of measuring improvements in quality of life. If we gave more weight to specifically help people get back to work, however, we would unfairly discriminate against people (i.e. children and older people) who do not do paid work.

QALYs are widely used by agencies like PHARMAC in other countries. There is also extensive literature about the measurement and assessment of QALYs. Other benefits from a medicine that are not expressed in the QALY can also be taken into account under PHARMAC's other decision criteria.

Assessing costs

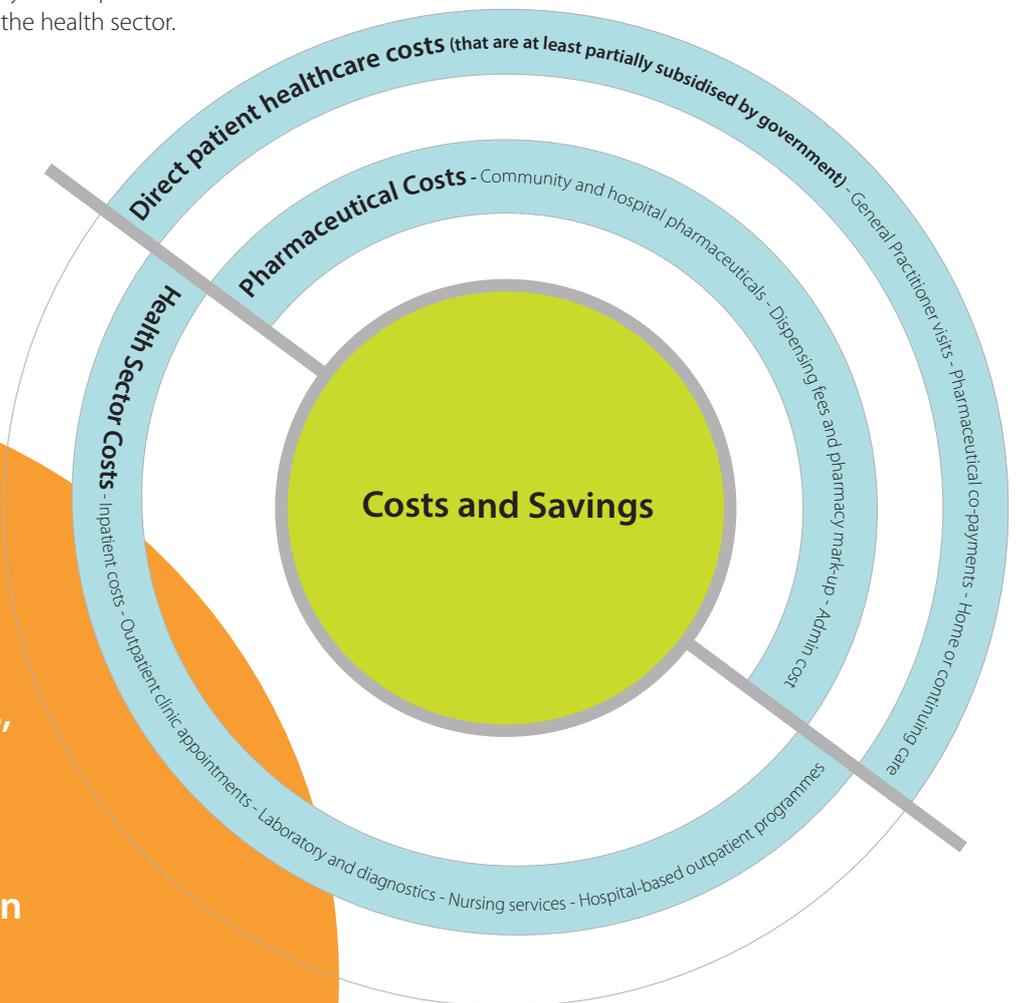
Linking money and health is always difficult. Because we are purchasing medicines from pharmaceutical companies, and spending taxpayer funding, however, PHARMAC has no choice but to consider costs. Just as we do with benefits, we want to ensure we undertake a robust assessment of what the costs would be from funding a medicine.

The obvious cost is what would be paid to the pharmaceutical company for the medicine. This is often called a 'direct cost'. It is relatively easy to determine initially, because it is the price that is offered to us, or a lower price that we believe we could negotiate by promoting competition between companies.

PHARMAC's decisions have long-term implications. Once a medicine is funded, it can be very difficult to stop funding it, even if the medicine has not proven to be as effective as initially thought and expenditure has 'blown out'. It is therefore essential that we get a good handle on the costs and the benefits to New Zealand, not just now but also into the future.

In assessing costs, we also take into account:

- direct patient healthcare costs that are at least partially subsidised by government (such as general practitioner visits, pharmaceutical co-payments, home or continuing care); and
- other costs to the health sector. For example, some medicines require additional services to be provided alongside them (such as diagnosis, testing or infusions), while others may reduce costs from, say, shorter stays in hospital. We consider all such costs and cost offsets to the health sector.



PHARMAC includes pharmaceutical costs, health sector costs (and cost savings) and direct patient healthcare costs when undertaking CUA

Managing risk

– for both benefits and costs

When you're spending your own money, you probably think about the likelihood of getting the benefits you hope to achieve. PHARMAC does the same when deciding about funding a medicine.

Evidence of long-term benefits from medicines can often be lacking, and costs in future years can also be difficult to predict. As a manager of public funding, we need to think carefully about these risks. We want to make prudent investments in medicines, not gamble with the budget.

In general, the more uncertain we are of future benefits and costs, the more difficult it is to make a decision. There are different methods PHARMAC uses to manage risk. These can include:

- sharing risk and cost with pharmaceutical companies (negotiation, expenditure caps and rebates);
- defining access groups (targeting funding to groups most likely to benefit lowers the risk and increases the gains); and
- saying 'no' until better evidence becomes available.

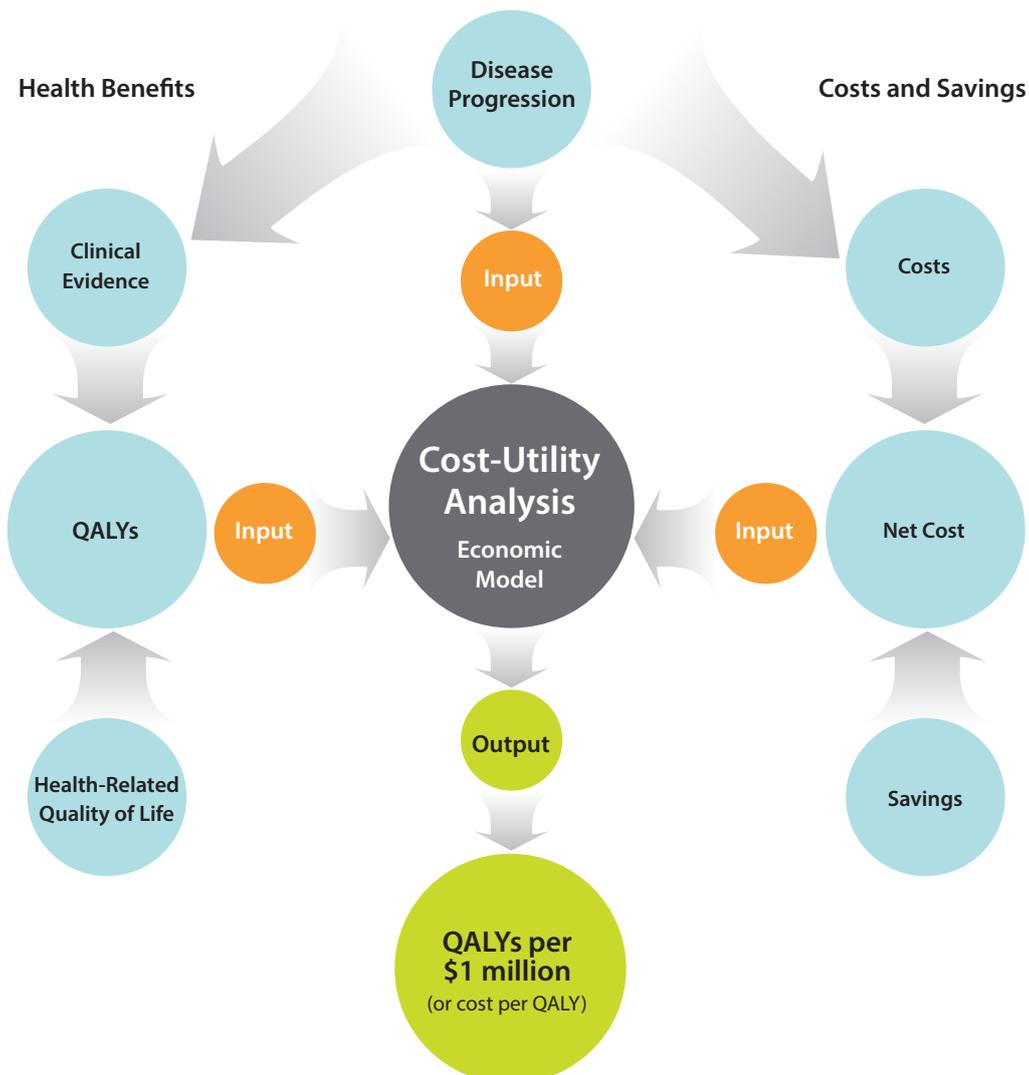
An important role of CUA is to test different scenarios, for both benefits and costs. This is called 'sensitivity analysis'. In this way, we can better understand risks and make more informed decisions.

The Model

– putting it all together

In CUA, neither benefits nor cost information is as meaningful by itself. It is most meaningful to PHARMAC when the information is combined to reflect the 'additional value' of a new treatment.

All of the inputs discussed above are put together into a combined assessment, called a model. This is illustrated in the diagram below.



It is important to remember that the information in the model is not just a single snapshot of time, but a series of information on benefits and costs that runs into the future. We then use standard techniques – widely used in business and across the health sector – to calculate the value of the medicine in today's dollars.

When we do that value assessment, we calculate the 'QALY gains per unit net cost', typically expressed as 'QALYs per \$1 million' – the additional net health benefit (QALYs) obtained for each additional million dollars of the health budget spent. This can also be expressed as the 'cost per QALY', which is the measure used by some health technology assessment and funding agencies elsewhere and previously used by PHARMAC. We often calculate a range for the 'QALYs per \$1 million' and 'cost per QALY' to take account of the risks around both benefits and costs as we discussed earlier.

Formula for QALY gains per \$1 million

$$= \frac{\text{QALYs new treatment} - \text{QALYs current treatment}}{\text{Cost new treatment} - \text{Cost current treatment}} \times 1 \text{ million}$$

(or, expressed differently, the additional cost per QALY)

$$= \frac{\text{Cost new treatment} - \text{Cost current treatment}}{\text{QALYs new treatment} - \text{QALYs current treatment}}$$

Example

A new medicine has become available for advanced bowel cancer. There is already a treatment funded and used widely for advanced bowel cancer. Patients require treatment for six months. You have been asked to assess whether the new treatment is relatively cost-effective to fund; that is, what are the additional health gains and costs of the new treatment compared with current treatment?

● Benefits (QALYs)

On reviewing the clinical evidence, you establish that there has been one randomised controlled trial that has assessed the effectiveness of the new treatment compared with current treatment for treating advanced bowel cancer.

The results of that clinical trial indicate that patients given the new treatment live approximately two months longer (average survival of approximately 12 months) compared with patients administered current treatment (average survival of approximately 10 months). In addition, patients given the new treatment are less likely to have treatment-related nausea and vomiting, therefore their quality of life improves.

Through the use of survey-derived Quality-of-Life scores you establish that patients given the new treatment have a health-related quality of life of 0.6 (on a scale of 0-1), and patients given current treatment have a health-related quality of life of 0.4.

You then calculate the QALY for patients administered the new treatment to be 0.60 for 1 year (1x0.6), compared with 0.33 for 10 months (10/12x0.4) for patients administered current treatment.

The additional QALY gain of the new treatment compared with current treatment is therefore estimated to be 0.27 (0.60 - 0.33).

● Costs

The total cost per patient of 6 months' treatment with the new treatment is \$5,500, compared with a cost of \$900 per patient for current treatment. In addition, current treatment is an infusion that people need to receive at a hospital outpatient unit. This costs approximately \$1,500 per patient. The new treatment is a pill, therefore people can take it at home.

The evidence indicates that 10% of patients in the clinical trial needed to be hospitalised due to severe nausea and vomiting with current treatment, compared with none (0%) of the patients taking the new treatment. The cost of going to hospital is estimated to be \$2,500. There is therefore a "saving" of \$250 per patient (\$2,500x10%) associated with the new treatment.

The total cost of the new treatment is therefore \$5,500, compared with a total cost of current treatment of \$2,650 (\$900+\$1,500+\$250).

The additional cost of the new treatment compared with current treatment is therefore estimated to be \$2,850 (\$5,500 - \$2,650).

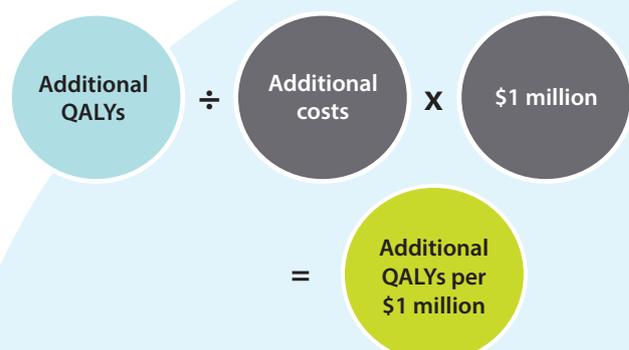
● Additional QALY gains per \$1 million

From your calculations you have established that the additional QALY gain of the new treatment compared with current treatment is 0.27.

You have also estimated the additional net cost of funding the new treatment is \$2,850.

The QALY gains per \$1 million are therefore the additional QALYs divided by the additional cost, multiplied by 1 million (0.27/\$2850x1M). This gives a result of about 95 QALYs gained per \$1 million spent.

Therefore, for every million dollars of the total health budget invested in the new treatment, an additional 95 units of benefit (QALYs) would be gained. This result can also be presented as a 'cost per QALY' (additional net cost divided by QALY gain), giving a result of approximately \$10,600 (i.e. cost of \$10,600 for each QALY gained).



Using the results of cost-utility analysis

Remember that CUA is done by comparing a new medicine with the existing standard treatment (taking into account the changes in both benefits and costs). This means that the 'QALYs gained per \$1 million spent' gives us information on the 'additional value' of a new treatment. The QALYs per \$1 million tells us how many QALYs we gain per million health dollars spent.

'Relative Value' – comparing the 'QALYs per \$1 million' result across different medicines

The 'QALYs per \$1 million' is also very useful for another reason. Remember that we use QALYs because they are a standard currency that can allow comparison of different treatments (e.g. cardiovascular medicines vs. cancer medicines). So using them allows us to compare the 'QALY gains per unit net cost' for different medicines – 'relative cost-effectiveness' – and improve our knowledge about which medicines offer the best health outcomes for New Zealand.

Relative value

– 'QALYs per \$1 million' compared across different medicines for different medical conditions

For example, the QALYs per \$1 million results for a variety of medicines for the following medical conditions could be:

High blood pressure 67-100 QALYs gained per \$1 million (or \$10,000-\$15,000 per QALY)

Epilepsy 20-33 QALYs gained per \$1 million (or \$30,000-\$50,000 per QALY)

Osteoporosis 100-200 QALYs gained per \$1 million (or \$5,000-\$10,000 per QALY)

Asthma 10-12 QALYs gained per \$1 million (or \$80,000-\$100,000 per QALY)

The QALYs per \$1 million results allow us to assess the 'relative cost-effectiveness' across different medicines that treat different medical conditions. Because a consistent method has been used across all medicines, we can make choices and prioritise treatments for investment decisions. Cost-effectiveness is one of the nine decision criteria PHARMAC considers when prioritising medicines for funding.

Use of a threshold?

We are often asked whether we have a 'cost per QALY' threshold - a particular trigger point for deciding when a medicine will be funded. We don't, for good reason. Remember CUA only helps us with some of our decision criteria, and other factors remain important.

A threshold is also incompatible with a fixed budget, however big. We can't guarantee to fund everything; we have to choose those with the best value within the funding available. This also has important implications for how we undertake CUAs.

When doing CUA we do as much work as we need to feel confident that we can rank one funding proposal against another, including consideration of the associated risks. This can sometimes be done quite quickly; in other cases more work is required.

Price setting?

We are also often asked whether the cost per QALY determines the price for a medicine that PHARMAC is happy to pay. It doesn't. The results of a CUA help us rank the funding options from best value to least value. We then try to agree with pharmaceutical companies for the supply of the better options, including negotiating on price or using other purchasing tools designed to promote competition between companies.

Summary

Why use CUA?

CUA is a tool that helps us better understand the costs and benefits of funding a medicine. CUA helps us understand how much better a new medicine is than existing medicine. CUAs for different medicines can also be compared, so that we choose to fund the medicines that offer the best health outcomes.

Assessing benefits using QALYs

In measuring QALYs, we are estimating a medicine's effect on **how much longer** we live and on **how much better** we live. QALY assessment is a standard tool used internationally.

Assessing costs

We include costs of the medicine itself, and any other costs to the health sector, or the ability of medicines to save other health sector costs.

Combining benefits and costs

By putting benefits and costs together in a model, we calculate the QALYs per \$1 million - the number of additional health benefit units (QALYs) gained from each additional \$1 million spent of the total health budget. We can also compare the additional QALYs per \$1 million across different medicines - helping us to make the best possible funding choices.

Cost-Utility Analysis (CUA) at PHARMAC

Questions and Answers

What is CUA?

An analytical tool that helps PHARMAC assess whether a medicine should be funded ahead of others. CUA helps PHARMAC assess the relative value of funding a medicine. Both benefits and costs of medicines are considered. CUA is widely used internationally by other medicine funders like PHARMAC. It is, however, only a part of our considerations under our decision criteria when making funding decisions, as not everything can be analysed in a CUA.

What is a QALY?

A QALY, Quality-Adjusted Life Year, is a measure of the benefits of a medicine. QALYs are a composite measure that combines a medicine's effects on how long we live with how well we live. The best way to think of QALYs is as a common currency. QALYs can be fairly and robustly calculated for medicines used for quite different purposes, making it possible to compare funding choices.

What about benefits of medicines that allow people to return to work?

When measuring changes in quality of life, we include any benefits from resuming normal activity, so return-to-work benefits are included. Placing more weight on returning to work than this would disadvantage people who do not do paid work.

What costs does PHARMAC consider?

We consider the direct costs of the medicine, plus any other relevant health costs, such as diagnostics or infusions. Some medicines can also save costs elsewhere in the health sector (e.g. reduced time in hospital), so we count those cost offsets as well. Healthcare costs to the patient (e.g. the full cost of a GP visit or rest home care) are also included.

What is 'QALY gains per unit net cost' or 'QALYs per \$1 million'?

We always look at both the benefits and costs of medicines. The QALYs per \$1 million is the overall value, taking into account both benefits and costs. It is the QALYs gained per dollar spend, and can also be expressed as the cost per QALY gained. It is the additional QALYs per unit net cost that is important - the change in QALYs and costs compared with standard treatment. This is because PHARMAC needs to assess how much better and how much more costly a new medicine is compared with current treatment when determining the value of a new medicine. The result will usually be expressed as QALYs per \$1 million rather than QALYs per dollar due to the scale of the values involved.

'QALYs per \$1 million' is used in the same way as the 'cost per QALY' measure is used by some health technology assessment and funding agencies elsewhere.

What is 'relative assessment'?

Different CUAs can be compared to help PHARMAC decide which medicines should be funded before others - this comparison is called 'relative assessment'. This is a very useful feature of CUA. PHARMAC's role is to achieve the best health outcomes from available funding, so we have to know that we are choosing the best funding options.

Does PHARMAC use a cost per QALY threshold?

No. CUA helps us rank funding options, so that we focus our work on the medicines offering the best health outcomes for New Zealand. We measure value by using nine decision criteria - a key reason why the results of CUA are not any sort of threshold. A threshold is also incompatible with a fixed budget - however big - because we could never guarantee to fund everything that met a threshold.

Why does PHARMAC not fund some medicines known to be effective?

Some medicines can be clinically effective, but also very expensive. We try to assess the amount of benefit achieved per unit net cost. This means that compared with other funding options we have, the medicine may not be the best choice to provide the best health outcomes for New Zealand. Some effective new medicines may also not provide 'additional value', meaning they may not be much better than existing medicines we already fund.

Does the amount of people who could benefit from a medicine make a difference?

CUA estimates the additional benefits of medicines being considered for funding on a per patient basis. So the size of the patient population does not affect the result of a CUA. Population size, however, does impact on the total health gains (total QALYs) across the entire patient population. The patient population size relates to the total cost of a proposal and therefore the budget impact and financial risk (one of PHARMAC's decision criteria) - and hence opportunity cost. So to this extent the size of the patient population does have an impact on PHARMAC's decisions.

Does CUA favour medicines that prolong life over those that improve quality of life?

No. The QALYs gained from a new medicine compared with existing standard treatment take into account both extension of life and improvements in quality of life. The QALY gains could be greater in either type of medicine. The balance between quality and quantity of life is a key consideration of the way QALYs are calculated.

PHARMAC

Pharmaceutical Management Agency

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