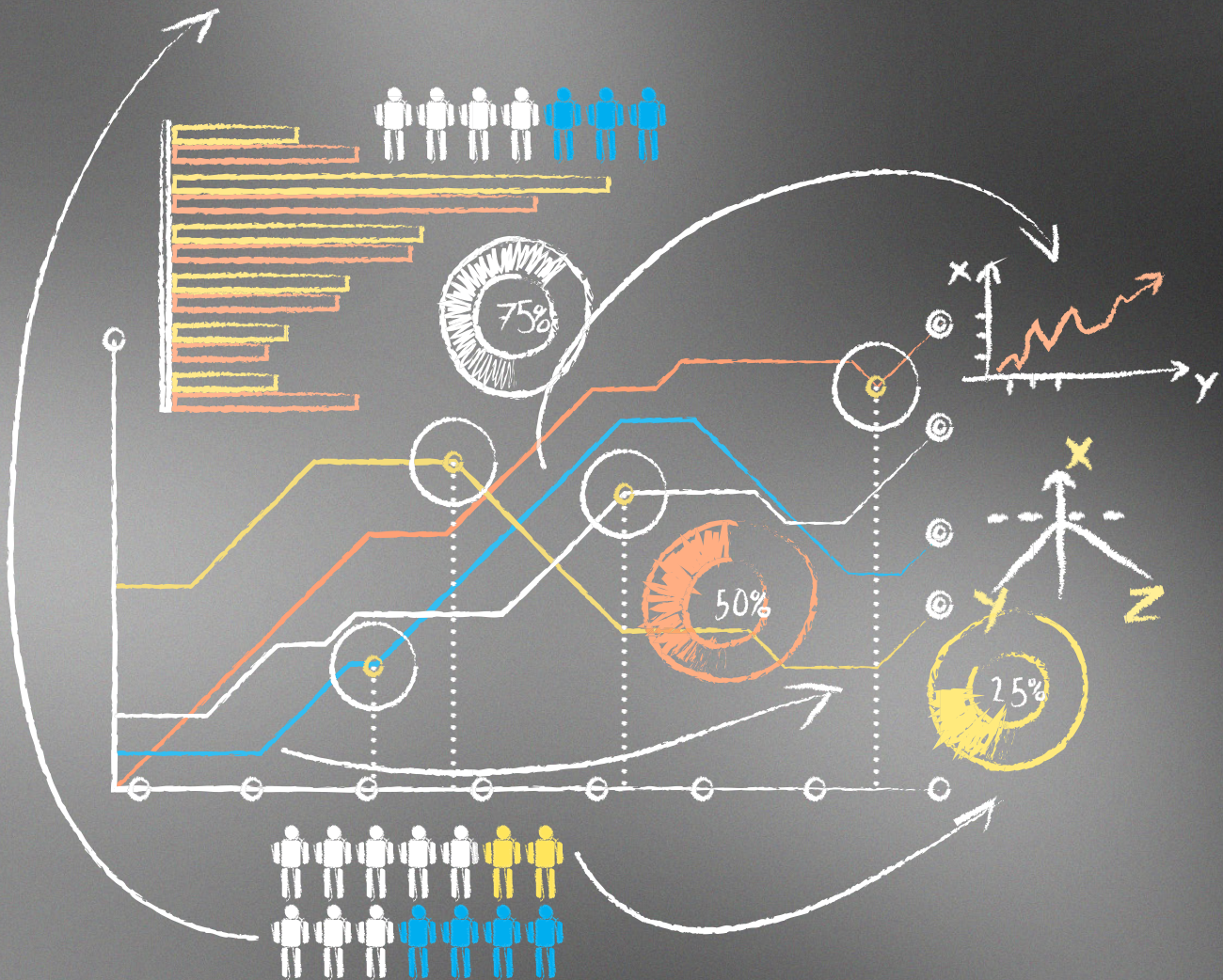


Pharmaceutical Management Agency

Cost-Utility Analysis (CUA) Explained



Cost-Utility Analysis (CUA) at PHARMAC

Questions and Answers go to page 9 >>

This document explains the process that PHARMAC generally uses 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.health.nz/economic-analysis

The PFPA is a more detailed guide to PHARMAC's general 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 'Your guide to PHARMAC':

www.pharmac.health.nz/your-guide-to-pharmac/

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Introduction

PHARMAC is the government agency charged with deciding which pharmaceuticals will be publicly funded. Our objective is to, in effect, get the best health outcomes we reasonably 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 help inform our decisions on which pharmaceuticals to fund. This guide explains the CUA process.

Why use CUA?

- How much better is this new pharmaceutical than those we already fund?
- What is the 'value' of the new pharmaceutical? Is any additional benefit worth it?
- Will funding this pharmaceutical 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 that can be applied to all kinds of health technologies, including the medicines, vaccines, and medical devices that fall within the statutory definition of 'pharmaceutical' for PHARMAC's purposes.

The type of questions we face might sound familiar to anyone on a limited budget. Would it be better overall to buy an expensive car, or buy a cheaper one and save for a holiday? Clearly every choice has benefits and also costs – 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 pharmaceutical has an 'opportunity cost' because there is less money for other pharmaceuticals 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 pharmaceuticals that could improve the lives of New Zealanders.

CUA: a quick overview

Pharmaceuticals treat a wide range of medical conditions, from minor to fatal, and with very different benefits and costs associated with their use. Similarly, PHARMAC may consider many kinds of health technologies to address these conditions. CUA is the assessment of the additional benefits and costs associated with treatments.

When assessing a new pharmaceutical, we want to know how much better it is, and how much more it costs, than the pharmaceuticals or other treatments that are publically available. This comparison is made against standard clinical practice in New Zealand.

Assessing benefits

The benefits of a pharmaceutical in CUA are estimated using 'quality-adjusted life years' (QALYs). QALYs are a measurement that can be used to compare benefits of different treatments in a consistent and standardised way. 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 and savings

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 pharmaceuticals 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 net benefits and net 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 pharmaceutical is than other pharmaceuticals. We can compare the results of assessments for different pharmaceuticals – this is called 'relative cost-effectiveness'.

CUA helps us apply our Factors for Consideration – the standard set of Factors we judge pharmaceuticals against (see below). CUA combines information from Factors relating to health benefits and Factors relating to costs and savings. Our base case CUAs focus on comparing the health benefits to the patient with the related costs to District Health Boards. More details are in the Prescription for Pharmacoeconomic Analysis, which sets out the technical details behind a CUA.

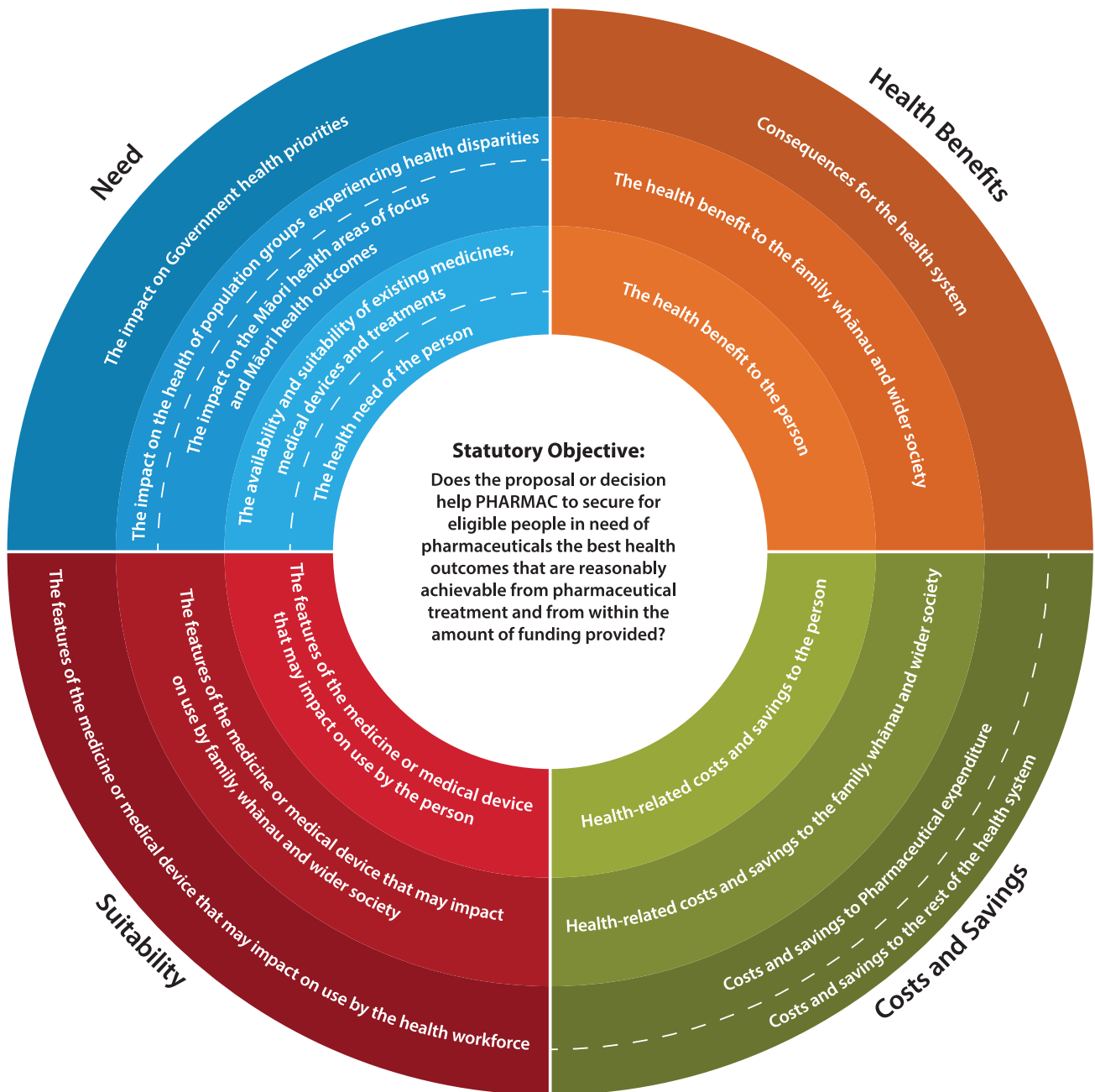
Although we have focussed above on assessing new pharmaceuticals, CUA is also helpful for assessing whether access to existing pharmaceuticals should be widened, to allow for different uses of the same pharmaceutical.

How we decide

PHARMAC's Factors for Consideration

The Factors for Consideration group into four different dimensions (Need; Health Benefits; Costs and Savings; and Suitability), and the three levels of impact (to the person; to the person's family, whānau and wider society; and to the broader health system), seen in the following diagram:

PHARMAC's Factors for Consideration ¹



¹ Please note that, although not explicit on this diagram, PHARMAC will take into account the health needs of the family, whānau, and wider society during our decision making process. More details on this are available on our website at www.pharmac.health.nz/medicines/how-medicines-are-funded/factors-for-consideration/supporting-information/.

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 pharmaceuticals are compared with currently funded alternatives. Clinical evidence comes in different forms and levels of quality. While the highest quality evidence for assessing the health benefits and risks of a pharmaceutical is usually in the form of well conducted randomised controlled trials, clinical evidence of any quality can support an application. PHARMAC receives applications supported by evidence of varying quality. Each application is assessed on the merits of the evidence available.

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 120 senior registered health professionals.

Members of PTAC have broad experience and knowledge of pharmaceuticals 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 Factors for Consideration, before recommending to PHARMAC whether a pharmaceutical should be funded, and at what priority. PTAC’s objective advice is one of the key pieces of information 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 also provides life-long benefits. As part of CUA we can test different future predictions and scenarios.

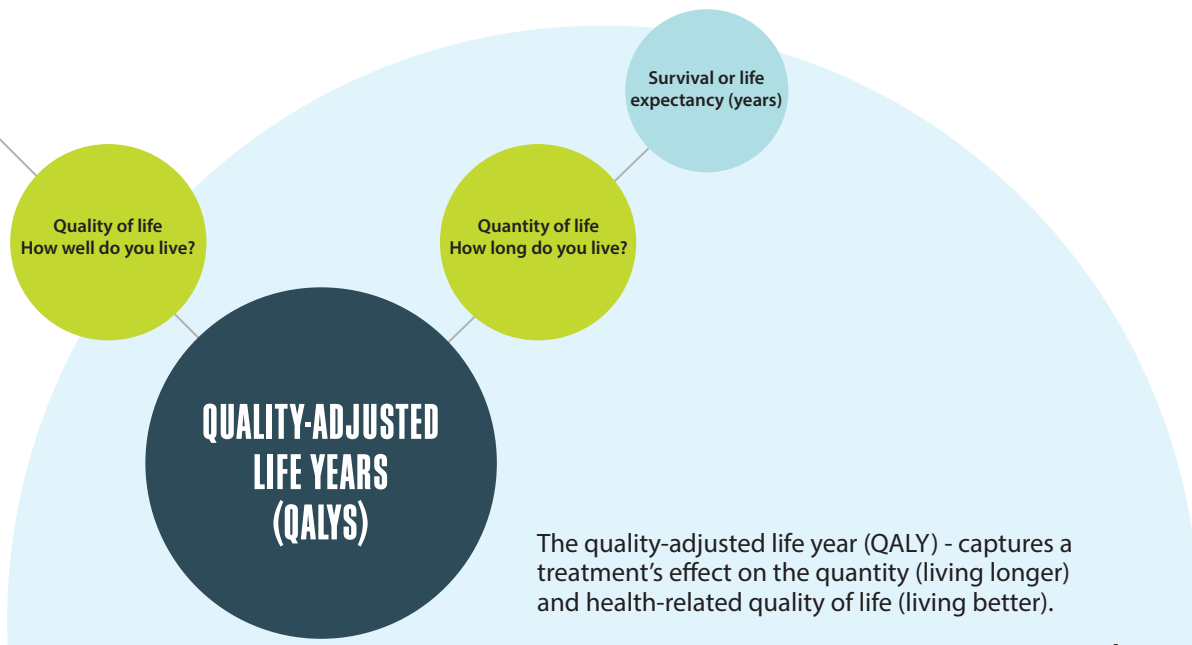


Assessing benefits

Quality-Adjusted Life Years (QALYs)

We estimate the benefits of pharmaceuticals 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 measure. This allows us to fairly compare the health outcomes associated with different pharmaceuticals, such as one for treating cardiovascular disease and one 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 aspects such as impact on mobility, ability to self-care, ability to undertake usual activities (eg work, study, or leisure), levels of pain and discomfort, and anxiety and depression.

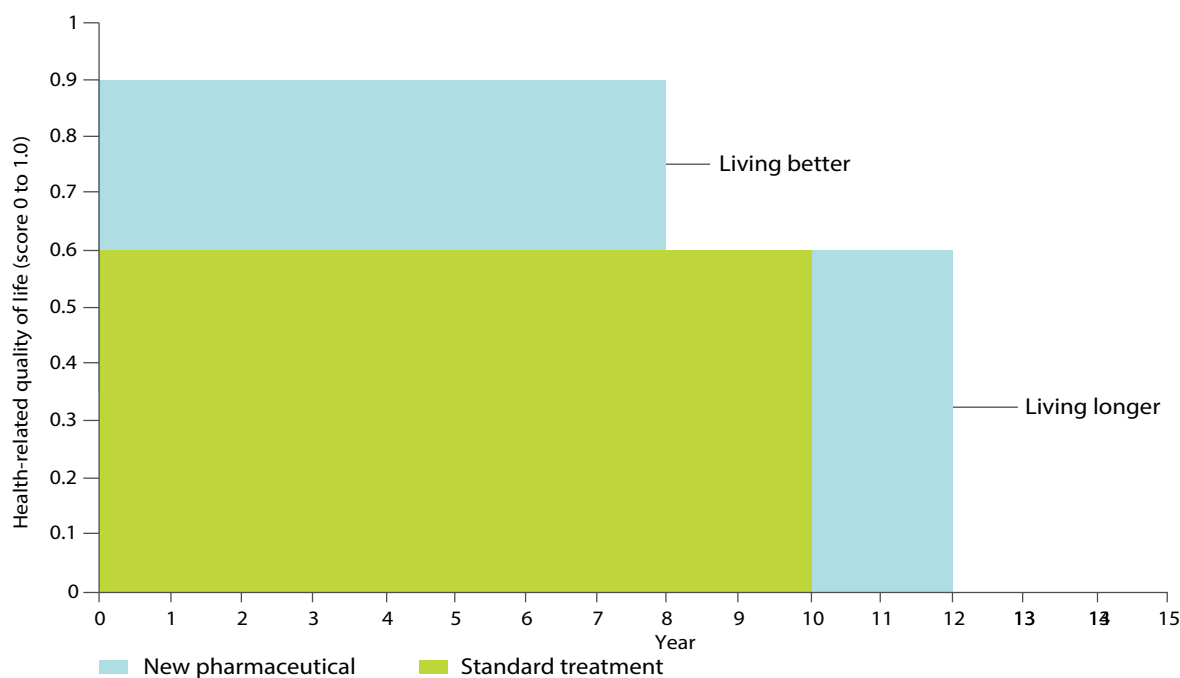
There is extensive international information about these aspects, which allow people to 'score' illnesses – and score the improvement in health from taking a pharmaceutical. We also have information from a survey of New Zealanders about their perspectives on illness and the impact of the illness on their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

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

$$\text{QALY} = \text{change in health-related quality of life} \times \text{change in quantity of life}$$

$$= (\text{living better}) \times (\text{living longer})$$

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 pharmaceutical allows someone to resume employment. In our assessments, we count inability to do 'usual activity' – including paid work – as a health loss. This way we fairly compare all loss of activity no matter whether it is lost by a child, an employed or unemployed person, or a retiree.

QALYs are widely used by agencies like PHARMAC in other countries. There is also extensive literature about the measurement and assessment of QALYs. Other health benefits from a pharmaceutical that are not expressed in the QALY will be taken into account under PHARMAC's other relevant Factors for Consideration.

Assessing costs and savings

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

The obvious cost is what would be paid to the supplier for the pharmaceutical. 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 pharmaceutical is funded, it can be very difficult to stop funding it, even if the pharmaceutical has not proven as effective as initially thought or if its expenditure exceeds our budgets. 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 if they 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 pharmaceuticals require additional services to be provided alongside them, such as administration costs or costs of training to use the product, 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 in CUAs

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 pharmaceutical.

Evidence of long-term benefits from pharmaceuticals can often be lacking, and costs in future years can also be difficult to predict. As a manager of public funding, we would be neglectful if we didn't carefully think about these risks. We want to make prudent investments in pharmaceuticals, 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
- deciding to wait until better evidence becomes available.

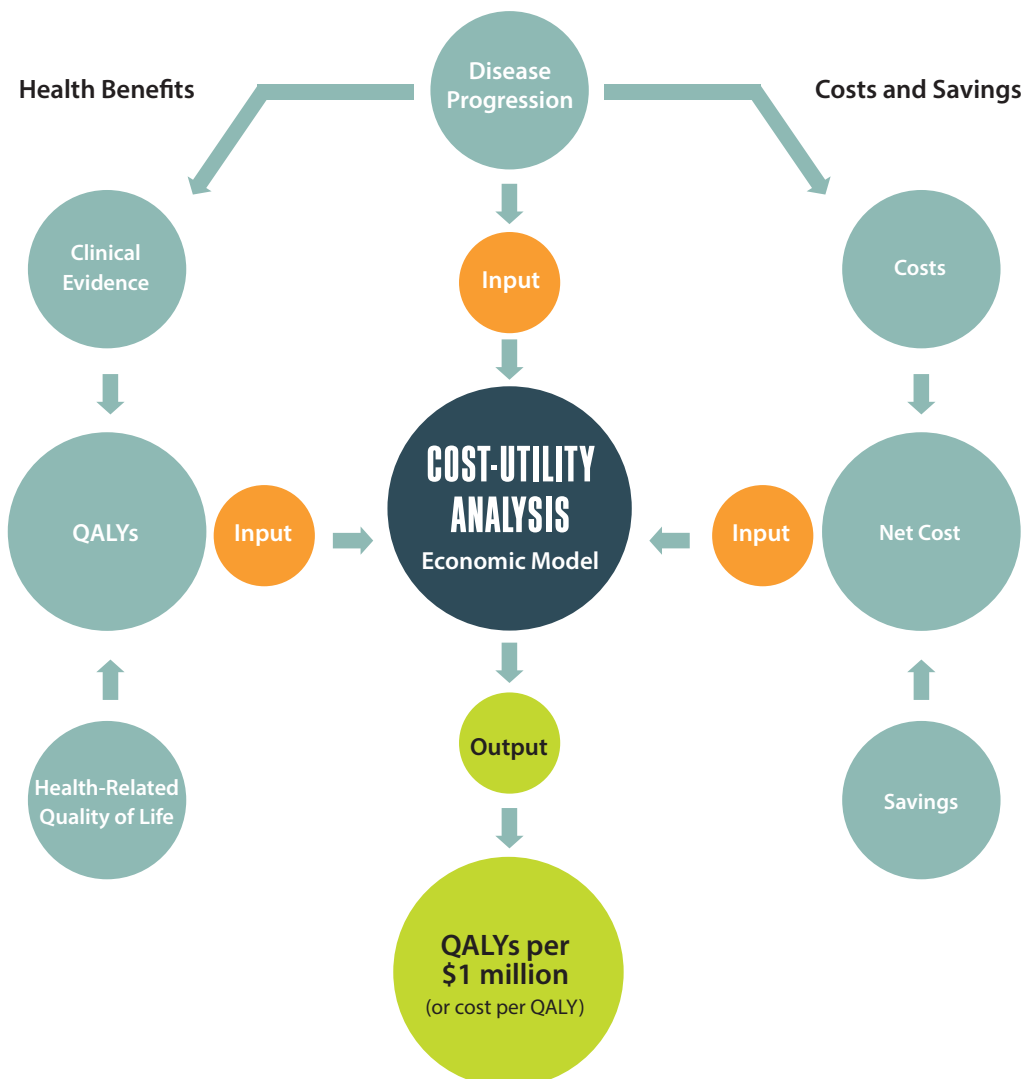
An important role of CUA is to test different scenarios, for both benefits and costs. This is called 'sensitivity analysis'. In this way, we can better understand risks and make more informed decisions.

The Model

Putting it all together

In CUA, neither benefits nor cost information are enough in themselves to inform a funding decision. 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. The model is what, overall, reflects CUA. 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, costs, and savings 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 pharmaceutical 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

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

Example

A new pharmaceutical 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 assessed the effectiveness of the new treatment compared with current treatment for treating advanced bowel cancer.

The results of that clinical trial indicated 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 were 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 (1×0.6), compared with 0.33 (10/12×0.4) 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 and savings

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. Total infusion cost is about \$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 (\$2,500×0.1) 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).

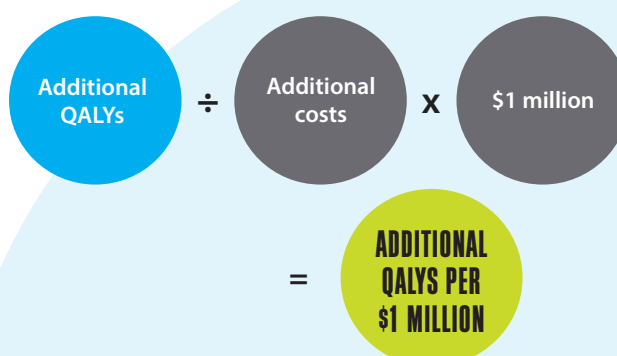
● Additional QALY gains per \$1 million

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

You also estimate 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/\$2,850×1m). 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 'cost per QALY' (additional net cost divided by QALY gain), giving a result of approximately \$10,600 (ie cost of \$10,600 for each QALY gained).



Using the results of cost-utility analysis

Remember that CUA is done by comparing a new pharmaceutical 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 cost-effectiveness result across different pharmaceuticals

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 (eg cardiovascular pharmaceuticals vs. cancer pharmaceuticals). So using them allows us to compare the 'QALY gains per unit net cost' for different pharmaceuticals – 'relative cost-effectiveness' – and improve our knowledge about which pharmaceuticals offer the best health outcomes for New Zealand.

Relative value

Comparing the cost-effectiveness across different pharmaceuticals for different medical conditions

For example, the cost-effectiveness results for a variety of pharmaceuticals for the following medical conditions could be:

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

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

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

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

The QALYs per \$1 million results allow us to assess the 'relative cost-effectiveness' across different pharmaceuticals that treat different medical conditions. Because a consistent method has been used across all pharmaceuticals, we can make choices and prioritise treatments for investment decisions. Cost-effectiveness combines information from Factors relating to Health Benefits and Costs and Savings that PHARMAC uses when making decisions for funding.

Use of a threshold?

We are often asked whether we have a 'cost per QALY' threshold – a particular trigger point for deciding a pharmaceutical will be funded. We don't, for good reason. Remember CUA only combines information from some of our Factors for Consideration, 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 pharmaceutical 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 pharmaceutical. CUA helps us understand how much better a new pharmaceutical is than existing uses of health funds. CUAs also allow different pharmaceuticals to be compared. This can help us identify which options offer the best health outcomes. The base case CUA provides a summary of the Factors relating to the benefits of a treatment for a patient when compared to Factors relating to costs and savings, and helps to inform the relative ranking of a proposal along with other information on all the Factors for Consideration.

Assessing benefits using QALYs

In measuring QALYs, we are estimating a pharmaceutical'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 and savings

We include costs of the pharmaceutical itself, and any other costs to the health sector, or the ability of pharmaceuticals to create savings elsewhere in the health sector.

Combining benefits and costs

By putting benefits, costs and savings 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 pharmaceuticals – helping us 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 pharmaceutical should be funded, relative to other funding options. CUA combines the benefits, costs and savings of a pharmaceutical. CUA is widely used internationally by other pharmaceutical funders. It is only a part of our considerations as not everything can be analysed in a CUA. Our Factors for Consideration detail the things we may take into account when making funding decisions or decisions around which proposals we should pursue.

What kinds of interventions can CUA assess?

CUA can be used to assess any intervention that would affect the health of patients and health system expenditure. This means that CUA can be used to examine everything that falls within the legislative definition of 'pharmaceutical' for PHARMAC's purposes, including medicines, vaccines, medical devices, and other kinds of interventions. The details of assessing different interventions will vary, including how the health gains are achieved and the kinds of costs and savings that are affected, but the premise – comparing net health gains to net costs – is applicable to all interventions PHARMAC looks at.

What is a QALY?

A QALY, Quality-Adjusted Life Year, is a measure of the benefits of a pharmaceutical. QALYs are a composite measure that combines 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 pharmaceuticals used for quite different purposes, so we can compare funding choices.

What about benefits of pharmaceuticals 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?

In a base case CUA, we consider all costs and savings to the health system. This includes the cost of the pharmaceutical, plus any other effects on health costs the pharmaceutical has, such as infusion costs or reduced hospital time. Healthcare costs to the patient are also included but only if the health sector pays for part of it, such as the full cost of a GP visit or rest home care. Direct costs to the patient, or to their family, whānau, or wider society, are not included in the base case CUA but can be considered in other scenarios, or outside the CUA through the relevant Factors for Consideration.

What is 'QALYs per \$1 million'?

QALYs per \$1 million is a way of reporting a CUA result. It reports the additional QALYs gained divided by the each additional \$1 million spent. As with CUA itself, it looks at the change in QALYs and in net costs compared with current treatment. This is because PHARMAC looks at how a pharmaceutical will change things compared with the current situation.

'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'?

Because PHARMAC needs to choose between different funding decisions, it is useful to have a measure which allows us to compare proposals against one another. CUAs, as part of the Factors assessment, allow a direct comparison of funding options to help PHARMAC decide which options should be funded before others. This comparison is called 'relative assessment'. 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. PHARMAC has multiple Factors for Consideration, so a threshold on one measure would be inappropriate. Further, PHARMAC has a fixed budget, which changes our ability to fund options. If we committed to funding anything above a certain threshold, we may not have the funds available to achieve that. Similarly, if we committed to rejecting options below a threshold, we may have to leave some of our budget unspent. The standard for funding in any given year depends on the budget and on the quality and number of funding options.

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

Some pharmaceuticals can be clinically effective, but also very expensive. To fund any pharmaceutical means that those funds cannot be spent on other alternatives. Those alternatives may be a better choice to provide the best health outcomes for New Zealand. Some effective new pharmaceuticals may also not provide 'additional value', meaning they may not be much better than an existing pharmaceutical we already fund.

Does the number of people who could benefit from a pharmaceutical make a difference?

CUA estimates additional benefits 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 cost of a proposal which affects financial risk. This is relevant to our Statutory Objective and some Factors for Consideration. So to this extent the size of the patient population does have an impact on PHARMAC's decisions.

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

No. The QALYs gained from a new pharmaceutical take into account both extension of life and improvements in quality of life. The QALY gains could be greater in either type of pharmaceutical. The balance between quality and quantity of life is a key consideration of the QALY composite measure.

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