

Pharmaceutical Management Agency

# Cost-Utility Analysis (CUA) Explained

## Cost-Utility Analysis (CUA) at PHARMAC

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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):

**<http://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:

**<http://www.pharmac.govt.nz/patients/AboutPHARMAC/infosheets>**

*Printed October 2009*

# 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?

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/  
Government objectives
9. Other criteria that PHARMAC thinks are relevant  
(with appropriate consultation).

# Clinical evidence

## – the key building block

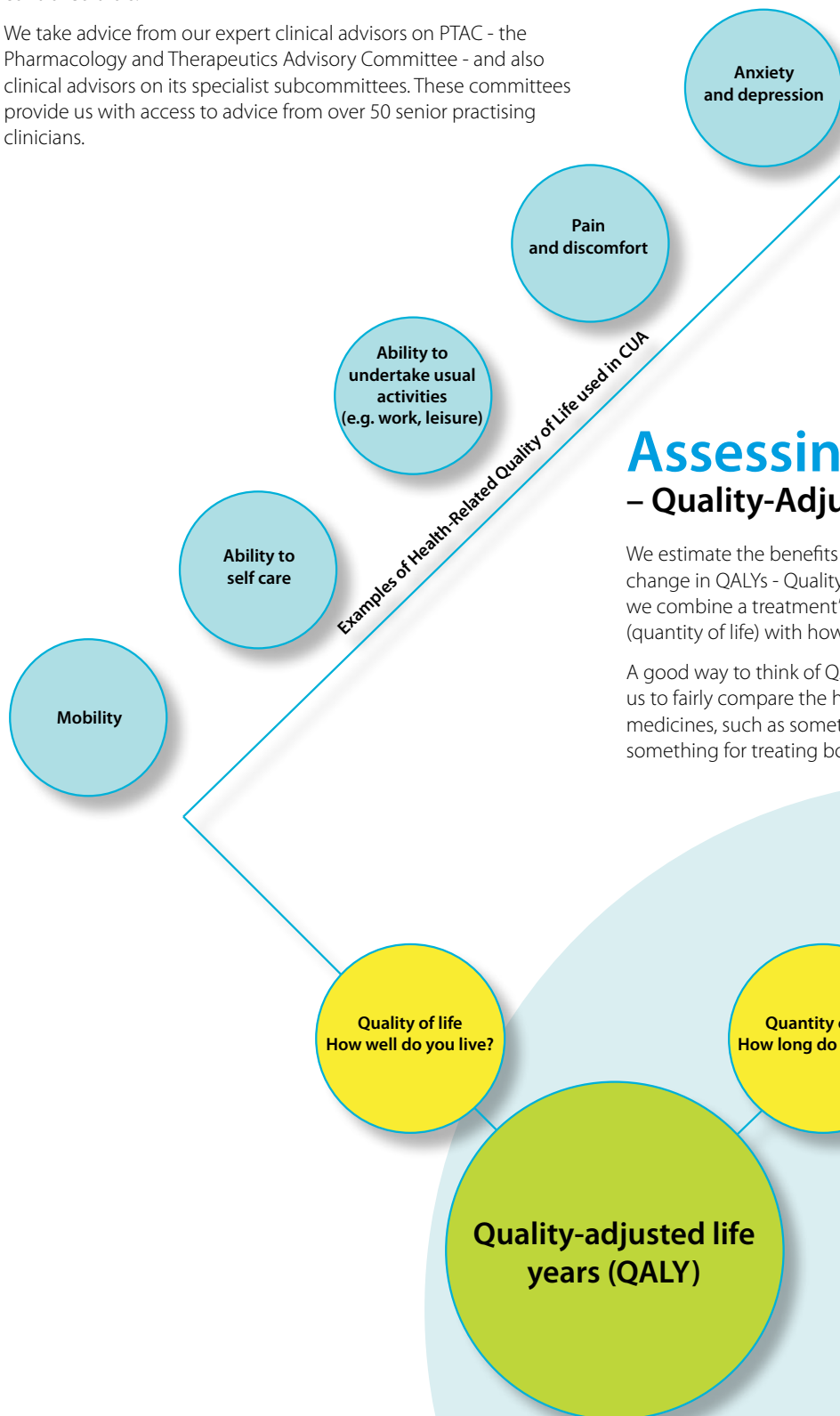
Clinical evidence is core and is the fundamental building block for all CUAs. We always want to conduct a robust and fair assessment of all relevant clinical evidence.

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**).

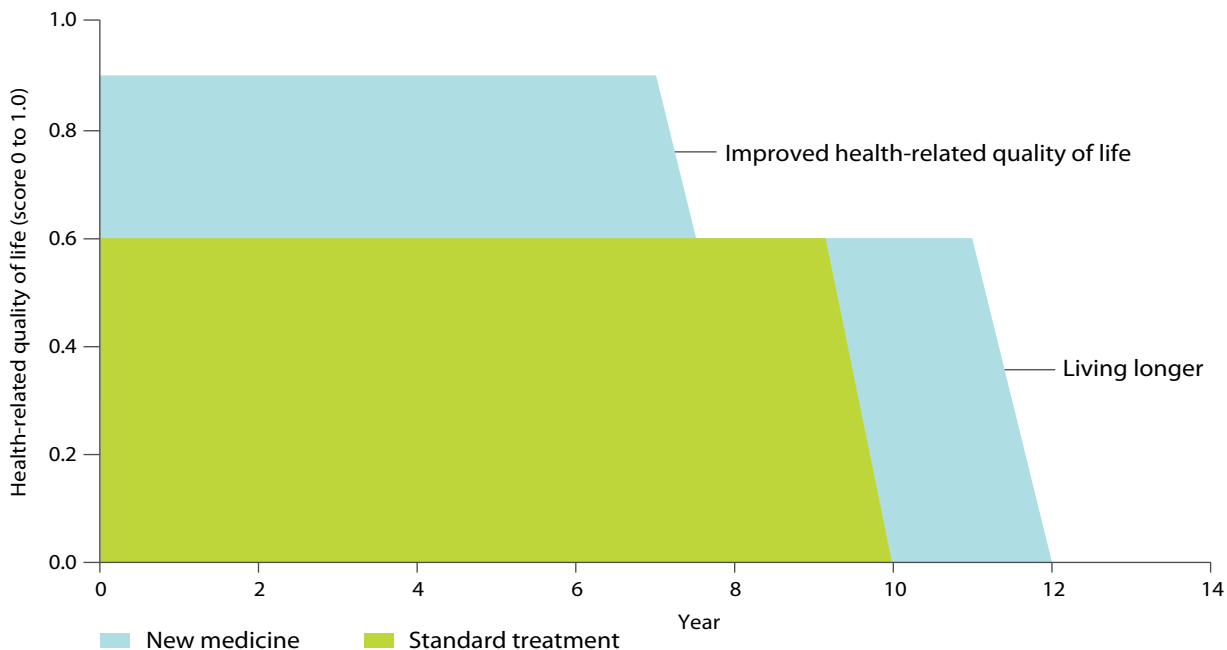
There are well-established ways of measuring the effect of a treatment on quality of life. These include looking at factors such as impact on mobility; ability to self care; ability to undertake usual activities (e.g. work, study, or leisure); levels of pain and discomfort; and anxiety and depression.

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.

$$\text{QALY} = \text{change in health-related quality of life (living better)} \times \text{change in quantity of life (living longer)}$$

## 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.

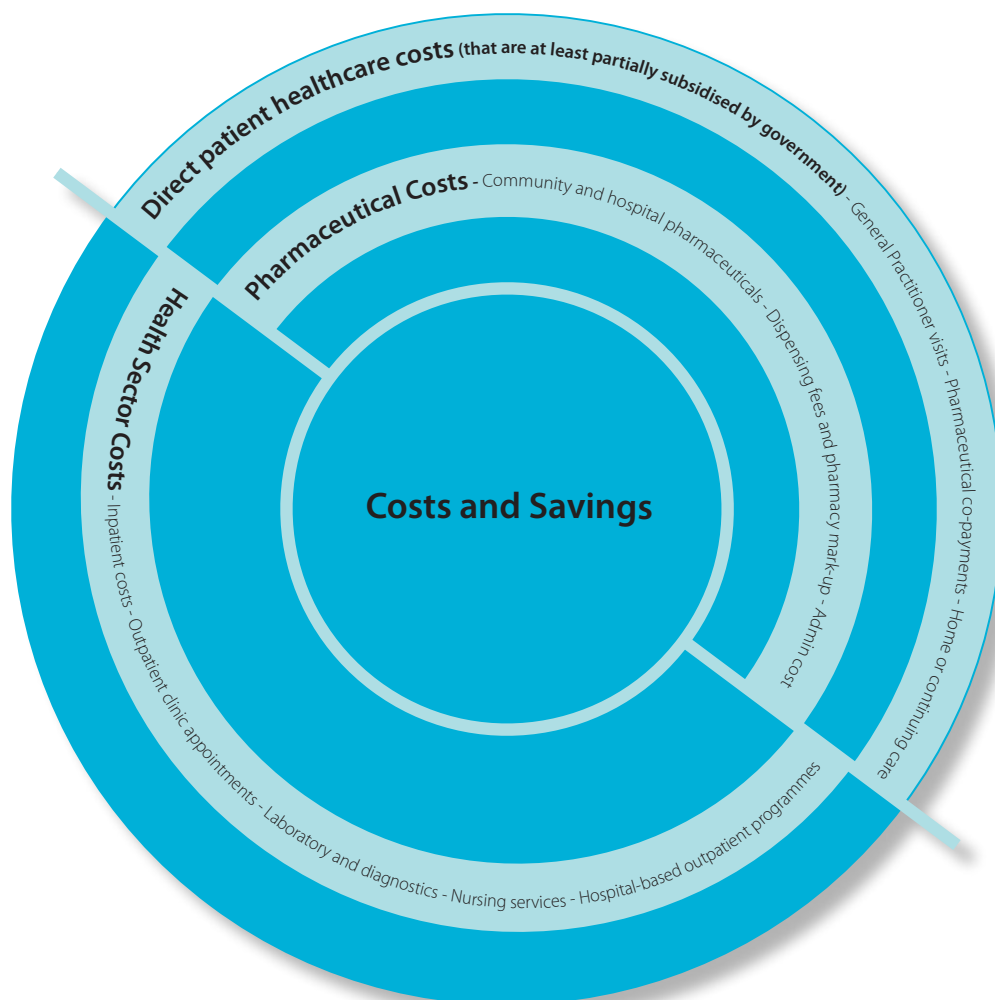
The obvious cost is what would be paid to the pharmaceutical company for the medicine. This is often called a 'direct cost'. It is relatively easy to determine initially, because it is the price that is offered to us, or a lower price that we believe we could negotiate by promoting competition between 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 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 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 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 would be neglectful if we didn't carefully think 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.

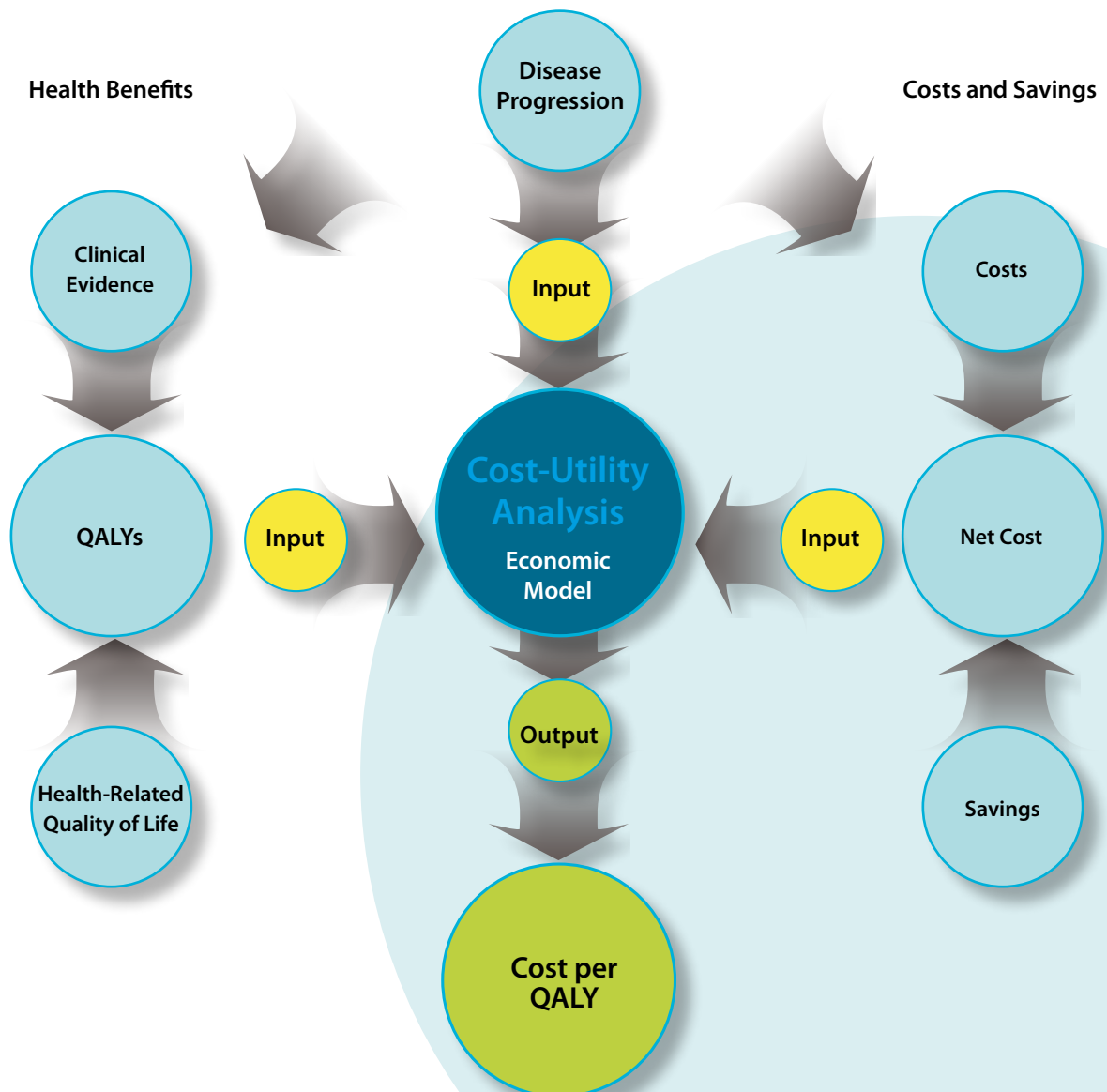
An important role of CUA is to test different scenarios, for both benefits and costs. This is called 'sensitivity analysis'. In this way, we can better understand risks and make more informed decisions.

# 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. The model is what, overall, reflects CUA.



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.

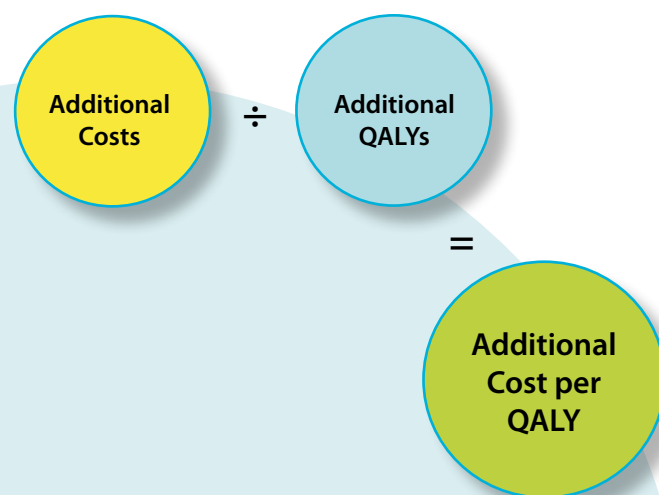
When we do that value assessment, we calculate the ‘cost per QALY’ – the additional net cost per additional health benefit (QALY). We often calculate a range for the ‘cost per QALY’ to take account of the risks around both benefits and costs as we discussed earlier.

## Formula

### – the additional cost per QALY

Net cost of the new medicine – Net cost of standard treatment

Net QALYs of new medicine – Net QALYs of standard treatment



## Example

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

### ● Benefits (QALYs)

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

The results of that clinical trial indicated that patients given Treatment A live approximately two months longer (average survival of approximately 12 months) compared with patients administered Treatment B (average survival of approximately 10 months). In addition, patients given Treatment A were less likely to have treatment-related nausea and vomiting, therefore their quality of life was improved.

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

You then calculate the QALY for patients administered Treatment A to be 0.60 (1x0.6), compared with 0.33 (10/12x0.4) for patients administered Treatment B.

The additional QALY gain of Treatment A compared with Treatment B is therefore estimated to be 0.27 (0.60 minus 0.33).

### ● Costs

The total cost per patient of 6 months treatment with Treatment A is \$5,500, compared with a cost of \$900 per patient for Treatment B. In addition, Treatment B is an infusion that people need to receive at a hospital outpatient unit. This costs approximately \$1,500 per patient. Treatment A 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 Treatment B, compared with none (0%) of the patients taking Treatment A. The cost of going to hospital was estimated to be \$2,500. There is therefore a ‘saving’ of \$250 per patient (\$2,500x0.1) associated with Treatment A.

The total cost of Treatment A is therefore \$5,500, compared with a total cost of Treatment B of \$2,650 (\$900+\$1,500+\$250).

The additional cost of Treatment A compared with Treatment B is therefore estimated to be \$2,850 (\$5,500 minus \$2,650).

### ● Additional Cost per QALY

From your calculations you establish that the additional QALY gain of Treatment A compared with Treatment B is 0.27.

You also estimate the additional net cost of funding Treatment A is \$2,850.

The cost per QALY is therefore the additional cost divided by the additional QALY (\$2,850/0.27). This gives a cost per QALY result of around \$10,600.

Therefore gaining an extra unit of benefit (1 QALY) from the new bowel cancer medicine (Treatment A) would be at an additional cost of ~\$10,600. This is the same as 95 QALYs per million dollars of the total health budget invested.

# Using the 'cost per QALY' information

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 'cost per QALY' gives us information on the 'additional value' of a new treatment. The cost per QALY tells us how many QALYs we gain per dollar spent.

## Relative Value – comparing the 'cost per QALY' result across different medicines

The 'cost per QALY' 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 'cost per QALY' for different medicines – 'relative cost-effectiveness' – and improve our knowledge about which medicines offer the best health outcomes for New Zealand.

## Relative value

### – comparing the cost per QALY across different medicines for different medical conditions

For example, the cost per QALY results for a variety of medicines for the following medical conditions could be:

#### High blood pressure

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

#### Epilepsy

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

#### Osteoporosis

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

#### Asthma

\$80,000-\$100,000 per QALY (or 10-12.5 QALYs per \$1 million invested)

The cost per QALY 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 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 'cost per QALY' helps 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 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 cost per QALY - the additional cost per unit of additional health benefit (QALY). The cost per QALY tells us how many QALYs we gain per dollar spent. We can also compare the additional cost per QALY across different medicines - 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 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 medicines 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 diagnosis 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 'cost per QALY'?

We always look at both the benefits and costs of medicines. The cost per QALY is the overall value, taking into account both benefits and costs. It can also be expressed as QALYs gained per dollar spend, the lower the cost per QALY the more QALYs are gained. It is the additional cost per QALY that is important - the change in costs and QALYs compared with currently funded medicine. 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.

### 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 dollar spent. This means that compared to 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 an existing medicine 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 to 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 QALY composite measure.



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PHARMAC is the Government agency responsible for deciding which medicines are subsidised for New Zealanders. It manages spending on pharmaceuticals for the District Health Boards, and ensures that a comprehensive list of medicines (the Pharmaceutical Schedule) is subsidised for New Zealanders, and that the list of medicines continues to grow to meet the needs of patients.

