

## **NEW ZEALAND'S PHARMACEUTICAL POLICIES: A FRESH LOOK**

by Brian Easton

This report, commissioned by Pharmac, reviews the report by Castalia Strategic Advisers *New Zealand Pharmaceutical Policies: Time to Take a Fresh Look*.

November 2005.

## INTRODUCTION

In August 2005, Castalia Strategic Advisers, published a report *New Zealand Pharmaceutical Policies: Time to Take a Fresh Look*. The report was commissioned by Pfizer New Zealand Ltd and was prepared by Alex Sundakov and Dr Viktoria Sundakov.

The report claims that the public pharmaceutical procurement policy is inefficient and could be improved. If the allegation were true, it would be serious, for it implies that there is severe fiscal waste.

But, as we shall see, the claim is largely tendentious, and often based on factual errors or misunderstandings. There is little rigorous analysis and certainly no discussion of an alternative procurement policy.

Pharmac (Pharmaceutical Management Agency Limited), the New Zealand public agency responsible for the procurement of pharmaceuticals, has commissioned this report to independently review the allegations of the Castalia report, and also to carry out an assessment of Pharmac's current evaluation assessment procedures.

This report is in three parts. Chapter 1 reviews the Castalia report. Chapter 2 describes the current procedures for deciding whether a pharmaceutical will be publicly funded. Chapter 3 assesses the extent to which the current procedures are economically efficient.

The conclusion of this report is that while there may always be opportunities for improving the administration of the existing procedures, their underlying principles are robust. However these were not issues to which the Castalia report contributes much.

This report reflects the analysis and views of its writer. It does not necessarily reflect Pharmac's views.

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For background see P. Davis (2004) "'Tough but fair"? The active management of the New Zealand drug benefits scheme by an independent Crown agency', *Aust Health Rev* 2004: 28(2): 171–181

## CHAPTER 1: THE CASTALIA REPORT

Here follows a slightly edited version of the Executive Summary of the Castalia Report, plus a commentary upon the summary and the main argument.

Some of the report is concerned with the central issue of the efficiency of the pharmaceutical procurement policy. That is mentioned, but largely left out, as it is not central to the issues being explored. In particular little attention is paid to the means by which Pharmac and the pharmaceutical industry negotiate the prices of the pharmaceutical (which sometimes involves restricting purchase of a particular medicine to a single supplier), once the treatment has been decided upon.

The Castalia report's Executive Summary is in two parts, a main text, and a summary commentary in the margin. The main text in *italics* and the summary commentary is set here in ***bold italics*** after the relevant section.

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*Two elements make New Zealand's policy for funding pharmaceuticals prescribed to patients not in hospitals fundamentally different from policies adopted in other countries:*

*\* First, where possible, New Zealand uses sole source tenders to bid down the price of prescription medicines. Under this policy, only one or a limited number of products are procured for each indication. The expectation is that the winning bidder would agree to a lower price in return for the opportunity to supply the entire New Zealand market demand for that medicine. In some cases, this is widened to preferential or sole supply access for a therapeutic group/sub-group. And the danger here is that patients react differently (benefits as well as side effects) to different medicines, hence the need to have multiple medicines in each therapeutic category.*

Pharmac recognises the danger. Where there are proven differences it will make the different pharmaceuticals available on the schedule. However, while it is said that there are such differences, in many instances there is little compelling scientific evidence.

*Second, the budget for the funding of pharmaceuticals (in other words, Pharmac's budget) is strictly capped.*

It is indicatively, not strictly, capped. See Chapter 2.

*... In New Zealand, it is the entitlement that gets adjusted through the year in order to remain within the set budget. New drugs tend to be approved only if savings are made on older drugs. Further, there is strict control over the discretionary approvals for drugs that are made*

*available under “special authority criteria”. The ability to grant “special authority” is limited by the budget cap, rather than by clinical considerations.*

What happens is somewhat more complicated than what is characterised here. (Chapter 2)

*In addition to these explicit restrictions on customer access, there are concerns about the transparency of the process for getting new medicines listed. In particular, we have encountered growing concern that the Pharmacology and Therapeutics Advisory Committee (PTAC), whose job it is to review the effectiveness of new medicines, is being influenced in their judgements by fiscal imperatives rather than by the relevant clinical considerations. While fiscal issues must be taken into account, there are risks to the integrity of the system and the quality of decisions if fiscal restraint is exercised implicitly under the guise of concerns about effectiveness or safety. The Castalia report does not indicate the sources of these concerns. It seems unlikely they were expressed by health professionals. While there may be concerns, they seem to be based on a misunderstanding of the issues. These are elaborated in Chapter 2.*

The crucial misunderstanding is that a treatment (pharmaceutical or other) is clinically effective, does not mean it is economically justified, or fiscally prudent. A quick illustration is that were a clinically effective treatment to cost a very large sum (say \$100m a dose) it could not be economically justified.

*Finally, New Zealand, like a number of other countries, also uses reference pricing to beat down prices of patented pharmaceuticals. ... We do not address the general effects of reference pricing in this report because it is not unique to New Zealand.*

This report does not address reference pricing either, except to note the fact that it is used by a number of countries, gives some weight to the probability the method is effective.

***New Zealand’s funding of pharmaceuticals is unusual: we subsidise only one drug for a relatively wide range of conditions, and access to drugs is varied during the year to keep to a budget.***

All national systems of funding pharmaceuticals are ‘unusual’ in that there are very great differences between them. The Castalia report does not make the case that New Zealand’s is more ‘unusual’ than any other one.

*This policy has been consistently implemented since 1997. As a result, in the last eight years, New Zealand’s pattern of expenditure on pharmaceuticals has diverged dramatically from other OECD countries. New Zealand’s pharmaceutical expenditure – both as a proportion of total health expenditure and of GDP – fell sharply during a period when, elsewhere in the OECD, these proportions have grown in response to innovative treatments and increased reliance on pharmaceutical rather than surgical interventions. In essence, New Zealand set out to achieve the lowest possible prices for pharmaceutical products but in the process, has*

*restricted the range and quantity of medicines available to prescribing medical professionals and their patients.*

There are a number of non-sequiturs here.

That pharmaceutical expenditure fell as a proportion of total health expenditure and GDP does not necessarily mean that pharmaceutical use has declined. First, expenditure has increased. Second, the analysis focuses on only a part of pharmaceutical use – publically funded primary care use, and private secondary care. It does not cover public secondary care use, nor private purchase. Third, the analysis makes no allowance for different costs of medicines over time and by country (where the different institutional arrangements need close attention when comparisons are being made).

In any case, it is possible that a reduction in drug use (or at least the elimination of some drugs) may be beneficial.<sup>1</sup>

The expression ‘choice’ and ‘customer’ is misleading. The choice is usually made by the prescribing agent (typically the general practitioner) for the patient typically follows the agents advice. He or she is not really a ‘customer’, nor is the prescriber.

Indeed the claim of reductions of ‘access’ and ‘choice’ are misleading reflecting a faulty framework of analysis. Once Medsafe, an agency totally independent of Pharmac, has approved a pharmaceutical, access is unrestricted, and the patient’s prescriber may choose the pharmaceutical if that is desired. However, the patient or some private source, would have to pay for it unless it is provider in some way through the public system.

What the public health system, with Pharmac as one of its agents, does is enable patients to have access to some pharmaceuticals without private payment (or sometimes with a public subsidy to the private payment). Thus the public health system increases access and choice which the private payment system restricts.

The Castalia report sets out the issue out as if all pharmaceuticals are in principle available (presumably without charge irrespective of their cost or effectiveness) and the Pharmac procedures restrict access and choice. In practice it is the other way around.

***As a result, our drug use has declined relative to other OECD countries but at a cost of reduced access and choice.***

Both the fact is unproven, because not all medicines were considered, and the conclusion is unproven, not only because it is based on an unproven fact, but because it misrepresents the situation.

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<sup>1</sup> Some examples, such as reductions in Hormone Replacement Therapy and antibiotics, are given in the Pharmac Annual Review, 2004. In neither case does there seem to have been an alternative treatment replacing them.

*This restraint can be considered good policy if and only if:*

*\* the restrictions on the availability of medicines do not result in adverse health and social outcomes and do not impose additional costs by leading to more costly surgical and other interventions, and*

*\* the restrictions are the only way to achieve reasonable prices for pharmaceuticals.*

Despite the ‘if and only if’ the apparent theorem is not proven, but stated. As stated it cannot be true as the following examples illustrate.

The first condition is not necessary. Suppose that the restrictions led to minor adverse health events for a few people and major gains for others, then the first condition is not fulfilled, but the restrictions might still be beneficial. Similarly the restrictions may lead to costly other interventions for some people but still save funds. (This is explained in Chapter 3.)

The second condition is not necessary either. There may be other means of achieving reasonable prices – such as the local production of medications without the payment of royalties – which would be considered inferior to the method used in New Zealand today.

Even were the two propositions necessary, they would not be sufficient.

The thinness of the argument becomes evident from the arguments which follows.

*Our analysis suggests that there are good reasons to believe the above conditions do not hold:*

*\* First, there is indicative evidence to suggest that restrictions on pharmaceuticals have had a negative impact on New Zealand’s disability burden and health outcomes. Our high level of welfare dependency, at a time of nearly full employment, has recently become a focus of political attention and has been attributed, in part, to a culture of dependency. However, our analysis suggests that New Zealanders may indeed be more disabled than comparable populations, in part due to poor management of conditions arising out of restrictions on pharmaceuticals.*

The use of the expression ‘indicative’ means that the evidence is consistent with the hypothesis, but that there are alternative hypotheses which are just as explanatory, or more so. The Castalia report does not rule out these alternatives, and so the indicative evidence is weak.

*\* Second, there also appears to be evidence that restrictions on pharmaceuticals are shifting costs to other more invasive, costlier treatments.*

That there ‘appears to be evidence’ rather than ‘there is evidence’ demonstrates the evidence is weak. No specific instances are sited.

*\* Finally, while the Pharmaceutical Management Agency (Pharmac) was successful in reducing New Zealand pharmaceutical prices at a more rapid rate than other OECD countries during the early years of the policy, New Zealand prices now appear to have settled*

*at a constant ratio to the level in Australia, with the gap between the two countries likely to narrow.*

It is unclear how this relates to the argument. (Even if it did, the Castalia reports asserts the ‘facts’, rather than demonstrates them.)

*Both countries are enjoying on-going reductions in the prices of medicines due to a mix of purchasing techniques (such as reference pricing) and the on-going substitution of brand products by generics as patents come off. While New Zealand prices are, on average, somewhat lower than those in Australia, our analysis suggests that these differences can not be attributed to the severe restrictions on access imposed on New Zealand consumers.*

There is no clear logic between the concluding statement, and the initial sentence, nor the first clause of the final sentence and its conclusion.

The use of the term ‘consumer’ rather than ‘patient’ is indicative of the confusion the report has between a private market and a public one.

*These possibilities are serious enough to warrant a thorough review of pharmaceutical funding. Since so many factors affect health outcomes, heroic assumptions would be required to isolate the impact of restrictions on pharmaceuticals from everything else that is going on to a standard of scientific proof. We do not pretend to be able to do that. However, we believe there is sufficient information to suggest that very serious questions should be asked about the effects of the current pharmaceutical benefits scheme on the well-being of New Zealanders.*

The Castalia Report’s argument is so thin – the possibilities so tenuous – that it has not made a case for a thorough review of pharmaceutical, and certainly it has made no contribution to any such review. The issues discussed in Chapter III reflect an ongoing discussion within the New Zealand health analysts community, which it is opportune to give a more public profile.

***This does not appear to be minimising total costs: restrictions may be worsening disability rates and increasing the need for more costly and intrusive treatment while achieving prices not much better than in Australia.***

The conditional ‘may’ is indicative that such analysis that is being provided is conjectural rather than based on sound investigation and analysis. This becomes even more evident in the detailed argument below.

*Strong suspicions of pharmaceutical companies’ motives and business tactics, combined with the successes achieved in the late 1990s in shifting New Zealand from a high pharmaceuticals price country to a low price one, have cemented in many peoples’ minds the idea that the policy works, and that complaints about the system are no more than self-interested lobbying.*

The ‘strong suspicions are not attributed to any particular party. It may be that there are sections of the community who are ‘suspicious’ of the pharmaceutical communities’ motives and certainly there is a vigorous international debate about whether current arrangements for the pharmaceutical industry lead to best outcomes.

However in the Pharmac documents I have read, and in discussions with Pharmac staff over a number of years – informally and more formally when discussing their practices – there has never been any hint of the suspicions alleged in the Castalia reports.

It is true there are tensions, as inevitably there must be where two economic actors are negotiating over deals involving very large sums of money. But the impression I have is that Pharmac respects the Big Pharma, and understands the contribution they and their products make to the public's health.

*However, in our view, it is risky to allow prejudices against the “big pharma” to obscure the costs that restrictions on access to pharmaceuticals are imposing on the New Zealand society. Even in the narrow fiscal sense, it is likely that the spectacular “achievement” of keeping spending on the funding of outpatient pharmaceuticals virtually unchanged for many years, simply masks higher costs and inefficiencies in other parts of the health and welfare budgets. It is agreed that such prejudices would be inimical to the public health. It is good that Pharmac does not have them.*

*A common mantra in defence of the current pharmaceutical funding policy is that New Zealand is a poor country, and while we would like to spend more, we can not afford it. However, this begs the question, confusing restraint on expenditure on pharmaceuticals with the overall fiscal constraint on health and welfare spending.*

The mantra is unsourced so it is difficult to comment upon it. In practice New Zealand experiences fiscal restrictions which limits it from providing all the health care that would be considered ideal. This is the situation faced by every public health system (including Medicare and Medicaid in the United States) whether they are in richer or poorer countries. It is true that because New Zealand is poorer than some countries its fiscal restriction on health spending is tighter. However the tightness applies equally to all sub-components of spending and not just to some components more than others.

The report provides no evidence of the fiscal restrictions applying more onerously to pharmaceuticals, and provides no evidence that is the intention of current funding arrangements.

***Suspicion of Pharmaceutical companies should not be allowed to prevent a careful check of whether drug cost containment is increasing costs elsewhere that is, whether we have the right cost mix.***

Agreed. It is fortunate those suspicions do not exist in the current processes.

*The real question going forward is whether it is possible to achieve better health and social outcomes by re-allocating resources from other areas of health expenditure to pharmaceuticals while remaining within the overall budget envelope.*

This is a good question, although not well formulated. Nor does the Castalia Report contribute to answering it.

*Our analysis suggests that the answer to this question is “yes”. It is certainly timely to examine alternatives. In preparing this report, we have not sought to design a detailed proposal for an alternative model. However, our analysis suggests there are strong indications that the costs of the current funding model are likely to exceed its benefits. The available evidence certainly raises sufficient concerns to recommend a wide-ranging inquiry into the policy.*

*In our view, the key challenge will be to design a funding regime which improves access to a broad range of pharmaceuticals and provides sufficient incentive to the pharmaceutical companies to market and trial their products in New Zealand, without undermining sound fiscal management and without creating conditions for unjustified price increases in New Zealand.*

The analysis in the report is so inadequate that it provides no guidance as to how one might go about answering it, which would normally be the outcome of a rigorous thorough approach. Which is the focus of the remainder of the report.

***It should be possible to do better by rebalancing overall spending between drugs and other treatments consistent with sound fiscal management and increasing patient choice.***

It would have been helpful had the Castalia Report made some suggestions as to how such a rebalancing might occur.

Under ‘sound fiscal management’ increasing patient access to publically subsidised pharmaceuticals will reduce their access to other treatment options. This is a key balance the public sector health system faces.

## **CHAPTER 2: HOW THE SYSTEM OF PUBLIC PHARMACEUTICAL PROVISION CURRENTLY WORKS**

### **Background**

Behind the current system are four key questions:

- (1) Is the medicine safe (have greater benefits than risks if appropriately used)?
- (2) Is the medicine clinically effective?
- (3) Is the medicine cost effective (give a return to justify its expenditure)?
- (4) Who pays for the medicine?

### **1. Is The Medicine Safe?**

Once a pharmaceutical becomes internationally available, usually following approval by the United States Food and Drug Administration, the provider typically approaches Medsafe a business unit agency within the Department of Health.

Medsafe is responsible for ensuring that, as far as possible, the medicines available in New Zealand can be expected to have greater benefits than risks if used appropriately. This is achieved through:

- \* assessing the safety, quality and efficacy of medicines before they are marketed;
- \* auditing manufacturers, packers and wholesalers of medicines to ensure their premises and practices meet an acceptable standard
- \* monitoring the safety of medicines on the market.

In the case of new medicines a company wishing to market a medicine that has not previously been available in New Zealand must obtain the consent of the Minister of Health (or her or his delegate) to distribute a "new medicine". Typically, in order to obtain consent, the company has to submit an application dossier containing detailed information about the safety, quality and efficacy of the medicine.<sup>2</sup> The application is considered by the Medicines Assessment Advisory Committee, which is a committee of experts set up to advise the Minister of Health. If the application is acceptable, the Minister of Health approves the medicine, and it can then be marketed.

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<sup>2</sup> It is unnecessary for these purposes to record the differing consent paths for innovator medicines, multi-source or generic medicines, and over-the-counter medicines.

At this point the medicine is available on prescription by any designated medical professional (typically a doctor, but for some medicines dentists, nurses and so on). However some medicines are classified as ‘over-the-counter’ (OTC) and supplied by a pharmacist or, in some cases, by an unqualified agent such as a supermarket.

Medsafe decisions pay only a limited attention for the question of clinical effectiveness (see section 2) and no attention to the other two questions, particularly who should fund the purchase (except for OTC medication, where implicitly they are allowing the user to pay).

## **2. Is The Medicine Clinically Effective?**

The British National Health service defines clinical effectiveness as ‘the extent to which specific clinical interventions, when deployed in the field for a particular patient or population, do what they are intended to do – i.e. maintain and improve health and secure the greatest possible health gain from the available resources’<sup>3</sup>

A shorter definition is that clinical effectiveness is ‘the extent to which a treatment achieves its intended purpose for the range of patients who will receive it in practice’<sup>4</sup>

There is no New Zealand government agency whose primary responsibility is to determine the extent to which a medicine is clinically effective. However, Medsafe has to pay attention to the question when it trades off the potential harm a medicine can do (say from side effects) against the potential benefits, while clinical effectiveness is the foundation of cost-effectiveness, and therefore of considerable concern to Pharmac.

In practice the prescriber makes the decision as to the effectiveness of the treatment. Typically the decision is made on the basis of the prescriber’s expertise and experience, augmented by such evidence there is from clinical and other trials.<sup>5</sup> The research evidence is usually filtered

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<sup>3</sup> *Promoting Clinical Effectiveness -A Framework for Action in and through the NHS.* NHS Executive (1996)

<sup>4</sup> Efficacy is defined by the same source as ‘the extent to which a treatment achieves its intended purposes under the strict conditions of randomised, controlled trials, in patients typically recruited to such trials.’ See

<http://www.nsfpathways.co.uk/glossary.html#anchor45412351>

<sup>5</sup> Pharmaceuticals generally experience extensive testing in order to obtain FDA approval. However the testing cannot be comprehensive, and often continues after the

through such channels as the pharmaceutical company's marketing, professional colleagues, and standard practice.

Many 'complementary medicines' have had no rigorous analysis of their medical effectiveness at all. (They are not covered by Pharmac and do not receive a public subsidy.)

### **3. Is the Medicine Cost Effective?**

The definition used here is that a 'cost effective treatment gives treatment outcome using least resources, where the treatment outcome is measured in terms of uniform units of health such as quality adjusted life years' (QALYs).<sup>6</sup>

Any purchaser of a pharmaceutical with a budget constraint explicitly or implicitly makes a cost effectiveness decision when they ask whether they are getting value for money. This applies just as much to the OTC purchaser as it does to the District Health Board.

However those who do not pay for the medicine need only be concerned about its clinical effectiveness. This applies to patients when the treatment is funded by the public sector or by private insurance, and it can apply to prescribers (although many are increasingly aware of the issue of value for money through public and professional education).

The government agency responsible for the evaluation of cost effectiveness of publicly provided medicines prescribed by the private sector is Pharmac. (Public secondary care involves a different method of evaluation.)

Pharmac cost-effective evaluations uses orthodox economic principles, adapted for the particularities of the circumstances they face. While sometimes the detailed analysis is unnecessary – as when a more expensive medicine is replaced by a cheaper one with the same clinical effectiveness – the broad pattern of their approach when a full decision has to be made is as follows. (A schematic diagram offering more detail follows on the next page.)

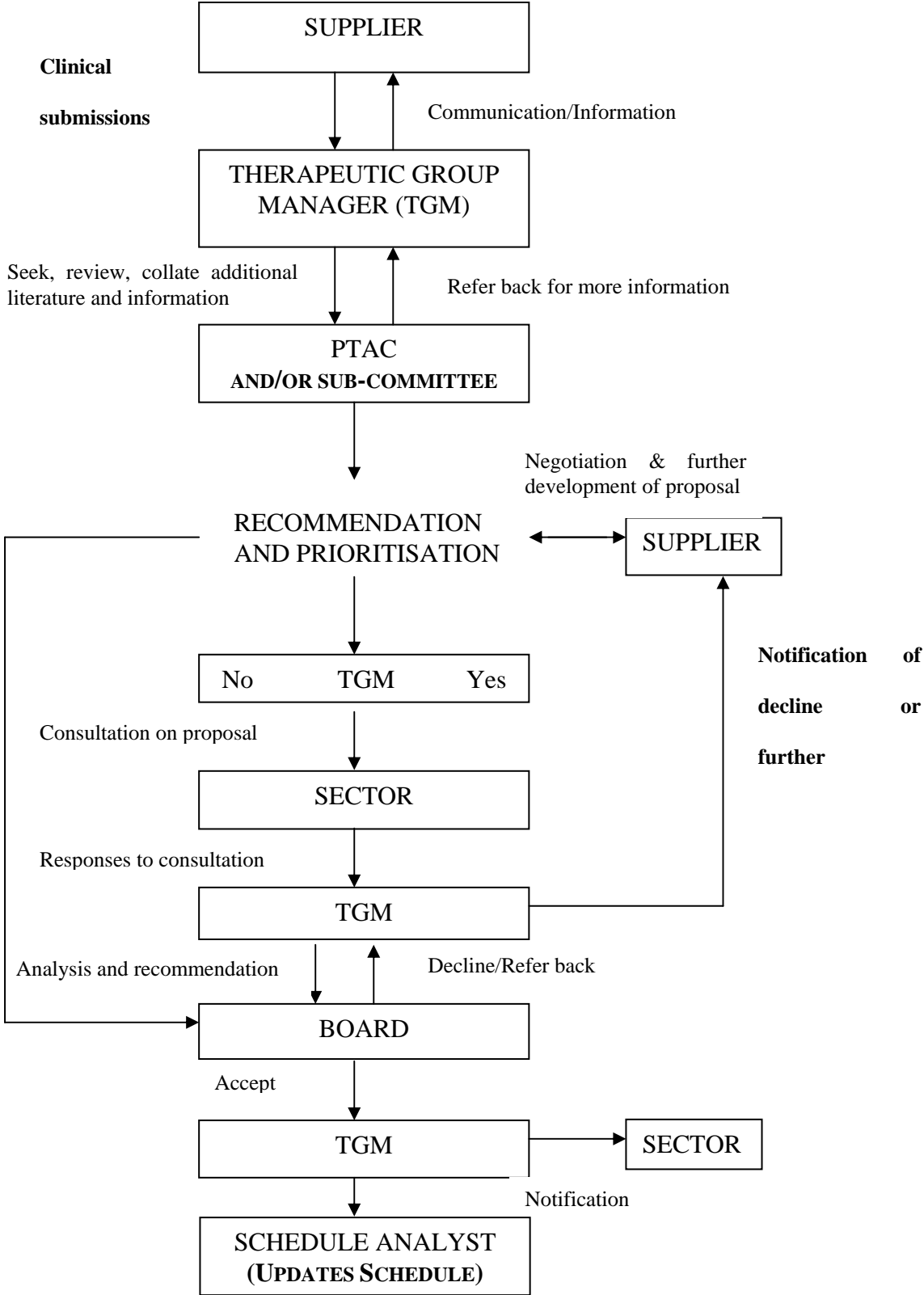
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approval, including by independent researchers.. There is much less testing of comparable clinical effectiveness where the performance of different drugs is compared.

<sup>6</sup> *International Guidelines for Estimating the Costs of Substance Abuse (2ed)*, E. Single, D. Collins, B. Easton, H. Harwood, H. Lapsley, P. Kopp & E. Wilson (WHO 2003). The expression 'cost effectiveness' has different definitions on either side of the Atlantic. Some might call this definition 'cost utility effectiveness'.

Pharmac is usually alerted to the potentiality of a new medication by the supplying pharma (which may involve more than one company). Often there will be supporting submissions from a voluntary group concerned with the health problem the medicine is intended to address.

**DECISION PROCESS**



The process set out in the diagram above is intended to be indicative of the process that may follow where a supplier wishes to list a new pharmaceutical on the Pharmaceutical Schedule.  
**PHARMAC MAY, AT ITS DISCRETION, ADOPT A DIFFERENT PROCESS OR VARIATIONS OF THIS**

Pharmac appoints a ‘Therapeutic Group Manager’ who seeks, reviews, and collates additional literature and information. This is submitted to the Pharmacology and Therapeutics Advisory Committee (PTAC) which assesses the evidence. (Not detailed here but shown in the accompanying diagram, the process has referral back where further clarification is required.)

The PTAC deliberations are based on criteria listed below. After sector consultation the recommendations (including prioritisation) goes the Pharmac Board for consideration. After sector consultation (and further Pharmac analysis), recommendations go to the Board which decides whether the new drug meets its criteria, and if there is the funding is available. If the Board’s decision is favourable to the medicine’s availability, the Therapeutic Group Manager notifies the sector and arranges for the medicine to be added to the Pharmaceutical Schedule of medicines available for use by approved prescribers. This means that the patient being treated with the drug does not have to pay its full price (and may not have to pay anything).

There is also a process of settling the price which the pharma will be paid for supplying the medicine. Although important in its own right, this negotiation is not central to the approval process, except insofar as it the cost of the medicine is important in the evaluation process.

Note that a favourable cost effectiveness finding may be set aside if there is insufficient funding in the ‘indicative budget’. This is an amount agreed between the government and Pharmac as to what the total government outlays for drugs for primary care purposes should be. (Section 4 elaborates this.) Each year there may be some room within the indicative budget from reducing the price of medicine (by, for instance, negotiations with pharma suppliers or lapsing of patents) or better management of their use. The released funds can be used for new medicines.

Pharmac also has the opportunity to bid for more funding for its indicative budget in the course of the government’s budgetary cycle. This is most likely to happen when the new medication is expected to be costly in terms of the total funds.

The effect of the indicative budget is to set the maximum cost of treatment for a given health outcome. The primary measure of health outcome used by Pharmac is ‘quality adjusted life years’, (QALYs) an internationally accepted measure. This measures any years of life gained from a treatment adjusted of life as the result of an improvement in the enjoyment of years from reduced pain, increased mobility and so forth.<sup>7</sup>

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<sup>7</sup> Single et al (2002) *op cit*. An alternative measure, not used in New Zealand, is ‘disability adjusted life years’ (DALYs) . Chapter 4 considers the relevance of each measure.

Essentially the criterion is the number of Quality Adjusted Life Years gained from the treatment in terms of a net outlay (say QALYs per million dollars spent – Q/\$m). Net outlay refers to all expenditures of the public health system, allowing that a pharmaceutical treatment may reduce other treatment costs, (This is one reason why the indicative budget may be increased, in effect shifting health funds from non-pharmaceutical treatments to pharmaceutical treatments.)

The effect of this procedure is to set a minimum level for Q/\$m. If the minimum, in future here called the ‘threshold’, is exceeded then the medicine is placed on the schedule (subject to funding availability). The threshold is, in effect, set by the indicative budget. An increase in the indicative budget reduces the minimum (there being more funds available, less cost effective treatments can be funded), an increase in the threshold increases the minimum (and less medicine is funded).

Calculation of the ratio is not easy. Often there is not the detailed clinical effectiveness information available to make an exact judgement.<sup>8</sup> Very often, the best available assessment procedure is to take overseas cost effectiveness studies and adjust them for New Zealand conditions (especially the costs). There may be a number of such international assessments with inconsistent findings. Thus there is a need for careful judgements in any cost-effectiveness assessment, the reviewing of which is one of the tasks of the PTAC.

The PTAC is directed to take into consideration the following matters:

- the health needs of all eligible people<sup>9</sup> within New Zealand;
- the particular health needs of Maori and Pacific peoples;
- the availability and suitability of existing medicines, therapeutic medical devices and related products and related things;
- the clinical benefits and risks of pharmaceuticals;
- the cost-effectiveness of meeting health needs by funding pharmaceuticals rather than using other publicly funded health and disability support services;
- the budgetary impact (in terms of the pharmaceutical budget and the Government’s overall health budget) of any changes to the Pharmaceutical Schedule;
- the direct cost to health service users;

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<sup>8</sup> Sometimes this is impossible. For instant assessing beta-interferon on multiple sclerosis involves making judgements about the impact on the drug on patients many decades in the future, for which there is no evidence, and cannot be available given the recent use of the drug for this purpose.

<sup>9</sup> As defined by the Government’s rules of eligibility.

- the Government’s priorities for health funding, as set out in any objectives notified by the Crown to Pharmac, or in Pharmac’s Funding Agreement, or elsewhere; and
- such other criteria as Pharmac thinks fit. Pharmac will carry out appropriate consultation when it intends to take any such “other criteria” into account.

As explained in chapter 3, many of the criteria are directly covered by the cost effectiveness evaluation.

Completeness requires a brief account of the practice of public secondary care, which may provided medicines without charge which are not on the Pharmac schedule. They are also under a budget constraint. A not uncommon practice is for a hospital to have a ‘preferred medicine lists’ which is a list of medicines which doctors may use without restriction, and a procedure of referral to senior colleagues for approval to use a medicines not on either list. Neither the preferred medicines list nor the referral process usually use as rigorous a cost-effectiveness evaluation as does Pharmac, but costs are taken into account in the decisions.<sup>10</sup>

Cost effectiveness determines the degree of public funding available for a treatment. The New Zealand application involves a political decision at the highest level – the amount of funds voted by parliament – but it designed that once the overall funds are set, the decisions are made by experts (at the Pharmac level including the medical experts involved in the decision), while the the medical professional (usually) focusses only on clinical effectiveness at the patient level. This separation of powers is characteristic of all decisions in the public health system.

#### **4. Who Pays for the Medicine?**

Once the medicine has been approved by Medsafe as effective, any approved prescriber may obtain it on behalf of a patient.<sup>11</sup> Patients may pay for the medication themselves or have it paid for some other private agent such as a private insurer or a friendly society. In such cases there is no charge to the public purse.

If the patient is in a public hospital, there is no charge to the patient. The cost of the medication used is charged to the hospital, which receives its funding from the District Health Board.

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<sup>10</sup> Pharmac provides advice to hospitals on request.

<sup>11</sup> The requirement of an approved prescriber is because there may be potential side effects from treatment or misuse. However in the case of OTCs, these risks are considered sufficiently small to allow the ‘patient’ to make the decision, although the pharmacist and information included with the medication way add to the patient’s knowledge.

The public funding provisions for Pharmac scheduled medicines in primary care has recently changed. Today the District Health Board in the region where the medicines are prescribed pays the subsidy on the medication. Pharmac collects the information on actual DHB outlays and compares that total with the indicative budget for pharmaceuticals. While the amounts need not be the same (and are never exactly the same), too large a divergence could require a major review.

The advantage of having pharmaceutical funding within DHB budgets is that it gives them an incentive to better manage primary care in order to reduce their overall costs, thus reinforcing the cost effectiveness valuation by complementary management.

### **Coda**

This chapter and the next have concentrated upon only one aspects of Pharmac's activities. Other activities include the purchase of the pharmaceuticals on behalf of the DHBs, and thereby the price setting for them (it also does this for some hospital pharmaceuticals), and the influencing of prescriber behaviour.

## CHAPTER 3: CAN THE CRITERIA BE IMPROVED? <sup>12</sup>

Systematic decision-making requires a rational criteria. Quality Adjusted Life Years per million dollars spent (Q/\$m) is such a criteria, developed out of the microeconomic framework of Cost Benefit Analysis. Use of it for decision making, leads to efficient outcomes, in the sense of the maximum QALYs for the outlays.

However, application of the principles always involves some practical compromises, so it is worth reviewing the criteria in detail. In particular it sometimes occurs that the compromises sometimes lead to unintended anomalies, although none were found in the Pharmac procedures.

This chapter is in five sections. The first section reviews the notion of health outcome which is the numerator of the criteria ratio, the second reviews the notion of cost which is denominator of the criteria ratio, the third reviews other factors, the fourth the discount rate and the fifth reviews the procedure to select the new pharmaceuticals.

### 1. Health Outcome

The circumstances in which Pharmac makes decisions involves the use of medication for the treatment of different disease. The health outcome has therefore to be measured in a way in which the effects of different diseases and their alleviation or cure can be compared.

The health outcome used by Pharmac is QALYs or Quality Adjusted Life Years lost or gained. This is a more general measure than morbidity, because increasingly the effect of various treatments is to give patients a higher quality life as well as to postpone death.

One year of ordinary healthy life is measured as one QALY. If the individual suffers discomfort or restrictions on their life during the year, then its QALY value is discounted. A dead individual's QALY is valued at zero. Surveys of opinion are used to interpolate the intervening steps. The surveys have found that some life states are judged worse than death, and are so are measured as negative. <sup>13</sup>

Some examples of standard QALY measures are  
1.000 No problems;

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<sup>12</sup> Pharmac is currently reviewing its criteria.

<sup>13</sup> <http://www.evidence-based-medicine.co.uk/ebmfiles/WhatisaQALY.pdf>

- 0.760 No problems walking about; no problems with self-care; some problems with performing usual activities; some pain or discomfort; not anxious or depressed;
- 0.516 Some problems walking about; some problems washing or dressing self; some problems with performing usual activities; moderate pain or discomfort; moderately anxious or depressed;
- 0.329 No problems walking about; some problems washing or dressing self; unable to perform usual activities; some pain or discomfort; not anxious or depressed;
- 0.222 Some problems walking about; no problems with self-care; no problems with performing usual activities; moderate pain or discomfort; extremely anxious or depressed;
- 0.079 Some problems walking about, unable to wash or dress self, unable to perform usual activities, moderate pain or discomfort, moderately anxious or depressed;
- 0.000 Death;
- 0.429 Confined to bed; unable to wash or dress self; unable to perform usual activities; extreme pain or discomfort; moderately anxious or depressed.<sup>14</sup>

A rating has to be assigned to each disease, which involves judgement because different patients may have different condition states, and because their conditions change over time.

QALYs are used extensively in cost effectiveness and related studies .While they are by no means perfect (and are being continually improved) there is probably no better across-disease measure, for the comparison of outcomes.

For a while, QALYs were challenged by DALYs (Disability Adjusted Life Years) which were proposed by WHO. A fundamental weakness is they use a 5% p.a. discount rate which limits the use of alternative rates. (See Section 4.) They also seem to become less popular for various reasons. It is not recommended that the Pharmac analysis switch to DALYs.

Very often, the QALY is applied only to the patient. But in some cases the patient's health state can place a considerable burden on the patient's close associates – family and friends. The counsel of perfection is that such a burden should be incorporated into the measure of QALYs gained or lost from a treatment or fail to treat. In practice it may be quite difficult to do this, especially as the external burden varies from patient to patient. There are also raises ethical issues. The current practise is to alert the TPAG when the disease commonly involves a lot of external burden (say on the parents and siblings of a sick child) , and for them to take it into consideration where it is particularly important. Until progress is made with this issue at the international level, this would seem to be the recommended practice.

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<sup>14</sup> Based on EQ-5D health state valuations.

For some purposes, such as the cost burden of disease and cost-benefit analysis it is necessary to put a dollar value on a QALY, This valuation is problematic and controversial. However, it is unnecessary to do this for Pharmac's purposes. since the Q/\$m ratio gives a satisfactory ranking of comparable health outcomes for its decision-making purposes.

### Recommendations

- 1.1. QALYs (Quality of Life Years) are the best available measure for comparing the effects of disease and the impact on health outcomes where different diseases are involved.
- 1.2. It is not recommended to change to DALYs (Disability Adjusted Life Years).
- 1.3. Although the lost of QALYs by associates of the patient is relevant, and should be taken into consideration where the impact is large, it should not be included in the calculation of the criteria ratio.
- 1.4. It is not necessary to put a dollar valuation on QALYs for Pharmac's purposes.

### **2. Costs**

The denominator of the criteria ratio is expenditure by the health system. This includes outlays on the medicine, which may offset to some degree by outlays on other treatments which are avoided as a consequence of the use of the pharmaceutical.

Since the other health expenditures are taken into account, it is hard to understand the source of the claim of the Castalia report that *'there also appears to be evidence that restrictions on pharmaceuticals are shifting costs to other more invasive, costlier treatments'*, except as an inappropriate application of the criteria. (Some possibilities are discussed later.) A deceptive case would be where a particular medicine is not included because of cost effectiveness but where a small proportion (allowed for in the cost effectiveness evaluation) led to expensive treatment, it being cheaper to do them than outlay on all the potential suffers only a few of who require the expensive treatment.<sup>15</sup>

A more serious concern is the exclusion from the denominator of other costs involved in the cost effective comparison.

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<sup>15</sup> Suppose a medicine was applicable to 1000 patients, costing \$1000, and that one of those patients would subsequently require treatment costing \$10,000. There would be a net cost to the health system of \$90,000 to prescribe the medication. In one case (out of 1000) the failure to include the medicine on the schedule could have resulted in a more invasive, costlier treatments.

Some of these are public sector costs, including costs incurred by Work and Income (say payments of sickness benefit) and any loss of tax revenue due to the failure of the patient to work because of the disease. A whole-of-government approach should require the inclusion of these costs in the denominator of the criteria ratio. It should be noted that overseas cost-effectiveness studies often do not include such phenomenon, and local protocols would need to be developed.

The extension of the denominator to cover net private costs is more problematic. The most important (other than the time and other costs to family and friends) may be the gains to the individual from an early return to employment.<sup>16</sup> The effect of including such gains for employment would be to prioritise treatment which enabled patients to return to the workforce.<sup>17</sup> Given the expectation that unemployment will remain low, and the need to maintain high labour force participation rates for high GDP per capita, such an outcome would seem consistent with the government's wider objectives (as well as reducing fiscal pressures).<sup>18</sup> This issue needs to be explored further, especially as it has an ethical dimension.

By adding further savings from the treatment recommended changes here would tend to increase the Q/\$m ratio. This need not impact on the total pharmaceutical spending, but rather would raise the threshold by which new medicines were introduced were the indicative cap be kept at its current level. However it could change the prioritisation of medication (particularly in terms of these recommendations towards the treating of diseases which reduce labour force participation.) As is explained in section 5 the suggested changes may also lead to a redeployment of government funding to the pharmaceutical budget from other public sector budgets, with a net gain to the effectiveness of government spending and improvements in economic performance.

## Recommendations

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<sup>16</sup> The resource gains to the economy as a whole from a return to employment are usually measured as the gains to the individual worker, and the taxes paid from the worker's employment. There is a further adjustment in a cost-benefit or a cost-effectiveness study where there are transfers, such as social security payments.

<sup>17</sup> Attention would have to be given to returning to the informal workforce, as when medication enables a parent to better look after the children.

<sup>18</sup> This will be particularly important with the growing proportion of sickness and invalids beneficiaries, many of whom are highly skilled, as the result of psychiatric disability and drug addiction. (The appropriate treatment may not always be pharmaceuticals.) The recently announced pilot work-focussed service program is a recognition of its increasing significance.

- 2.1 The notion of cost in the expenditure should, as it does now, include all treatment costs, including the savings from treatment forgone as a result of the medication.
- 2.2 There is a strong case for extending costs to a whole-of-government basis, by including net savings to the entire government budget from the medication.
- 2.3 The case for extending costs to include employment and other private costs needs to be explored. This is likely to become more important in the future with the need to make better employment utilisation of the population.
- 2.4 The recommended whole-of-government approach could lead to a redeployment of public funds to the indicative pharmaceutical budget with improvements in the effectiveness of government spending (as could the inclusion of private cost savings, were they also to be included).

### **3. Other Factors to Be Taken Into Consideration**

The following matters are taken into consideration by the Pharmacology and Therapeutics Advisory Committee (PTAC) when making its recommendations. (The ordering differs to from the Pharmac list):

#### General Matters

- the budgetary impact (in terms of the pharmaceutical budget and the Government's overall health budget) of any changes to the Pharmaceutical Schedule;
- the Government's priorities for health funding, as set out in any objectives notified by the Crown to Pharmac, or in Pharmac's Funding Agreement, or elsewhere;
- such other criteria as Pharmac thinks fit. Pharmac will carry out appropriate consultation when it intends to take any such "other criteria" into account.

#### Matters included in a Cost Effective Analysis:

- the health needs of all eligible people <sup>19</sup> within New Zealand;
- the availability and suitability of existing medicines, therapeutic medical devices and related products and related things;
- the clinical benefits and risks of pharmaceuticals;
- the cost-effectiveness of meeting health needs by funding pharmaceuticals rather than using other publicly funded health and disability support services;
- the direct cost to health service users.

#### Other Matters Not included Above

- the particular health needs of Maori and Pacific peoples.

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<sup>19</sup> As defined by the Government's rules of eligibility.

Except that the direct costs to health service users may not cover net costs such as loss of employment (as discussed in section 2), the current practices are well covered by the current procedures.

The last listed here (the second in the Pharmac list) reflects a weakness in cost-effective and related analyses (including cost benefit analysis). They do not allow for fairness and other distributional matters. For instance suppose the ranking set only drugs for the elderly above the threshold determined by the indicative budget, and the next – immediately below the threshold – was for children (at roughly the same pharmaceutical cost). It would be quite understandable for the children’s medication to be prioritised a little higher. It would mean slightly lower QALYs. but equity considerations are important in a modern society. Currently the equity point only directly refers to a couple of ethnic minorities. It would conform more closely to the notions underpinning Cost-Effectiveness Analysis, if it were to be extended to ‘the particular health needs of minority groups including Maori and Pacific peoples’.

The list above applies to Pharmac’s concerns. Elsewhere in the health system, the principles of when to treat are taken on other criteria. Are they consistent, particularly where the pharmaceutical and other treatment regimes interact? If they are not, there could be inconsistency of optimal treatment and a loss of efficiency.<sup>20</sup> There would seem to be a case for a overall set of principles for the health system, similar but more general to the Pharmac ones, to ensure consistency.

It might be asked that given there is an apparently objective ranking from the Q/\$m ratio, is there any need for a PTAC and its sub-committees. It would be unwise to use an entirely mechanical mechanism, for the following reasons:

- The PTAC system acts as a quality review of the assessment;
- The Q/\$m ratio can be very sensitive to assumptions, many of which those who do the analysis (e.g economists) are not competent to judge. The PTAC brings a competence outside that of narrow disciplines involved in the ratio’s calculation.
- The Q/\$m ratio is subject to a margin of error which needs to be taken into consideration when ultimate decisions are made.

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<sup>20</sup> For instance, one group of treatments (say pharmaceuticals) may assess QALYs gained by children and elderly the same, and another (say surgery) give greater weight to children’s QALYs. This could result in the first group under-providing to children, resulting in the second group to use more expensive (less cost-effective) treatments. The example is chosen because there is a literature on QALY weightings by age, especially in the ‘fair innings hypothesis’, the noun indicating an awareness that there are ethical issues involved.

- The PTAC is able to make assessments of matters which the cost-effectiveness analysis cannot or has not taken into consideration. (There are examples of such matters in other sections .)
- The PTAC gives confidence to the professionals who prescribe the pharmaceuticals. This is particularly important where it is necessary, for whatever reason, to restrict the availability of the medicine.

Having accepted the need for a PTAC, it may be that its members should receive a briefing on the cost-effective analyses which they backgrounds their decisions.<sup>21</sup>

### Recommendations

- 3.1 That the current set of matters to be taken into consideration be retained with the second matter be extended to ‘the particular health needs of minority groups including Maori and Pacific peoples’.
- 3.2 That the PTAC procedure is appropriate, particularly to deal with deficiencies in the cost-effective ratio and to give confidence to prescribers. There may be a case for giving members of the PTAC and its subcommittees an annual briefing on cost-effective analysis, and recent developments in its application.

## **4. The Discount Rate**

Discounting is the means with which cost-effectiveness (and related procedures) deals with outcomes and costs over time. It is a routine procedure, well justified and accepted by economists. At issue is the appropriate discount rate, the parameter which controls the discounting.

The higher the discount rate, the greater the weight given to events in the immediate future compared to events in the distance future.<sup>22</sup> For instance a 10% p.a. discount rate reduces the return on an event 10 years into the future by over 60 percent, whereas a 2% p.a. discount rate reduces it by 18 percent. The effect of lowering the discount rate might be said to increase the ‘preventative’ element in the health system relative to the ‘urgent’ element.

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<sup>21</sup> The Castalia report suggests that briefings for health professionals may also be useful.

<sup>22</sup> When the evaluation of beta-interferon treatment to Multiple Sclerosis was undertaken, high discount rates emphasised the returns from reducing episodes in the year of prescription, but gave virtually no weight that it seems likely that the fewer episodes would mean that the patient would experience delays in the deterioration of the quality of life some decades later.

There is no agreement as to what is the correct discount rate. Currently the discount rate used in evaluations is 8% p.a..

It is good practice to test the sensitivity of the Q/\$m ratio to the choice of discount rate. A good set for tests would be 8% p.a. (the current rate); 5% p.a. (a moderate rate used by WHO for its DALYs), and 2% p.a. (which corresponds more closely to the sustainability rate). PTAC would want to be sure that its recommended prioritisation was as robust as possible to the choice of discount rate. (Note the use of different discount rates in different parts of the health system could lead to inefficient use of resources).

Part of the dispute over the appropriate discount rate involved the premium for risk. The further out the projection, the wider the margin for forecasting error.<sup>23</sup> There is not a robust theory of such risk which can be practically applied. Commonsense suggests that where there are these margins of error, that a sensitivity analysis should be applied to indicate the likely Q/\$m range.

#### Recommendations

- 4.1 Where discounting is necessary, the Q/\$m ratio should be calculated with different discount factors. (Suggested were 10% p.a., 5% p.a. and 2 % p.a.)
- 4.2 Where assumptions are subject to error, the Q/\$m should be presented in a likely range.

#### **Selecting the New Pharmaceuticals**

The Q/\$m ratio is used to prioritise the proposed pharmaceuticals. The priorities are then adjusted for the other factors that the ratio cannot encompass. The cost of introducing each pharmaceutical is then calculated.

There are also projections for the outlays on existing pharmaceuticals. The difference between this current aggregate and the indicative budget is then available for the prioritised pharmaceuticals, and the top pharmaceuticals on the list is approved until the difference is exhausted.<sup>24</sup> This has the effect of creating a threshold Q/\$m ratio (although the other factors

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<sup>23</sup> For instance in the beta-interferon for MS evaluation, it was assumed that it slowed down the later stages of multiple sclerosis some decades on. This a plausible conjecture, but based on thin scientific evidence. Such are the time horizons involved beta-interferon will be out of patent well before the scientific evidence is available to evaluate the conjecture.

<sup>24</sup> A complication, which need not detain us, is that if a new medicine involves great use, the total outlays may breach the expenditure cap. If some means cannot be found to

taken into consideration may make the precise threshold fuzzier than implied here). Medicines which exceed the threshold are added to the schedule, those that do not are not.

The threshold is a useful policy indicator. It helps assess the adequacy of the indicative budget. It would be expected to be relatively stable from year to year, but to fall in the long run with growing affluence (indicating the community is willing to spend more to improve quality of life)..

However, as described here, the procedure may not give an efficient use of the available funds on a whole of government basis. That is because the decision procedure (i.e the fiscal cap) uses the cost of the pharmaceutical in the denominator but the prioritisation ratio uses the net expenditure of health spending (the cost of the pharmaceutical less savings of other treatment costs) in the denominator (which we have argued is the correct concept although it could be extended to a whole of budget basis). In particular while the procedure maximises QALYs for a given outlay on pharmaceuticals, it does not guarantee a maximum of QALYs for a given outlay by the health system as a whole, if that be the goal.

Whether this is important and leads to significant distortions from the most efficient choice of medication is an empirical matter. If it were, it could be resolved in one of the following ways:

1. To redefine the indicative pharmaceutical budget as outlays on pharmaceuticals less other treatment savings. This is a counsel of perfection, but would be a very complicated operation. (I emphasises the point that the cost of the pharmaceuticals to the health system is considerably less than the amounts outlaid, because they save other treatment costs.)
2. Allow 'side-payments' from other agencies. For instance Work and Income might finance medications which would improve their ability to get their clients back to the workforce, DHBs would charge to themselves (rather than to their pharmaceutical budget) primary care outlays (or the part costs) which reduced their treatment costs. While such side-payments should never be ruled out, widespread use of them is likely to be unduly complicated.
3. Allow Pharmac to bid for more funds, increasing the indicative budget, in the annual Budget Round, making the case on the basis of savings by other government agencies in a whole-of-government context. This, in effect, centralises the side-payments and/or moves closer the counsel of perfection of a redefined indicative budget.

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reduce the outlay, then small outlay lower priority medicines may be substituted. The omitted medicine may then be reviewed, held over, or a special case made in the health budget round.

## Recommendations

- 5.1 Observing that the current indicative budget is confined to pharmaceutical spending only, and the prioritisation procedure uses net spending of the health sector, it is recommended that consideration be given to varying current procedures to ensure that decisions are not distorted by this inconsistency.