

*Preliminary Report*  
*Intended to Promote Discussion & Elicit Feedback*

**Review of Access to  
High-Cost, Highly-Specialised Medicines  
in New Zealand**

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# Contents

<b>Executive Summary</b>	Page 1
<b>1. Introduction</b>	Page 5
<b>2. Preliminary Discussion</b>	Page 7
<i>What are ‘high-cost’ medicines?</i>	
<i>What are ‘highly-specialised’ medicines?</i>	
<i>How are pharmaceutical funding decisions made in New Zealand?</i>	
<i>What does ‘value for money’ mean when thinking about which medicines to fund?</i>	
<b>3. International Comparisons</b>	Page 16
<i>How does New Zealand’s access to high-cost, highly-specialised medicines compare with other OECD countries (particularly Australia) on a population basis, and why do any such differences arise?</i>	
<i>What are the institutional arrangements by which prioritisation and funding decisions are made internationally (particularly in Australia and the UK)?</i>	
<i>What’s in the pharmaceuticals ‘pipeline’?</i>	
<b>4. Perceived Problems</b>	Page 25
<i>What are problems – perceived and real – with New Zealanders’ access to high-cost, highly-specialised medicines, and are certain people or groups of people particularly disadvantaged?</i>	
<b>5. Proposed Solutions</b>	Page 38
<i>What are practical and affordable solutions to the problems identified, including how might the role and administration of PHARMAC’s Exceptional Circumstances schemes be improved?</i>	
<i>More funding?</i>	
<i>More consistent decision-making processes?</i>	
<b>6. Conclusion</b>	Page 46
<b>References</b>	Page 47
<b>Appendix 1: Terms of Reference</b>	Page 49
<b>Appendix 2: Individuals and organisations with whom the Review Panel has consulted and/or from whom written submissions were received</b>	Page 51
<b>Appendix 3: Documents read by the Review Panel</b>	Page 54

## Executive Summary

In May 2008 the Minister of Health asked us to review access to high-cost, highly-specialised medicines in New Zealand. Our Terms of Reference are in Appendix 1.

This is our preliminary report, representing ‘our thinking and direction’ at this point in the review process. It is intended to promote discussion and elicit feedback. What have we got right? What have we got wrong? What have we missed? How might our recommendations be implemented (if you agree with them)? We would like to know what you think by the end of February 2010 (our contact details are in the Conclusion). We will incorporate all feedback, where appropriate, into the final version of the report, for presentation to the Minister by 31 March 2010.

In the course of our review we have consulted with many individuals and organisations (listed in Appendix 2) who kindly shared their expertise and opinions with us. Many problems concerning access to high-cost, highly-specialised medicines, and problems with the medicines system overall, were raised. As detailed in our recommendations below, we believe that significant improvements are possible to how New Zealand’s medicines system operates. Such improvements will likely increase access to high-cost, highly-specialised medicines (by which we mean medicine that are high-cost *and/or* highly-specialised).

However, because high-cost medicines are, by definition, expensive, there are no ‘free lunch’ ways of increasing access to them. We fully acknowledge that if high-cost, highly-specialised medicines are not provided to people who need them, and if other treatments are also unavailable, their health inevitably suffers. For some patients and their families this can be very serious – in the extreme, resulting in death.

Logically, in theory, there are four possible ways of increasing funding for (and thereby access to) high-cost, highly-specialised medicines:

1. Increase the Government’s spending on medicines overall (i.e. reduce spending elsewhere or raise taxes) and spend at least some of that increase on high-cost, highly-specialised medicines.
2. Switch spending away from other (lower-cost) medicines in favour of high-cost, highly-specialised medicines.
3. Raise additional funds for high-cost, highly-specialised medicines via higher patient co-payments, ‘risk sharing’, etc.
4. Reduce wastage in the medicines system (or health system in general) and spend the savings on high-cost, highly-specialised medicines (and perhaps other medicines as well).

All but option (4) are likely to be contentious, as they all have opportunity costs in terms of alternative possible uses for the resources involved. Their desirability or undesirability

respectively hinges on ethical (and political) considerations of the trade-offs associated with these alternative uses for the resources. There are no universally ‘right’ answers to the resource-allocation questions implied by options (1) - (3). Everyone has his or her own personal preferences, which depends on each individual’s value judgements (or ethical position), of which there is an infinite number theoretically possible.

Accordingly, we are not advocating for nor against options (1) - (3). We – and also most of the individuals and organisations with whom we have consulted – are not uniquely qualified to stipulate how much should be spent on medicines (including high-cost, highly-specialised ones). The amount spent on the public health system (‘Vote Health’) – from which medicines are funded – is a political decision determined by political processes. It depends on the Government’s priorities and its assessments of the trade-offs associated with alternative uses of the available Government Budget funds (e.g. Vote Health versus Vote Education, etc).

On the other hand, we believe that changes should be made to how decisions are reached about what Vote Health (whatever the amount available) is spent on. Specifically, across the health system as a whole, we would like to see greater efforts made to achieve consistency in funding decision-making processes and ultimately the value for money of spending on medicines (including high-cost, highly-specialised ones) and all other types of ‘health technologies’ such as devices, vaccines and medical and surgical procedures and equipment.

Currently, it seems that medicines are subjected to much greater analytical scrutiny in assessing their ‘value for money’ – especially medicines on the Community Pharmaceutical Schedule (e.g. prescribed by family doctors) – than other health technologies. Similar inconsistencies are evident for medicines available on the Community Pharmaceutical Schedule (decided by PHARMAC<sup>1</sup>) relative to medicines available from hospitals (decided mostly by individual DHBs<sup>2</sup>).

Broadly speaking, our recommendations are also intended to reduce complexity, increase transparency (except where there are commercial sensitivities such as PHARMAC’s negotiations with pharmaceuticals companies), and ultimately to reduce the associated frustration evident throughout the health system.

Although many of the following recommendations do not mention high-cost, highly-specialised medicines explicitly, the objective is that, by improving the medicines system overall, access to high-cost, highly-specialised medicines will be increased.

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<sup>1</sup> New Zealand’s Pharmaceutical Management Agency.

<sup>2</sup> District Health Boards, of which there are 21 nationwide.

Our recommendations thus far are:

1. That prioritisation and funding decisions concerning high-cost, highly-specialised medicines continue to be made in the same way as such decisions for other medicines. To be clear, we are *not* recommending that new prioritisation processes and pools of funding be established for high-cost, highly-specialised medicines per se.
2. That the multiple pharmaceutical schedules and Exceptional Circumstances schemes currently in existence be replaced by a single ‘New Zealand Pharmaceutical Schedule’ (covering community, cancer and hospital pharmaceuticals) and a single Exceptional Circumstances scheme.
3. That PHARMAC be responsible for all the pharmaceuticals on the New Zealand Pharmaceutical Schedule and the Exceptional Circumstances scheme referred to above. This would involve clinical and economic assessments, funding decision-making and the procurement of pharmaceuticals (i.e. in addition to community pharmaceuticals, PHARMAC should also take responsibility for all hospital and cancer pharmaceuticals).
4. That DHBs continue to be responsible for managing their spending on pharmaceuticals (through the above-mentioned New Zealand Pharmaceutical Schedule and Exceptional Circumstances scheme). Particularly for small DHBs (without the necessary financial capacity due to their small scale), for some parts of the Exceptional Circumstances scheme this may involve risk pooling with other DHBs.
5. That a New Zealand pharmaceutical formulary be established to support clinical decisions regarding the use of pharmaceuticals.
6. That PHARMAC establishes new processes, and refines existing ones, to ensure effective and timely funding decision-making. Such decisions should not be made unreasonably slower than current funding decisions for hospital medicines.
7. That PHARMAC establishes new processes to ensure decisions about the New Zealand Pharmaceutical Schedule and the Exceptional Circumstances scheme are communicated more effectively to all stakeholders – especially prescribing and dispensing clinicians.
8. That PHARMAC shares its clinical and economic assessments (and its expertise) with clinicians around New Zealand to support their clinical decision-making with respect to implementing the New Zealand Pharmaceutical Schedule and the Exceptional Circumstances scheme from within DHBs’ budgets.
9. That PHARMAC engages more effectively with both clinical networks and DHB funding managers to increase transparency and create a broader sense of involvement and ‘ownership’ of decisions.

10. That PHARMAC improves and extends its Pharmaceuticals & Therapies Advisory Committee (PTAC) and specialist advice processes, including greater involvement from specialists and other interested clinicians.
11. That MedSafe and PHARMAC are directed to ensure low-cost and highly-specialised medicines (as described elsewhere in the Report) are more readily available in New Zealand than they are now.
12. That PHARMAC be responsible for clinical and economic assessments, funding decisions and procurement with respect to *all* health technologies (e.g. pharmaceuticals, devices, vaccines and medical and surgical procedures and equipment).
13. That across the health system as a whole, greater efforts are made to achieve consistency in funding decision-making processes and ultimately the value for money of spending on all types of health technologies.
14. That further attempts are made to encourage a constructive national discussion about the ethical issues and funding dilemmas related to high-cost, highly-specialised medicines.
15. That the Ministry of Health further examines the incentives in place to encourage the optimal and ethical use of medicines in New Zealand.

## **1. Introduction**

The Minister of Health asked us to review access to high-cost, highly-specialised medicines in New Zealand. Our Terms of Reference are in Appendix 1.

This is our preliminary report, representing ‘our thinking and direction’ at this point in the review process (1 December 2009). The report is intended to promote discussion – to be something of a ‘stalking horse’ for eliciting feedback from readers. We would like to know what you think by the end of February 2010 (our contact details are in the Conclusion), including:

- What have we got right?
- What have we got wrong?
- What have we missed?
- How might our recommendations be implemented (if you agree with them)?

If you know of others who might be interested in the report, but who we have missed when we distributed copies, please send the report to them or let us know. We will incorporate all feedback, where appropriate, into the final version of the report, to be presented to the Minister by 31 March 2010.

Our panel was convened in June 2009. Since then the Ministerial Review Group (MRG 2009) has recommended major reforms to New Zealand’s health system. In writing our report, we are mindful of the MRG’s recommendations that relate to our areas of interest.

Our thinking has also been informed by the individuals and organisations listed in Appendix 2 who kindly shared their expertise and opinions with us. The usual disclaimer applies: responsibility for the ideas in this report lies with us, the members of the Review Panel. In addition, we have benefited from reading a wide range of written material, as recorded in Appendix 3.

We would like to acknowledge that none of us (members of the Panel) are experts with respect to the operational details of New Zealand’s medicines system.<sup>3</sup> Compared to the many highly skilled individuals who work within the system, we are ‘outsiders looking in’. Accordingly, the analysis and recommendations in this report are of a relatively general (overview) nature. This means that implementing our recommendations (should the Minister and his Government choose to do so) will ultimately hinge on the leadership and specialised knowledge of the above-mentioned individuals working within the system.

Our report is organised as follows. In the following section, we discuss what we think ‘*high-cost, highly-specialised* medicines’ are. We also briefly review how pharmaceutical funding

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<sup>3</sup> See the footnote on the title page outlining our respective backgrounds.

decisions are made in New Zealand, and also consider the fact that there are many possible – and inevitably conflicting – ethical positions with respect to the meaning of ‘value for money’ when thinking about which medicines to fund. This ‘preliminary discussion’ is in general terms and is intended to help ‘frame’ the remainder of the report.

The subsequent three sections address each the following three questions (and related issues), consistent with the main issues raised in our Terms of Reference (see Appendix 1):

- How does New Zealand’s access to high-cost, highly-specialised medicines compare with other OECD countries (particularly Australia) on a population basis, and why do any such differences arise?
- What are problems – perceived and real – with New Zealanders’ access to high-cost, highly-specialised medicines, and are certain people or groups of people particularly disadvantaged?
- What are practical and affordable solutions to these problems, including how might the role and administration of the Exceptional Circumstances schemes be improved?

The report concludes by repeating our request for feedback from readers by the end of February 2010 and providing our contact details. (The report’s main conclusion and recommendations are presented in the Executive Summary.)

We have tried to be concise in the main body of the report. Particularly in the next section, elaborations of the main points are in the footnotes, for readers who are interested in such details. Arguably, the footnotes are excessive; however, given the wide range of backgrounds and interests of potential readers, we decided to err on the side of over-explaining rather than under-explaining things.

Finally, we would like to thank all the people with whom we have consulted (listed in Appendix 2 – and please accept our apologies if we have not acknowledged you, and please get in touch so we can remedy our mistake). We have been particularly impressed by the expertise and the enormous goodwill and dedication of these people – clinicians, managers, policy-makers, patients and their advocates, scientists, pharmaceutical industry representatives, etc. Thanks to Michael Hampl and Megan Simmons of the Ministry of Health for policy advice and administrative support, and to the staff at PHARMAC, in particular Fiona Rutherford.

## 2. Preliminary Discussion

What are ‘high-cost’ medicines?

What are ‘highly-specialised’ medicines?

How are pharmaceutical funding decisions made in New Zealand?

What does ‘value for money’ mean when thinking about which medicines to fund?

During our consultations with the individuals and organisations listed in Appendix 2 we encountered a variety of definitions of ‘high-cost, highly-specialised medicines’. Not surprisingly, we also came across a variety of opinions – many strongly expressed – about whether particular medicines should be funded or not.

Therefore, with the objective of ‘framing’ the other sections in the report, in this section we discuss in general terms what we think ‘high-cost, highly-specialised medicines’ are. We also briefly review how pharmaceutical funding decisions are made, and also consider the fact that there are many possible – and inevitably conflicting– ethical positions with respect to the meaning of ‘value for money’ when thinking about which medicines to fund.

Consistent with most of the people with whom we have consulted, we have chosen to interpret ‘high-cost, highly-specialised medicines’ to mean *high-cost and/or highly-specialised*. Figure 1 represents the simplest possible combinations of these two dimensions. The subject of this review – *high-cost and/or highly-specialised* – corresponds to the three coloured cells in the figure.

Costliness	<b>High Cost and Not Highly Specialised</b>	<b>High Cost and Highly Specialised</b>
	Low-Cost and Not Highly-Specialised	<b>Low Cost and Highly Specialised</b>
	Degree of Specialisation	

**Figure 1: Simple schematic representation of high-cost and/or highly-specialised medicines**

We begin by discussing these two dimensions individually, before combining them as *high-cost and/or highly-specialised* medicines.

## What are *high-cost* medicines?

*High-cost* medicines may be defined as medicines that, compared to others, are expensive relative to the health benefits patients receive from them. As we discuss later, the number of patients treated and the corresponding total cost at the health-system level may be small or large.

When comparing different medicines, as well as other health ‘technologies’ (i.e. devices, medical and surgical procedures and equipment), with respect to their cost-effectiveness<sup>4</sup> it is common practice for their health benefits to patients to be measured in generic units, known as “Quality-Adjusted Life Years (QALYs) gained” (i.e. “gained” from using the medicines).<sup>5</sup> Thus, consistent with the definition in the previous paragraph, high-cost medicines have a high cost per QALY gained.

How high is a *high* cost per QALY? There is no precise definition for New Zealand. According to PHARMAC (2006):

There is no formal dollar value at which a pharmaceutical is termed “high cost”, as over time what constitutes high cost has and will change. Funding of a medicine 5 years ago at \$20,000 for each person over a year was very high cost, while now it is much more in the order of \$20,000 to \$100,000. In future, “high cost” could be much more.<sup>6</sup>

At the other extreme, high-cost medicines can be potentially *very, very* high cost (and so be unlikely to be funded in New Zealand). For example, during our consultations we were told of biological medicines costing in the order of \$500,000 per patient per year (i.e. more than that amount in cost per QALY terms), and that are sometimes required for the patient’s whole life (e.g. 80 years).

Corresponding to a high cost per QALY, the *total cost* for a high-cost medicine at the health-system level may be relatively small or large, depending on the number of patients treated.

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<sup>4</sup> Such comparisons are necessary when health care spending is being allocated across alternative possible uses – e.g. when allocating Vote Health, or prioritising pharmaceuticals for the Pharmaceutical Schedule, etc. This is discussed in more detail later in this section.

<sup>5</sup> QALYs (Quality-Adjusted Life Years) combine length of life, measured as the number of years of life, with health-related quality of life (HRQoL). HRQoL is represented on a scale where 1 = perfect health and 0 = dead, and with negative values corresponding to health states considered to be worse than dead. Years of life × HRQoL = QALYs. For more information about QALYs, see Drummond et al. (2005), for example. For information about how PHARMAC employs QALYs for performing Cost-Utility Analysis (used for assessing the relative cost-effectiveness of pharmaceuticals considered for funding), see PHARMAC (2007a).

<sup>6</sup> Note, though, that PHARMAC does not have an ‘official’ cost-per-QALY threshold – as, amongst other reasons, this would constrain PHARMAC in its price negotiations with pharmaceutical companies.

Clearly, for a given cost per QALY, the more patients treated the greater the total cost of the medicine.

The number of patients to be treated also determines how the decision whether or not to fund a medicine is reached from an administrative point of view. With more than nine patients nationwide<sup>7</sup> PHARMAC<sup>8</sup> follows its standard decision-making processes (discussed below) for managing the Community Pharmaceutical Schedule. With fewer than ten patients nationwide (who are usually known by name), the decision is reached via the Community Exceptional Circumstances scheme (PHARMAC 2008).

### **What are *highly-specialised* medicines?**

Relative to ‘high-cost’ medicines, defining ‘highly-specialised’ medicines is less clear-cut. One possibility is that they are medicines that are targeted at relatively few patients; that is, they are *highly specialised* (targeted) with respect to who they treat. This includes medicines administered through the Community Exceptional Circumstances scheme (fewer than ten patients), as referred to above.

Another interpretation of ‘highly-specialised’ medicines is that they are medicines that are either technically sophisticated (i.e. *highly specialised*) in their manufacture; for example, so-called ‘large molecule’ medicines (which also tend to be ‘high cost’). Or, if they are not technically sophisticated, there is ‘something special’ in their procurement, manufacture or chemical stability. Thus, although they may be inexpensive, they are not easily sourced through the usual channels; examples include indomethacin, amiloride, quinine, captopril, and KCl (potassium chloride). Also some ‘orphan medicines’ create difficulties for particular population groups – e.g. the treatment of gout for Māori. Due to the relatively high cost of registering some of these low-cost (and low profitability) medicines, some are unavailable in New Zealand.

Combining the individual definitions above of *high-cost* and *highly-specialised* medicines respectively, *high-cost*, *highly-specialised* medicines are, as explained earlier, *high-cost and/or highly-specialised* – corresponding to the three coloured cells in Figure 1 above.

Not surprisingly, as discussed in Section 4, most of the concerns raised by the people with whom we have consulted were about difficulties accessing high-cost medicines (highly specialised or not – i.e. the top two cells in the figure). Hence most of the report is devoted to this group.

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<sup>7</sup> We understand that nine patients is, in effect, the threshold for defining a medicine as being delivered at the population level (rather than to known individuals). Clearly, this distinction is arbitrary – e.g. the difference between the total cost of treating nine and ten patients (i.e. the threshold for defining small versus large numbers of patients for the purpose of administering the CEC scheme) is small. For some high-cost medicines the total cost of treating nine patients will be less than the total cost of treating ten patients for other high-cost medicines.

<sup>8</sup> New Zealand’s Pharmaceutical Management Agency.

Nonetheless, despite their relatively low cost, we also heard of difficulties accessing low-cost and highly-specialised medicines (the bottom-right cell in Figure 1). For example, we heard of difficulties experienced by clinicians at Starship Children’s Hospital obtaining medicines appropriately formulated for children. And so, this group of medicines is also discussed in the report.

## **How are pharmaceutical funding decisions made in New Zealand?**

Before focussing exclusively on funding decision-making with respect to pharmaceuticals, it is worthwhile recognising, as discussed in Devlin and Hansen (2004), that in New Zealand there are, in essence, four inter-related levels for allocating (or ‘rationing’) health care spending. Each level can be thought of as corresponding to a question that must be answered:

Level 1. How many resources at the New Zealand-wide level – i.e. Vote Health – should be devoted to producing health care and disability support services (including spending on medicines)?

Level 2. How much of Vote Health should each of the 21 District Health Boards (DHBs) receive (including their share of the national pharmaceuticals budget)? (In addition, some of Vote Health is spent nationally; for example, on public health programmes and running the Ministry of Health, etc.)

Level 3. Which services and medicines (and in what quantities) should each DHB and PHARMAC fund?

Level 4. Which patients should receive these services and medicines?

Level 1 is largely determined by political processes, based on the Government’s priorities and its assessments of the trade-offs associated with alternative uses of the available Budget funds (resources) across alternative Votes – e.g. Vote Health versus Vote Education, etc. Nearly \$13 billion was allocated to Vote Health for 2009/10, out of a total of approximately \$65 billion of government spending (The Treasury 2009).

Level 2 is determined by the ‘population-based funding formula’. Each DHB’s share of Vote Health is calculated according to the number of people in its district, weighted by their age profile and other factors such as ethnicity and the proportion of people living in rural areas (who are more expensive to provide services to), etc.

The amount of Vote Health allocated to the Community Pharmaceutical Budget (\$694 million for 2009/10) – to be spent by DHBs – is decided by the Minister of Health, based on joint or separate recommendations from PHARMAC and the DHBs. In formulating their recommendations, PHARMAC provides DHBs “with a budget proposal based on an analysis of medicine usage trends and potential new medicines investments” (PHARMAC 2009a).

As when determining Vote Health, determining the size of the Community Pharmaceutical Budget depends on considerations of the trade-offs associated with alternative uses of the available funds. In this case, though, the trade-offs are largely with respect to alternative uses *within* the health system – e.g. community pharmaceuticals versus other types of ‘health technologies’ such as medical and surgical procedures and equipment, etc.

Level 3 with respect to decisions about which pharmaceuticals to fund are determined by both PHARMAC and DHBs. PHARMAC decides on behalf of the DHBs which medicines (including high-cost ones) to fund via its management of the Community Pharmaceutical Schedule<sup>9</sup> (and associated Community Pharmaceutical Budget) and the Exceptional Circumstances schemes.<sup>10</sup> In addition, DHBs decide which ‘Hospital’ and ‘Cancer’ medicines (typically, high-cost medicines too) to fund – and how much to spend – within their hospitals.<sup>11</sup>

PHARMAC decides which medicines to fund from the Community Pharmaceutical Budget according to nine decision criteria, as explained here (PHARMAC 2009b; also see PHARMAC 2009c):

In deciding which medicines to fund, PHARMAC seeks to balance the needs of patients’ access to healthcare against its responsibilities to the taxpayer. Given PHARMAC is managing taxpayer funding, PHARMAC’s decisions need to represent good value for money for the benefit of all New Zealanders.

PHARMAC uses the criteria set out below, where applicable and giving such weight to each criterion as PHARMAC considers appropriate, to make decisions about proposed amendments to the Pharmaceutical Schedule. Where PHARMAC makes decisions that do not involve amendments to the Schedule (for example, decisions relating to PHARMAC’s access and optimal use activities), it endeavours to use

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<sup>9</sup> The Community Pharmaceutical Schedule consists of over 1600 subsidised pharmaceuticals, as prescribed by family doctors (GPs), specialists, some nurse practitioners and midwives. We understand that at a national level, prescriptions for medicines on the Community Pharmaceutical Budget written directly by specialists accounted for 7% in numbers and 25% of value of national pharmaceuticals in 2008/09 (Fiona Rutherford, PHARMAC, personal communication).

<sup>10</sup> There are three Exceptional Circumstances (EC) schemes: Community EC, Hospital EC and Cancer EC. These are discussed later.

<sup>11</sup> Decisions about the use of these drugs are made within local DHB funding environments. We understand that there is wide variation in the types of clinical and management processes across the DHBs to manage these areas of significant clinical activity (and also expenditure). With 21 different DHBs negotiating access to some of the higher cost medicines used in New Zealand (and in the process sometimes undermining the effectiveness of PHARMAC in its negotiating on behalf of the country), there were some inherent weaknesses. We did not see any evidence that the good practices followed in some DHBs were applied across other DHBs. We met senior personnel in one DHB who explained the clinical engagement processes were utilised to ensure broad clinical support. Our view is that although this is worthwhile, it is also an inefficient and probably inadequate process for managing the more than \$200m of Vote Health resources involved nationwide.

these criteria, to the extent that they can be applied to those decisions. The criteria for decisions about proposed amendments to the Schedule are:

1. The health needs of all eligible people within New Zealand;
2. The particular health needs of Māori & Pacific peoples;
3. The availability and suitability of existing medicines, therapeutic medical devices and related products and related things;
4. The clinical benefits and risks of pharmaceuticals;
5. The cost-effectiveness of meeting health needs by funding pharmaceuticals rather than using other publicly funded health & disability support services;
6. The budgetary impact (in terms of the pharmaceutical budget and the Government's overall health budget) of any changes to the Schedule;
7. The direct cost to health service users;
8. 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
9. Such other criteria as PHARMAC thinks fit. PHARMAC will carry out appropriate consultation when it intends to take any such "other criteria" into account.

When considering which medicines to fund, PHARMAC's assessment process includes Cost-Utility Analysis – specifically, estimates of cost per QALY (related, arguably, to criterion 5 above) (see PHARMAC 2007a). PHARMAC also considers a medicine's total cost (i.e. the impact on the Community Pharmaceutical Budget – criterion 6 above), as well as the other decision criteria noted above. All else being equal, the higher the cost per QALY and/or the greater the total cost, the less likely a medicine will be funded.

By definition, high-cost medicines are expensive – as discussed earlier, both in terms of their *cost per QALY* and also, potentially, their *total costs*. And so PHARMAC may decide not to fund a particular high-cost medicine – because, in PHARMAC's judgement (based on the nine decision criteria above), the medicine does not represent (good) value for money. In fundamental terms, benefits of greater value could be realised (at a health-system level) if other, cheaper medicines were bought instead. (In other words, the 'true' cost of high-cost medicines is their *opportunity* cost in terms of the value that could be realised from lower-cost alternatives that must be foregone.)

Clearly, when a particular high-cost medicine is not funded this results in patients being denied it. If other treatments are unavailable, this inevitably results in the health of these patients suffering (as it would if any particular health intervention is delayed or denied). (Concern about this reality presumably motivated this review into access to high-cost and/or highly-specialised medicines in New Zealand.)

Fundamental to the discussion above about how pharmaceutical funding decisions are made is the notion of 'value for money'. As mentioned at the beginning of this section, during our

consultations we have come across a variety of opinions about whether particular medicines should be funded or not. In the remainder of this section, we discuss the fact that there are many possible – and inevitably conflicting– ethical positions with respect to the meaning of ‘value for money’ when thinking about which medicines to fund.

### **What does ‘value for money’ mean when thinking about which medicines to fund?**<sup>12</sup>

As discussed earlier, when different medicines – as well as other health ‘technologies’ (i.e. devices, medical and surgical procedures and equipment) – are being compared relative to each other with respect to their cost-effectiveness, their health benefits to patients are usually measured in terms of QALYs (Quality-Adjusted Life Years) gained. Considering the QALYs gained from using a medicine makes sense, as the objective of treating patients is to improve their health (and clearly this is of value).

However, there are other potentially important sources of value associated with improving patients’ health. In general terms, these other sources of value include the ‘equity’ or ‘social justice’ gains (arguably criteria 2, 8 and 9 in PHARMAC’s list above) from treating patients who are in relatively poor health – i.e. patients whose lives are threatened or whose health-related quality of life is poor – relative to treating relatively healthier patients with cheaper medicines. (This is especially germane in the present context, given the patients treated with high-cost medicines are often in poorer health relative to other patients.)

All such evaluations and comparisons of value across different patients and patient groups, and the funding decisions that follow from them, are inherently *normative* in nature. Inevitably they depend on decision makers’ value judgements (or ethical positions), which, in general terms, depend on their beliefs about social justice (or equity). In theory, there is an infinite number of such value judgements (ethical positions) possible. For illustrative purposes it is perhaps instructive to contrast two of the most well-known ethical positions – utilitarianism (or ‘Benthamism’)<sup>13</sup> and Rawlsianism<sup>14</sup> – with respect to their implications for deciding which medicines to fund.

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<sup>12</sup> This sub-section is based on ideas discussed in Hansen (2006), which includes diagrammatic illustrations of some of the ideas discussed here (and others).

<sup>13</sup> Named after Jeremy Bentham (1748-1832), an English philosopher and legal and social reformer. Bentham originally expressed what has become known as the “greatest happiness principle” in terms of “the greatest happiness of the greatest number of people”; however, he later realised that it included two different and potentially conflicting principles (maximands), and so he abandoned the second part (“the greatest number of people”).

<sup>14</sup> Named after John Rawls (1921-2002), an American philosopher. Rawls advanced his well-known theory of justice, “Justice as Fairness”, as an alternative to utilitarianism (Rawls 1971). Central to the development of his theory is a famous ‘thought experiment’ in which people are asked to choose the moral principles they would like the society they inhabited to operate under. However, they have to make their choices from behind a ‘veil of ignorance’; that is, in ignorance of their own particular characteristics, such as their wealth and natural abilities (in the present context, their health), in that society. Rawls argues that most people would prefer a world in which the well-being of the worst off

The utilitarian ethical position seeks to maximise the total number of QALYs gained from a given amount of spending (the Community Pharmaceutical Budget). This means eschewing relatively high-cost medicines (i.e. with a high cost per QALY gained) in favour of lower cost medicines (with a lower cost per QALY gained). In effect, utilitarianism treats each QALY as being of equal value regardless of to whom it accrues, and all that matters is the total number of QALYs gained (in other words, one person's QALY gains are regarded as being as valuable as any other person's).

In contrast, the Rawlsian ethical position favours patients (and patient groups) with relatively poor health (e.g. in QALY terms) over patients with better health. Thus, all else being equal, medicines benefiting sicker patients should be funded in preference to medicines benefiting healthier patients, with the ultimate objective being the equalisation of people's health. Rawlsianism is likely to result in fewer QALYs gained from the Community Pharmaceutical Budget than the utilitarian value judgement (i.e. the number of QALYs gained will not be maximised). Relative to utilitarianism, Rawlsianism is more likely to favour the funding of high-cost, highly-specialised medicines.

As mentioned above, there is an infinite number of such value judgements (ethical positions) available in theory. With reference to PHARMAC's nine decision criteria reproduced earlier above, in general terms, PHARMAC's ethical position lies (as you would expect it to) somewhere between these two extremes of utilitarianism and Rawlsianism.

Naturally, the people with whom we have consulted endorsed, implicitly or explicitly, a variety of ethical positions. Some are apparently very much at odds with PHARMAC's ethical position, whereas others seem quite close. The end result is that some people are in favour of much greater funding of high-cost medicines than is currently the case; and others are in favour of less funding. Such disagreements are natural.

As clearly articulated by Hope et al. (2002, pp. 147-51), the following ethical considerations (expressed as questions) succinctly illustrate a range of ethical positions that would likely find some degree of support from most people (arguably they are consistent with PHARMAC's nine decision criteria reproduced earlier, albeit they are more clearly articulated).<sup>15</sup> According to Hope et al, these considerations "arise in practice and ... raise the question of whether more (or less) should be spent per QALY than the [threshold amount – in the context of the United Kingdom]."

- "Should treatments for the young have a greater priority than treatments for the old?"

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in society was maximised (because they might turn out to be that person when the veil of ignorance is lifted). This is often known as the 'maximin' rule: the *maximisation* of the *minimum* (or worst possible) outcomes.

<sup>15</sup> This list of possible value judgements is not exhaustive; for a more comprehensive survey, see Schwappach (2002) for example.

- “Should identifiable patients be favoured over non-identifiable patients?<sup>16</sup> The rule of rescue.”
- “Should palliative care be given higher priority than would result from the QALY calculation?”
- “Should higher priority be given to those who are particularly badly off with regard to their health?”<sup>17</sup>
- “Should higher priority be given if there is no alternative treatment?”
- “How is ‘double jeopardy’ to be dealt with?”<sup>18</sup>

The challenge for people in New Zealand as a whole, including policy-makers, is to reach some kind of national consensus about how to deal with the above ethical considerations (and others). In particular, for example:

- How ought these and other ethical considerations be traded off against each other when they are in conflict (as they almost always are in practice)?
- How much are New Zealanders willing to pay through the public health system for high-cost medicines to treat very serious illnesses (i.e. life-threatening and/or serious in terms of health-related quality of life). In particular, how should ‘end of life’ treatments’ be dealt with?
- And hence what other treatments (or other things) are New Zealanders willing to give up to pay for such medicines?

Naturally, we (members of the Review Panel) have our own views (ethical positions) – but they are no more or less relevant than anyone else’s. There are no universally ‘right’ answers per se. As discussed earlier, attempts at arriving at such answers inevitably depend on individuals’ value ethical positions, which, in general terms, depend on their beliefs about social justice (or equity).

Simply ignoring the above issues (which are often extremely difficult, and sometimes painful, to contemplate) will not make them go away. One way or another they have to be faced. The issue, therefore, is how ‘best’ to resolve them? By not facing up to these issues, poorer-quality decisions may be arrived at by default, resulting in resources being wasted.

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<sup>16</sup> The value judgement implied by this question being answered in the affirmative is that the QALYs gained from preventing the immediate premature death of, in the extreme, a single identifiable individual (e.g. a heart attack sufferer) are inherently more important (valuable) than the same number of QALYs gained from slightly improving the health (or reducing the risk of ill health) of a group of people.

<sup>17</sup> This is qualitatively the same as the Rawlsian ethical position discussed earlier.

<sup>18</sup> ‘Double jeopardy’ relates to the possibility of co-morbidities in the patient group being considered for a medicine, that would have the effect of lowering the QALYs gained from using the medicine, all else being equal.

### 3. International Comparisons

*How does New Zealand’s access to high-cost, highly-specialised medicines compare with other OECD countries (particularly Australia) on a population basis, and why do any such differences arise?*

*What are the institutional arrangements by which prioritisation and funding decisions are made internationally (particularly in Australia and the UK)?*

*What’s in the pharmaceuticals ‘pipeline’?*

In this section we attempt to answer the first question above by comparing access to high-cost, highly-specialised in New Zealand vis-à-vis Australia. Given the economic and cultural similarities and the relative ease of migrating between the two countries, Australia is the country that New Zealanders most often compare their own country against.

Also of interest, especially to health care professionals and policy-makers, are the approaches and institutional arrangements by which prioritisation and funding decisions are made internationally. In this respect, PHARMAC is often compared to its proximate counterparts in Australia and also the UK – specifically, Australia’s Pharmaceutical Benefits Advisory Committee (PBAC) and the UK’s National Institute for Clinical Excellence (NICE). Thus, with reference to the second question above, in this section we also compare how prioritisation and funding decisions for such medicines are made in New Zealand vis-à-vis both Australia and the UK.

In addition, at the end of this section we briefly discuss what we learned during our consultations about possible pharmaceuticals available in the near future (i.e. the ‘pharmaceuticals pipeline’).

#### **Positive versus normative analysis**

When comparing access to high-cost, highly-specialised medicines in New Zealand vis-à-vis Australia, it is useful to appreciate the difference between *positive* and *normative* types of analysis.

A *positive* analysis addresses quantifiable differences in the medicines that are available in the two countries – in effect, based on a comprehensive stocktake of each country’s schedule of publicly-funded pharmaceuticals. In contrast, a *normative* analysis considers the extent to which such international differences are desirable or undesirable respectively. In other words, a *positive* analysis is concerned with ‘what *are*’ the differences between countries, and a *normative* analysis with ‘what *should* be’ (or *should* not be) the differences.

Both types of analysis are problematic in the present context – for different reasons. For a positive analysis, a comprehensive stocktake of each country’s schedule of publicly-funded

pharmaceuticals is very information intensive. It is not enough to simply note that a given medicine is available in a country; other, more contextual, information is required to be able to evaluate the extent to which the medicine is accessible. Such information includes the specific illnesses a medicine that is nominally available are used to treat, treatment guidelines, dosages, patient co-payments, etc.

A normative analysis is, arguably, even more problematic. If a particular medicine is not available in a country (as revealed via a positive analysis), this does not necessarily mean that that is undesirable (normative analysis). Further analysis into the reasons for the medicine's unavailability is required – i.e. for it not being a funding priority in one country when it is in another. Such reasons, in broad terms, will likely include differences between the countries in their populations (i.e. health needs) and economic circumstances, differences in their priorities for their societies in general and their health systems in particular (including access to medicines), and differences in their approaches to deciding on such priorities and implementing them.

Having thus introduced these two types of analysis and their attendant difficulties, we now offer some observations about each of them individually.

### **Positive-analysis observations**

Most people with whom we have consulted believe that, overall, New Zealand has less access to high-cost, highly-specialised medicines than Australia.

This popular viewpoint is reflected in this excerpt from The National Party's medicines policy document (23 October 2008) reproduced in our Terms of Reference (Appendix 1):

Access to high cost highly specialised medicines in New Zealand is very limited compared to other countries. For example, in the six years to mid-2006, only 20 innovative new medicines were subsidised by New Zealand authorities, while in the same period 78 innovative new medicines were subsidised in Australia.

Similarly (in more detail and updated), according to a recent RMI (Researched Medicines Industry) newsletter (RMI 2009, our bolding):

The gap between New Zealand and Australian access to innovative medicines has grown over the three years since the original comparative analysis on access to medicines in the two countries was reported in 2006. The initial work was by Michael Wonder, from Novartis Pharmaceuticals Pty Australia. At the time he reported on access between May 1, 2000 and June 30, 2006, he found that 58 more innovative prescription-only medicines were funded in Australia than New Zealand during that period. Of the 78 new medicines funded in Australia during this time, Wonder found that only 20 of those were funded in New Zealand. Updated analysis shows that since July 1, 2006 PHARMAC has funded nine of those 58 medicines. However, during the same period, a further 35 innovative new prescription-only medicines, that are still not funded in New Zealand, have since been made available to patients

**in Australia. The gap between the two countries has now reached 84; i.e. 84 more medicines have been made available to Australians through the PBS (the equivalent to our Pharmaceutical Schedule) than were made available here in the same time period.**

In response to our request (consistent with our Terms of Reference), PHARMAC progressed their stocktake of each country's schedule of publicly-funded pharmaceuticals. PHARMAC summarises its analysis thus far as follows (Fiona Rutherford, PHARMAC; personal communication).

In summary, we have identified that of the 84 medicines on the RMI list:

- 6 are not currently funded in Australia
- 7 are now funded by PHARMAC
- 11 are hospital treatments which DHBs are responsible for making funding decisions on (we do not have readily accessible information about whether these are funded)
- 1 is a fertility treatment, which the Ministry [of Health] is responsible for funding
- 35 have been, or are being, assessed by PHARMAC, of which
  - 5 PTAC [Pharmaceuticals & Therapies Advisory Committee] has recommended by declined;
  - 6 PHARMAC has declined; and
  - 10 - 15 appear to offer little or no additional benefit over those funded in NZ.
- 1 has been discontinued by the supplier in NZ
- 16 are registered by Medsafe but have not been submitted to PHARMAC for funding consideration, of which half appear to offer little or no additional benefit over those funded in NZ
- 7 are not registered by Medsafe and have not been submitted to PHARMAC; of which half appear to offer little or no additional benefit over those funded in NZ.

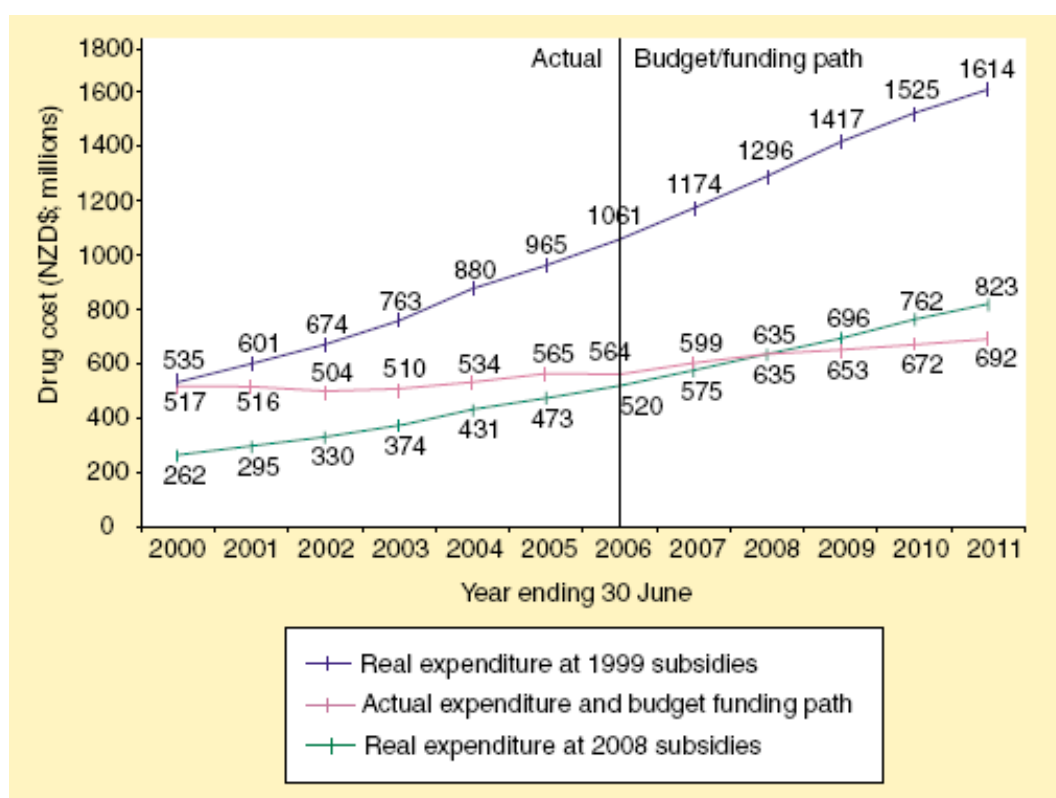
Our analysis reveals that 58 of the 84 medicines the RMI lists are not actually funded in New Zealand. On the face of it, only a small proportion of the 58 non-funded community pharmaceuticals medicines are likely to offer substantial therapeutic benefit.

As part of the same body of research, PHARMAC acknowledges that many caveats are necessary when performing international comparisons of publicly-funded pharmaceuticals. As mentioned earlier, as well as 'basic' information about whether or not a given medicine is available, 'contextual' information is also very important (i.e. specific illnesses, treatment guidelines, dosages, patient co-payments, etc), as are 'normative-analysis' elements discussed in the next section. Without such extra information, comparisons of countries' schedules of publicly-funded pharmaceuticals can be confusing. We understand that PHARMAC's

ongoing programme of research includes studying expenditure patterns for treatment groups between the two countries and volumes and costs.

Interpreting international comparisons of pharmaceutical spending as proportions of countries' gross domestic products (GDP) is also problematic. Aside from the fact that countries have different priorities for how they spend their national incomes (as will be discussed in the next sub-section), they also pay different prices for their pharmaceuticals and so they get different 'pharmaceutical bangs for their GDP bucks'. In other words, two countries could be spending the same amount (and, if they had the same incomes, the same proportion of their GDPs), and yet be receiving very different quantities of pharmaceuticals.

For the money that New Zealand spends on pharmaceuticals, there is clear evidence that it gets exceptional value – certainly relative to the likely counterfactual if PHARMAC did not exist. PHARMAC's putative impact on the Community Pharmaceutical Budget is illustrated in Figure 2, where actual expenditure (the relatively flat line) is contrasted with what spending would have increased to (the very steep line) had pharmaceutical prices not fallen in the last decade (in part as a result of PHARMAC's activities, but also due to 'natural' price decreases, as discussed below).



**Figure 2: Impact of PHARMAC on drug expenditure over time (reproduced from Grocott 2009)**

The dramatic outcome illustrated in Figure 2 is summarised by PHARMAC (2009d) as being evidence that "PHARMAC's purchasing power has tripled since 1993. This means we can

now subsidise about three times the amount of medicines that could have been bought with the same money in 1993.”

A large part of this threefold increase in PHARMAC’s purchasing power is attributable to PHARMAC’s success – probably greater than for any other country – at negotiating lower pharmaceutical prices from pharmaceutical companies. PHARMAC has successfully exploited its monopsony<sup>19</sup>-pricing power (arising from it being New Zealand’s main buyer of pharmaceuticals) by pursuing a range of competitive pricing strategies<sup>20</sup> and operating with a binding budget constraint. In addition, though, most pharmaceuticals’ prices have also ‘naturally’ fallen over time (in real, and sometimes nominal, terms) as a result of economies of scale in their production,<sup>21</sup> and as they eventually ‘come off patent’ and face competitive pressures.

A striking illustration of how dramatically some medicines have fallen in price in New Zealand is provided in Figure 3, which tracks the fall in price of fluoxetine from \$1.92 per capsule in 1993 to 5¢ now. There are many other examples of similarly dramatic price falls. For example, during our consultations we heard (and supported by PHARMAC 2007b) that New Zealand pays approximately half the price for statins that Australia pays.

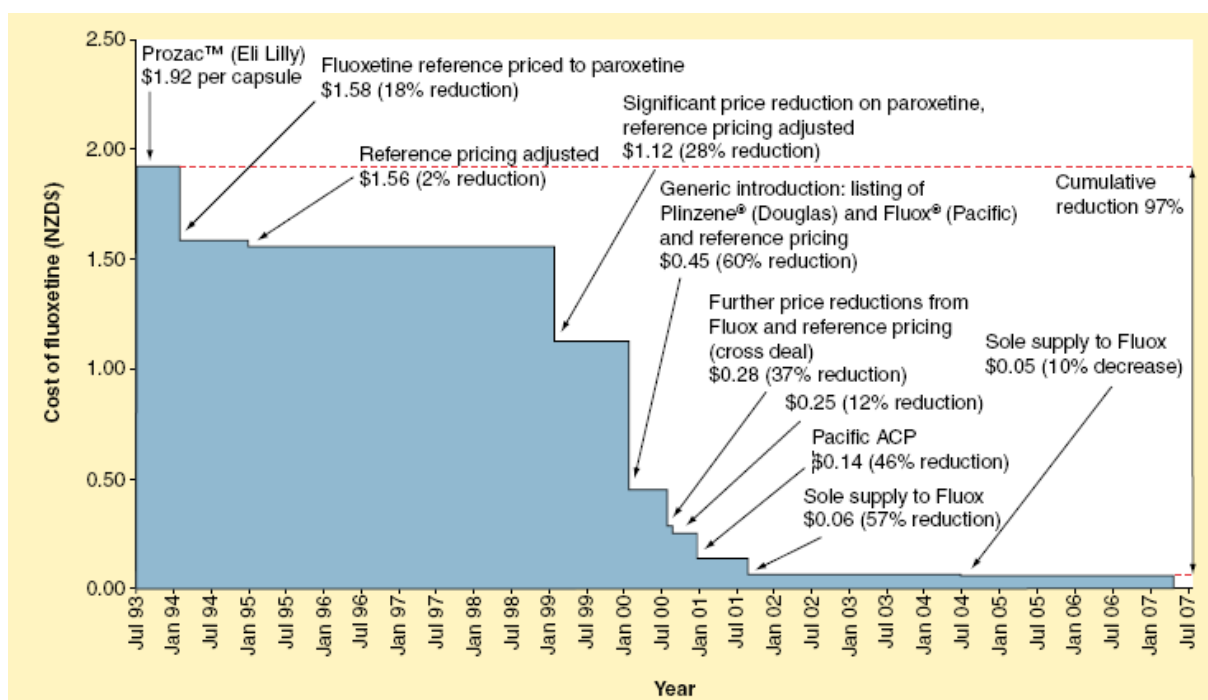


Figure 3: Price reductions for fluoxetine 1993-2007 (reproduced from Grocott 2009)

<sup>19</sup> A monopsony is a single buyer of a good (here pharmaceuticals), who, as a result of being the only buyer, is able to exploit its market power over sellers. [A monopsonist (buyer) can be thought of as being the equivalent of a monopolist (seller), but on the other ‘side’ of the market.]

<sup>20</sup> Including tendering, sole supply and reference pricing. See PHARMAC (2009e) for details.

<sup>21</sup> As increasing volumes of a pharmaceutical are produced, its average cost of production tends to fall.

On the other hand, as raised by many people with whom we have consulted, there are concerns that PHARMAC's competitive pricing strategies have resulted in a smaller range of medicines being available for particular conditions. A significant difference between New Zealand and Australia appears to be that PHARMAC often funds just one medicine in each therapeutic subgroup,<sup>22</sup> whereas Australia funds all brands within the sub-group (to the lowest priced brand, and typically to a different level than in New Zealand).

This tends to result in patients in Australia having a wider range of subsidised medicines available (albeit they face higher co-payments) than patients in New Zealand. For example, there is only one TNF-alpha inhibitors class of medicines – Humira (adalimumab) – on the Community Pharmaceutical Schedule (New Zealand), whereas, we were told that doctors in Australia have a choice of up to five or six medicines. Apparently 80% of people respond positively to a single TNF medicine but for the 20% who do not, they have no second-line alternative.

In our opinion, and as we discuss in the next section, such restricted choices in general in New Zealand is a legitimate concern. We acknowledge, though, that funding a single medicine in each therapeutic sub-group gives PHARMAC greater power to negotiate lower prices.

Another criticism that we heard relatively frequently during our consultations – especially from representatives of pharmaceuticals companies – is that, as a consequence of some high-cost, highly-specialised medicines not being funded in New Zealand (in contrast to other countries), undertaking pharmaceuticals research in New Zealand is not worthwhile for pharmaceutical companies. Other people with whom we have consulted (not from the pharmaceuticals companies) disputed this; they counter-argued that there are other more significant determinants of where pharmaceuticals research is based than whether or not the medicines being researched are likely to be funded in the country concerned. Overall, to us, this is a moot point that we are unable to resolve.

## **Normative-analysis observations**

As mentioned above, most people with whom we have consulted believe that, overall, New Zealand has less access to high-cost, highly-specialised medicines than Australia. If we accept this 'stylised fact' (and we have so far come across no evidence to reject it), then what, if anything, can we say about the desirability or undesirability respectively of this situation?

How might we go about explaining the difference between New Zealand and Australia in access to high-cost, highly-specialised medicines? As alluded to earlier, in broad terms, the two countries have different economic circumstances and different populations (as well as cultural differences). It is not unreasonable to suppose that they also have different priorities

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<sup>22</sup> A therapeutic subgroup is a group of medicines that have the same or similar therapeutic effects.

for their societies in general and their health systems in particular (including with respect to access to medicines). They also have different approaches to deciding on such priorities and implementing them.

Thus, the first thing to recognise is that New Zealand is much poorer economically than Australia. New Zealand's GDP per capita is approximately 80% of Australia's (World Bank 2008). This means that, all else being equal, New Zealand is less able than Australia to afford all things that are not available for free, including high-cost, highly-specialised medicines.

Moreover, the Treasury forecasts that New Zealand's relative prosperity will not improve in at least the next five years (The Treasury 2009). The government's fiscal deficits (from which the Community Pharmaceutical Budget is funded) and debt levels are also forecast to increase greatly. Hence, finding money for high-cost, highly-specialised medicines will be more difficult in the future than it is now.

Second, and obviously, New Zealand's and Australia's populations have different characteristics. Amongst other differences, New Zealand has more Māori and Pacific Island peoples respectively than Australia. Australia has more Aboriginal people, and other ethnic groups, etc. The effects on the demand for high-cost, highly-specialised medicines of such (and other) population differences are unclear to us; nonetheless, logically, such differences may explain part of the differences between the two countries in access to such medicines.

Third, and the main focus of the remainder of our discussion in this section, there are likely to be significant differences between New Zealand and Australia with respect to their priorities for their societies in general and their health systems in particular, and also differences in their approaches to deciding on such priorities and implementing them.

Specifically, it seems natural to expect that both countries have different preferences (or 'tastes') with respect how many resources at the national level – i.e. Vote Health in New Zealand – should be devoted to producing health care and disability support services. Likewise, the two countries are likely to also have different preferences with respect to how much they allocate to pharmaceuticals vis-à-vis other types of health care (or 'health technologies': devices, medical and surgical procedures and equipment).<sup>23</sup>

There are no 'magic' optimal proportions of GDP for these amounts. This is partly for the reasons discussed in the previous sub-section associated with the different prices countries pay for their pharmaceuticals (i.e. differences in their 'pharmaceutical bang for their GDP buck'), but also because this is a matter of 'national preferences'. Some countries would rather spend, say, an extra billion dollars of national income on defence, or education, or sport, etc., than spend it on health. And who is to say that is necessarily right or wrong?

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<sup>23</sup> In the previous section we briefly reviewed how pharmaceutical funding decisions are made in New Zealand, including deciding how many resources to allocate to health care spending in general and pharmaceuticals in particular.

National priorities depend on a wide variety of factors: historical, cultural, institutional, political, social, etc.

Similarly, from the resources allocated for pharmaceuticals, there is no ‘magic’ optimal (or universally accepted) formula for allocating it across the available pharmaceuticals competing for funding, including high-cost, highly-specialised medicines. New Zealand and Australia, and also the UK – as discussed at the beginning of this section, the two other countries that New Zealand compares itself against with respect to how prioritisation and funding decisions are made<sup>24</sup> – have significant differences in these respects.

## **Pharmaceutical funding decision-making in Australia and the UK**

The Australian medicines system is overseen by the Pharmaceutical Benefits Advisory Committee (PBAC). PBAC is an independent statutory committee appointed by the Minister of Health. The PBAC generally does not set its own work agenda, but reviews applications (usually submitted by manufacturers) for the listing of new medicines or of additional uses of already-listed ones. Evidence submitted by applicants is evaluated by the Department of Health, with assistance from contracted academic groups (e.g. health economist, etc).

Based on the evaluation, the PBAC may recommend an unrestricted listing on the formulary, or listing for specified indications only, and in some cases may require prior authorisation to prescribe. There is no specific cost-effectiveness threshold for approval. For example, a relatively high cost per QALY may be accepted for medicines for life-threatening conditions for which there are no effective alternative treatments, whereas a medicine with a relatively low cost per QALY may not be recommended if there is significant uncertainty in the estimate of cost-effectiveness. A medicine that has not been recommended by the PBAC cannot be added to the Pharmaceutical Benefits Scheme (PBS) formulary, but the Minister of Health may decide not to list a recommended medicine.

PBS processes are intended to ensure value for money for Australian taxpayers and to support affordable, equitable access to prescription medicines for all Australians, and are not intended as a mechanism for cost containment. The PBS operates under the umbrella of Australia’s National Medicines Policy, which has as its overall aim “to meet medication and related service needs, so that both optimal health outcomes and economic objectives are achieved.” (Lopert 2009).

According to Australia’s National Medicines Policy (Australian Government 2000), one of its strategic aims is the maintenance of:

a responsible and viable pharmaceutical industry ... It is essential that industry policy and health policy be coordinated, providing a consistent and supportive environment for the industry, and appropriate returns for the research and development, manufacture, and supply of medicines.

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<sup>24</sup> For a recent survey of another 11 countries, see Nikolentzos, Nolte & Mayes (2008).

In the UK, the medicines system comprises a national body, the National Institute for Health and Clinical Excellence (NICE), establishing standards, inviting independent academic institutions to review the evidence, and ultimately the individual Primary Care Trusts making purchasing decisions.

In contrast to these two systems, as is well known, the context for the New Zealand health system is one of explicit and binding budgetary constraints and careful prioritisation. As discussed in the previous section, decisions about which medicines to fund are determined by both PHARMAC and DHBs. Political interventions in funding decisions are rare (and in our opinion, and of most of the people with whom we have consulted, should remain so).

### **What's in the pharmaceuticals 'pipeline'?**

We conclude this section with a brief discussion of what we learned during our consultations about possible pharmaceuticals available in the near future. This 'pipeline' will be an important determinant (but not the only one, as alluded to earlier) of New Zealand's access to high-cost, highly-specialised medicines in the future, and of the corresponding funding pressures.

As the old saying goes: *Forecasting the future is easy; it's being right that is hard!* Nonetheless, the consensus from the experts in this area with whom we have consulted is that the medicines likely to be available in the future will be increasingly technically sophisticated and targeted at relatively few patients (i.e. *highly specialised* in both respects discussed in the Introduction to the report). Although new scientific discoveries are going to produce some exciting new options for treatment, we were also advised to be wary of some of the more extraordinary claims. Most future medicines will also be more expensive than the ones currently available.

Overall, it is not expected that there will be many new 'blockbuster' medicines that will have a high uptake and be able to be provided at relatively low cost over time. Instead, we were told, there will be increasingly narrow sub-types of medicines for treating smaller groups of patients, and these medicines will be increasingly expensive, with lower returns to their developers than in the past.

One important example is treatment for cancer, which is beginning to be treated as a chronic disease. Pharmaceuticals-based treatment will involve a more targeted attack on the mutant cells, with a 'wait-and-see' approach to new cancerous cells, rather than using a more toxic broad-based approach. Such medicines will be expensive, and will be prescribed to specific patients according to markers on individual genes. This will mean that there will be very few patients from whom pharmaceuticals companies are able to recoup the development costs of these new medicines.

## 4. Perceived Problems

*What are problems – perceived and real – with New Zealanders’ access to high-cost, highly-specialised medicines, and are certain people or groups of people particularly disadvantaged?*

In this section we report on the problems raised by the individuals and organisations (listed in Appendix 2) who kindly shared their expertise and opinions with us. We have done our best to record *all* problems raised with us. In the interests of confidentiality (and the implied terms of our consultation and submission process), none of the problems are attributed to the individuals or groups who raised them.

Not surprisingly, some of the problems raised are contradictory (*vis-à-vis* each other). ***And some we do not agree with.*** Nonetheless, in the interests of being fair to everyone, we have done our best to record them all. Our intention is that this section serves as an inventory of problems raised by the various people with whom we have consulted. They are grouped under the following dozen headings, presented as bullet points; and, where appropriate, we have added our own comments.

We would be interested in hearing from readers if we have missed any problems (e.g. with reference to the question above). As mentioned in the Introduction, we will incorporate all feedback, where appropriate, into the final version of the report (due for delivery to the Minister of Health by 31 March 2010).

Our ultimate reaction to the problems raised in this section are revealed by our response in the following section to the third and final question we seek to address (“*What are practical and affordable solutions to these problems, including how might the role and administration of PHARMAC’s Exceptional Circumstances schemes be improved?*”)

### **Total pharmaceuticals spending**

#### ***Problems reported to us:***<sup>25</sup>

- The Community Pharmaceutical Budget is insufficient for PHARMAC to be able to fund the high-cost, highly-specialised medicines that New Zealand should have access to (e.g. arguably, to ensure the same access as in Australia, as discussed in the previous section). As shown in Figure 2 above, total pharmaceuticals expenditure has grown very slowly (notwithstanding the tripling of PHARMAC’s purchasing power).

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<sup>25</sup> Note, as mentioned above, we have done our best to record *all* of the problems raised by the various individuals and organisations with whom we have consulted. We do not agree with some of them.

- The main beneficiaries of PHARMAC’s success at negotiating lower prices from pharmaceutical companies are DHBs (rather than patients who use pharmaceuticals), as they have been able to use the savings for other purposes (given that DHBs fund the Community Pharmaceutical Budget).
  - More specifically, low growth in the Community Pharmaceutical Budget has been used to enable relatively high growth in expenditure on surgical diagnostics and procedures.
  - Spending relatively small amounts on pharmaceuticals, particularly high-cost, highly-specialised medicines, might suggest the Government’s and New Zealand’s priorities are misplaced in broad terms. For example, it is reputed to cost \$90,000 per year to keep a prisoner in goal – in contrast, that amount (or even less) spent per patient on medicines is apparently considered to be excessive and not good value for money.
- If it is not possible to increase the Pharmaceuticals Budget, the available funds could be made to go further by increasing the range of safe and low-cost medicines (e.g. common painkillers, condoms, etc) that are not subsidised and are only available as ‘over-the-counter’ medicines.

***Our comments:***

- As illustrated in Figure 2, notwithstanding the low growth in the Community Pharmaceutical Budget, the dramatic increase in purchasing power (corresponding to lower pharmaceutical prices overall) has expanded the range of medicines and number of prescriptions from the Community Pharmaceutical Schedule.
- In the 2009 Budget, the Government increased the Community Pharmaceutical Budget by \$180 million during the next three years (i.e. \$40 million, \$60 million and \$80 million in each year).
  - This has had a noticeable impact this year. As result of this additional expenditure and continuing PHARMAC activity, eight new medicines have been funded and access widened for a further 55 medicines (PHARMAC 2009f).
  - We are mindful, though, that such increases in funding need to be considered in the context of New Zealand’s economic situation, which, as noted in the previous section, according to Treasury forecasts, is relatively bleak for at least the next five years.
- We do not know how large the Community Pharmaceutical Budget should be. As discussed in the previous section, international comparisons, in particular with Australia, are problematic.
- The Government has a small number of ‘levers’ it could use to release funds for additional high-cost medicines. One possibility (perhaps controversial), as noted above, would be to

remove some safe and low-cost medicines (e.g. common painkillers, condoms, etc) from the Community Pharmaceutical Schedule and have patients pay for them privately.

- Another possibility is to increase the patient co-payment for some patients to generate additional income from the approximately 60 million prescriptions annually in New Zealand (e.g. increasing the co-payment from \$3 to \$4, for say 50% of prescriptions, would raise \$30 million, all else being equal).

## **Budget-setting for pharmaceuticals and across the health sector overall**

### ***Problems reported to us:***

- The process that sets the size of the Community Pharmaceutical Budget is not transparent. It appears to be, in effect, a ‘residual’ item after DHBs’ funds have been allocated to other purposes.
- Relative to pharmaceuticals, expenditure on other health technologies is typically subjected to much less evaluation with respect to their ‘value for money’ (e.g. cost per QALY calculations). This is both ‘unfair’ and likely to result in allocative inefficiencies.
- In practice, DHBs that are in financial deficit argue (veto power?) to limit increases in the size of the Community Pharmaceutical Budget (which all DHBs contribute to pro rata).

### ***Our comments:***

- We suspect that the process by which DHBs and PHARMAC work together to agree on the Community Pharmaceutical Budget to propose to the Minister of Health is somewhat fraught.
  - This is especially so given there are 21 DHBs involved in negotiations with PHARMAC, and the relatively poor information available concerning the costs and benefits of spending on pharmaceuticals relative to other health technologies.
  - We note and support the current PHARMAC consultation on pharmaceutical subsidy eligibility (PHARMAC 2000g).
- We agree that it appears that prioritisation and funding decisions elsewhere in the New Zealand health system are not subjected to the same degree of rigour and scrutiny as in the pharmaceuticals area.

## **Exceptional Circumstances Schemes**

### ***Problems reported to us:***

- There are many perceived problems with the operation of the three Exceptional Circumstances (EC) Schemes – Community EC, Hospital EC and Cancer EC. These perceived problems include:
  - The efforts of EC applicants (medical professionals in hospitals) are duplicated in terms of gathering the required ‘background’ information (e.g. from the literature) – i.e. in effect, as a result of having to apply from scratch for medicines that other applicants have already applied for.
  - The ‘nine patient’ maximum (threshold) required for an application to be eligible under the Community EC scheme is too low.
  - Too much paperwork is required when making an EC application. As well as being inefficient, this is bad for morale as some health professionals feel insulted by having to justify their reasons for the application.
- Relative to the Community Pharmaceutical Budget, it is not clear how much money is spent on the three EC Schemes.
  - There is an overlap in budget figures for hospital expenditure and cancer medicine costs because hospital cancer treatments are a subset of all hospital medicines. We understand that DHB hospital expenditure on medicines is around \$150 million (although this figure is not up-to-date and may have increased). Of this \$150 million, the cost of hospital cancer treatments is about \$50 million.
  - Community EC has a budget of \$3 million per year currently and has spent slightly less than this in recent years.
  - There is also some expenditure on community pharmaceutical cancer treatments (as distinct from those provided in hospitals) of approximately \$30-\$40 million (it is difficult to be precise about this figure as quite a few treatments used for forms of cancer are also used for other diseases).
  - There is no central register of data on DHB expenditure on hospital medicines. PHARMAC used to receive data from the industry (the \$150 million estimate above is based on this) but this stopped several years ago. PHARMAC receives data from DHBs on what they purchase and uses this to understand usage of individual products. However, this process requires significant resources.
  - There is no set budget for Hospital EC. When assessing HEC applications, PHARMAC’s EC Panel may recommend the funding of a medicine for use in the community by a specific patient from the DHB’s own budget (to enable hospital specialists to legally prescribe medicines not on the Schedule). In these cases, the DHB may – but is not obliged to – fund the medicine.

- We are aware that in some cases clinicians have sought HEC approval for treatments that the hospital subsequently decides it will not or cannot fund. If the EC Panel recommends against funding of a medicine for use in the community by a specific patient, the DHB Hospital must not fund the medicine from its own budget. DHBs do not provide information that distinguishes between what their hospitals spend on medicines that they use in the hospital compared to what is dispensed to out-patients under HEC.
- The EC process does not suit paediatrics medicine, which needs appropriate formulations and access for children. This is discussed later below.
- The current policy (which needs to be changed) is that PHARMAC will not consider a Cancer EC application if the medicine is under review via the standard PHARMAC funding decision-making process. The latter often takes a long period of time, and many patients miss out on the opportunity to receive HCHS medicines during this time.

## **Patients or health service users**

### ***Problems reported to us:***

- Many groups and individuals are being denied pharmaceutical treatments for their illnesses. Some of these illnesses included rheumatoid-arthritis, renal lupus, scleroderma, lysozymal enzyme disorders, some cancers, etc.
- Some neonates are also denied treatment, as discussed in the following sub-section.
- No specific areas of concern for Māori and Pacific Islands peoples with respect to their access to high-cost, highly-specialised medicines were raised by the people with whom we have consulted.
  - However, there are issues around access, public education, use of medication (prescribing, pick up, use of medication).
- As discussed in the previous section, there are concerns that a negative consequence of PHARMAC's pricing practices is a smaller range of available medicines for particular conditions. PHARMAC often funds just one medicine in each therapeutic subgroup.
  - Hence, if that medicine is ineffective for a given patient then there are few, if any, substitutes available (in contrast to the, say, five or six options in Australia). For example, 20% of patients with rheumatoid arthritis are in this situation in New Zealand.

***Our comments:***

- As we discussed in Section 2, when high-cost medicines are not funded this results in patients being denied it, which, if other treatments are unavailable, inevitably results in the health of these patients suffering.
  - For some patients and their families this can be very serious – in the extreme, resulting in death.
- In our opinion, as we discussed in the previous section, this restricted choice in New Zealand is a legitimate concern.
  - On the other hand, as we noted in the previous section, funding a single medicine in each therapeutic sub-group gives PHARMAC greater power to negotiate lower prices.

**Paediatric medicines**

***Problems reported to us:***

- In general, because there are fewer trials of medicines in forms appropriate for children than for adults, there are relatively few medicines registered for children.
- Pharmaceutical companies register fewer medicines for children in New Zealand for a range of cost, profitability and scale reasons.
  - Hence some children's medicines that are registered, funded and available in other parts of the world are not available in New Zealand.
- As a consequence of other PHARMAC decisions, disruptions in medicine supply pose problems for paediatric patients.
- Pharmacists require stable formulations and funded ingredients to prepare medicines to replace those available overseas.
  - A readily available suspending agent (e.g. Ora-Plus/Ora-Sweet) is not funded in New Zealand as PHARMAC thinks it is too expensive and there is no competition (to bring prices down).
  - There are concerns about the chemical stability of formulations for children created by pharmacies, especially community pharmacies.
  - Patients (parents) need to travel to larger centres to pick up medicines because available stable formulations are not available.
- Paediatric Specialists suggest:
  - PHARMAC consider paediatric medicines as part of its tender processes.

- An evidence-based and available suspending agent is funded for paediatric specialist-prescribed medicines.
- Community pharmacists are given access to an approved database of approved formulations.
- Best-practice guidelines are introduced to ensure adequate notification of product discontinuation and brand changes.
- The EC process does not suit paediatrics, which needs appropriate formulations and access for children.
  - Children are not equivalent to ‘small people’ and they do not necessarily simply need smaller doses; rather, they may need to be reformulated (usually in a liquid form). As such, most paediatric medicines are low-cost, highly-specialised medicines.
  - The EC process is time consuming and inefficient.

## **Patient advocacy groups**

### ***Problems reported to us:***

- Some advocacy groups have been very successful in lobbying for their particular patients who need access to high-cost medicines to gain health or prolonged life.
  - This can sometimes be referred to as the high-profile media approach that tugs at the nation’s heartstrings. For example, public profiling of children with serious illnesses (or their parent with a serious illness) can influence public sentiment.
- There have also been instances over the past two decades where political parties in Opposition have made promises to specific groups that have subsequently been kept when there is a change in Government.
  - This process has not been generally well-received by the majority of advocacy groups who firmly believe that the process should be uniform for all groups of patients.
  - However there appears to be a view from some advocacy groups that some very high-need groups should be treated as special cases due to rarity and/or cost.

### ***Our comments:***

- We are impressed by the professional yet friendly demeanour of advocates who work tirelessly for their particular cause.
  - Some may be personally affected with either themselves or family members being afflicted, while others are employees or volunteers with a passion for their clients.
  - They play a multi-faceted role in educating the public about the needs of their particular group; educating family and affected members about how best to live with a

particular condition; whilst endeavouring to raise funds from the public and/or private sectors in order gain access to better treatments for their clients.

- The difficult reality is that there is no definitive ethical basis on which one group of patients can be unambiguously favoured over others – e.g. with respect to their type of illness, level of disability, age, extension of life, quality of life, family commitments, etc.

## **PHARMAC**

- As well as the ‘issues’ noted below,<sup>26</sup> there were many compliments directed at PHARMAC, including:
  - PHARMAC is staffed by many highly motivated, capable and focussed people.
  - The rationing role played by PHARMAC is broadly understood and accepted by most people with whom we have consulted.
  - Dealing with PHARMAC has become much easier – in particular, following improvements over the past few years.

### ***Problems reported to us:***

- The following criticisms were raised by a number of the people with whom we have consulted.<sup>27</sup>
  - Dealing with PHARMAC is in an environment of “low trust & high bureaucracy”, which is bad for morale of health professionals.
  - PHARMAC’s nine assessment criteria (reproduced in Section 2) are inherently subjective, and it is not clear how they are implemented.
  - PHARMAC’s analyses (i.e. Cost-Utility Analysis, QALYs and the incorporation of the other decision criteria) is fundamentally flawed.
  - Additional considerations such as ‘access’, ‘ethics’, ‘equity’, ‘affordability’ and ‘community values’ should be explicitly included in PHARMAC’s decision-making processes.
  - Overall, PHARMAC does not have a principle-based process for determining the Community Pharmaceutical Budget in consultation with DHBs.
  - Clinicians applying for a particular medicine to be funded often feel ‘in the dark’ with respect to the progress of the application, and also the reasons for the application being rejected (if this is the outcome).

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<sup>26</sup> Recall, the purpose of this section is to report on issues pertaining to the question posed in the section’s heading – e.g. it is not a review of PHARMAC per se.

<sup>27</sup> As we noted at the beginning of this section, in the interests of fairness to the people with whom consulted we have done our best here to record *all* of the issues that they raised, albeit some are contradictory (vis-à-vis each other) and some we do not necessarily agree with.

- Clinicians used words like “hassle”, “frustration” and “time wasting” to describe their access to PHARMAC decisions. One said (and others expressed similar sentiments): “We want to do the best for our patients and we cannot.”
- A perceived increase of emphasis on medicines for end-of-life treatments was mentioned – especially with high-cost cancer medicines (cancer is now the leading cause of death in New Zealand as cardiovascular risks fall with better prevention and management).
- The following allegations questioning PHARMAC’s integrity were raised by some of the people with whom we have consulted.<sup>28</sup>
  - As well as PHARMAC’s analyses (i.e. Cost-Utility Analysis, QALYs and the incorporation of the other decision criteria) being fundamentally flawed (as noted above), they are biased and dishonest.
  - PHARMAC is unreasonably slow to implement medical evidence, and sometimes it manipulates and misrepresents the clinical evidence in its prioritisation and funding decisions.
  - PHARMAC takes (unreasonable) pride in not increasing the Community Pharmaceutical Budget.
  - PHARMAC’s decision-making processes overall are deeply mistrusted, and there is concern that PHARMAC’s board and the Pharmaceuticals & Therapies Advisory Committee (PTAC) needs more ‘new blood’.
- As mentioned in the previous section, PHARMAC’s success at negotiating lower pharmaceuticals prices from pharmaceutical companies has caused many international pharmaceuticals companies to leave New Zealand.
  - One consequence is that there are very few clinical trials in New Zealand.
  - This reduces New Zealand’s capacity to retain highly-qualified clinical scientists.
- PHARMAC’s relationship with PTAC is inappropriate, as discussed in the following sub-section.

***Our comments:***

- Overall, we have been impressed in our dealings with PHARMAC by the high calibre of staff and the apparent quality of their work.
- Contrary to the allegations raised above, we have seen no evidence of anything that would lead us to question PHARMAC’s integrity.

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<sup>28</sup> See footnote 27 again.

- PHARMAC’s recent efforts at increased transparency and broader engagement seem to have reduced the frustration of clinicians and the patients they serve (e.g. the Pulmonary Hypertension process was positively commented on by a number of clinical observers.) It seems that this should continue.
  - We acknowledge, however, that PHARMAC’s negotiating positions and commercial sensitivities need to be protected.

## **Pharmaceuticals & Therapies Advisory Committee (PTAC)**

### ***Problems reported to us:***<sup>29</sup>

- There are concerns that as PTAC is dependent on PHARMAC for administrative support, this means PHARMAC does not receive independent advice.
  - Information is filtered by PHARMAC staff who have their own ‘world views’.
  - PTAC is not neutral – instead it is, in effect, PHARMAC’s lackey.
- PTAC members are not subject-area experts, and therefore they are unable to understand the evidence presented and so are not able to make the complex decisions required.
  - There should there be more specialists on PTAC and or specialist PTAC subcommittees – e.g. for paediatrics, cancer, rheumatoid arthritis, etc.
- A number of people with whom we have consulted recommended the establishment of a separate high-cost medicines committee with its own independent experts and external support.
- PTAC unreasonably considers the cost of medicines in its deliberations.
- In reaching its prioritisation decisions, PTAC should only assess the clinical effectiveness of medicines, and leave it to PHARMAC to worry about the cost side.
- Some clinicians expressed concerns about national and local funding decisions that did not make sense to them.
  - Example of failures to take a ‘systems’ view include: mycophenolate versus cyclosporine for renal lupus; and erythropoietin versus dialysis for renal failure.
- There is no right of appeal against PTAC decisions except through the courts.

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<sup>29</sup> See footnote 27 again!

***Our comments:***

- The Review Panel chair, Paul McCormack, was invited to attend a regular meeting of PTAC as an observer under the same obligations of confidentiality as all other visitors to PTAC.
  - He was impressed with the evident quality of the PTAC process. The PTAC members were demonstrably well prepared and independent.
  - More specifically, they were focused for the most part on the evidenced identified by PHARMAC and also from other literature discovered as part of their independent review, on the human impact of their considerations and the relative value of the medicines being compared, and finally on considerations of cost.

**Medsafe<sup>30</sup>**

- Some pharmaceutical formulations (including low-cost ones), especially for children, are unavailable in New Zealand.
  - One reason for this has been the fee charged by Medsafe to register medicines in New Zealand (historically, \$125,000; now \$80,000). This fee makes bringing many low-cost, highly-specialised medicines into New Zealand unprofitable (e.g. it was claimed that revenue of at least \$50,000 is required for it to be profitable to bring a medicine into New Zealand).
  - Medsafe can substantially lower the cost of registration but for some low-cost medicines there will still be few, if any, suppliers of the medicine. For some medicines it would still not be profitable given the low expected revenues and other expenses and compliance costs of bringing medicine into New Zealand, such as packaging and labelling.
- We heard that there is an Australian company specialising in picking up ‘orphan drugs’ – might it be possible to have a single market across the two countries?
- New Zealand seems to like having its own system for registering medicines. (Perhaps using other countries’ systems undermines New Zealand’s national prestige?) But is this efficient?

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<sup>30</sup> Medsafe – otherwise known as the ‘New Zealand Medicines and Medical Devices Safety Authority’ – is the business unit of the Ministry of Health responsible for the regulation of therapeutic products in New Zealand.

## **DHBs**

- 21 DHBs around New Zealand, all involved in clinical assessment and prioritisation processes, is a wasteful duplication of processes, imposing high administrative ('transactions') costs across the country.
- Despite agreements amongst the CEOs of DHBs about access to medicines in hospitals, there are national inconsistencies (especially for high-cost medicines) as a result of 21 DHBs reaching different decisions and facing different financial constraints. This phenomenon of national inconsistencies in access to medicines is often referred to as 'post-code prescribing'.
  - There is the challenge of accountability when a clinician pushes the pharmaceutical-availability boundary, without funding authority, in the interest of their patient.
- Clinicians are troubled about the inequity that results from these differential DHB funding decisions.
  - Some clinicians recommended a single streamlined process at the national level with one binding national decision for a particular medicine. The decision should then be widely disseminated so that all involved were informed (with the objective of a balanced single centralised national system supported by the appropriate local processes).
  - Some clinicians supported the process used by PHARMAC for Pulmonary Hypertension.
- 'Cost shifting' is an issue. This occurs when patients get started with a high-cost medicine at a tertiary hospital in a DHB area away from where they live, and then when they return to their 'home' hospital in another DHB area they bring the continuing expense with them (incurred by the second DHB). This can be financially crippling for small DHBs.

## **Prescribers**

- New Zealand does not have a formulary. Some people with whom we have consulted recommended that one be created.
- Many doctors are uncertain about PHARMAC's processes or previous decisions.
- Despite the STAT scheme<sup>31</sup> being in place since October 2003, many clinicians perceive the three-month prescribing regime is wasteful and the scheme underpinning is unduly complex.

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<sup>31</sup> This scheme involves dispensing medicines in quantities sufficient for a three-month supply all in one go.

## **Pharmaceuticals Industry**

### ***Our comments:***

- Overall, we were impressed by the calibre of the people we met with from the pharmaceuticals industry, and their apparent willingness to engage constructively in discussing ways of improving New Zealand's medicines system.
- However, we were somewhat surprised by the apparent lack of awareness and perpetuated mis-information from *some* pharmaceutical-industry insiders and their consultants about how PHARMAC makes decisions.
  - Specifically, some seem to be unaware of how Cost-Utility Analysis and QALYs work.
  - Are they truly unaware or are they disingenuous?
  - If they are truly unaware, this could easily be remedied.

## 5. Proposed Solutions

*What are practical and affordable solutions to the problems identified, including how might the role and administration of PHARMAC's Exceptional Circumstances schemes be improved?*

Having reported the problems raised by the people with whom we have consulted in the previous section, we now address the third and final of the questions consistent with the main issues raised in our Terms of Reference, as above. This section is largely the same as the Executive Summary, except that here (below) we also explain (justify) our recommendations.

As detailed in our recommendations below, we believe that there are significant improvements that can be made to how New Zealand's medicines system operates. Such improvements will likely increase access to high-cost, highly-specialised medicines.

However, because high-cost medicines are, by definition, expensive, there are no 'free lunch' ways of increasing access to them. We fully acknowledge that if high-cost, highly-specialised medicines are not provided to people who need them, and if other treatments are also unavailable, their health inevitably suffers. For some patients and their families this can be very serious – in the extreme, resulting in death.

### **More funding?**

Logically, in theory, there are four possible ways of increasing funding for (and thereby access to) high-cost, highly-specialised medicines:

1. Increase the Government's spending on medicines overall (i.e. reduce spending elsewhere or raise taxes) and spend at least some of that increase on high-cost, highly-specialised medicines.
2. Switch spending away from other (lower-cost) medicines in favour of high-cost, highly-specialised medicines.
3. Raise additional funds for high-cost, highly-specialised medicines via higher patient co-payments, 'risk sharing', etc.
4. Reduce wastage in the medicines system (or health system in general) and spend the savings on high-cost, highly-specialised medicines (and perhaps other medicines as well).

All but option (4) are likely to be contentious, as they all have opportunity costs in terms of alternative possible uses for the resources involved. Their desirability or undesirability respectively hinges on ethical (and political) considerations of the trade-offs associated with these alternative uses for the resources. There are no universally 'right' answers to the resource-allocation questions implied by options (1) - (3). Everyone has his or her own

personal preferences, which depends on each individual's value judgements (or ethical position), of which there is an infinite number theoretically possible.

Accordingly, we are not advocating for nor against options (1) - (3). We – and also most of the people with whom we have consulted – are not uniquely qualified to stipulate how much should be spent on medicines (including high-cost, highly-specialised ones). The amount spent on the public health system ('Vote Health') – from which medicines are funded – is a political decision determined by political processes. It depends on the Government's priorities and its assessments of the trade-offs associated with alternative uses of the available Government Budget funds (e.g. Vote Health versus Vote Education, etc).

### **More consistent decision-making processes?**

On the other hand, we believe that changes should be made to how decisions are reached about what Vote Health (whatever the amount available) is spent on. Specifically, across the health system as a whole, we would like to see greater efforts made to achieve consistency in funding decision-making processes and ultimately the value for money of spending on medicines (including high-cost, highly-specialised ones) and all other types of 'health technologies' such as devices, vaccines and medical and surgical procedures and equipment

Currently, it seems that medicines are subjected to much greater analytical scrutiny in assessing their 'value for money' – especially medicines on the Community Pharmaceutical Schedule – than other health technologies. Similar inconsistencies are evident for medicines available on the Community Pharmaceutical Schedule (decided by PHARMAC) relative to medicines available from hospitals (decided mostly by individual DHBs).

Broadly speaking, our recommendations are also intended to reduce complexity, increase transparency (except where there are commercial sensitivities such as PHARMAC's negotiations with pharmaceuticals companies), and ultimately to reduce the associated frustration evident throughout the health system.

Our recommendations thus far are:<sup>32</sup>

1. That prioritisation and funding decisions concerning high-cost, highly-specialised medicines continue to be made in the same way as such decisions for other medicines. To be clear, we are *not* recommending that new prioritisation processes and pools of funding be established for high-cost, highly-specialised medicines per se.

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<sup>32</sup> As noted above, these recommendations are the same (verbatim) as in the Executive Summary, except that here we also discuss or justify our recommendations.

***Explanation:***

- We have considered proposals for new processes and special pools of funding for high-cost, highly-specialised medicines suggested by some of the people with whom we have consulted, and as used in some other countries. We were not convinced that they are superior to the status quo (with our other recommendations below). We believe that any new additional processes and funding pools are more likely to unnecessarily increase complexity.
  - In particular, decisions about how much money should be allocated (‘top-sliced’) for a ‘high-cost, highly-specialised medicines pool’ would still require comparative value-for-money assessments relative to other (lower-cost) medicines (and other health technologies, as recommended below).
2. That the multiple pharmaceutical schedules and Exceptional Circumstances schemes currently in existence be replaced by a single ‘New Zealand Pharmaceutical Schedule’ (covering community, cancer and hospital pharmaceuticals) and a single Exceptional Circumstances scheme.

***Explanation:***

- We believe that the multiple budgets, processes and schemes are legacies of how PHARMAC and the DHBs evolved, and that they poorly serve their originally-intended purposes. We have been disturbed that there seems to be a relatively poor understanding of pharmaceutical expenditures in hospitals, and that they are less likely to be subject to value for money assessments and are relatively ‘hidden’ from scrutiny.
  - An Exceptional Circumstances scheme is essential – as well as legislatively required. In essence, *Exceptional* Circumstances are simply that: ‘exceptional’. There will always be such circumstances given humans are idiosyncratic and medical science brooks variations in treatments.
3. That PHARMAC be responsible for all the pharmaceuticals on the New Zealand Pharmaceutical Schedule and the Exceptional Circumstances scheme referred to above. This would involve clinical and economic assessments, funding decision-making and the procurement of pharmaceuticals (i.e. in addition to community pharmaceuticals, PHARMAC should also take responsibility for all hospital and cancer pharmaceuticals).

***Explanation:***

- PHARMAC has significant expertise in these areas. Arguably, it is a world leader.
- This might require the creation of some new schedule categories to include areas such as the support of clinical research.

4. That DHBs continue to be responsible for managing their spending on pharmaceuticals (through the above-mentioned New Zealand Pharmaceutical Schedule and Exceptional Circumstances scheme). Particularly for small DHBs (without the necessary financial capacity due to their small scale), for some parts of the Exceptional Circumstances scheme this may involve risk pooling with other DHBs.

***Explanation:***

- Despite our recommending a single New Zealand Pharmaceutical Schedule, we propose that DHBs continue to manage their own budgets as they do currently. This is in the interests of minimising the disruption that might otherwise occur from requiring DHBs to de-construct their costing models and information systems (with very few off-setting benefits).
  - This should also maintain the current incentives for clinicians within DHBs to manage their locally-controlled budgets.
  - One possibility might be to utilise the Operating Policy Framework (OPF) to define access rules for DHBS – in other words, to obligate DHBs to comply with a schedule. This should reduce concerns about ‘post-code prescribing’ across New Zealand, thereby achieving greater equity, while still requiring DHBs to continue managing their own budgets.
5. That a New Zealand pharmaceutical formulary be established to support clinical decisions regarding the use of pharmaceuticals.

***Explanation:***

- Many of the prescribing and dispensing clinicians with whom we have consulted pointed out the deficiency of there not being an up-to-date and relevant pharmaceutical formulary in New Zealand. We believe a formulary will improve the quality of both prescribing and dispensing in New Zealand.
  - We acknowledge, though, that there are significant costs associated with establishing such a formulary.
6. That PHARMAC establishes new processes, and refines existing ones, to ensure effective and timely funding decision-making. Such decisions should not be made unreasonably slower than current funding decisions for hospital medicines.

***Explanation:***

- If, as at Recommendation 3, PHARMAC is to be responsible for *all* pharmaceuticals on the New Zealand Pharmaceutical Schedule and the Exceptional Circumstances scheme, then PHARMAC must reach funding decisions not unreasonably slower than they are currently by DHB decision-makers (for hospital medicines, under the current regime). In other words, shifting such decisions to ‘the centre’ must not introduce undue new obstacles to clinical practice.
7. That PHARMAC establishes new processes to ensure decisions about the New Zealand Pharmaceutical Schedule and the Exceptional Circumstances scheme are communicated more effectively to all stakeholders – especially prescribing and dispensing clinicians.

***Explanation:***

- This follows-on from Recommendation 6 – our goal overall is to reduce complexity, increase transparency, and reduce frustration.
8. That PHARMAC shares its clinical and economic assessments (and its expertise) with clinicians around New Zealand to support their clinical decision-making with respect to implementing the New Zealand Pharmaceutical Schedule and the Exceptional Circumstances scheme from within DHBs’ budgets.

***Explanation:***

- This recommendation is intended to more fully utilise the capacity in the centre (PHARMAC), where it can add value, and then localise that so that local clinicians can effectively and efficiently make their prescribing decisions.
  - We believe that PHARMAC should make its first-level assessments available in the form of relevant evidence-bases and draft guidelines to relevant prescribers and dispensers across New Zealand so that the information can be assessed and ‘socialised’ at the local level.
9. That PHARMAC engages more effectively with both clinical networks and DHB funding managers to increase transparency and create a broader sense of involvement and ‘ownership’ of decisions.

***Explanation:***

- We believe that PHARMAC should further develop a national network of clinicians and DHB funding managers (i.e. ‘implementation-type’ people) to more fully engage with them for a ‘whole of system’ perspective with respect to the implications of funding decisions.

- PHARMAC should actively promote its ‘0800 PHARMAC’ helpdesk service to clinicians, where they can discuss their intentions to apply for medicines for funding with a competent PHARMAC staff member. One thing that might be discussed, for example, are funding-request precedents (to eliminate duplications of funding applications by clinicians).
10. That PHARMAC improves and extends its PTAC and specialist advice processes, including greater involvement from specialists and other interested clinicians.

***Explanation:***

- We believe that PHARMAC should have more specialist advisory committees to inform PHARMAC and PTAC decisions. Although we recognise that critical (generalist) analytical skills are essential in this kind of work, greater involvement by specialists could help allay some concerns that they (and via them, their patients) are not adequately represented and consulted.
  - Similarly, we believe that clinicians should be invited to present to PTAC. This would increase confidence that PTAC’s recommendations to PHARMAC are robust and independent.
  - We believe consideration should be given to allowing pharmaceutical companies the opportunity of presenting to PTAC, subject to their presentations being, in effect, cross-examined by PTAC and PHARMAC, as appropriate. This could help allay some concerns that clinical evidence and economic assessment data are misrepresented to PTAC by PHARMAC. However, any practical issues associated with this suggestion should be carefully considered.
  - We believe that PTAC should continue to consider value for money (i.e. cost-effectiveness) when making funding recommendations.
11. That MedSafe and PHARMAC are directed to ensure low-cost and highly-specialised medicines (as described elsewhere in the Report) are more readily available in New Zealand than they are now.

***Explanation:***

- We believe that the burden of Medsafe’s registration processes and costs act as a major barrier to access to low-cost and highly-specialised medicines in New Zealand. It seems that no agency currently tracks which medicines are ‘falling through the gaps’, and none accepts responsibility for addressing this problem.

- Medsafe and PHARMAC should clarify which low-cost and highly-specialised medicines are no longer available in New Zealand – and why. They should then work together to ensure a pathway for accessing medicines that are clinically important.
  - Medsafe and PHARMAC should ensure that it is as easy as possible for low-cost and highly-specialised medicines to be registered in New Zealand, subject to safety requirements.
  - Consideration should be given to establishing a national dispensing service for highly-specialised medicines that are used infrequently but which have important chemical stability characteristics, etc.
12. That PHARMAC be responsible for clinical and economic assessments, funding decisions and procurement with respect to *all* health technologies (e.g. pharmaceuticals, devices, vaccines and medical and surgical procedures and equipment).

***Explanation:***

- As noted earlier, PHARMAC has significant expertise at assessing treatments (currently medicines) in both clinical and economic terms. We believe it is possible for PHARMAC to extend its scope, relatively cost-effectively, to include other health technologies.
13. That across the health system as a whole, greater efforts are made to achieve consistency in funding decision-making processes and ultimately the value for money of spending on all types of health technologies.

***Explanation:***

- As discussed at the beginning of this section, it seems that medicines are subjected to much greater analytical scrutiny in assessing their value for money – especially medicines on the Community Pharmaceutical Schedule – than other health technologies.
- Similar inconsistencies are evident for medicines available on the Community Pharmaceutical Schedule (decided by PHARMAC) relative to medicines available from hospitals (decided mostly by individual DHBs).
- We believe that significant increases in allocative efficiency across the health system can be realised by extending the decision-making framework based on methodical clinical and economic assessments (e.g. as exemplified by PHARMAC) to all health technologies.

14. That further attempts are made to encourage a constructive national discussion about the ethical issues and funding dilemmas related to high-cost, highly-specialised medicines.

***Explanation:***

- For example, such dilemmas would include: How much are New Zealand residents willing to pay through the public health system for high-cost medicines to treat very serious illnesses that are life-threatening or serious in terms of health-related quality of life?
  - In other words, what other treatments (or other things) are New Zealanders willing to give up so that the country can afford to pay for such medicines?
  - Despite the National Advisory Committee on Core Health Services (established in 1992 and led by Sharon Crosbie), and the earlier Core Services work on prioritisation, the general public appears to have a relatively poor appreciation of the need for prioritisation decisions, and the effects of such decisions on what services are available through the public health system.
  - We believe that a community discussion, perhaps initiated and led by clinicians, would increase understanding of these complex dilemmas.
15. That the Ministry of Health further examines the incentives in place to encourage the optimal and ethical use of medicines in New Zealand.

***Explanation:***

- We have heard from many of the people with whom we consulted of significant wastage in prescribing, dispensing and patient use of medicines. Such waste should be reduced by better aligning the incentives for all involved to use medicines properly.
- Any such reductions in wastage will result in resource savings, which could be spent on high-cost, highly-specialised medicines (and perhaps other medicines and health technologies as well).

## **6. Conclusion**

This is our preliminary report from our review of access to high-cost, highly-specialised medicines in New Zealand. Our main conclusions and recommendations (i.e. from the previous section) are summarised in the Executive Summary at the beginning of the report.

As mentioned in the Introduction, this report is intended to promote discussion and elicit feedback. We would like to know what you think by the end of February 2010, including:

- What have we got right?
- What have we got wrong?
- What have we missed?
- How might our recommendations be implemented (if you agree with them)?

We will incorporate all feedback, where appropriate, into the final version of the report, for presentation to the Minister of Health by 31 March 2010.

Please send us your written comments, addressed to:

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Email: [hchsmedicines@moh.govt.nz](mailto:hchsmedicines@moh.govt.nz)  
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In addition, we intend to hold a ‘feedback forum’ on the morning of 17 February 2010 in Wellington, and we invite you to attend. Please contact Megan (as above) to indicate your interest.

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## **Appendix 1: Terms of Reference – Review of access to high cost, highly specialised medicines**

### **Preamble**

The National Party's medicines policy document (23 October 2008) identified access to highly specialised medicines as a priority:

*Access to high-cost highly specialised medicines in New Zealand is very limited compared to other countries. For example, in the six years to mid-2006, only 20 innovative new medicines were subsidised by New Zealand authorities, while in the same period 78 innovative new medicines were subsidised in Australia.*

*Highly specialised medicines often benefit comparatively few people. They provide individual patients with a great benefit that can be life changing, but the number of patients who benefit is not large. Nor is the cost low.*

*In order to access an unfunded, high-cost, or highly specialised community medicine, the only option for an individual who cannot pay for the medicine themselves is to apply to PHARMAC's Community Exceptional Circumstance Committee, one of three exceptional circumstance committees operated by PHARMAC. The budget for this committee is small and the eligibility criteria is so restrictive that doctors advise that they have given up making applications because they are so often turned down.*

*National believes access to such medicines needs to be improved. We will work with PHARMAC and stakeholders (including community groups) to investigate the best mechanism for this to occur.*

*National will work with stakeholders to investigate ways to improve access to high-cost highly specialised medicines.*

The Minister of Health is establishing a three-person review panel to progress this commitment.

### **Purpose**

The review panel will:

- review access to high cost, highly specialised medicines in New Zealand and review Exceptional Circumstances funds
- work with stakeholders to investigate ways to improve this access
- advise the Minister of Health on practical and affordable means to improve this access.

### **Scope**

In developing recommendations on high cost, highly specialised medicines, the panel should:

- consider how New Zealand's access to high cost, highly specialised medicines compares with other OECD countries on a population basis, and why any differences arise

- consider whether certain people or groups of people experience particular disadvantage in accessing high cost, highly specialised medicines
- consider the role and administration of PHARMAC's Exceptional Circumstances schemes
- have regard to:
  - health outcomes for those seeking access to high-cost highly specialised medicines
  - health outcomes for users of other health and disability services, and the effects of any decrease in services to allow increased access to high cost, highly specialised medicines
  - fiscal and operational implications of any proposals for PHARMAC, the health system and the Government's budget
  - incentive effects on individuals and pharmaceutical companies
  - relevant international developments
  - the existing *Medicines New Zealand* and work occurring under *Actioning Medicines New Zealand*
- engage widely with stakeholders, including by holding public meetings and through the use of electronic, teleconferencing and video-conferencing communication.

### **Deliverables**

The panel will provide final recommendations to the Minister of Health by 31 March 2010.

All requests for public comment on the panel's work should be referred to the Minister of Health. Panel members will not make any public comment unless authorised by the Minister of Health.

The Chair will provide regular reports on progress to the Minister of Health, as and when required by the Minister.

### **Membership**

The panel will be chaired by Dr Paul McCormack, and will include Joy Quigley and Associate Professor Paul Hansen.

### **Administrative and secretariat support**

The panel will receive administrative and secretariat support from the Ministry of Health.

**Appendix 2:** Individuals and organisations with whom the Review Panel consulted and/or from whom written submissions were received<sup>33</sup>

**Consulted with during the ‘induction’ phase of Review (in no particular order):**

<b>Name</b>	<b>Organisation</b>
Svend Petersen (Roche), Alan Carter (Sanofi-Aventis)	Researched Medicines Industry Association of New Zealand Inc (RMI)
Colin Tukuitonga	Ministry of Pacific Island affairs
Chai Chuah	Hutt Valley DHB
Teresa Wall	Māori Health Directorate, Ministry of Health
Robert Logan, Colette Burns	Hutt Valley DHB
Ruth Isaac, Andrew Davies	Health Section, The Treasury
Mathew Brougham, Peter Moodie, Rachel Grocott, Mathew Poynton, Peter Alsop, Sean Dougherty, Scott Metcalfe, Fiona Rutherford, Steffan Crausaz, Rico Schoeler,	PHARMAC
Katherine Silvester, George Slim	Ministry of Research, Science & Technology
MoH Cancer team	Ministry of Health
MoH Policy Unit	Ministry of Health
Julian Inch, Murray Georgel	DHB New Zealand

In addition, the Review Panel attended the “Forum on Access to High Cost Highly Specialised Medicines” (Wellington, 5 June 2009).

**Consulted with (including receiving presentations from) during the ‘invited submissions’ and ‘consultation’ phase of review:**

<b>Name</b>	<b>Organisation</b>
June Tordoff	School of Pharmacy, University of Otago
Andrea Grant	Roche Products (New Zealand) Ltd
Bill Denny	University of Auckland
Andrew Sutton, William Wong, Caroline De Luca, Brenda Hughes	Starship Children’s Hospital
Geoff McDonald, Katherine Lester, Frances Benge, Alan Carter, Pippa MacKay	Researched Medicines Industry Association of New Zealand Inc (RMI)

<sup>33</sup> As we mentioned in the Introduction, please accept our apologies if you communicated with us and you are not acknowledged here. Please contact us (again), so that we can remedy our mistake.

Brian Rousseau	CEO Otago & Southland District Health Boards
Sandra Kirby, Natalia Valentino	Arthritis New Zealand
John Forman	New Zealand Organisation for Rare Disorders
Richard Furneaux	Industrial Research Ltd (IRL)
Stewart Jessamine	Medsafe
Les Toop, Dee Mangin	Christchurch School of Medicine
Nicola Austin	Chief of Child Health, Canterbury DHB
Peter Chapman, John O'Donnell, Lisa Stamp	Department of Rheumatology/Immunology, Canterbury DHB
Tony Fraser, Sharyn Willis Dave Woods	bpac nz Ltd (Best Practice Advocacy Centre)
John Highton, Simon Stebbings, John Schollum, Rob Walker, Chris Jackson, David Reith, Annette Neylon, Michael Schultz, Jocelyn Livesey, Christopher Jackson	(Senior Medical Officers) Otago DHB
Most of the PHARMAC staff noted in the previous table, plus Jan Quin, Dilky Rasiah	PHARMAC

In addition, the Review Panel attended the PHARMAC Forum (Wellington, 9 October 2009).

**Written submissions received from:**

<b>Name</b>	<b>Organisation</b>
Andrew Harrison	New Zealand Rheumatology Association
Sarah Perry	Cancer Society
Rosemary Marks, Brenda Hughes	Starship Children's Hospital
John Highton	(Rheumatologist) Otago DHB
Callum Wilson	Starship Children's Hospital
J Alasdair Millar	(Consultant Physician) Southland DHB
Rosemary Marks	Paediatric Society of New Zealand
Daniel Ching	(Consultant Physician & Rheumatologist)
Sandra Kirby	Arthritis New Zealand
Richard Isaacs	Mid-Central Cancer Service, Palmerston North
David Pullar	Genzyme Australasia Pty Ltd
Pippa McKay	Researched Medicines Industry Association of New Zealand Inc (RMI)
Svend Petersen	Roche Products (New Zealand) Ltd

George Laking	Apparently on behalf of “a group of researchers and administrators from disciplines including Oncology, Rheumatology, Neurology, Respiratory Medicine, Clinical Pharmacy, and Clinical Trials.”
Peter Chapman, John O’Donnell, Lisa Stamp	(Rheumatologists) Canterbury DHB

### Appendix 3: Documents read by the Review Panel<sup>34</sup>

(In addition to the documents in the References.)

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