Report to Minister of Health, Hon Tony Ryall

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

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EXECUTIVE SUMMARY

Commissioned by you, the Minister of Health, this report presents the results of our review of access to high-cost, highly-specialised medicines in New Zealand – which we have interpreted to mean medicines that are high cost and/or highly specialised. You also asked us to work with stakeholders to investigate ways of increasing access, and to advise you on practical and affordable means of achieving this. For the past nine months, we have consulted with and received submissions from many interested parties from around New Zealand and also overseas.

Although New Zealand spends less on medicines per capita than other comparable countries, New Zealand pays lower prices for medicines, thereby achieving better value for money than most, if not all, other countries. This is largely a result of PHARMAC’s effectiveness at prioritising and procuring medicines. Nonetheless, we have been convinced during the course of our review that New Zealand has less access to new, high cost medicines than other countries, in particular Australia (an obvious comparator).

Somewhat surprisingly, there are also problems accessing some highly specialised but low cost medicines in New Zealand. We believe that, as addressed in our recommendations, increasing access to such medicines should be relatively easy.

With respect to high cost medicines, at an individual patient level, the impact of not having access to some medicines can be severe. Some patients (and their friends and families) pay a high personal price in terms of their poor health – in the extreme, resulting in death.

At an aggregate level, though, because New Zealand achieves relatively good value for money from the medicines it buys overall – and recognising that total health-care spending as a percentage of GDP is above the OECD average – we have seen no evidence that health outcomes are worse overall than for comparable countries. It is important to bear in mind that differences between countries in how much they spend on medicines reflect differences in their economic circumstances, their populations, and their priorities for their societies in general and their health systems in particular (including with respect to access to medicines).

In theory, if access to high cost medicines were to be increased then, logically, the funds to pay for more high cost medicines must come from at least one of these three main sources: (1) increased government spending, (2) increased patient co-payments on prescriptions overall, and (3) reduced wastage (i.e. better value for money) in the medicines system and/or health system overall.
We recommend that you pursue the third of these options. Many problems concerning access to high-cost, highly-specialised medicines were identified in the course of our review, along with significant problems with New Zealand’s medicines system overall. By improving the medicines system and also the health system overall, we expect that access to high cost medicines, as well as other medicines, will increase.

We are not necessarily recommending against the first two options above; ultimately, though, they depend on your and your Government’s priorities and assessments of the trade-offs associated with each of them. In any case, if more funds were to become available for spending on medicines, it is not obvious that they should necessarily be spent on high cost medicines per se. Depending on the amount of extra funding available, there are also other, lower cost medicines that are currently unfunded that, arguably, represent better value for money.

We offer 17 specific recommendations in Section 2 below (see pp. 15-30). One of our main recommendations is 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 (subject to our other recommendations aimed at improving how such decisions are made in the future). We do not recommend that new prioritisation processes and pools of funding be established for high-cost, highly-specialised medicines per se.

We would be very happy to discuss this report with you.
1. Introduction

In May 2009 you asked us to review access to high-cost, highly-specialised medicines in New Zealand, to work with stakeholders to investigate ways of increasing access, and to advise you on practical and affordable means of achieving this, as outlined in our Terms of Reference (Appendix 1). This is our report.

As you know, we also released a ‘preliminary report’ in December 2009, representing some of our thinking at that point and intended to promote discussion and elicit feedback. Subsequently we received many more submissions, complementing those we had received earlier.

We have also consulted with many individuals and organisations (Appendix 2) who kindly shared their expertise and opinions with us. Naturally, 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 (Appendix 3). Informed by these submissions, consultations and written material, this (final) report presents our findings, including our conclusions and recommendations.

Unfortunately and probably inevitably, some of the individuals and organisations with whom we consulted will be disappointed by this report. Access to high cost medicines in particular is a very sensitive and personally distressing issue for patients (and their friends and families) who do not have access to them. If such medicines are not provided to people who need them, and if other effective treatments are also unavailable, their health inevitably suffers. For some patients this can be very serious – in the extreme, resulting in death.

Many problems concerning access to high-cost, highly-specialised medicines – which we have interpreted to mean medicines that are high cost and/or highly specialised – were identified in the course of our review, along with significant problems with New Zealand’s medicines system overall. We believe that significant improvements should be made to how the medicines system operates, which we expect will increase access to high-cost, highly-specialised medicines.

This report proceeds as follows. In Section 2, our conclusions and recommendations, as well as other options we considered, are presented. In Section 3, as background to the report, we consider the meaning of ‘high-cost, highly-specialised medicines’ and briefly review how pharmaceuticals funding decisions are made in New Zealand. We also 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.
In Section 4, we consider how New Zealand’s access to high-cost, highly-specialised medicines compares with other OECD countries, particularly Australia, on a population basis, and why differences arise.

In Section 5, we catalogue problems – perceived and real – that have been communicated to us by people we have met or received submissions from concerning New Zealanders’ access to high-cost, highly-specialised medicines, including the extent to which certain people or groups of people are particularly disadvantaged.

We would like to thank everyone with whom we have consulted (Appendix 2). We are very impressed by the expertise and the enormous goodwill and dedication of these people – patients and their advocates, clinicians, managers, policy-makers, scientists, pharmaceutical industry representatives, etc. Thanks also 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.

Finally, we would like to acknowledge that none of us (the members of the Panel) are experts with respect to the operational details of New Zealand’s medicines system.\textsuperscript{1} Compared to the many highly skilled individuals who work in this complex system, we are ‘outsiders looking in’. Accordingly, the analysis and recommendations in this report are of a relatively general nature. Moreover, despite our best efforts, it is likely that we have missed some subtleties of the medicines systems that are important. Minister, should you choose to implement our recommendations, achieving the outcomes we envisage will ultimately hinge on the leadership and specialised knowledge of the people working within the system.

\textsuperscript{1} See the footnote on the title page summarising our respective backgrounds.
2. Conclusions and Recommendations

- What are high-cost, highly-specialised medicines?
- By definition, high cost medicines are expensive
- How do we compare internationally?
- Options available, in theory, to increase access to high cost medicines
- Increased government spending on high cost medicines?
- Increased patient co-payments on prescriptions overall?
- High-cost, highly-specialised medicines should be treated the same as other medicines
- Normative and subjective decisions are inevitable
- Other countries’ approaches to funding decisions for high cost medicines
- Better value for money
- Greater consistency across the health system overall
- Reduced complexity, increased transparency
- Specific recommendations
- Acknowledgement of the recommendations’ resource implications

What are high-cost, highly-specialised medicines?

For the sake of clarity, we begin by defining ‘high-cost, highly-specialised medicines’, which, in general terms, we have interpreted to mean medicines that are high cost and/or highly specialised. This definition permits two main groups of medicines to be recognised: (1) high cost medicines, highly specialised or not; and (2) highly specialised medicines that are low cost.

This second group comprises medicines for which there is ‘something special’ – i.e. ‘highly specialised’ in a broad sense of the term – in their manufacture, procurement, low frequency of prescription, or chemical stability, so that they are not easily sourced through the usual channels. Somewhat surprisingly, despite their relatively low cost, there are currently problems accessing some such medicines in New Zealand. We believe that, as addressed in our recommendations below (see Recommendation 5, pp. 20-21), increasing access to low cost medicines that are highly specialised should be relatively easy.

Hence, our main focus is on the first group of medicines distinguished above: high cost medicines, highly specialised or not. In this context, ‘highly specialised’ has two possible meanings. One interpretation is that they are medicines targeted at relatively few patients; that is, they are highly specialised with respect to whom they treat. This

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2 For a simple schematic representation, see Figure 1 in the next section.
3 Examples include indomethacin, amiloride, quinine, captopril and potassium chloride.
includes medicines administered through the Community Exceptional Circumstances scheme (currently for people with conditions affecting fewer than ten patients in New Zealand). Such medicines tend to be high cost as well. Another interpretation is that ‘highly specialised’ medicines are medicines that are manufactured in a technically sophisticated (‘highly specialised’) manner – e.g. ‘large molecule’ medicines – and so they also tend to be high cost.

**By definition, high cost medicines are expensive**

Fundamentally, there is less access to high cost medicines (highly specialised or not) than to other, lower cost medicines for the simple reason that high cost medicines are more expensive. High cost medicines have a high ‘opportunity’ cost in terms of the benefits that could be realised from spending the money on other, lower cost medicines.

How ‘high’ are high cost medicines? Although there are no precise definitions for New Zealand, their cost is likely to be in excess of $20,000 per patient per year, and sometimes considerably more. In the extreme, we were told during our consultations of biological medicines that are available costing $500,000 or more per patient per year, potentially to be taken for the patient’s whole life (e.g. 80 years).

In essence, when the decision to not fund a high cost medicine is reached that is because, in the opinion of PHARMAC⁴ (or a DHB,⁵ as for most hospital pharmaceuticals currently), the medicine does not represent good value for money. Benefits of greater value could be realised (usually at a health-system level) if other, cheaper medicines were bought instead. We will return to the issue of how such funding decisions are made later below.

**How do we compare internationally?**

Although it is difficult to precisely evaluate New Zealand’s access to high cost medicines (highly specialised or not) relative to other comparable countries, the following stylised facts seem incontrovertible to us.

New Zealand’s total health-care spending as a percentage of GDP is above the OECD average (OECD 2009). And yet New Zealand’s pharmaceutical spending per capita is one of the lowest in the OECD. This relatively low per capita expenditure on pharmaceuticals does not, however, translate to poor access to pharmaceuticals overall. New Zealand pays lower prices for medicines and so achieves better value for money.

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⁴ New Zealand’s Pharmaceutical Management Agency.

⁵ District Health Board, of which there are 21 nationwide.
than most, if not all, other countries. This is largely as a result of PHARMAC’s effectiveness at prioritising and procuring medicines.6

Nonetheless during the course of our review we have been convinced that New Zealand has less access to new, high cost medicines than other countries, in particular Australia (an obvious comparator). But the question needs to be asked: So what? Is this necessarily a ‘bad’ thing at an aggregate level?

Clearly, at an individual patient level, the impact of not having access to some high cost medicines can be severe. Some patients (and their friends and families) pay a high personal price in terms of their poor health – in the extreme, resulting in death.

However, at an aggregate level, because New Zealand achieves relatively good value for money from the medicines it buys overall, we have seen no evidence that health outcomes are worse overall than for comparable countries. For example, albeit it is a very crude indicator, life expectancy for people in New Zealand is about the same as in other developed countries, including Australia and the UK (OECD 2009), where access to new, high cost and highly specialised medicines is greater.

It is important to bear in mind that differences between countries in how much they spend on medicines reflect differences in their economic circumstances, their populations, and their priorities for their societies in general and their health systems in particular (including with respect to access to medicines). Different countries also have different approaches to deciding on such priorities and implementing them.

Arguably, relative to other countries, New Zealand has been more successful at making the difficult prioritisation decisions involved in assessing the opportunity costs inherent in each purchasing decision – so that, in our opinion, New Zealand achieves relatively more value per health dollar spent. Other countries have other arrangements, including greater political involvement in deciding which drugs to fund, greater use of insurance and private and charitable funding.

**Options available, in theory, to increase access to high cost medicines**

As we mentioned above, during the course of our review we have been convinced that New Zealand has less access to new, high cost medicines than other countries, in particular Australia. As well as to review access to high cost medicines, you asked us to advise you on practical and affordable means of increasing access to them.

Therein lies the main issue: as we discussed earlier, high cost medicines are expensive, and so any increase in access to them will inevitably require that more money is spent

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6 See Figures 2-4 in Section 4 for evidence to support these assertions.
on them. Logically, the funds to pay for more high cost medicines must come from at least one of these three main sources:

- Increased government spending.
- Increased patient co-payments on prescriptions overall (thereby ‘freeing up’ government money for high cost medicines).
- Reduced wastage (i.e. better value for money) in the medicines system and/or health system overall.

Each of these three sources of funding represents an option available, in theory, for you to increase access to high cost medicines. However, their overall desirability, respectively, hinges on the extent to which they are practical and affordable.

In short, whether or not you decide to increase government spending on high cost medicines (the first option above) depends on your and your Government’s priorities and assessments of the trade-offs associated with alternative uses of the available government funds.

Likewise, whether or not you decide to increase patient co-payments on prescriptions overall (the second option above) depends on your and your Government’s assessment of the option’s relative merits overall – i.e. not just with respect to the capacity to ‘free up’ government money for high cost medicines.

Whether you decide to pursue the first and second options or not, we believe the third option is highly desirable, and we recommend that you pursue it. As we discuss in this report, in the course of our review we identified significant problems with New Zealand’s medicines system. By improving the medicines system and also the health system overall, we expect that access to high cost medicines, as well as other medicines, will increase.

Before discussing this third option, we consider the first two options in more detail.

**Increased government spending on high cost medicines?**

In theory, if you were to decide to increase government spending on high cost medicines – and we note options for doing so below – what spending in other areas of Vote Health would you be willing to cut? Or if Vote Health were to be increased, which other Votes would your Government be willing to decrease? Or would you raise taxes? Clearly, such tradeoffs are problematic, particularly given fiscal deficits are forecast for the next five or more years and the New Zealand economy is growing slowly.

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7 Spending could also be switched away from other (lower-cost) medicines in favour of high cost ones.
As you know, 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 your Government’s priorities and assessments of the trade-offs associated with alternative uses of the available Budget funds – e.g. Vote Health versus Vote Education versus Social Welfare, etc.

If you were to decide to increase government spending on high cost medicines then, in theory, there are at least three options for distributing it:

- Allocate the (hypothetical) increased government spending specifically to the single Exceptional Circumstances scheme proposed in our recommendations and direct that it all be spent on high cost medicines (in effect, lower the access criteria for high cost medicines). Alternatively, PHARMAC could be directed to allocate more of its pharmaceuticals budget to the Exceptional Circumstances scheme.

- Allocate the (hypothetical) increased government spending to the Ministry of Health’s Special High Cost Treatment Pool and extend access to include high cost medicines. Although this Pool is not currently intended for high cost medicines, including them would seem to fit the scope of the fund by including “medical treatment that is only available outside New Zealand, or treatment that is only currently available outside the public health system” (see footnote 9).

- Use the (hypothetical) increased government spending to establish a special pool of new funding dedicated exclusively to high cost medicines.

Although we have identified these options here, we do not recommend any of them. As we discuss later in this section, one of our main recommendations overall is 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 (subject to our other recommendations aimed at improving how such decisions are made in the future).

Accordingly, if you were to decide to increase government spending on pharmaceuticals, we recommend that you do so by increasing the budget available for

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8 One of our recommendations (Recommendation 2) is that the three Exceptional Circumstances schemes currently in existence be replaced by a single Exceptional Circumstances scheme.

9 According to the Ministry of Health (2010):

The Special High Cost Treatment Pool is money set aside by the Ministry of Health for one-off treatments not otherwise funded by the public health system. District Health Board specialists apply to the Ministry of Health on a patient's behalf.

... Special high cost treatments includes medical treatment that is only available outside New Zealand, or treatment that is only currently available outside the public health system.
all pharmaceuticals competing for funding, as currently managed by PHARMAC – rather than creating (or increasing) separate pools of funds targeted at high cost medicines per se. In our opinion, it is important that all medicines spending proposals are assessed and compared against each other in a consistent fashion – in essence, to ensure they ‘stack up’ against each other with respect to their value for money.

It is possible that as a result of such comparisons it, if you were to decide to increase government spending on pharmaceuticals, you would not necessarily want to allocate the extra money to high cost medicines per se. Likewise, if more money were to be made available to DHBs for buying pharmaceuticals, they would not necessarily choose to allocate all of it to the Community Exceptional Circumstances scheme. In both cases, depending on how much extra funding were to be made available, there are also other, lower cost medicines that are currently unfunded that, arguably, represent better value for money.

Especially with respect to the first and second options above, it is also worthwhile noting that they represent, in essence, political ‘over-rides’ with respect to PHARMAC’s assessment, prioritisation and decision processes. If you are thinking of adopting such options, we recommend that you also consider the possible effects of reinforcing such precedents that already exist, and also about how, arguably, this could undermine how such assessment, prioritisation and decision processes work at a system level. In our opinion, it is more important to work towards improving PHARMAC’s processes (and those of other agencies too), and that is one of the main objectives of our Specific Recommendations later below.

In the context of the three options above, we also considered ‘risk sharing’ between PHARMAC and pharmaceutical companies – in essence, whereby PHARMAC agrees to fund a medicine for a maximum number of patients, and the company agrees to fund any additional patients (i.e. the company ‘shares’ the financial risk of the medicine being funded). Such arrangements, in various forms, have existed in New Zealand for years. They could be utilised in conjunction with any of the three options above; however, as always, it would be important to ensure that any such arrangements are fully understood and agreed by the parties involved (in particular, so that patients are not vulnerable to being unreasonably denied access to their medicines if circumstances change in the future).

**Increased patient co-payments on prescriptions overall?**

In theory, another possible way of raising funds in order to buy more high cost medicines would be to increase the amount that people pay as a contribution to their routine prescriptions (i.e. pharmacy co-payments). An increase in pharmacy co-payments could be levied across the whole population or targeted at people with higher incomes. It is not clear, though, how much government money this would ‘free up’ – further research (‘number crunching’) is required here.
We are, of course, aware that there are other important considerations associated with setting ‘optimal’ pharmacy co-payments. For example, higher charges at the ‘front door’ of the health system are likely to decrease access to medicines so that more patients end up in the hospital system instead of being treated in the community. On the other hand, for example, higher co-payments would increase incentives to reduce the wastage and misuse of prescriptions. And so on.

In any case, as mentioned in the previous section, if more funds were to become available for spending on medicines, it is not obvious that they should necessarily be spent on high cost medicines per se. Depending on how much extra funding is available, there are also other, lower cost medicines that are currently unfunded that, arguably, represent better value for money.

**High-cost, highly specialised medicines should be treated the same as other medicines**

As referred to above, one of our main recommendations below (Recommendation 1, pp. 15-16) is 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 (subject to our other recommendations aimed at improving how such decisions are made in the future). We do not recommend that new prioritisation processes and pools of funding be established for high-cost, highly-specialised medicines per se.

An implication of this recommendation is that if you were to decide to increase government spending and/or raise funds by increasing pharmacy co-payments you should allocate the additional funds to the budget for all medicines competing for funding – rather than targeting high-cost, highly-specialised medicines per se. Moreover, you should leave it to professional decision-makers (currently PHARMAC) to decide how to spend it.

As in most other developed countries, in New Zealand when medicines are being considered for funding their health benefits to patients are usually measured in terms of ‘QALYs (Quality-Adjusted Life Years) gained’. QALYs gained reflect predicted changes in patients’ life expectancies and/or health-related quality-of-life as a result of treatment. In addition to QALYs gained, other potentially important sources of value associated with ‘equity’ or ‘social justice’ gains from treating patients who are in relatively poor health are also considered (sometimes also referred to as ‘community values’).

Thus when PHARMAC makes funding decisions (including with respect to high-cost, highly-specialised medicines) it considers each medicine’s cost per QALY as well as,
broadly speaking, the above-mentioned equity or social justice gains (including considering “the particular health needs of Māori and Pacific peoples”).

When PHARMAC declines a medicine for funding, it does so because, in PHARMAC’s opinion, the medicine does not represent good value for money, in terms of its benefits relative to its costs, compared to other medicines that could be purchased with the available funds.

**Normative and subjective decisions are inevitable**

Inevitably, such evaluations – involving comparisons of costs per QALY and ‘equity’ or ‘social justice’ gains across different patients and patient groups – are normative and inherently subjective. They depend on decision-makers’ value judgements or ethical positions, which, in general terms, depend on their beliefs about social justice or equity.

The inevitability of normative and subjective evaluations is at the centre of the current debate about access to high-cost, highly-specialised medicines in New Zealand that we hope this report contributes to. Some people – especially those affected by illnesses treatable by such medicines – will think that such medicines should be funded because, according to *their* beliefs about social justice or equity, they represent good value for money. In contrast, other people will think that such medicines should not be funded because, according to *their* beliefs about social justice or equity, they represent poor value for money.

Such disagreements are entirely natural because there are no universally ‘right’ answers to the questions implied by such resource allocation problems. Everyone will have his or her own personal preferences, depending on his or her own value judgements or ethical positions – of which there is an infinite number of such value judgements or ethical positions theoretically possible.

Clearly, someone has to make these difficult decisions. The important thing is that such decisions are made in the ‘best’ way possible (itself, inevitably, a normative and subjective evaluation too). Every country grapples with this issue. In New Zealand, such decisions are made by PHARMAC, arguably a world leader in the area of pharmaceutical funding decision-making and procurement.

**Other countries’ approaches to funding decisions for high cost medicines**

It is clear from our consultations and reading the international literature, that due to scarce health-care resources and on-going technological advances in medicines – many

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10 More specifically, PHARMAC decides which medicines to fund according to nine decision criteria, as reproduced on p. 37 below.
of which are increasingly expensive – all health systems face the challenge of prioritising new medicines for funding.

For example, from a conversation with senior people involved in health technology assessments in Australia, we understand that in Australia, The Netherlands, the UK and Canada funding decisions for high cost medicines for very rare diseases are made outside of the main funding decision-making system for most medicines. In Australia, for example, each application is examined on a case-by-case basis and is subject to the approval of the Minister of Health. If the cost is over $10 million, for a group of patients, then the decision is taken to the Cabinet. This is not an approach that we recommend for New Zealand.

We also understand that in the countries mentioned above there are serious concerns about the sustainability of their decision-making processes for high cost medicines, so that these countries are considering changing their processes. Specifically, at least in Australia, there appears to be a growing movement towards bringing high cost medicines ‘back inside’ the main funding decision-making system (perhaps with necessary modifications). You might consider discussing this issue with your colleagues from these countries when you meet with them next.

**Better value for money**

Although we are not recommending that more money be allocated to high-cost, highly-specialised medicines per se, we are strongly recommending that you continue to pursue reduced wastage (i.e. better value for money) in New Zealand’s medicines system (and health system overall).

With approximately $900 million per annum from Vote Health currently being spent on pharmaceuticals, we think that getting better value for money from this spending should be your main focus. More generally, as we discuss below, getting better value for money from spending across the health system overall would be desirable.

When you are confident that this is being achieved, both across the medicines sector and the health system overall, then your Government might consider increasing spending on medicines – i.e. in addition to the 2009 Budget increase in spending by $180 million over three years.

As mentioned earlier, we recommend that if you were to decide to increase government spending on pharmaceuticals, you do so by increasing the budget available for all pharmaceuticals competing for funding, as currently managed by PHARMAC – rather

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11 In addition, we understand that approximately $5 million is spent on pharmaceuticals by the Accident Compensation Corporation, and other amounts by the Ministries of Economic Development and Social Development respectively.
than creating a separate pool of funds targeted at high-cost, highly-specialised medicines per se. All medicines spending proposals should be assessed and compared against each other in a consistent fashion – in essence, to ensure they ‘stack up’ against each other with respect to their value for money.

Accordingly, our recommendations reflect a broad medicines-system perspective, seeking greater efficiency and effectiveness in the use of current spending. By improving the medicines system overall, we expect that access to high-cost, highly-specialised medicines, as well as other medicines, will increase.

This over-arching philosophy of better value for money, and also many of our Specific Recommendations below, is consistent with *Actioning Medicines New Zealand 2009* (Associate Minister of Health, Minister of Health 2009).

This is also consistent with the recommendations of the *Report of the Ministerial Review Group* (Ministerial Review Group 2009); in particular, and as proposed by you and agreed to be the Cabinet (Ryall 2009), that the National Health Committee be reconfigured and strengthened to evaluate and prioritise new and a selection of existing health technologies and interventions.

**Greater consistency across the health system overall**

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 (as prescribed by family doctors, specialists and midwives) – 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 (mostly decided by individual DHBs).

**Reduced complexity, increased transparency**

Finally, broadly speaking, our recommendations are also intended to reduce complexity, increase transparency, and ultimately to reduce the associated frustration evident in the health system.

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12 *Actioning Medicines New Zealand* is the action plan for Medicines New Zealand, the medicines strategy for New Zealand supported by the Ministry of Health.
We would like to emphasise, however, that in seeking to increase transparency, we consider it imperative that PHARMAC is not impeded in performing its pharmaceutical purchasing activities, which often involve sensitive information (as most business negotiations do). In other words, increased transparency should be balanced against PHARMAC’s continuing ability to perform its purchasing activities.

In the remainder of this section we present our recommendations and then acknowledge, in very general terms, that their likely resource implications are likely to be significant.

**Specific recommendations**

Here are our specific recommendations, each accompanied by an explanation and additional details.

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 (subject to our other recommendations aimed at improving how such decisions are made in the future). To be clear, we do not recommend that new and separate prioritisation processes and pools of funding be established for high-cost, highly-specialised medicines.

*Explanation/details:*

- Implicit in this recommendation is the over-arching principle that the main rationale for funding medicines is to improve health outcomes rather than the particular characteristics of the medicines themselves being of fundamental importance. PHARMAC’s statutory objective in this respect – to obtain the best health outcomes for New Zealanders from the funds available – requires a focus on the relative value for money of all medicines available for this purpose.

- This implies, as we discussed earlier, that if you were to decide to increase government spending and/or raise funds by increasing pharmacy co-payments and/or release resources by reducing wastage that you should allocate the additional funds to the budget for all medicines competing for funding.

- Nonetheless, we considered proposals for new and separate prioritisation processes and pools of funding for high-cost, highly-specialised medicines, as suggested by some of the people with whom we have consulted and as used in some other countries, including Australia. We were not convinced that such proposals are superior to the status quo (with modifications, as in our other recommendations below).
• In our opinion, likely problems with such proposals include the difficulty of determining the appropriate size of the separate funding pool. Deciding how much money should be allocated or ‘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, in general).

• Other likely disadvantages include the likely increase in bureaucracy needed to assess high-cost, highly-specialised medicines separately, and also possible perverse incentives if funding could be more easily obtained through a separate funding process for such medicines.

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/details:

• We believe that the multiple budgets, processes and schemes are largely legacies of how PHARMAC and the DHBs evolved. Although they were an improvement on the systems then in place, we believe that New Zealand would be best served now by taking the best of these processes and schemes and simplifying and improving them further.

• Many people in the hospitals sector regarded the introduction of the Discretionary Community Supply (DCS) and Hospital Exceptional Circumstances (HEC) scheme as having encouraged greater national consistency by increasing information about what was available and what was not. The HEC scheme was established as an independent process for deciding whether, given a patient’s specific circumstances, it was cost-effective for the patient to be allowed access to a particular medicine. In contrast, the Community Exceptional Circumstances (CEC) scheme was set up for managing access to medicines for people suffering from very rare conditions (fewer than 10 patients nationally).

• Both processes are considered to be irritating by many hospital clinicians. The CEC form is very long and requires considerable clinician time to complete. The HEC form is shorter but still regarded as ‘overkill’ by doctors, who feel they are too busy and should not be doing excessive amounts of paperwork.

• When treating patients with severe and life-threatening illnesses in hospitals, clinicians require rapid access to the medicines that are available with clear guidance on prescribing. In our opinion, new pharmaceutical schedule criteria
and supporting processes will be required to direct access to where the evidence suggests the greatest value can be gained. We believe that this can be best achieved by creating a single source of that information. There will be a continuing tension, though, between enabling clinicians to as effective as possible given the available resources and managing medicine costs.

- As well as ‘positive’ lists for both the New Zealand Pharmaceutical Schedule and Exceptional Circumstances scheme, ‘negative’ lists, specifying medicines that are not and will not (currently) be funded should be considered as a means of increasing transparency and reducing duplicate applications for funding.

- Our proposed single Exceptional Circumstances scheme should systematise EC precedent decisions on to the proposed New Zealand Pharmaceutical Schedule as soon as it is clear what the place of a new medicine is in New Zealand. We have not attempted to define the criteria for the proposed new EC scheme, as there are many people in the health sector who are better qualified to determine this than us.

- In our opinion, an Exceptional Circumstances scheme for pharmaceuticals 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.

- In future, the New Zealand Pharmaceutical Schedule and Exceptional Circumstances scheme should be a more flexible, electronic system that enables new and existing medicines to be added, edited or removed regularly with changing indications and access criteria as knowledge, funding and availability changes. Email alerts to specialists would limit surprises when they got to prescribe a drug that is relatively rarely used.

- Improved management of hospital medicines should result in these benefits: improved national consistency in the availability of information and the use of new medicines; an improved interface between the funding of community and hospital medicines; and improved value for money from spending in hospitals (particularly in relation to patented medicines) which will free up funds for other health services (including, potentially, increasing access to pharmaceuticals).

3. **That PHARMAC be responsible for the clinical and economic assessments and funding decision-making of all the pharmaceuticals on the New Zealand Pharmaceutical Schedule and the Exceptional Circumstances scheme referred to above. This would mean that in addition to community pharmaceuticals,**
PHARMAC would also take responsibility for all hospital and cancer pharmaceuticals.

Explanation/details:

- PHARMAC has significant expertise in these areas. Arguably, it is a world leader.

- This recommendation will significantly change how medicines are made available within hospitals. When a clinician prescribes a high cost medicine from the proposed New Zealand Pharmaceutical Schedule the information should be available at that time so that the clinician knows the access criteria, the process for authority, and the likely outcome from that application.

- The manner in which this recommendation is implemented will be very important. The aim is not to establish new restrictions for hospital-based clinicians, but rather to increase efficiency in national assessing and purchasing and to facilitate clinical decisions so that clinicians are clear about the medicines available to them.

- We have noted in other parts of this report some of the negative outcomes arising from PHARMAC taking a single-supplier approach (and see Recommendation 7 below). We have heard clinicians’ concerns that having just one medicine available in each therapeutic sub-group causes problems for patients who cannot tolerate or do not respond to the medicine, with no alternatives available. It is possible however that some restrictions might be made more transparent than at present.

- Many hospital clinicians consider the change to Schedule H (the section in the pharmaceutical schedule that refers to hospital medicines) to have been successful. It is understandable that there is a desire to introduce a consistent and transparent approach to new, expensive pharmaceuticals and this was also debated at length by the Hospital Pharmaceutical Advisory Committee (HPAC).

- Clinicians have asked that if it is decided that PHARMAC will take over all pharmaceutical purchasing, then the current process of restricting within class should be either changed or not applied to the hospital sector. We defer to sector experts on this matter.

4. That DHBs continue to be responsible for managing their total spending on pharmaceuticals through the above-mentioned New Zealand Pharmaceutical Schedule and Exceptional Circumstances scheme.
Explanation/details:

- Notwithstanding our recommending a single New Zealand Pharmaceutical Schedule (Recommendation 2 above), we propose that DHBs continue to manage their own budgets, as they do currently.

- Our understanding is that within a notional national pharmaceuticals budget, individuals DHBs are responsible for both their community and hospital pharmaceuticals spending. (And yet PHARMAC – in effect, the 21 DHBs on aggregate – has always managed to live within the Community Pharmaceuticals Budget.) Our recommendation is that this aspect of the current system continues if a single New Zealand Pharmaceutical Schedule and Exceptional Circumstances scheme are introduced.

- This recommendation reflects our desire to maintain the current incentives for clinicians and managers in DHBs to manage their own budgets locally. When funding both pharmaceuticals and other health interventions, it is in a DHB’s interest to seek to maximise the value from that spending. If pharmaceutical funding were provided from a separate pool and it had no direct impact on the total amount of DHB funding, DHBs and their clinicians could have an incentive to cost-shift to the pharmaceutical budget where otherwise this would not make sense.

- Our reluctance to recommend change here is also in the interests of minimising the disruption that might otherwise occur from requiring DHBs to de-construct their costing models and information systems with, in our opinion, very few off-setting benefits.

- One possibility might be to utilise the Operating Policy Framework (OPF) to define access rules for DHBs – in other words, to oblige DHBs to comply with a schedule. This should reduce concerns about ‘post-code prescribing’ across New Zealand, thereby achieving greater national consistency, while still requiring DHBs to continue managing their own budgets. Our understanding of the OPF is that this requirement already exists in Section 4.15.3 (p. 47) of the current OPF that you signed in September 2009. Another possible mechanism is via the Service Coverage Framework.

- Due to small DHBs not having the necessary financial capacity to manage the financial risks associated with all or part of the proposed Exceptional Circumstances scheme, they may decide to develop pharmaceutical risk pooling with other small DHBs. Similarly, at a regional level, DHBs may join together to risk pool against the same risks.
5. That the Ministry of Health, Medsafe and PHARMAC are directed to ensure low cost and highly specialised medicines (or ‘low volume’ medicines, as described elsewhere in the Report) are more readily available in New Zealand than they are now. A comprehensive framework for ensuring this should be developed that addresses the following issues (and almost certainly others).

*Explanation/details:*

- The Medicines Act requires that an ‘entity’ acts as a sponsor and takes responsibility for each low cost and highly specialised medicine bought into New Zealand. This role involves tasks like identifying suppliers, negotiating supply contracts, applying for permission to market a medicine in New Zealand, assuming legal liability, distributing, and supporting the ‘informed’ use of these medicines by clinicians and consumers, etc.

- We understand that for some low cost and highly specialised medicines pharmaceutical companies consider the cost of these ‘sponsoring’ activities to be greater than the revenue to be earned from bringing some low cost and highly specialised medicine into New Zealand, so that it is not profitable to do so. Medsafe’s regulatory requirements and costs are a relatively small proportion of these total costs, and Medsafe currently has a number of schemes that reduce the regulatory burden on New Zealand-based companies wishing to supply medicines in New Zealand. And yet some low cost and highly specialised medicine are not available in New Zealand.

- We understand that something of a ‘Catch 22’ situation sometimes arises, whereby a pharmaceutical company is advised it cannot obtain funding until the product is approved by Medsafe, but is not prepared to apply to Medsafe until it is confident its product will be funded.

- Without compromising on the quality, safety or efficacy of medicines, Medsafe should further investigate what further steps it can take to ensure low cost and highly specialised medicines are available. This would require a lower regulatory burden on manufacturers than that required under S29 of the Medicines Act.

- PHARMAC could be encouraged when offering sole supply contracts for large volume medicines to include ‘companion’ low cost and highly specialised medicines in the contract. An alternative approach may be contracting for a group of named low cost and highly specialised medicines at a guaranteed price, regardless of the volume supplied. In effect, this would ensure a fixed income to a company prepared to supply a range of such medicines.
• As well, an entity to undertake the ‘product sponsor’ role discussed above could be established as a unit in PHARMAC, the National Health Board or a DHB; or an external body could be contracted to do this. An expert advisory panel, drawing on expertise within Medsafe and PHARMAC’s clinical subcommittees and with links to the prescribing community, should be established to advise on low cost and highly specialised medicine to be managed in this way. A national dispensing service for highly specialised medicines that are used infrequently but which have important chemical stability characteristics, etc. should also be considered.

• In our opinion, better communication and cooperation is needed across the health sector, including government agencies and other entities and prescribers, with respect to the management of low cost and highly specialised medicines. For example, when a medicine becomes unavailable because a supplier has discontinued it, this information needs to be more widely known than it is now. We encourage Medsafe to increase its efforts to increase the information about discontinued products that it is supplied with.

• Overall, Medsafe’s ability to respond to these and other issues is limited by the existing Medicines Act legislation. For example, there are currently no legislative requirements for a sponsor of a medicine to inform Medsafe that it is withdrawing from the market. Without this information it is difficult for Medsafe to advise on the use of alternative medicines. This also prevents Medsafe from approaching other manufacturers of the medicine to encourage them to apply to market their brand in New Zealand. As part of the Actioning Medicines New Zealand work programme, the Medicines Act should be further improved in these respects.

6. That New Zealand-based public good research be applied to identifying targetable individuals with specific characteristics who are expected to receive the benefits from high cost medicines.

Explanation/details:

• It is increasingly clear that high cost medicines do not deliver equal benefit across treated populations. Specific individuals receive substantial benefits compared to the treated group. Arguably, the incentive for pharmaceutical companies is to demonstrate value across large population groups – and it is not in their best interests to demonstrate efficacy in small sub-populations.

• It has been proposed to us that access to high cost medicines can be improved within a framework of ‘public good’ research applied to identifying targetable individuals with specific characteristics who are expected to receive the benefits
from such medicines. The tools used would include investigator-led clinical research and health informatics integrative databases. Such research could be in collaboration with international researchers and organisations.

- Expansion of access to newer, higher cost medicines would offer an important opportunity to conduct public good research. Although it would require a trade-off between the purchase of medicines and the purchase of research and database infrastructure, in the longer term research may be the better investment. The costs will be recouped in later years when treatment can be targeted towards appropriate population sub-groups, doses and durations are optimised, and relevant alternatives are established.

- Public good research into high cost medicines could be funded via one of more of these institutions:
  - The Health Research Council – via its existing research peer-review and funding mechanisms.
  - A newly-established agency to manage such R&D – funded by DHBs’ pharmaceutical purchasing allocations.
  - PHARMAC – as part of its funding process for high cost medicines, whereby, in the first instance, access and the selection of patients would be ‘optimised’ with respect to the research (pending the results of the research). Due to greater care and attention associated with research protocols, participation in such research should be intrinsically advantageous to patients.

7. That, where appropriate, PHARMAC be directed to fund second-line and perhaps third-line medicines within a therapeutic sub-group – so that most patients who do not benefit from the first-line medicine have an alternative treatment available. This recommendation is contingent on the treatments offered being strictly sequenced – i.e. the second-line medicine is only available if the first-line treatment fails, and the third-line treatment is only available if the second-line treatment fails.

Explanation/details:

- This sequencing restriction – which should be strictly adhered to – is intended to enable PHARMAC to continue to be able to negotiate favourable prices on the first-line treatment which should still be guaranteed the largest market share, albeit it will probably be reduced.
• The aim of this recommendation is to increase the range of medicines available for particular conditions, while preserving as much as possible PHARMAC’s power to negotiate lower prices than if a full range of medicines were available.

8. That a New Zealand pharmaceutical formulary be established to support clinical decisions regarding the optimal use of medicines.

Explanation/details:

• Simply put, although the proposed New Zealand Pharmaceutical Schedule describes the what and when of medicines available to clinicians, a formulary might describe how medicines might be best prescribed in New Zealand.

• 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.

• To be clear, the intention underlying this recommendation is not to restrict drug availability or choice, or to use a formulary for cost containment, but rather to make transparent the agreed guidelines for using medicines that are available in New Zealand. Instead of being prescriptive, the formulary should be supportive of clinical decision-making given the resources available.

• We understand that a national formulary project is currently underway within the Ministry of Health as part of the Actioning Medicines New Zealand work programme. This is being co-ordinated through the Safe Medication Management Programme as the New Zealand Universal List of Medicines. We understand that the prime purpose for the national formulary work underway is as a platform for electronic messaging and decision support. It seems that other key implementation aspects are integration with the Pharmaceutical Schedule and functionality reinforcing key messages from optimal medicine-use campaigns. In our opinion, this work is important.

• We acknowledge that there will be significant costs associated with establishing a formulary. Naturally, these should be carefully considered before accepting or rejecting our recommendation.

9. That consideration be given to extending the budgeting period for funding medicines that PHARMAC must live within from one year, as it is now, to two years – i.e. so that PHARMAC is required to meet its budget over two years rather than one.
**Explanation/details:**

- The objective here is to reduce the criticality of the one-year budgeting period, so that PHARMAC is less risk-averse with respect to living within its annual budget. Nonetheless, PHARMAC must still operate within its budget constraint, but this is measured over two years rather than one.

- The obvious practical implication of this recommendation is that PHARMAC can run a (hopefully small) budget deficit in the first year, but this must be offset by an equal surplus in the second year.

10. That, if directed to implement the single New Zealand Pharmaceutical Schedule and the Exceptional Circumstances scheme that we recommend, PHARMAC should establish new processes, and refine existing ones, to ensure effective and timely funding decision-making. We expect that such decisions should not be made unreasonably slower than current funding decisions for hospital medicines; and on many occasions, those decisions might be made more quickly than at present.

**Explanation/details:**

- 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 new obstacles to clinical practice ‘at the edges’.

- It is important to recognise that there is an inherent tension between clinicians’ need for timely access to medicines and the time it takes to PHARMAC to properly assess them.

- We defer to experts inside and outside PHARMAC with respect to the details of such recommended new processes and refinements of existing ones.

11. That PHARMAC improves its PTAC and specialist advice processes, including greater involvement from medical specialists and where appropriate GPs, pharmacists, pharmacological practitioners and other interested clinicians. In addition, processes should be strengthened for patients and their advocates to offer advice to PTAC; however, they should not to be involved in making funding decisions. Consideration should also be
given to allowing pharmaceutical companies the opportunity of presenting to PTAC.

Explanation/details:

- We understand that PHARMAC currently receives clinical advice from PTAC, 16 clinical advisory sub-committees, the Hospital Pharmaceutical Advisory Committee (HPAC), the Exceptional Circumstances Panel, and from the members of six specific medicine decision-making panels. This involves over 100 health professionals (some on multiple panels) from over 50 specialties and sub-specialities. In addition, there are seven Medical Colleges and 125 clinicians currently registered on PHARMAC’s consultation databases.

- Furthermore, we understand that PTAC seeks independent advice on clinical issues outside the collective knowledge base when required. This is essential given the complexity and rarity of some of the disorders for which high cost medicines are requested. Experts in the field bring up-to-date knowledge from personal clinical practice or from communication with international peers – invaluable when diseases are rare and drug experience limited and not all PTAC committees will have members with such backgrounds.

- Despite these impressive consultation statistics, we believe that PHARMAC could improve its connection with clinicians by providing more information about PHARMAC and PTAC decisions.

- We believe that clinicians should be invited to present to PTAC. Although we recognise that critical generalist analytical skills are essential in this kind of work, greater involvement by medical specialists and where appropriate GPs, pharmacists, pharmacological practitioners and other interested clinicians could help allay some concerns that they and, via them, their patients are not adequately represented and consulted.

- We think that PTAC should continue to consider value for money (i.e. cost-effectiveness) when making funding recommendations. Efficient resource allocation is an important ethical consideration for all clinical decision-makers and essential for a population health perspective in relation to PTAC’s role.

- We acknowledge that the potential disadvantage of greater involvement by participants outside current PTAC processes is likely to be slower decision-making overall and higher costs. Therefore, any modifications to PTAC need to reflect a balance between these time delays and other costs and having appropriate clinical input into decisions so that well-informed decisions can be made (and seen to be made).
• In addition, processes should be strengthened for patients and their representatives and advocates to offer advice to PTAC; however, they should not to be involved in making funding decisions. We note that the draft Terms of Reference for PHARMAC’s Consumer Advisory Committee (CAC) proposes more interaction between CAC meetings and PHARMAC staff and Board meetings as observers and advisers (PHARMAC 2009a, 2010).

• In our opinion, patients and their representatives and advocates have the legitimate role of ensuring that the medicines system takes into account the human impact of the assessment and prioritisation decisions in their processes. However, their position with respect to a particular illness is hard to reconcile with the prioritisation processes involved in assessing a medicine for its value for money relative to other medicines (and treatments more generally) competing for funding.

• Hence, although we are supportive of ‘the public’ or ‘the community’ in a generic sense being part of these processes to add ‘the reasonable person’ test and in the interests of transparency and public participation, we are not persuaded that patients and their representatives and advocates should be involved in making funding decisions.

• 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 staff, 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 – e.g. issues of confidentiality, time constraints, etc.

12. That PHARMAC be further encouraged to build additional ‘sensitivity’ into its processes to increase the efficiency of medicine funding applications, as illustrated below.

Explanation/details:

• For example, there is a single metabolic genetic specialist in New Zealand who knows each individual with specific rare disorders. In our opinion, it does not make sense that he is required to make annual applications for medicines, including a declaration that each patient still has the same genetic disorder.

• Likewise, it would make more sense to require that each application for a high cost medicine for this group of rare disorders should either be made or supported
by the single specialist expert. Such a specialist could also be responsible for authorising these medicines.

13. 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/details:**

- This recommendation follows-on from Recommendation 10 – our goal overall is to reduce complexity, increase transparency, and reduce frustration.

- We understand that commercial drivers reasonably cause PHARMAC to hesitate to disclose the state of any particular medicine funding application. Nevertheless, we believe that after any initial medicine assessment, PHARMAC should declare the status of the application – e.g. “accepted for funding”, “still being assessed”, “not yet funded” due to evolving medical evidence, indications, cost or insufficient funding available to PHARMAC, etc.

- As we emphasised earlier, though, in seeking to increase transparency, we consider it imperative that PHARMAC is not impeded in performing its pharmaceutical purchasing activities, which often involve sensitive information (as most business negotiations do). In other words, increased transparency should be subject to PHARMAC’s continuing ability to perform its purchasing activities being protected.

14. 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. This should result in increased transparency and instil a broader sense of involvement and ‘ownership’ of decisions.

**Explanation/details:**

- This 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 ‘embedded’ at the local level.

- We believe that PHARMAC should further develop a national network of clinicians (i.e. medical, pharmacy, pharmacological practitioners and others) and DHB funding managers (‘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.

15. That PHARMAC and other agencies also performing clinical and economic assessments, funding decision-making and procurement with respect to other health technologies (e.g. devices, vaccines and medical and surgical procedures and equipment) work together. To maximise value for money, these processes, and ultimately the decisions reached, should be benchmarked across the agencies involved to ensure consistency across the groups of technologies assessed and across the health system overall. Across the health system, greater effort should be made to achieve consistency in funding decision-making processes and ultimately the value for money of spending on all types of health technologies.

Explanation/details:

- Ideally, we believe that there is value in all health technology assessments and prioritisation decisions being made by a single agency, preferably based on PHARMAC. PHARMAC has significant expertise at assessing treatments (currently medicines) in both clinical and economic terms. This would ensure maximum consistency across the types of technology assessed and reduce duplication.

- For example, the agencies referred to in this recommendation include the Accident Compensation Corporation and the Ministries of Economic Development and Social Development respectively (though, strictly speaking, arguably they are outside the ‘health system’). As noted in footnote 11 above, these government agencies also buy pharmaceuticals, and presumably other types of health technologies too.
• However, we are aware that the Ministerial Review Group (MRG 2009) recommended – and you and the Cabinet agreed (Ryall 2009) – that the National Health Committee be reconfigured and strengthened to evaluate and prioritise new and a selection of existing health technologies and interventions. The MRG identified prioritisation as a way to manage costs and improve the safety and effectiveness of health services, as has been achieved by PHARMAC with respect to the Community Pharmaceutical Schedule.

• We also understand that there are various processes involving consultation and the development of advice currently underway. We note that you recently initiated a consultation process concerning clinicians’ views about PHARMAC’s role being expanded to include hospital pharmaceuticals and devices (Ryall 2010).

• Where different evaluation and prioritisation processes are used by different agencies in the health sector, their rationale should be strong and explicit so as not to undermine confidence in the validity of these processes. Where possible, spending proposals assessed by different agencies should be compared against each other – in essence, to ensure they ‘stack up’ against each other with respect to their value for money.

• The capacity for rigorous health technology assessments is limited within New Zealand. In our opinion, PHARMAC could offer support to the National Health Committee in a relatively cost-effective way.

• In our opinion, medicines are currently subjected to much greater analytical scrutiny in assessing their ‘value for money’ – especially medicines on the Community Pharmaceutical Schedule (as prescribed by family doctors, specialists and midwives) – 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 (mostly decided 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, as exemplified by PHARMAC for example, to all health technologies.

16. That further attempts are made to encourage a constructive national discussion about the ethical issues and funding dilemmas within the health system.
Explanation/details:

- 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, and the effects of such decisions on what services are available through the public health system.

- Many New Zealanders are intellectually willing to accept the need for prioritisation at an aggregate (health system) level. Understandably, when the need for treatment becomes very personal strong emotions are often involved.

- We believe that a community-based discussion, perhaps initiated and led by clinicians (or other groups), would increase the understanding of these ethical issues and funding dilemmas within the health system.

17. That the Ministry of Health and PHARMAC continue to examine the incentives and institutional arrangements in place to encourage and achieve the optimal and ethical use of medicines in New Zealand.

Explanation/details:

- During the course of our consultations we heard from many people about significant wastage in the prescribing, dispensing and use of medicines. Such waste should be reduced by better aligning the incentives for all involved to use medicines properly. This will result in resource savings, which could be spent on more medicines overall, including high-cost, highly-specialised medicines.

- We note that prescribing for the elderly and people in the terminal stage of their life presents particular challenges with respect to supplying optimal amounts of the most appropriate medicines and other forms of treatment.

- PHARMAC has a statutory role in optimising the prescribing of medicines in New Zealand. PHARMAC’s Access and Optimal Use team develops and implements campaigns aimed at increasing access to funded medicines where beneficial, and decreasing the inappropriate use of medicines.

- The Actioning Medicines New Zealand work programme directed at the optimal use of medicines should be continued in these respects. Amongst other things, better using the clinical skills of pharmacists to ensure appropriate, cost-effective prescribing and that patients take their medicines properly is an area that is still under-utilised, in our opinion.
Acknowledgement of the recommendations’ resource implications

Should the above recommendations be implemented, there will be significant financial, personnel, time and ‘focus’ costs. We are unable to estimate the likely magnitude of these costs, other than to predict they will be significant. Not all of the recommendations will be able to be implemented simultaneously, so prioritising them will be necessary.

One concern we have is that if additional participants are included in decision-making processes – as we have recommended – then decision-making may take longer. Serious efforts should be made to mitigate this as much as possible.
3. Background

- What are ‘high cost’ medicines?
- What are ‘highly specialised’ medicines?
- How are pharmaceuticals 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, for the purpose of ‘framing’ the rest 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 pharmaceuticals 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 vs Degree of Specialisation](image)

**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, vaccines and medical and surgical procedures and equipment – with respect to their cost-effectiveness\(^\text{13}\) 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).\(^\text{14}\) 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.\(^\text{15}\)

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 that are available costing in the order of $500,000 or more per patient per year (i.e. more than that amount in cost per QALY terms), potentially 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

\(^\text{13}\) 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.

\(^\text{14}\) 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).

\(^\text{15}\) Note, though, that PHARMAC does not to have an ‘official’ cost-per-QALY threshold – as, amongst other reasons, this would constrain PHARMAC in its price negotiations with pharmaceutical companies.
patients treated. 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 with the condition to be treated with the medicine nationwide, PHARMAC 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 whom 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 potassium chloride.

Also some highly specialised and low cost 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 volume (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.

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16 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.

17 New Zealand’s Pharmaceutical Management Agency.
Not surprisingly, as discussed in Section 5, 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.

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, clinicians at Starship Children’s Hospital experience difficulties obtaining medicines appropriately formulated for children. And so this group of medicines is also discussed in the report.

**How are pharmaceuticals 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 2009b).

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 health services and other types of ‘health technologies’ such devices, vaccines and 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 Schedule18 (and associated Community Pharmaceutical Budget) and the Exceptional Circumstances schemes.19 In addition, DHBs decide which ‘Hospital’ and ‘Cancer’ medicines (typically, high cost medicines too) to fund, and how much to spend, within their hospitals.20

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

18 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).

19 There are three Exceptional Circumstances (EC) schemes: Community EC, Hospital EC and Cancer EC. These are discussed later.

20 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 overall), 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. In our opinion, although this is worthwhile, it is also an inefficient and probably inadequate process for managing the more than $200 million of Vote Health resources involved nationwide.
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 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 not having access to 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 pharmaceuticals 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?²¹

As discussed earlier, when different medicines – as well as other health ‘technologies’ (i.e. devices, vaccines and 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.

²¹ This sub-section is based on ideas discussed in Hansen (2006), which includes diagrammatic illustrations of some of the ideas discussed here (and others).
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’){22} and Rawlsianism{23} – with respect to their implications for deciding which medicines to fund.

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. 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

{22} 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”).

{23} 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 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.
reproduced earlier, 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).24 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?”
- “Should identifiable patients be favoured over non-identifiable patients?25 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?”26
- “Should higher priority be given if there is no alternative treatment?”
- “How is ‘double jeopardy’ to be dealt with?”27

24 This list of possible value judgements is not exhaustive; for a more comprehensive survey, see Schwappach (2002) for example.

25 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.

26 This is qualitatively the same as the Rawlsian ethical position discussed earlier.

27 ‘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.
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). For example, how should ‘end of life’ treatments’ be dealt with?
4. International Comparisons

- Positive versus normative analysis
- Positive-analysis observations
- Normative-analysis observations
- Pharmaceutical funding decision-making in Australia and the UK
- What might be in the pharmaceuticals ‘pipeline’?

In this section, we compare access to high cost medicines 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, 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 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 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 particular 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 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 medicines than Australia.

This 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 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 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 they could 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: PHARMAC’s impact on drug expenditure over time (reproduced from Grocott 2009)](image-url)
The dramatic outcome illustrated in Figure 2 is summarised by PHARMAC (2009e) 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 pharmaceuticals prices from pharmaceutical companies. PHARMAC has successfully exploited its monopsony\textsuperscript{28}-pricing power (arising from it being New Zealand’s main buyer of pharmaceuticals) by pursuing a range of aggressive pricing strategies\textsuperscript{29} 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,\textsuperscript{30} 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 5c 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.

\textsuperscript{28} 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.]

\textsuperscript{29} Including tendering, sole supply and reference pricing. See PHARMAC (2009f) for details.

\textsuperscript{30} 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 aggressive 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 sub-group, 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% in New Zealand who do not there is no second-line alternative.

In our opinion, such restricted choice 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.

Nevertheless, if we examine access in a broader sense, the base pharmaceutical co-payment in New Zealand of NZ$3 is very low compared to the equivalent co-payment in Australia of A$29.40 for unsubsidised people. ‘Access’, therefore, has a number of defining characteristics.

31 A therapeutic sub-group is a group of medicines that have the same or similar therapeutic effects.
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 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 in 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 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 medicines? As referred 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 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 medicines.

Moreover, the New Zealand 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 medicines will be more difficult in the future than it is now.

Second, 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, vaccines and medical and surgical procedures and equipment).\(^{32}\)

Figure 4 reports the proportions of GDP that New Zealand and Australia, as well as other OECD countries, devote to health care, publicly and privately funded. As can be seen, as a percentage of GDP, New Zealand’s total health-care spending, as well as publicly funded health-care spending, is greater than Australia’s and the OECD average.

![Figure 4: Expenditure as a percentage of GDP on health, publicly and privately funded, by OECD countries, including New Zealand and Australia (reproduced from OECD 2009)](chart)

1. Public and private expenditures are current expenditures (excluding investments).
2. Current health expenditure...
3. Health expenditure is for the insured population rather than resident population.


There are no ‘magic’ optimal proportions for how much of GDP should be devoted to producing health care and disability support services or spent on medicines, etc. This is

\(^{32}\) In the previous section, we briefly reviewed how pharmaceuticals funding decisions are made in New Zealand, including deciding how many resources to allocate to health-care spending in general and pharmaceuticals in particular.
partly for the reasons discussed in the previous sub-section associated with the different prices countries pay for their pharmaceuticals, 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 or medicines. And who is to say that is necessarily right or wrong? 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 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\footnote{For a recent survey of another 11 countries, see Nikolentzos, Nolte & Mayes (2008).} – have significant differences in these respects.

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

The Australian publicly-funded medicines system is known as the Pharmaceutical Benefits Scheme (PBS).\footnote{Most of this material is based on the ‘country report’ for Australia contained in Nikolentzos, Nolte & Mayes (2008).} The PBS is overseen by the Pharmaceutical Benefits Advisory Committee (PBAC), an independent statutory committee appointed by the Minister of Health. Mostly the PBAC does not set its own work programme, but instead reviews applications (usually submitted by manufacturers) for the listing of new medicines or of additional uses of already-listed ones. Evidence submitted in applications is evaluated by the Department of Health, with assistance from contracted academic individuals and groups (e.g. health economists, etc).

Based on an evaluation, 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, in general, 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).

In marked contrast to New Zealand, 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.

From conversations with senior people involved in health technology assessments in Australia, we understand that Australia also operates a ‘Life-Saving Drugs’ programme, which sits outside the PBS. ‘Life-Saving Drugs’ are medicines that have been rejected by the PBAC for not being cost effective, but for which there is evidence that they will extend life (albeit at relatively high cost). Applications to fund such medicines (usually one or two per year) go to the Minister of Health to make the decision. If the cost is over $10 million, for a group of patients, then the decision is taken to the Cabinet.

The proximate counterpart of New Zealand’s PHARMAC and Australia’s PBAC in the UK is the National Institute for Health and Clinical Excellence (NICE). In brief, amongst other activities, NICE makes recommendations concerning the use of new and existing health technologies (including medicines) within the National Health Service (NHS) (NICE 2010).

Based on these recommendations individual Primary Care Trusts (PCTs) make purchasing decisions. PCTs are legally bound to fund all medicines and other health technologies recommended by NICE. According to NICE (2010):

NICE is asked to look at particular drugs and devices when there is confusion or uncertainty over the value of a drug or device or when prescribing practices vary across the country - so that patients may be receiving different prescribed treatments, depending on where they happen to live, rather than on the state of their health.

While a drug or device is being appraised by NICE, NHS organisations should make decisions on its use locally, using their usual arrangements. A recommendation by NICE ends any uncertainty and inequality about prescribing. Once national guidance has been issued by NICE, it replaces local recommendations and promotes equal access for patients across the country.

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35 Reproduced from NICE’s webpage (retrieved 30 March 2010): www.nice.org.uk/aboutnice/whatarewedo/niceandthenhs/nice_and_the_nhs.jsp
In contrast to Australia’s and the UK’s 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 might be 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 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 Section 3).

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.

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, and that there will continue to be an increasing medicalisation of previously accepted ‘normal’ conditions. We were also told that older, and cheaper, medicines and treatments should not be dismissed in favour of the new as many still ‘do the job’ and can cause fewer side effects.

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. Most new medicines are likely to be more expensive than the ones currently available. We were also told that, at this stage in the development of these new medicines, it is much more difficult to produce corresponding generic (and therefore likely cheaper) forms once they come off patent.
5. Problems Identified during our Consultations

- Total pharmaceuticals spending
- Budget-setting for pharmaceuticals and across the health system overall
- PHARMAC
- Pharmaceuticals & Therapies Advisory Committee (PTAC)
- Exceptional Circumstances Schemes
- Formulary
- Patients and health-service users
- Paediatric medicines
- Patient advocacy groups
- Medsafe
- DHBs
- Prescribers
- Pharmaceuticals Industry

In this section, we report on all the problems raised by the individuals and organisations (Appendix 2) who kindly shared their expertise and opinions 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. 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 or so headings; and, where appropriate, we have added our own comments.

Total pharmaceuticals spending

Problems reported to us:

- 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).

36 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).
  
  o More specifically, low growth in the Community Pharmaceutical Budget has been used to enable relatively high growth in expenditure on surgical diagnostics and procedures.
  
  o 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).
  
  o This has had a noticeable impact in 2009. As a result of this additional expenditure and continuing PHARMAC activity, eight new medicines have been funded and access widened for a further 55 medicines (PHARMAC 2009g).
  
  o 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. We considered the option of removing some safe and low cost medicines and devices (e.g. common painkillers, condoms, etc) from the Community Pharmaceutical Schedule and have patients pay for them privately. However, we received advice that this would release relatively small amounts of funding and may lead to both adverse health effects and higher costs overall as a result of prescribers switching to higher costs medicines that are subsidised. On the other hand, some patients may experience personal cost savings by purchasing OTC medicines and devices instead of paying the cost of visiting a doctor and making the co-payment.

  o Another option we considered was for the patient pharmacy co-payment to be increased for some patients, thereby generating additional income from the approximately 60 million prescriptions annually in New Zealand. Again we received advice that the impact of doing so would release relatively small amounts of funding and again may lead to adverse health effects.

**Budget-setting for pharmaceuticals and across the health system 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.

• In practice, DHBs that are in financial deficit argue (have veto power?) to limit increases in the size of the Community Pharmaceutical Budget (which all DHBs contribute to pro rata).

• 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.

• Political decisions to implement programmes and treatment options result in confusion and sometimes frustration for clinicians and advocacy groups as one type of illness appears to be favoured over others.

*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 there is relatively poor information available about the costs and benefits of spending on other health technologies relative to pharmaceuticals.

We note 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.

PHARMAC

As well as the ‘issues’ noted below,37 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.38

- Dealing with PHARMAC is in an environment of “low trust & high bureaucracy”, which is bad for the 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) are 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.

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37 Recall, the purpose of this section is not to present a review of PHARMAC per se.

38 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 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).

Clinicians used words like “hassle”, “frustration” and “time wasting” to describe their access to PHARMAC decisions. One said (and many 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).

PHARMAC should specify a threshold above which medicines will be funded and below which they will not.

PHARMAC’s consultation periods are too short, especially when they occur during holidays.

The following allegations questioning PHARMAC’s integrity were raised by some of the people with whom we have consulted.\textsuperscript{39}

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.

\textsuperscript{39} See footnote 38 again.
• There was concern that PHARMAC’s effectiveness would be diluted if its role was expanded to cover all Health Technologies Assessment, as described in a recommendation in our preliminary report.

• PHARMAC’s relationship with PTAC is inappropriate, as discussed in the following sub-section.

Our comments:

• Overall, in our dealings with PHARMAC we have been impressed by the high calibre of the 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.

• 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.
  o We acknowledge, however, that PHARMAC’s negotiating positions and commercial sensitivities need to be protected.

• We are not convinced that there should be a cost-effectiveness threshold above or below which medicines will or will not be funded. As well as being impractical because of the other relevant considerations (see PHARMAC’s nine decision criteria reproduced on p. 37 above), we believe this would constrain PHARMAC in its the negotiations with pharmaceutical companies.

Pharmaceuticals & Therapies Advisory Committee (PTAC)

Problems reported to us:

• There are concerns that as PTAC is dependent on PHARMAC for administrative support, this means PHARMAC does not receive independent advice.
  o Information is filtered by PHARMAC staff, who have their own ‘world views’.
  o 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.

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40 See footnote 38 again!
There should 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.

- Advocates do not believe that their views are sufficiently represented.

- Pharmaceutical companies would like to have more input into the PTAC process.

- 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: mycophenalate 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.

**Our comments:**

- The Review Panel chair, Paul McCormack, attended 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 competent, thoughtful, well prepared and independent.
  - More specifically, they were focused for the most part on the evidence 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.
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.
  - There is too much paperwork 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.
  - A much broader range of medicines are available in 2010 than in 2002, and that the criteria for the EC scheme need to be revised.

- 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 $205 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 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.

- The EC process is not seen to be effective when PHARMAC approves a new treatment but an individual DHB will not fund it

**Our comments:**

- We understand that the existing EC processes have different criteria and reasons for being in existence. Hospital Exceptional Circumstances (HEC) was set up to cater for situations where patients could not be accommodated under the Discretionary Community Supply (DCS) arrangement. Discretionary Community Supply was to enable hospitals to provide medicines listed in Part IV of Section H of the Pharmaceutical Schedule to patients for use in the community

- This arose from work by the Hospital Pharmaceutical Advisory Committee (HPAC) aimed at resolving historical problems with access and equity. Prior to DCS and HEC being established (i.e. pre-2001), there had been no consistent approach to whether a patient would be supplied with non-funded medicines as part of a hospital visit and then allowed a supply to go home with. Often the cost of the medicine if supplied in the community was all that prevented the patient from going home. It was seen as more cost-effective to send people home with supplies of the ‘unfunded’ medicine in order to minimise the length of stay. We support that outcome and would expect that to continue after any revision of these arrangements.

- Subsequent to our preliminary report, many submissions supported our preliminary recommendation to combine the EC schemes. Reservations were expressed about
the level of funding, some ongoing inequities between DHBs, and different requirements for children.

Patients and health-service users

Problems reported to us:

- Many groups and individuals do not have access to new innovative pharmaceutical treatments for their illnesses. Some of these illnesses included rheumatoid arthritis, renal lupus, scleroderma, lysosomal 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 medicines (prescribing, pick-up, use).
  - It was pointed out to us that there are subsets of most ethnic groups that are more likely to be prone to specific diseases and syndromes. Genome analysis is enabling more of these to be identified, and in due course more treatments will be available.

- As discussed in the previous section, there are concerns that a negative consequence of PHARMAC’s pricing and single-supplier practices is a smaller range of available medicines for particular conditions. PHARMAC often funds just one medicine in each therapeutic sub-group.
  - As an example of the impact of this practice, 80% of patients with severe rheumatoid arthritis in New Zealand treated with Humira, a TNF alpha, have a positive response to that treatment but this means that 20% of patients with very severe rheumatoid arthritis have no alternative innovative treatment in New Zealand. Hence, if that medicine is ineffective for a given patient then there are few, if any, effective substitutes available.
  - This in contrast to the five or six treatment options for people with severe rheumatoid arthritis in Australia who do not respond to the earlier lines of treatment. These drugs are subject to strict criteria and if there is no improvement then the treatment with the second, third, fourth tier drug is discontinued.
  - Some submitters maintained that the other options could sometimes be cheaper than the supplied drug, and the health consequences for individuals, who may be able to live a much more productive life, certainly improved.
Some submitters believe that individual New Zealanders should be able to more easily access medicines that are not currently funded in New Zealand by purchasing the medicines themselves or via some other private arrangement with a pharmaceutical company.

Our comments:

- As we discussed in Section 2, when high cost medicines are not funded this results in not having access to 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
  - We have recommended that other treatments could be considered in specific cases with defined criteria.

Paediatric medicines

Problems reported to us:

- Simply put, in terms of medicines, children are not just little adults. In general, because there are fewer trials of medicines in forms appropriate for children reducing the availability of appropriate dosage forms for this population, Pharmaceutical companies will not produce and register a child-friendly formulation if they not have dosing or safety information to use the medicine in 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 single-supplier decisions, disruptions in medicine supply pose problems for paediatric patients as the newer medicines may not be able to be formulated in the same way as the previous one.

- Pharmacists require stable formulations and funded ingredients to prepare medicines to replace those available overseas.
o 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).

o There are concerns about the chemical stability of formulations for children created by community pharmacies.

o Patients (parents) need to travel to larger centres to pick up medicines because available stable formulations are not available.

**Paediatric Specialists suggest:**

o PHARMAC consider paediatric medicines as part of its tender processes.

o An evidence-based and available suspending agent is funded for medicines prescribed for paediatric patients.

o Community pharmacists are given access to an approved database of approved formulations.

o 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.**

o 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.

o 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.**

o 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:**

- Clearly, patient advocacy groups play a multi-faceted role, including: educating the public about the needs of their particular group, educating patients and their family about how best to live with a condition, and raising funds from the public and private sectors in order gain access to better treatments for their clients. It is also understandable that patient advocacy groups have a strong interest in decisions that will significantly affect people who are important to them.

- We accept that involving the community in medicine assessment and prioritisation processes is valuable for transparency and public participation reasons. But the value of single-issue advocates is less clear. The dilemma presented by single-issue advocates contributing to prioritisation processes is that 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.

- Patients and their representatives and advocates need to be heard but their position is hard to reconcile with the prioritisation processes involved in assessing a medicine for its value for money relative to other medicines competing for funding. 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.

- We understand that PHARMAC is currently reviewing the terms of reference for its Consumer Advisory Committee (PHARMAC 2009a, 2010).

**Medsafe**

**Problems reported to us:**

- New Zealand represents less than 0.1% of the global pharmaceutical market. Due to our isolation and the lack of local manufacturers, the cost of importation of medicines to New Zealand is relatively high. The loss of niche low cost and highly specialised (low volume) medicines from the market is a global problem.

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41 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.
The management of New Zealand’s medicines system by imposing a cap on spending and tendering for sole supply of some medicines, while responsible for lowering costs, has likely been a disincentive for bringing, or maintaining, low cost and low volume medicines into the New Zealand market.

Traditionally some low-cost, low-volume medicines were provided to New Zealand by pharmaceutical companies where the profit earned from another product subsidised the supply of the low volume product. Increased pressure on the profits of the pharmaceutical industry is causing pharmaceutical companies to merge, which is leading to rationalisation of the products that they supply and divestment of low-cost, low-volume medicines.

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, low-volume 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 low-cost, low-volume medicines – 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?

The processes required to comply with Section 29 of the Medicines Act can be onerous.

Our comments:

- In our opinion, the arguments advanced in the second and third bullet points above are moot. More research could be done to investigate these issues.
DHBs

Problems reported to us:

- 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 and patient advocates are troubled about the inequity that results from these different 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.

- Applications under S29 of the Medicines Act can be frustrating and time-wasting, and so some aspects of this Act should be revised.

Our comments:

- PHARMAC currently manages the list of subsidised community pharmaceuticals, and hospital pharmaceutical cancer treatments (PCTs). It also negotiates national supply agreements for some hospital medicines on behalf of DHBs, which are listed in section H of the Pharmaceutical Schedule relating to hospital medicines. We have
been disturbed that there seems to be a relatively poor understanding of pharmaceutical expenditures in hospitals, that they are less likely to be subject to value-for-money assessments and are relatively ‘hidden’ from scrutiny.

- PHARMAC’s current hospital medicines activity is enabled by its establishment provisions in the New Zealand Public Health and Disability Act, but is also specifically provided for in a Ministerial authorisation issued in September 2001. Other than PCTs, PHARMAC does not manage decision-making with respect to medicines that are funded by DHBs for the treatment of patients in a hospital and it is not involved in the ordering of, payment for or distribution of pharmaceuticals to pharmacies (whether hospital or community based).

Prescribers

Problems reported to us:

- 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\(^\text{42}\) being in place since October 2003, many clinicians perceive the three-month prescribing regime is wasteful and the scheme underpinning it is unduly complex.

Our comments:

- The Community Pharmaceutical Schedule describes the pharmaceuticals available in New Zealand, their costs and access criteria. A formulary would describe how to dispense and prescribe those drugs, along with some agreed care pathways.

- Possible definitions of the word “formulary” are broad, such that it means different things to different people. For example, this is the definition from Wikipedia (http://en.wikipedia.org/wiki/Formulary_(pharmacy); retrieved 22 March 2010):

  At its most basic level, a formulary is a list of medicines. Traditionally, a formulary contained a collection of formulas for the compounding and testing of medication (a resource closer to what would be referred to as a pharmacopoeia today). The main function of formularies today is to specify which medicines are approved to be prescribed under a particular contract. The development of formularies is based on evaluations of efficacy, safety, and cost-effectiveness of drugs.

\(^{42}\) This scheme involves dispensing medicines in quantities sufficient for a three-month supply all in one go.
Depending on the individual formulary, it may also contain additional clinical information, such as side effects, contraindications, and doses.

**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 a small minority of pharmaceutical-industry insiders and their consultants (e.g. as contained in several reports and background papers to various fora) about how PHARMAC makes decisions. Specifically, some seem to be unaware of how Cost-Utility Analysis and QALYs work.
  
  o An earlier version of the comments immediately above upset some people following the release of the ‘preliminary report’. We explained ourselves to concerned individuals, and stand by our comments in the context in which they are made.
References


www.beehive.govt.nz/release/ministerial+review+group+report+released

Ministry of Health, Special High Cost Treatment Pool, Ministry of Health website.
www.moh.govt.nz/moh.nsf/indexmh/special-high-cost-treatment-pool#examples

National Institute for Health and Clinical Excellence (NICE), What we do, NICE website. www.nice.org.uk/aboutnice/whatwedo/what_we_do.jsp


PHARMAC, *Draft Terms of Reference for the PHARMAC Consumer Advisory Committee*, March 2010. [www.pharmac.co.nz/2010/03/05/Final%20Draft%20CAC%20ToR.pdf](http://www.pharmac.co.nz/2010/03/05/Final%20Draft%20CAC%20ToR.pdf)


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

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

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

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) during the ‘invited submissions’ and ‘consultation’ phase of the review:

<table>
<thead>
<tr>
<th>Name</th>
<th>Organisation</th>
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<tbody>
<tr>
<td>June Tordoff</td>
<td>School of Pharmacy, University of Otago</td>
</tr>
<tr>
<td>Andrea Grant</td>
<td>Roche Products (New Zealand) Ltd</td>
</tr>
<tr>
<td>Bill Denny</td>
<td>University of Auckland</td>
</tr>
<tr>
<td>Andrew Sutton, William Wong, Caroline De Luca, Brenda Hughes</td>
<td>Starship Children’s Hospital</td>
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<tr>
<td>Geoff McDonald, Katherine Lester, Frances Benge, Alan Carter, Pippa MacKay</td>
<td>Researched Medicines Industry Association of New Zealand Inc (RMI)</td>
</tr>
<tr>
<td>Brian Rousseau</td>
<td>CEO Otago &amp; Southland District Health Boards</td>
</tr>
<tr>
<td>Sandra Kirby, Natalia Valentino</td>
<td>Arthritis New Zealand</td>
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<tr>
<td>John Forman</td>
<td>New Zealand Organisation for Rare Disorders</td>
</tr>
<tr>
<td>Richard Furneaux</td>
<td>Industrial Research Ltd (IRL)</td>
</tr>
<tr>
<td>Stewart Jessamine</td>
<td>Medsafe</td>
</tr>
<tr>
<td>Les Toop, Dee Mangin</td>
<td>Christchurch School of Medicine</td>
</tr>
</tbody>
</table>
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 and Pharmacists) 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 (before the ‘preliminary report’ on 1 December 2009) from:**

<table>
<thead>
<tr>
<th>Name</th>
<th>Organisation</th>
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<tbody>
<tr>
<td>Andrew Harrison</td>
<td>New Zealand Rheumatology Association</td>
</tr>
<tr>
<td>Sarah Perry</td>
<td>Cancer Society</td>
</tr>
<tr>
<td>John Highton</td>
<td>(Rheumatologist) Otago DHB</td>
</tr>
<tr>
<td>Callum Wilson</td>
<td>Starship Children’s Hospital</td>
</tr>
<tr>
<td>J Alasdair Millar</td>
<td>(Consultant Physician) Southland DHB</td>
</tr>
<tr>
<td>Rosemary Marks</td>
<td>Paediatric Society of New Zealand</td>
</tr>
<tr>
<td>Daniel Ching</td>
<td>(Consultant Physician &amp; Rheumatologist)</td>
</tr>
<tr>
<td>Sandra Kirby</td>
<td>Arthritis New Zealand</td>
</tr>
<tr>
<td>Richard Isaacs</td>
<td>Mid-Central Cancer Service, Palmerston North</td>
</tr>
<tr>
<td>David Pullar</td>
<td>Genzyme Australasia Pty Ltd</td>
</tr>
<tr>
<td>Pippa McKay</td>
<td>Researched Medicines Industry Association of New Zealand Inc (RMI)</td>
</tr>
<tr>
<td>Svend Petersen</td>
<td>Roche Products (New Zealand) Ltd</td>
</tr>
<tr>
<td>David Simpson</td>
<td>(Consultant Haematologist) Waitemata DHB</td>
</tr>
<tr>
<td>Peter Chapman, John O’Donnell, Lisa Stamp</td>
<td>(Rheumatologists) Canterbury DHB</td>
</tr>
</tbody>
</table>

Following the release of the ‘preliminary report’ on 1 December 2009, a ‘Feedback Forum’ was held on 17 February 2010 to which everyone consulted with was invited, as well as anyone else who was interested (invitations were included with the preliminary
Following the release of the ‘preliminary report’ (1 December 2009), we consulted with:

<table>
<thead>
<tr>
<th>Name</th>
<th>Organisation</th>
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<tbody>
<tr>
<td>Callum Wilson</td>
<td>Starship Hospital</td>
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<tr>
<td>Stewart Jessamine</td>
<td>Medsafe</td>
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<tr>
<td>Andrea Grant, David Chisholm</td>
<td>Roche Products (New Zealand) Ltd</td>
</tr>
<tr>
<td>Denise Wood, Kevin Sheehy, Alan Carter</td>
<td>Researched Medicines Industry Association of New Zealand Inc (RMI)</td>
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<tr>
<td>Dan Brown, Ric Sicurella, David Pullar,</td>
<td>Genzyme Australasia Pty. Ltd</td>
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<tr>
<td>Barbara Wood</td>
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<tr>
<td>John Forman</td>
<td>New Zealand Organisation of Rare Diseases</td>
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<tr>
<td>Frances Benge</td>
<td>Pfizer New Zealand</td>
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<tr>
<td>Peter Dunne</td>
<td>Associate Minister of Health</td>
</tr>
<tr>
<td>Melissa Young and colleagues</td>
<td>Waikato DHB</td>
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<tr>
<td>Carolyn Gullery</td>
<td>GM Planning and Funding, Canterbury DHB</td>
</tr>
<tr>
<td>Callum Wilson</td>
<td>Starship Hospital</td>
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<tr>
<td>Barbara Woods</td>
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<tr>
<td>Most of the PHARMAC staff noted in the</td>
<td>PHARMAC</td>
</tr>
<tr>
<td>previous tables</td>
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<tr>
<td>George Laking</td>
<td>On behalf of Auckland Medical Oncologists</td>
</tr>
</tbody>
</table>

Following the release of the ‘preliminary report’ (1 December 2009), written submissions were received from:

<table>
<thead>
<tr>
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<tr>
<td>Andrew Harrison</td>
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</tr>
<tr>
<td>Callum Wilson</td>
<td>Starship Children’s Hospital</td>
</tr>
<tr>
<td>Caroline De Luca, Brenda Hughes, Rosemary</td>
<td>Pharmacy &amp; Therapeutics Committee of the Paediatric Society of New Zealand</td>
</tr>
<tr>
<td>Marks, Andrew Sutton, William Wong</td>
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<tr>
<td>Sandra Kirby</td>
<td>Arthritis New Zealand</td>
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<tr>
<td>John Foreman</td>
<td>New Zealand Organisation of Rare Diseases</td>
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<td>Kevin Sheehy</td>
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<tr>
<td>Svend Petersen</td>
<td>Roche Products (New Zealand) Ltd</td>
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<tr>
<td>Name and Affiliation</td>
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<td>----------------------------------------------------------</td>
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</tr>
<tr>
<td>George Laking, Reuben Broome, Dragan Damianovich, Michael Findlay, Peter Fong, Vernon Harvey, David Porter, Rita Sasidharan and Richard Sullivan</td>
<td>Medical Oncologists in Auckland</td>
</tr>
<tr>
<td>Peter Chapman, John O’Donnell, Lisa Stamp</td>
<td>Rheumatologists Canterbury DHB</td>
</tr>
<tr>
<td>Cristine Della Barca</td>
<td>Subscripts Limited</td>
</tr>
<tr>
<td>David Hadorn</td>
<td>Centre for the Study of Assessment &amp; Prioritisation in Health, University of Otago School of Medicine</td>
</tr>
<tr>
<td>Peter Foley</td>
<td>New Zealand Medical Association</td>
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<tr>
<td>William (Billy) Allan</td>
<td>New Zealand Hospital Pharmacists’ Association Inc</td>
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<tr>
<td>Chris Walsh</td>
<td>Breast Cancer Aotearoa Coalition</td>
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<td>John O’Donnell</td>
<td>Christchurch Hospital</td>
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<td>Elisabeth Burgess</td>
<td>Breast Cancer Aotearoa Coalition</td>
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<td>Alan J Shirley</td>
<td>Wairarapa DHB</td>
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<td>Elizabeth Plant</td>
<td>Taranaki District Health Board</td>
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<td>Adele Harrex</td>
<td>Auckland DHB</td>
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<td>Pru Etcheverry</td>
<td>Leukaemia &amp; Blood Foundation</td>
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<td>Matthew Brougham</td>
<td>PHARMAC</td>
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<tr>
<td>Tim Wood</td>
<td>Northern DHB Support Agency Ltd</td>
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<td>Alistair Barkhouse</td>
<td>Gilead Sciences</td>
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<td>Anna Mitchell</td>
<td>Canterbury Arthritis Advocates</td>
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<td>Christy Parker</td>
<td>Women’s Health Action Trust</td>
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<tr>
<td>Andrew Wilson, Annabel Dunn, J Robinson and colleagues</td>
<td>Palliative Care Medications Work Group</td>
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<tr>
<td>Jan Goddard</td>
<td>Waikato District Health Board</td>
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<tr>
<td>John Barnard</td>
<td>Waikato District Health Board</td>
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<tr>
<td>Karen Thomas</td>
<td>The Royal New Zealand College of General Practitioners</td>
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<tr>
<td>Tim McCormick</td>
<td>Bio gen Idec</td>
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<td>Annabel Young</td>
<td>Pharmacy Guild of New Zealand</td>
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<tr>
<td>Vanessa Brown</td>
<td>Cranford Hospice</td>
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<tr>
<td>Sunita Goyal</td>
<td>Accident Compensation Corporation</td>
</tr>
<tr>
<td>Andrew Moore&lt;sup&gt;43&lt;/sup&gt;</td>
<td>Department of Philosophy, University of Otago</td>
</tr>
</tbody>
</table>

<sup>43</sup> Andrew’s submission arrived very late, so we were not able to fully consider it.
Appendix 3: Documents read by the Review Panel\textsuperscript{44}

(In addition to the documents in the References earlier.)


L Beil (2009), Targeted therapy: Hope or hype, curetoday.com. \url{www.curetoday.com/index.cfm/fuseaction/article.PrintArticle/article_id/1133}


D Cohen (2009), Academics criticise plan to allow new drugs to bypass NICE, \textit{British Medical Journal} 339, b2938.


TB Cueni (2008), Can Europe afford innovation? \textit{Eurohealth} 14, 8-10.


\textsuperscript{44} Including material supplied to us by individuals and organisations with whom we consulted.


J Gever (2009), Cost of cancer drugs to force hard decisions, medpageTODAY. www.medpagetoday.com/publichealthpolicy/healthpolicy/14899


PHARMAC, *Further Supplementary Technology Assessment Report No. 75c. 12 month trastuzumab (Herceptin) treatment in HER-2 positive early breast cancer, compared with 9 week concurrent treatment*, July 2008.  

PHARMAC, *Having Your Say in Our Decisions*.  


PHARMAC, *Information Sheets*.  
[www.pharmac.govt.nz/dhbs/AboutPHARMAC/infosheets](http://www.pharmac.govt.nz/dhbs/AboutPHARMAC/infosheets)

PHARMAC, *Introduction to PHARMAC, PHARMAC Information Sheet*.  
[www.pharmac.govt.nz/2008/12/16/01_PHARM_Infsheet_INTRO.pdf](http://www.pharmac.govt.nz/2008/12/16/01_PHARM_Infsheet_INTRO.pdf)


[www.pharmac.govt.nz/Schedule](http://www.pharmac.govt.nz/Schedule)

[www.pharmac.govt.nz/AboutPHARMAC/procedures](http://www.pharmac.govt.nz/AboutPHARMAC/procedures)


PHARMAC, PHARMAC’s Exceptional Circumstances Schemes, 2009.


PHARMAC, Section H for Hospital Pharmaceuticals, Effective 1 March 2009. www.pharmac.govt.nz/Schedule/SectionH


PHARMAC, Targeting Medicines, PHARMAC Information Sheet. www.pharmac.govt.nz/2008/12/16/PHARM_Infosheets_TARGETING_MEDICINES.pdf


PHARMAC, Terms of Reference for the Pharmacology & Therapeutics Advisory Committee (PTAC) and PTAC Sub-Committees, 2008. www.pharmac.govt.nz/2008/10/30/2008%20PTAC%20ToR.pdf


J Raftery (2009), NICE and the challenge of cancer drugs, British Medical Journal 338, b67.


L Toop, D Richards, T Dowell, M Tilyard, T Fraser & B Arroll, *Direct to Consumer Advertising of Prescription Drugs in New Zealand: For Health or Profit?* University of Otago, 2008.

(Unattributed), *Affordable Access to New Medicines with Particular emphasis on High Cost, Highly Specialised Medicines. Position Statement*.


A Wishart, The unbearable cost of living, *The Sunday Times* (UK), June 7 2009. [www.timesonline.co.uk/tol/news/uk/health/article6430926.ece](http://www.timesonline.co.uk/tol/news/uk/health/article6430926.ece)