



PHARMAC REVIEW
Te Arotake i Te Pātaka Whaioranga



Te Kāwanatanga o Aotearoa
New Zealand Government

Pharmac Review

Final Report: Executive Summary

February 2022



E kore e taea e te whenu kotahi ki te raranga i te whāriki kia mōhio tātou ki ā tatou. Mā te mahi tahi ō ngā whenu, mā te mahi tahi ō ngā kairaranga, ka oti tēnei whariki

Standing alone, a strand of flax cannot achieve but woven together is strong and enduring. Collective efforts often result in more meaningful and sustainable outcomes.

Each thread of our enquiry led us to the view that a more coordinated approach was needed, with Pharmac working in a more integrated way with the health sector. This whakataukī also reflects the coming together of the panel to provide what we hope is a report that will lead to meaningful change.

Acknowledgements: The Pharmac Review Panel (Chair Sue Chetwin, Professor Sue Crengle, Associate Professor Tristram Ingham, Frank McLaughlin, Heather Simpson, Leanne Te Karu) is grateful to the feedback received from stakeholders throughout its work. Many of these views are captured in our interim report, published in November 2021. Since finalising our interim report, we have also received further feedback from patient groups, Māori scholars, clinicians, advocates, and Pasifika clinicians, which has been invaluable. We are also grateful to Ministry of Health officials, the Human Rights Commissioner, Disability Commissioner, Children’s Commissioner, Te Aho o Te Kahu, the Health Transition Unit, Treasury officials, specialist medical committees within Pharmac, in particular the pharmacology and therapeutic advisory committee heads, the Māori Advisory Rōpū, the consumer advisory committee and of course Pharmac itself, all who have contributed analysis and insights to the review.

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Message from the Chair

It was the stoicism of the young woman who, along with her companion colostomy bag, had dragged herself out of bed to attend a meeting with me as head of the Pharmac review, that simply took my breath away. She was speaking on behalf of Crohn's disease sufferers. She had no bowel left to speak of. Her prognosis, too bleak to mention.

Every country wrestles with the challenge of funding an ever-increasing array of new and expensive medicines. For 27 years in Aotearoa New Zealand that task has fallen to Pharmac, and with it the responsibility for managing the hugely sensitive trade-offs involved in securing pharmaceuticals for our hospitals, primary healthcare and ultimately consumers like this Crohn's sufferer.

It has been my privilege to lead the first review of how well Pharmac meets its objective of achieving the best health outcomes for all New Zealanders, within a capped budget. It comes at a time when the entire health and disability system is going through the biggest reform in decades. The review has been mindful that its recommendations must support and enable Pharmac to become more closely knitted into this new integrated health system.

Our terms of reference essentially asked us to consider Pharmac's systems and processes and assess whether they achieve equitable health outcomes for all New Zealanders, but in particular for Māori, Pasifika, disabled people, and other priority populations. All our discussions, observations and recommendations have been considered from an equity perspective.

We've spent many hours examining the engine room of Pharmac – the decision-making processes it uses for prioritising which medicines to fund. Some of our recommendations, resulting from that analysis, will make hard reading, particularly those about improving equity outcomes. The review notes deficiencies in the nature of the decision-making process (from the board down) and the quality of the decisions that came out of it. The result has been inequitable outcomes for Māori, Pasifika, disabled people and other priority populations. Essentially our recommendations call for better oversight, better processes and more voices to be heard in deciding which medicines will be funded and for whom. However, we do note that while processes at Pharmac need improvement, their development has to be seen against a backdrop of an entire health system that has failed to properly honour te Tiriti o Waitangi principles. The reform of the health and disability system is designed to redress this, and our recommendations are in keeping with the proposed Pae Ora (Healthy Futures) legislation.

There is still a lot of which Pharmac can be proud. Its immensely skilled staff work in an agency that is unique, in that it combines medical assessment with procurement and budget management. Tens of thousands of New Zealanders benefit every day when they pick up their medicines or receive them in hospitals – mostly unaware of the work Pharmac does. And it would be fair to say that over the years Pharmac has been seen in some respects as like the 'little engine that could'. It has been given the complex task of applying the model it uses to drive sharp prices for pharmaceuticals and using the savings to fund more, as well as procuring medical devices and, latterly, vaccines. Neither of these new responsibilities, as we explain, sits well with Pharmac.

It also operates in an environment where international pharmaceutical companies insist on confidential deals, involving complicated rebate and discount schemes, all designed to ensure countries and jurisdictions pay top dollar and cannot compare prices paid.

Our interim report assembled much of what the review heard from patients, advocates, clinicians, industry lobbyists, pharmaceutical companies, Māori and Pasifika health providers and Pharmac itself.

Access to medicines is just one part of what determines a 'healthy outcome'. Some of our recommendations call for this new integrated system to work collaboratively to help provide better health outcomes for priority groups such as those with rare disorders. Pharmac must be part of that collaboration. It will need to work more openly for this to happen well.

The review panel has met frequently since it was formed in March last year. Covid-19 has presented challenges, but it has not stopped us from going about our work. Along with the groups mentioned above, we have met officials in the Ministry, the Human Rights Commissioner, Disability Commissioner, Children's Commissioner, Te Aho o Te Kahu, the Health Transition Unit, Treasury officials, specialist medical committees within Pharmac, in particular the Pharmacology and Therapeutic Advisory Committee heads, the Māori Advisory Rōpū, the Consumer Advisory Committee and of course Pharmac itself. In our interim report we noted the difficulty of extracting information from Pharmac. I am pleased to say we reached agreement to receive most of the data needed to complete our analysis. And I thank Pharmac for making its staff available to answer our many questions.

Coming from a consumer and journalist background but with little knowledge of the health system may have been a blessing because I brought fresh eyes. What I can say is that without the support, commitment, and specialist knowledge of each of the panellists (Sue Crengle, Tristram Ingham, Frank McLaughlin, Heather Simpson and Leanne Te Karu) we could not have completed such a thorough review. In addition, there has been the extraordinary dedication of our secretariat, in particular head of secretariat Sarah Davies, and our tireless consultants Sapere Research Group and Gabrielle Baker.

My parting thought is for the young woman who shared her story with me. In recognising that not all medicines can be publicly funded, I hope this report and our recommendations make a difference.

I commend the review and its recommendations to the Minister.



Sue Chetwin CNZM
Chair

Introduction and context

For Pharmac to be effective and deliver its core objectives, it needs to be far more integrated into the health system. This will require more substantial commitments and effective actions both by Pharmac and by the key health agencies it must work with, to ensure a more joined-up, effective and equitable health system. Evaluating and funding pharmaceuticals and the management of their supply are critically important, and these activities must be informed by the new health system frameworks and the priorities they establish.

The review recommends some current roles undertaken by Pharmac would be better advanced by other agencies, namely what goes on to the vaccination schedule, cataloguing and contracting of medical devices, and holding the leadership role in promoting responsible use of pharmaceuticals. The health system is changing, and the review has identified other lead agencies that are better placed to advance these functions. This would also free up Pharmac to focus more closely on its core role as a centre of excellence in respect of the assessment and evaluation, and purchase of, pharmaceuticals. We also suggest Pharmac takes on an enhanced role relating to the sustainable supply of pharmaceuticals.

Pharmac operates in a challenging space where staff consider highly technical material and make recommendations that have far-reaching impacts. Investment in medicines in a publicly funded health system is highly contested by those who manufacture the medicines and those who need them, which means Pharmac is often criticised.

Savings have historically been a dominant focus for Pharmac. Global practices favour confidentiality and pricing strategies such as rebates, volume discounts and bundling.

The review believes claims about the quantum of savings should be treated cautiously but that a fixed-budget, centralised agency, with expertise in pharmaceutical evaluation and commercial negotiation, has assisted, and will continue to assist, the New Zealand public health system.

In this final report we focus on:

- what changes are required to Pharmac's objectives, functions, governance and accountability arrangements to better enable the best health outcomes for New Zealand and enhance public trust and confidence in the functions Pharmac undertakes
- Pharmac's pharmaceutical investment decision-making processes and in particular how equity is considered. These processes underpin Pharmac's core function to maintain and manage the pharmaceutical schedule and ultimately are crucial to Pharmac achieving its legislative best health outcomes objective
- Pharmac's other functions, including how it promotes the responsible use of medicines, its expanding role contracting medical devices, its decision-making and purchasing role for vaccines, and what role it currently plays in supporting security of pharmaceutical supply

- the growing area of rare disorders and how well people with rare disorders are served
- cancer treatments including how New Zealand can manage the increasing demand for and cost of new treatments.

Throughout this process the review has:

- used an equity lens to understand whether and how Pharmac can achieve equity in investing public funding to achieve improved health outcomes
- focused on the purposes and implications of the reformed health system: where within the health system responsibility best sits for particular functions currently carried out by Pharmac; and, for the functions and activities that Pharmac is best placed to lead, how to ensure they are better grounded in the broader needs of the health system
- been guided by the importance of what needs to change to enhance public trust and confidence in Pharmac.

It is clear to us that 27 years is too long to wait for a first review. As an organisation it has evolved considerably – both for the good and for the not so good. It would be unnecessary and impractical to undertake a review of this nature frequently, but we do recommend an external quality assurance of some elements of the business – in particular the decision-making processes and equity outcomes – be carried out regularly, for example in alignment with the health strategic plan cycle.

Our approach to this report

For this report we carried out a thorough analysis of large data sets that looked at volumes of pharmaceuticals dispensed, considering ethnicity, geography and age, which showed us how Pharmac's investments were being spent. We made comparisons with Australia, Canada and the United Kingdom – both for the approach they take to assessing medicines for public funding and how they use expert advice and consumer and patient voices in their decision-making. We have also completed detailed reviews of how Pharmac makes decisions, who it involves and how it considers equity. Finally, we continued to consider the views of stakeholders, including further meetings and hui.

We have sought to understand how Pharmac, as a Crown-owned entity, is meeting government and Māori expectations around te Tiriti o Waitangi. It is clear that a significantly stronger te Tiriti o Waitangi response is needed from Pharmac.

We think the question of how mātauranga Māori is embedded across the full range of medicines activities is relevant to every part of the health system and best led by the Māori Health Authority.

In keeping with our terms of reference, the health and disability system reforms and the proposed Pae Ora legislation, we have considered how Pharmac's actions and inactions impact on three priority populations: Māori whānau, hapū and iwi; Pasifika; and disabled people. Findings on equity are woven throughout our report and are particularly important in the chapter on decision making.

Pharmac's operating environment

Pharmac was established in response to the high prices New Zealand was paying for publicly funded pharmaceuticals, demonstrably higher than Australia. A fixed-budget, single buying agency was seen as the best way to evaluate pharmaceuticals, negotiate prices and develop the capabilities needed for pharmaceutical management.

The primary reasons for a single agency approach were set out in the interim report. Those reasons are reinforced by current changes in the global pharmaceutical market, including:

- Pharmaceuticals are increasingly expensive
- International pharmaceutical regulatory standards have changed, and many pharmaceuticals are launched with less evidence of their efficacy
- Technologies have changed and this is reducing competition in some areas
- Pharmaceutical companies have separated markets and made it more difficult to understand market pricing.

The pressures on the pharmaceutical budget are pressures faced by all countries and increasingly tough decisions have to be made almost irrespective of the budget allocated. Given this environment, it is difficult to conceive of a situation where anything other than a fixed-budget, centralised specialist function would achieve better pharmaceutical assessment and cost management results. Our terms of reference required us to assume Pharmac would continue as a Crown-owned entity. We think this is sensible given the significant health reforms being undertaken. However, the review believes it would be worth reassessing Pharmac's role in the health system and whether it should continue as a stand-alone entity once the reforms are embedded, and the new agencies are fully operational.

In considering Pharmac's operating environment the review undertook analysis that examined Pharmac's investment profile including the number of new medicines that were added between 2010 and 2020 and which therapeutic groups they benefitted. The review also looked at which population groups benefitted from the new investments.

We make the following observations regarding our findings:

- Lower new listing expenditure for some populations (particularly Māori and Pasifika) is the result of poor access to medicines, barriers to accessing care, differences in quality of care, or a combination of these factors
- Some of the effect is due to Pharmac's prioritisation and funding processes. Lower per capita expenditure for priority populations is consistent with the review's analysis of Pharmac's decision-making process that shows equity considerations are not given due weight in its investment decisions
- Investment in cancer medicines, which appears to favour non-Māori/non-Pacific populations and those living in urban areas, has been made at the expense of other treatments.

In general, the review would expect to see more new expenditure favouring priority populations or being put towards reducing existing inequities. We believe Pharmac could provide more insight into changing patterns of investment in pharmaceutical use in New Zealand. Equity capability and capacity, and horizon scanning, are important to support this work.

The review agreed maintaining supply was particularly important. New Zealand is vulnerable to international changes in demand and supply of medicines. These changes can be difficult to predict.

Pharmac has done well to monitor and manage stock issues with pharmaceutical suppliers to keep frequency of stock outages to low levels. However, there have been some notable supply outages which have caused significant disruption and a disproportionate impact on at risk populations.

In the context of increasing global medicines and vaccine shortages and ongoing supply chain disruptions caused by Covid-19, the risks of supply outages impacting patients become greater. The review thinks there is a case for an enhanced role for Pharmac as an advisor to the Government on supply chain risks.

Governance and accountability

There is a need to make equity requirements more explicit in Pharmac's objectives and for Pharmac to be guided by the reformed health system's principles. This would require a change to the legislation. We also consider some Pharmac functions would be better performed by other agencies, but that it should have a new function relating to supply chain oversight as discussed under Pharmac's Operating Environment.

The review looked at the effectiveness of Pharmac's governance and accountability arrangements and in particular at the board, its responsibilities and its performance in overseeing Pharmac. We identified the board's oversight needs to focus more on Pharmac's core function of assessing and funding medicines and ensuring Pharmac operates collaboratively and effectively within the new health system. We also looked at the advisory groups which support the board with its governance role. We think the Consumer Advisory Committee (CAC) could play a stronger governance role but that to do this its members should be appointed by the Minister in consultation with the board. The CAC terms of references should be subject to the Minister's approval. This would provide a strong signal that consumer input needs to be, and is seen to be, independent of Pharmac's preferences of the day. We think this is ultimately in Pharmac's best interests.

We identified the importance of a revised system-wide medicines strategy to provide a framework for Pharmac to work within. The existing strategy is widely considered to be out of date because of changes in the medicines and wider health sectors in the past 15 years. We think work on a revised strategy should be progressed quickly and in consultation with stakeholders.

We identified the need for better system level horizon scanning to look for emerging trends domestically and overseas that might warrant funding. While the responsibility for this will primarily reside with various combinations of the Ministry of Health (the Ministry), Health NZ and the Māori Health Authority, Pharmac must be actively involved. We also noted a need to share more information across the health system and for a fairer representation of Māori, Pasifika and disabled people within Pharmac's own ranks and committees.

Pharmac also needs to change how it considers and manages confidentiality. Treasury alongside Health NZ and the Māori Health Authority will need to have sight of how public money is being invested and the outcomes Pharmac is achieving. The review believes that this can be achieved without compromising the confidentiality measures required by the pharmaceutical companies.

Recommendations

The review recommends the Minister:

- change the Pae Ora (Healthy Futures) Bill so that Pharmac's best health outcomes objective includes securing equitable health outcomes for Māori and other populations
- make explicit the expectation that in seeking the best health and equity outcomes, Pharmac must work collaboratively with the Ministry, Health NZ, and the Māori Health Authority
- ensure all health system guiding principles in the Bill apply to Pharmac
- amend Pharmac's functions to:
 - transfer responsible use of medicines to Health NZ and Māori Health Authority
 - enhance its role as an advisory agency in security of supply for pharmaceuticals
- agree the membership of the Consumer Advisory Committee should be appointed by the Minister
- direct the Ministry to develop an updated medicines strategy in consultation with stakeholders (including Māori, Pasifika, disabled people) on its contents over the next 12 months
- require Pharmac to ensure its contractual obligations do not preclude the sharing of commercially sensitive information with the key monitoring agencies such as Health NZ / Māori Health Authority, the Treasury
- require Pharmac to improve the transparency and accessibility of its systems, processes, resources, and communications to allow disabled people to participate and contribute on an equal basis
- direct Pharmac, and other agencies in the health sector to review how the different operating approaches used in the Covid-19 response could be applied to business-as-usual, including working collaboratively and speedily, sharing data, and using streamlined processes.

Pharmac's decision-making

We found Pharmac's decision-making processes did not always follow its own internal guidance. Further, it could be more transparent and explicit, and could address equity considerations much more rigorously and directly.

We looked at Pharmac's decision-making in three ways:

- How it applied analytical decision-making tools and followed its own defined medicine assessment process. We used six case studies Pharmac had assessed to evaluate whether the assessment matched Pharmac's internal guidance. The variety of case studies were deliberately chosen to provide insights into how the process of assessing applications and determining funding priorities worked in practice across different therapeutic areas.
- How it made funding decisions, including who reviews the analysis, what voices were heard in decision-making, and how decisions were communicated to the public.
- The extent to which inputs into decision-making – such as reports on Pharmac's factors for consideration framework, the technology assessment reports, the prioritisation dossiers and the one decision-making paper – were appropriate, particularly in light of equity considerations.

Overall, we found a fragmented documentation process which did not consistently follow internal guidance and was lacking strong governance oversight. For example, Pharmac's internal guidance recommends separate cost-utility analysis for different population groups if an inequity is likely to cause significant differences. However, while Pharmac recognises Māori, Pasifika and priority groups are disproportionately affected by diabetes and lung cancer, it did not recognise this fact in the analyses we reviewed. We also noted a dependence on the use of trial population information provided by the pharmaceutical companies rather than considering the characteristics of the New Zealand population. This would also impact on Pharmac delivering equitable outcomes.

The mechanisms designed to take equity into account did not do so and it was difficult to ascertain exactly when a decision had been made. Overall, we observed through the case studies the focus on utilitarianism suggested Pharmac's decision-making errors and omissions could be increasing inequities.

We looked at the factors for consideration in more detail and found they were too wide-ranging with no formal means of evaluating how they had been applied. It was also difficult to assess whether they were being applied consistently across applications and over time. Through our analysis we found that even if the factors for consideration had been applied, they did not make a material difference to the outcome when the application was being ranked on the options for investment list.

Overall, when considering applications Pharmac needs to put in place an integrated analytical framework to address the issues that the review identifies. There also needs to be greater clarity on what constitutes 'a decision' and we would like to see a robust assurance process that supports the board in its governance role.

We also see the need for greater diversity of voices in the decision-making process including from its Consumer Advisory Committee, its Pharmacology and Therapeutic Advice Committee, the Māori Advisory Rōpū, the various specialist committees and patient/carer representatives.

There is room to improve communication, both with the public – the various stages of the assessment process are unclear to the members of the public who rely on Pharmac's website for information – and with applicants. We remain concerned about Pharmac's ability to express in clear terms the basis for its decisions and suggest it adopt a proactive release approach to its decisions, rather than waiting for Official Information Act requests. We also suggest this release take the form of a templated one-page statement setting out the reasons for a decision, including, where applicable, a medicine's ranking on the options for investment list.

Recommendations

The review recommends the Minister directs Pharmac to:

- develop an integrated analytical framework for the assessment of pharmaceuticals that incorporates:
 - enhanced cost-benefit analysis with strengthened distributional elements
 - strengthened equity analysis in all its decision-making processes
 - reviewing and revising the factors for consideration to ensure a proper analytical framework for their application, which can be demonstrated to make a material impact on the outcomes of funding decisions and advance the agency's equity goals
 - more formal structure to consider the prioritisation of the options for investment list currently performed by Pharmac staff, with greater input from its advisory committees
 - more generally, role clarity at each step of the decision-making process, including what information should be taken into account when preparing material to support decisions.
- have stronger oversight by the board of the pharmaceutical investment decision-making, with a focus on what is not funded alongside what is funded. This should include:
 - ongoing quality assurance oversight of the investment decision-making process
 - regular evaluations of the impact of investment decisions and assurance that the Pharmaceutical Schedule more generally is advancing Pharmac's objectives, including those of achieving equitable health outcomes.

Cancer medicines

Cancer is New Zealand's leading cause of death, and as the population ages the number of people diagnosed with cancer is forecast to increase. Māori, Pasifika, people with mental illness and disabled people all experience disproportionately worse cancer-related outcomes. For Māori, cancer is the cause of more than a quarter of all deaths. A recent study shows Māori continue to have poorer survival rates than non-Māori for nearly all the most common cancers. This is due to inequities in the social determinants of health, differential access to cancer services, and inequities in cancer treatment. Simply put, Māori are twice as likely to die from cancer as non-Māori.

Scientific advances in cancer medicines mean more people may be able to be treated. But these new medicines are expensive and some may not necessarily be more effective in treating and curing cancer. Trends include the increasing number of medicines under development, weaker evidence of the benefits being required by regulatory bodies (in particular in the United States).

A pharmaceutical company's core purpose is to develop, promote and profitably sell its pharmaceutical products. By contrast, Pharmac's core purpose is to make an assessment across various and often competing products as to which pharmaceuticals should be bought, and at what price, to best meet the public health needs of New Zealanders while keeping within its fixed budget. Inevitably these two purposes lead to tension – a by-product of making trade-offs about which medicines to fund within a limited budget.

There is no doubt New Zealand lags other countries in the provision of cancer medicines. Recent research shows the gap is widening, particularly between Australia and New Zealand. Pharmac is under increasing pressure from pharmaceutical companies, patients, advocacy groups, and the media to fund these 'missing medicines'. However, these discussions on access rarely consider health benefits, risks, affordability, and the likely impact on population health outcomes.

Cancer pharmaceuticals are assessed by Pharmac using the same process as non-cancer pharmaceuticals. The only exception is that Pharmac will start to assess an application for a cancer pharmaceutical before the MedSafe approval has been granted. The review looked at whether the assessment process was appropriate and if, as suggested by stakeholders, there should be a separate ring-fenced fund for cancer pharmaceuticals. The review concluded cancer pharmaceuticals should be considered like other pharmaceuticals. The emphasis needs to be on severity of disease, clinical alternatives and cost for benefit and that ring-fenced funding for cancer could lead to prioritising over other conditions.

Cancer treatments account for a large proportion of Pharmac's new investment spend but the number of pharmaceuticals available is only one part of treating cancer. There is a government priority to improve care through the Faster Cancer Treatment pathways. Pharmac has a role to play and needs to work closely with Te Aho o Te Kahu and Health NZ to ensure New Zealanders are getting the best cancer care across cancer services generally, not just pharmaceuticals.

Recommendations

Pharmac is faced with a hard question of how it manages cancer alongside other conditions it must fund. Cancer medicines are only one type of cancer treatment, and care needs to be taken not to over-invest in pharmaceuticals to the detriment of other cancer services.

The review recommends the Minister:

- agree cancer pharmaceuticals should be considered like other pharmaceuticals. The emphasis needs to be on severity of disease, clinical alternatives and cost for benefit
- note the review considered ring-fenced funding for cancer would lead to prioritising over other conditions
- direct Pharmac and Te Aho o Te Kahu to develop a partnership to enable closer integration with the cancer health sector, with a focus on ensuring equitable access to funded cancer medicines.

Rare disorders

Rare disorders, contrary to their name, are not uncommon, although each disorder itself affects only a small number of people. They are often genetic, meaning they run in families, and people have them from conception. About half of those with a rare disorder are children, and the conditions are usually life-long and debilitating, often resulting in death at a young age. Only a small proportion of rare disorders have a proven effective treatment. For those that do have treatments options, they are typically costly and often do not meet the evidence threshold of common disorder treatments. People with a rare disorder face a disproportionate variety of challenges in dealing with the health system, starting, in many cases, with misdiagnoses and extensive – and sometimes inappropriate – interventions by numerous specialists before arriving at a diagnosis.

The effects on individuals' material and social quality of life (and that of their whānau and carers) are considerable, as a survey of 300 individuals by advocacy group Rare Disorders New Zealand in 2019 documented. It found 75 percent of people had some or a lot of difficulty seeing, hearing or moving; 80 percent suffered a loss in income and 30 percent were unemployed, because of their disorder; 35 percent often felt unhappy and depressed; 31 percent felt unable to overcome their problems; 60 percent felt communication between service providers was poor; 40 percent could not afford the recommended treatment; and 49 percent spent more than two hours a day on disease-related tasks.

Rare disorders also pose a particular equity challenge. In addition to the barriers faced by people diagnosed with rare disorders, including accessing healthcare and medicines, many people find even getting a diagnosis incredibly difficult. Internationally, we know inequitable access to healthcare disproportionately impacts the opportunities for Indigenous populations, people in rural and remote areas, ethnic minorities and those who are economically disadvantaged, to be diagnosed with a rare disorder.

We looked at what other countries do and examined how Pharmac approached assessment and funding rare disorder medicines. We note the absence of a high-level strategy or formal definition for rare disorders, which has wider impacts than just on Pharmac, and we also note the need to make improvements to the way the Pharmac Rare Disorders Advisory Committee works, for example by including other experts and patient voices. We also considered the findings of the Pharmac commissioned evaluation of the rare disorders pilot and that the outstanding recommendations including the need for better horizon scanning of medicines for rare disorders should be implemented. As with the decision-making section we note the need to involve patients in decision-making and to make the process more transparent.

Recommendations

The issue of medicines for rare disorders will become more fraught as the number of new, high-cost medicines to treat these diseases rises. Like other countries, New Zealand cannot fund all medicines and our ability to negotiate lower costs for these newer medicines may be hampered, because of our smaller size. However, if we do want to fund more of these medicines, consideration needs to be given to where in the general appropriation for health this money will come from. There is no easy way forward and so the suggestions we make are a pragmatic extension of what Pharmac currently does.

The review recommends the Minister directs the Ministry to:

- lead the development of a rare disorders strategy to coordinate efforts to address and improve the lives of people with rare disorders. This strategy will need to:
 - agree an official New Zealand definition of rare disorder
 - be a system view and based on a commitment to ensuring more equitable access to appropriate healthcare services from diagnosis through to treatment and other supports
 - consider the challenge of funding medicines for rare disorders, taking into account the increasing scale of the problem and the impact that this will have on health services more generally.

The review recommends the Minister directs Pharmac to:

- fully adopt the recommendations of a Pharmac-commissioned pilot evaluation:
 - Pharmac's rare disorders advisory committee needs to meet frequently enough to undertake and/or consider horizon scanning
 - Pharmac needs to demonstrate it is acting on the recommendation to have in place more regular calls to suppliers seeking applications
- support the chair of the rare disorders advisory committee to ensure the right expertise is invited to provide advice on applications where there is currently no member of the committee covering that specialism. This may mean involving experts from other countries
- involve the lived experience of patients with rare disorders in the decision-making process
- extend the role of the Rare Disorders Advisory Committee to monitor and review pharmaceuticals once funded, to gauge their efficacy. This could be achieved through the development of a register for funded medicines
- become more transparent about the decision on applications for rare disorders, including under exceptional circumstances
- formalise the discretion currently applied within the exceptional circumstances process to minimise barriers to access for rare disorders, including greater clinical oversight.

Vaccines

Pharmac manages the assessment and purchase of vaccines, a role it took over from the Ministry. Pharmac tenders for supply of vaccines on a three-yearly cycle. New vaccines are assessed by Pharmac and compete with pharmaceuticals for funding. We looked at whether Pharmac's approach to procuring vaccines fits with New Zealand's public health priorities. In our view, it does not.

We identified these concerns with vaccine arrangements:

- There is a tension between Pharmac and the Ministry over which agency should set national policy on which vaccines to buy and what their eligibility criteria should be. In the countries we looked at, this function sits, with a central public health agency or Minister, advised by experts. Given vaccines' role in ensuring and protecting public health, there is a strong argument for this function moving to the Ministry (and eventually to the new Interim Public Health Agency, which will develop policy and strategy within the Ministry).
- A memorandum of understanding between the Ministry, the District Health Boards and Pharmac is inadequate and limits the Ministry's ability to influence Pharmac's decisions. It requires the Ministry and Pharmac meet regularly to review, and where necessary update, the document, but no substantive review has ever happened. Neither agency, to our knowledge, has ever used the escalation process for any disagreements or disputes, despite Ministry concerns over some Pharmac decisions.
- The success of the memorandum of understanding depends on a good relationship between the two agencies, but the relationship is fragile, although improving.
- The Ministry is not represented on Pharmac's Immunisation Advisory Committee, having only an observer present.
- Pharmac applies the same decision-making approach to funding vaccines as it does to pharmaceuticals, despite fundamental differences between the two.
- Pharmac appears to give little weight to equity considerations in its decision-making.
- A number of supply chain coordination issues have been raised around vaccine forecasting, supply and demand. These issues were health-system-wide and subject to two substantive recent independent reviews that made recommendations around improving supply chain management and visibility of stocks.
- Accountability for the supply chain is too complex, and no one organisation is responsible for it from start to finish. Roles and responsibilities are not clear.

Recommendations

The review recommends the Minister:

- transition the prioritisation of vaccines and their eligibility criteria to the newly established Interim Public Health Agency
- direct the Interim Public Health Agency to consider equity as part of the processes it adopts
- direct Pharmac to continue to negotiate the price, supply and terms of conditions of supply but not decide which vaccines are listed on the schedule or the eligibility criteria
- transition these new arrangements over a sufficient time period to enable the Interim Public Health Agency to establish the requisite capability
- direct the Ministry, the Interim Public Health Agency, Health NZ and Pharmac to revise the memorandum of understanding to reflect clear roles and functions, including the primacy of the Interim Public Health Agency in ensuring the vaccine schedule is up-to-date and relevant to the health needs of New Zealanders
- allocate responsibility for overseeing the entire vaccine supply chain to Health NZ
- direct Health NZ to undertake detailed policy work to design the system needed to ensure comprehensive, real-time monitoring of vaccines along the supply chain.

Medical devices

Pharmac was given responsibility for managing hospital medical devices in 2012. The rationale, as with vaccines, included that it would be able to negotiate more competitive prices, just as it does for pharmaceuticals. However, the savings have been slower to come and harder to make than Pharmac envisaged. In the past decade, it has put considerable effort into compiling a catalogue of all medical devices used in hospitals and other healthcare settings (for example, district health board supplied equipment for patients to use at home) and negotiating contracts with suppliers for the delivery of these items. These are important tasks, but the scale of work that was needed has meant Pharmac has had to focus more on managing the current approaches than being able to innovate to make savings.

Medical devices span a wide range of equipment – everything from swabs, bandages and surgical gowns through to stents, orthopaedic joint kits and respirators. They are critical to successful healthcare, and there are systems to purchase and warehouse them and distribute them to sites across the health system including outpatient clinics, wards and theatres. A major category of equipment is devices for disability and rehabilitation and the Government intends to provide money for disability support services that will include funding for rehabilitation equipment.

Pharmac has unquestionably done good work in starting a catalogue of medical devices but buying from the catalogue is not compulsory, and hospitals and health services can – and often do – order off their own catalogues. As a result, suppliers are also reluctant to cede control by signing contracts with Pharmac.

Like pharmaceuticals, new medical devices will need to go through assessment to see if they should be publicly funded. Pharmac has not yet completed its work to establish the process for undertaking health technology assessments of medical devices, especially those devices which are both high-cost and high-risk. We think it would be useful for Pharmac to complete this work as part of the wind-down and transition of contracting and cataloguing activities to Health NZ. Whether this health technology assessment work is developed as part of Pharmac's service offering to Health NZ – or whether Health NZ wishes to go in a different direction – is a matter for Health NZ to decide.

The review considers under the reformed health system Pharmac is no longer the most appropriate agency to lead the management of hospital medical devices. It should move to Health NZ, which is responsible for establishing a national approach to managing the supply of medical devices. Pharmac might, however, have a continuing supporting role in this area by conducting health technology assessments of hospital medical devices as required by Health NZ.

Recommendations

The review recommends the Minister:

- transfer cataloguing and contracting medical devices from Pharmac to Health NZ, which is better placed to manage procurement and supply chain for medical devices
- direct that this transition happens at the speed Health NZ determines
- direct Pharmac to work with Health NZ to complete the design of the health technology assessment process
- direct Pharmac and Health NZ to report to the Minister on any ongoing role for Pharmac with medical devices.

Promoting responsible use of pharmaceuticals

The New Zealand Public Health and Disability Act 2000 requires Pharmac to 'promote the responsible use of pharmaceuticals', although it does not elaborate on what this should entail. Pharmac is the only health entity with such a statutory obligation.

According to its own definition of "responsible use" Pharmac currently spends about \$2 million a year on related activities. We examine here whether this work is effective and whether Pharmac is, indeed, the right agency to be performing this work. As with other chapters, we have paid particular attention to questions of equity and whether all parts of our population benefit from medicines in line with health need.

In our interim report, we noted responsible use of pharmaceuticals was closely related to Pharmac's work to ensure equity of access to medicines. These activities are built on strong communication and engagement with both health professionals and the wider community (especially those who are or should be prescribed medicines). However, we heard stakeholders' views that Pharmac was not meeting its obligations in this respect. This contributed to our initial impression that Pharmac could be relying too heavily on its website and social media as communication tools without enough focus on building relationships and working alongside patient groups and special interest clinical groups.

For this report we take a closer look at what Pharmac does to promote responsible use, from information and support to prescribers to targeted use of special authorities, and social marketing to consumers. We also look at the ways Pharmac works, including how it collaborates with partners in the health sector to achieve its equity aims.

Our conclusion in this report is that a broader approach to optimal medicines use, incorporating a strong focus on equity, needs to be led by an agency that has the responsibility of leading, overseeing and co-ordinating professions, providers, and agencies across the health system. This is likely to be a shared function of Health NZ and the Māori Health Authority. Pharmac should concentrate on ensuring equity is a core part of technical assessments, funding decision-making and negotiations.

Recommendations

Most of the recommended action in relation to responsible use can be found in the governance and decision-making sections of the report. The review also recommends the Minister:

- agree Pharmac's role in optimising the use of medicines should focus on ensuring medicines are assessed with an equity approach and undertaking any agreed activities that follow on from the proposed medicines strategy and associated action plans.