

Pharmac Review

Final Report

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 have 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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1 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 health care 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
Pharmac Review
Te Arotake i Te Pātaka Whaioranga

2 Introduction

For Pharmac to be effective and deliver its core objectives, it needs to be far more integrated into the health system as a whole. 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. The evaluation and funding of 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 of the current roles undertaken by Pharmac should be advanced by other agencies, namely what goes on to the vaccination schedule, cataloguing and contracting medical devices, and the leadership of promoting responsible use of pharmaceuticals. The reasoning for the reallocation of those roles is primarily that the health system is changing, and the review has identified other lead agencies that are better placed to advance these functions. This also has the benefit of freeing 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 are also suggesting Pharmac take on an enhanced role relating to the sustainable supply of pharmaceuticals.

Context for the review

Pharmac operates in a challenging space where staff have to 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, which claims success in procuring medicines at lower prices through its negotiations with pharmaceutical companies. The difficulty for the review, and indeed Pharmac, in determining what level of savings is occurring, is that it is not possible, particularly for pharmaceuticals under patent, to confidently determine what the true market price (or net price) of a pharmaceutical is. Global practices favour confidentiality and pricing strategies such as rebates, volume discounts and bundling. Thus, health systems commonly do not know the prices paid by others for individual products. This means there is limited transparency on net prices.

While these issues are less significant for generic pharmaceuticals, and some analyses show New Zealand performing well on price compared to other countries on some generic pharmaceuticals, our work concluded that no meaningful conclusions can be drawn.

For these reasons the review believes claims about the quantum of savings should be treated cautiously. Nevertheless, Pharmac exists to counter the power dynamics that exist in the global market for pharmaceuticals. It is designed to create leverage for New Zealand as an evidence-based buyer in the pharmaceuticals market, where the country only makes up a very small proportion of the overall market. There is little reason to believe that a move away from the existing model would achieve better prices than Pharmac gets. For the reasons set out in this report, 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.

Our interim report reflected an intense period of consultation with a broad range of people and organisations. We met many stakeholders and examined 213 submissions. As well as highlighting the issues we saw and heard about, the interim report made preliminary observations, and indicated areas the review wanted to explore further before making final recommendations.

In this final report we have focused on:

- Pharmac's pharmaceutical investment decision-making processes, which underpin Pharmac's core function to maintain and manage the pharmaceutical schedule and ultimately are crucial to Pharmac achieving its legislative best health outcome objective
- Pharmac's other functions and core activities, including how it promotes the responsible use of medicines, its expanding role as a purchaser of 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
- 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.

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 and activities 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 the review that 27 years before this first review of Pharmac is too long. 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, decision-making processes and equity outcomes – be carried out regularly, for example in alignment with the health strategic plan cycle.

Scope

We are required to make recommendations on two key matters:

- how well Pharmac performs against its objectives and whether and how its performance against these could be improved
- whether those objectives maximise its potential to improve health outcomes for all New Zealanders (in particular, equitable outcomes for Māori and Pacific people) as part of the wider health system, and whether and how these should be changed.

In considering equity, we use the Ministry's definition of health equity: 'People have differences in health that are not only avoidable but unfair and unjust. Equity recognises different people with different levels of advantage require different approaches and resources to get equitable health outcomes.' This is in line with the World Health Organization definition of health equity, which is 'the absence of avoidable, unfair, or remediable differences in funded medicines access among groups of people, whether those groups are defined socially, economically, demographically, or geographically or by other means of stratification'.¹

We examined whether Pharmac's model for assessing and managing the pharmaceutical budget remains suitable (and if not, what should change).

Out of scope

The terms of reference exclude consideration of:

- the commercial arrangements Pharmac negotiates with pharmaceutical companies
- whether Pharmac should remain a separate Crown-owned entity
- the size and fixed nature of its budget
- the day-to-day operations of Pharmac
- the appropriateness of specific decisions made by Pharmac.

¹ https://www.who.int/health-topics/health-equity#tab=tab_1

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, rurality² and age, which showed us how Pharmac's investments were being spent. We have undertaken international comparisons of Australia, Canada and the United Kingdom – both for the approach they take to assessing medicines for public funding and how they utilise 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.

Structure of this report

The report is divided into the following sections:

Operating environment: We looked at Pharmac's reporting on cost savings and tested whether they were correct by looking at how savings are reported overseas. We also looked at where Pharmac was allocating its funds and who was benefiting from this spending.

Governance and accountability: We examined Pharmac's governance and accountability arrangements and their effectiveness in ensuring Pharmac met its objective and fulfilled its various functions. We looked particularly at Pharmac's board, its responsibilities and its performance in overseeing the agency. We considered whether the relevant legislation should emphasise Pharmac's equity obligations as part of achieving its objective and should also require it to work collaboratively with other health sector agencies in doing that.

Decision-making: We reviewed how well Pharmac is performing in applying its analytical decision-making tools and following its own assessment process. We also looked at how this applied in practice to six medicines Pharmac had assessed. We also looked at other elements of a funding decision, including oversight of analysis, voices heard in decision-making and public communication mechanisms. Finally, we considered the appropriateness of all the matters taken into account in a decision, such as the factors for consideration framework, and whether they contributed to achieving equitable outcomes.

Cancer: We examined the nature and prevalence of this disease, and how Pharmac's approach to assessing and funding cancer medicines compared with those taken overseas.

Rare disorders: We examined how Pharmac approached assessment and funding of rare disorder medicines and looked at overseas examples.

Vaccines: We examined when and why Pharmac became the lead agency for purchasing vaccines. We considered whether the current arrangement was working and whether Pharmac should continue in this role.

² Rurality is defined using the geographical Classification for Health (GHC). Whitehead et al 2021.

Medical devices: We examined Pharmac’s role in contracting medical devices, how savings have proved elusive, and how Pharmac is building up a catalogue of all medical devices as part of its work. We considered the rationale for where medical devices should sit in the context of the health reforms.

Responsible use of medicines: We examined Pharmac’s responsibility for promoting the responsible use of medicines and what a coordinated, system-wide approach might look like.

The role of Pharmac in the health and disability sector

Medicines, or pharmaceuticals, are an integral part of health and disability care. Pharmac’s purpose is to ‘secure for eligible people in need of pharmaceuticals, the best health outcomes that are reasonably achievable from pharmaceutical treatment and from within the amount of funding provided’.³ Pharmac assesses medicines through an evidence-based process, considering whether benefits can be demonstrated to a sufficient level that justifies public funding. The assessment requires analysis against scientific, technical, and ‘social utility’ criteria. We consider this process and how it may or may not be achieving Pharmac’s objective later in this report.

Pharmac’s fixed budget forces prioritisation and trade-offs across illnesses and medicines. Pharmac is only able to fund a relatively small number of medicines annually, and the decision to fund a medicine or not has direct impacts, both positive and negative, on individuals, their whānau, and their community. For these reasons alone, Pharmac must adhere to a high standard of professionalism in the transparency of reasoning behind its decisions, the process by which it makes decisions, and how it explains its decisions to the public.

Pharmac undertakes other activities, including providing, in exceptional circumstances, funding for medicines not on the pharmaceutical schedule, research, promotion of responsible use of medicines, along with the already mentioned cataloguing and contracting of medical devices, and the evaluation and funding of vaccines.

³ Section 47 (a) of the New Zealand Public Health and Disability Act 2000.

The role of Pharmac in the health sector reforms

Increases in the size and age of the population, and the burden from chronic disease, are predicted to lead to increased dependence on, and demand for, health and disability services and a commensurate increased demand for pharmaceuticals. Health sector reforms announced in April 2021 (after this review started) are intended to better prepare the system to deliver the health outcomes New Zealanders require. The key elements of the reformed system are:

- strengthening **Manatū Hauora | the Ministry of Health's** role (the Ministry) as chief strategic advisor and kaitiaki (steward) of the health and disability system
- a new **Interim Public Health Agency**, within the Ministry of Health, will be responsible for public health policy, strategy, monitoring and intelligence
- a new Crown entity, **Health New Zealand (Health NZ)**, will replace 20 district health boards to run hospitals and commission primary and community health services
- a new statutory entity, the **Māori Health Authority**, will commission services, develop strategy and policy and monitor performance to improve Māori health.

On 29 October 2021 the Government also announced it was establishing a **Ministry for Disabled People** which would work for better outcomes for disabled people by leading policy for support services across the wider health system and all of government.

The review believes the reform promises a shift towards a system that is characterised by:

- being people-centred to bring together the voices of all communities
- having equity at the forefront of the system, focused on working in partnership with Māori, honouring te Tiriti o Waitangi and the responsibility Pharmac has as a treaty partner
- providing more accessible, equitable, convenient, and integrated services for all New Zealanders, particularly groups typically underserved by the sector, such as Māori, Pasifika, and disabled people
- a cohesive health system that delivers locally, supported by coordinated planning and oversight.

Te Tiriti o Waitangi

We have sought to understand how Pharmac, as a Crown-owned entity, is meeting government and Māori expectations around te Tiriti o Waitangi. For this, we have relied on Cabinet Office guidance on te Tiriti o Waitangi CO(19)5. These guidelines stress the importance of context to interpreting te Tiriti o Waitangi. For Pharmac, the review has tried to understand what Māori and Crown interests are in the work of Pharmac, what Pharmac's contribution is to good government in terms of te Tiriti o Waitangi, how Māori are (and could be) involved in Pharmac's work, and to understand how Māori wellbeing outcomes are achieved and inequities eliminated when it comes to medicines.

In thinking about Pharmac’s role and its connection to the wider health sector, the review has been influenced by the Waitangi Tribunal’s 2019 *Hauora* report, which set out decisions on stage one of the Health Services and Outcomes Kaupapa Inquiry, and the five principles of the Treaty of Waitangi articulated by the Tribunal, set out in the table below. We have also been guided by government strategy documents,⁴ published approaches to te Tiriti o Waitangi analysis⁵ and the extensive work of Māori scholars.⁶ We also observe the Pae Ora (Healthy Futures) Bill, particularly clauses 3 and 7, if passed, will provide Parliament’s direction on how te Tiriti o Waitangi should be applied by Pharmac.

Table 1: The principles of te Tiriti o Waitangi / Treaty of Waitangi, based on the Waitangi Tribunal’s Hauora report (2019)

The guarantee of Tino Rangatiratanga	Māori self-determination and mana motuhake in the design, delivery and monitoring of services.
The principle of equity	Requires the Crown to unequivocally commit to achieving equitable health outcomes for Māori.
The principle of active protection	The Crown should act, to the fullest extent practicable, to achieve equitable health outcomes for Māori and be fully informed of Māori health outcomes and inequities.
The principle of options	The Crown is obliged to ensure that all health services are provided in a culturally appropriate way that recognises and supports the expression of Māori models of care. It also requires the Crown to support Māori health and disability providers to fully participate in service provision.
The principle of partnership	Requires the Crown and Māori to work in partnership in the governance, design, delivery and monitoring of primary health care services.

Throughout our review, we have been grateful for the Māori stakeholders who have shared their views, aware that throughout much of our review period many Māori health professionals and organisations have been at the front line of responding to Covid-19 and protecting Māori whānau. We also reviewed submissions made by Māori health organisations to the 2020 Select Committee Inquiry into Health Inequities for Māori, which included looking at Pharmac’s terms of reference.⁷ We shared some of these views in our interim report and have included more throughout this report.

Overall, it is clear those who are familiar with Pharmac’s work are looking for significantly stronger te Tiriti o Waitangi responses from Pharmac. Specific areas of concern included the lack, until recently, of Māori in leadership and decision-making roles,⁸ the lack of detail in the current *Te Whaioranga – Māori Responsiveness Strategy*,⁹ and a lack of te Tiriti o Waitangi-based partnership with Māori.

⁴ For example, Ministry of Health 2014b; Ministry of Health 2020.
⁵ For example, Baker et al 2021, Came et al 2020.
⁶ For example, Mikaere 2011.
⁷ Māori Affairs Committee. 2020. Inquiry into Health Inequities for Māori. Available online https://www.parliament.nz/resource/en-NZ/SCR_100072/fbcffc6f0b843cb0adcbe1cbbe29fcb45b1f1d48.
⁸ Key informant interviews, July 2021.
⁹ Māori stakeholder hui.

‘We have an Indigenous population in this country that we have a responsibility to, and it requires us to move in and undertake additional unique strategies to enable Māori to have the same level of outcome.’¹⁰

Feedback from stakeholders and our own analysis of Pharmac’s approaches to te Tiriti o Waitangi have informed our recommendations, particularly around accountability, decision-making and optimal use of medicines, which see the need to have greater involvement of Māori in decision-making and the need for monitoring to ensure outcomes for Māori are being achieved.

An overarching theme from Māori stakeholders was the need for Pharmac to be pro-equity and make sure everything it does works towards the overarching aim of equitable outcomes for Māori, which we discuss further below.

Mātauranga Māori

Throughout our engagements with Māori, the importance of mātauranga Māori in ensuring Māori health and wellbeing was emphasised. Closely related to this, we were also told of the critical importance of Kaupapa Māori solutions in health.

Ma te mātauranga Māori ka ora ai te whānau, te hapū, te iwi.
Through Māori knowledge, the family, the sub tribe and the tribe prosper.¹¹

While mātauranga is the domain of Māori whānau, hapū, iwi and Māori communities, Pharmac (and indeed all health sector agencies) must recognise its value and understand its role in ensuring mātauranga Māori thrives. Not only is this what Māori stakeholders have told us, but it is consistent with the health and disability system review and health sector reforms which support mātauranga Māori and Kaupapa Māori solutions as an integral part of achieving hauora.

Given our overall recommendations, 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, but leading work on mātauranga Māori, including rongoā Māori, is something that sits best with the Māori Health Authority. Pharmac must play its role and ensure mātauranga Māori and Kaupapa Māori are incorporated into its processes and analysis.

¹⁰ Key informant interview, November 2021.

¹¹ Health and Disability System Review Final Report, p 29.

How we considered equity

As mentioned above, equity was a central part of our terms of reference. The review adopted the Ministry's definition of equity because it broadly aligns with the guidance from the World Health Organization and, crucially, because it is the definition used by the majority of the health system. A whole-of-system approach to equity is essential and can only be achieved where health sector organisations are working in unison towards the same equity goals.

As outlined in our interim report, we have drawn on a wide variety of academic and other expert sources in New Zealand and overseas, as well as Māori and Pasifika communities and scholars, to give context to what health equity should look like and how inequities arise and can be eliminated.

We note at the outset that different population groups:

- have different degrees of access to the things that help them stay healthy
- have different degrees of exposure to the things that put their health and wellbeing at risk
- have different degrees of access to health care services (including medicines), depending on such factors as location, physical access to services, the availability of services, the cost to patients of receiving care, and eligibility and funding rules
- receive different levels of care, including unjust or unfair differences in the kinds of referrals they receive, and are subject to differences in prescriber behaviour and quality of care.¹²

Throughout our report we have considered how Pharmac's actions and inactions impact on three priority populations in particular: Māori whānau, hapū and iwi; Pasifika; and disabled people. This approach is in keeping with our terms of reference, the direction taken across the health and disability system reforms and is also reflected in the proposed Pae Ora legislation.

These three groups are disproportionately impacted by negative health outcomes and the kind of systemic failings that drive health inequities. Often these inequities are compounding, with racism, ableism and gender discrimination each impacting on many tāngata whaikaha Māori (Māori with lived experience of disability), for example. Where possible, we have sought to understand these intersections, but as indicated in our interim report, the review has come up against data limitations, particularly around disability-related information.

The lack of high-quality health data for disabled people impacts the health system as a whole and has limited the ability for the review to take a more detailed look into, for example, medicine access equity for disabled people. We are also unable to get data on the numbers of disabled people employed by Pharmac or on Pharmac committees because it is not collected routinely. Pharmac is not alone in this regard, but it is a serious concern and something we hope will be addressed by the Pae Ora legislation's commitment to a disability health strategy and through the establishment of the Ministry for Disabled People.¹³

¹² This framework is based on Jones (2001) as quoted in Reid et al *Understanding Health Inequities* 2006, Hauora IV, University of Otago.

¹³ <https://www.odi.govt.nz/whats-happening/a-milestone-in-the-establishment-of-the-new-ministry-for-disabled-people/>.

The review notes the establishment of the Ministry for Disabled People creates an opportunity for Pharmac to engage with, and consider, the needs and concerns of disabled communities in relation to pharmaceuticals. As it will take time for the new Ministry to be in a position to engage with Pharmac, we recommend as a first step the board of Pharmac invite, as the review did, Disabled Persons Organisations (as the UNCRPD mandated representative bodies) and the Disability Rights Commissioner to meet with them and understand what action it could be taking now.

Stakeholders noted Pharmac had little visibility within Pasifika communities. This concern has been further validated in our consideration of Pharmac's performance in delivering its Pacific Responsiveness Plan, where it has not been able to deliver on the actions that would have had a positive impact.

Those with rare disorders are also disproportionately impacted by negative health outcomes and the kinds of systemic failings that drive health inequities. The review considers the wider health system, including Pharmac, needs to demonstrate a good understanding of the challenges people with rare disorders experience and develop an integrated approach to their needs.

To further understand Pharmac's approach to equity and the opportunities to maximise its contribution to improving health outcomes for all New Zealanders (in particular, equitable outcomes for Māori and Pasifika), the review commissioned an expert review of Pharmac's cost-utility analysis modelling approaches in relation to Māori health equity. This looked at how Pharmac's medicine assessments had taken into account and reflected matters relating to equity, identified whether there were any omissions of information that constituted a serious gap in Pharmac's analysis, and provided recommendations on how Māori health equity in cost-utility analysis modelling may be strengthened. This has influenced our findings on decision-making.

While equity findings are woven throughout our report, there are areas we wish to highlight as having particular equity implications:

- In section 4 we consider Pharmac's governance arrangements and ask how the existing arrangements provide an opportunity to enhance Māori and overall New Zealand wellbeing; and how Pharmac's governance is addressing the elimination of inequities and bias both within the organisation and in the work it carries out.
- In section 5 we take a deeper look at Pharmac's decision-making processes and how they are applied, to understand how equity is considered at every stage of decision-making and whether the right expertise and information is included when it comes to pharmaceutical funding decisions.
- In section 6 we look at cancer medicines, including implications for equity.
- In section 7 we consider rare disorders. While small patient numbers can mean people with rare disorders are rendered invisible in cost-benefit analyses, we have applied an equity lens to understand how this population is impacted by pharmaceutical funding decisions. We have also looked at whether and to what extent other jurisdictions have approached rare disorders as an equity issue.
- In section 10 we use an equity lens to look at optimal use of medicines across the health system and Pharmac's role within this.

Applying human rights frameworks

The review has applied an equity focus to every aspect of Pharmac's work. While equity is part of some human rights frameworks, we received a number of submissions seeking more explicit recognition of human-rights-based approaches by Pharmac. In particular, we were directed to consider:

- the United Nations Convention on the Rights of the Child (UNCROC) 1989 (ratified by New Zealand in 1993)
- the United Nations Convention on the Rights of Persons with Disabilities (UNCRPD) 2006 (ratified by New Zealand in 2008)
- the United Nations Declaration on the Rights of Indigenous Peoples (UNDRIP) 2010 (New Zealand is currently developing a declaration plan for consultation in 2022).¹⁴

There was considerable debate on how a human rights framework should apply to Pharmac. This is not surprising since the job of allocating a limited pharmaceutical budget raises complex questions around human rights and the progressive realisation of the right to health.

As we observed in our interim report, Pharmac has tended to make decisions explicitly based on a utilitarian perspective (how to get the greatest impact for the most people from a fixed budget). In its most pure form, utilitarian approaches create tensions with equity frameworks, which require an uneven distribution of resources to avoid unfair or unjust differences in outcomes between people. Human-rights-based frameworks include a focus on equality and non-discrimination.¹⁵

Given that one of our overarching views is the need for Pharmac to be better integrated into the wider health system, we consider it impossible for us to reconcile these tensions and articulate an allocation framework informed by a human-rights-based approach to be driven through Pharmac alone. That framework would need to apply to the whole health system, guiding allocation of funding in all areas of health investment. The role for developing such a framework sits with the Ministry, not Pharmac; rather it would be Pharmac's job (along with other health agencies) to apply this framework.

In coming to this view, the review does, however, recognise Pharmac can take practical steps now that will ultimately improve its decision-making. These improvements are informed by human rights approaches, such as those shared with us by the Human Rights Commissioner, the Disability Commissioner and the Children's Commissioner, and are consistent with current equity commitments. These practical steps broadly equate to improved procedural fairness and include:

- greater use of participatory processes, ensuring a wider range of external voices is heard during decision-making (especially Māori, Pasifika and disabled people)
- broadening the membership of advisory groups, such as the Rare Diseases Advisory Committee, to include consumer/patient representatives when needed

¹⁴ <https://www.tpk.govt.nz/en/whakamahia/un-declaration-on-the-rights-of-indigenous-peoples>.

¹⁵ University of Otago, Bioethics Centre presentation to Pharmac Panel (November 2021).

- collecting statistics on disability to help identify gaps for Pharmac's inclusion of disabled people
- increasing transparency around decision-making, including providing information on decisions in a range of accessible formats.

In deciding on these practical steps, we have also been cognisant of the importance of the Enabling Good Lives principles¹⁶ in achieving the government's future vision for disability supports. These principles further reinforce the need to involve disabled people and families in governance, system and service design and monitoring.

The Human Rights Commissioner shared work on the Guidelines to a Decent Home and suggested the model used to develop the guidelines in partnership with others (in this case the Iwi Chairs Forum and Community Housing Aotearoa), and this may be worth Pharmac exploring as it goes about these practical improvements.

¹⁶ <https://www.enablinggoodlives.co.nz>.

3 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.

In this section, we measure the success of this operating model, including whether Pharmac gets the best prices and achieves the levels of savings it claims. We also look at the future of the agency against the backdrop of significant changes occurring for the wider health system. Finally, we consider the implications of global shifts in the manufacture of medicines and the impact Covid-19 is having on supply chains. We consider Pharmac's role as an expert adviser to the Government on the supply chain should be strengthened.

The primary reasons for a fixed-budget pharmaceutical purchasing function for the New Zealand health system, such as Pharmac, are 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 reform being undertaken, with the need for those reforms to be embedded and for the new agencies to demonstrate their effectiveness. However, in the future it may be worth reassessing Pharmac's role in the health system and whether it should continue as a stand-alone Crown-owned entity.

Global pharmaceutical market trends

Pharmac operates in a global market for pharmaceuticals. The industry is characterised by high innovation and a focus on research and development of new technologies and treatments. New Zealand has very limited domestic production capability and capacity.

As the review highlighted in its interim report, pharmaceutical companies are highly driven to seek public funding for their new products as quickly as possible. That is because new pharmaceuticals enjoy patent protection for a period, creating a time-limited monopoly.

Key trends that Pharmac faces include:

- **Marked increase in development of medicines.** There has been a 27 percent increase in the number of pharmaceutical companies worldwide between 2017 and 2021, and a 25 percent increase in the number of pharmaceuticals under development. There are substantial increases in the number of medicines being produced for cancer and rare diseases. We discuss the challenges this creates in our sections on cancer and rare disorders.
- **Regulatory changes and lower evidence thresholds** mean a lot more pharmaceuticals are available quickly in global markets. This increases expectations that New Zealanders should also have access to these pharmaceuticals. A recent OECD report¹⁷ expressed concern about the sustainability of member countries' health budgets in the face of the growing number of new, high-priced cancer medicines. It noted spending on such medicines had been steadily rising as a result of higher launch prices and increases in the number of patients being treated, which in turn was due to more cancer cases, new treatment options and longer treatment times. To make matters more difficult, some pharmaceuticals (particularly for cancer) arrive with lower levels of international evidence than was previously expected.
- **Precision medicine** involves individualised treatment based on a greater genetic understanding of diseases and environmental variations among patients. This minimises the side effects experienced during broad treatment approaches. To date, precision medicine has mostly been used to treat cancers (accounting for 90 percent of such approved treatments in 2018). Biological pharmaceuticals, as opposed to traditional synthetic chemical pharmaceuticals, are expected to dominate the industry by the end of the decade. They currently account for 43 percent of medicines under development.

The rising number of available medicines is increasing public expectations of access based on global availability. However, for Pharmac, the lower evidence thresholds, increasing prices and uncertainty around the fairness of deals, and personalised medicines combine to create continual and growing funding pressures. These factors combined make it increasingly difficult for Pharmac to deliver the purchasing impact that it has done historically.

Supply disruptions

Maintaining supply is particularly important for certain classes of medicines. New Zealand is vulnerable to international changes in demand and supply of medicines. These changes can be difficult to predict, and may arise because of manufacturing shortages, increases in demand because of an epidemic, natural disasters, or changes in regulatory rules in other countries.¹⁸ Supply disruptions have come to the fore with Covid-19.

¹⁷ OECD. 2020. <https://www.oecd.org/health/health-systems/Addressing-Challenges-in-Access-to-Oncology-Medicines-Analytical-Report.pdf>.

¹⁸ <https://pharmac.govt.nz/medicine-funding-and-supply/medicine-supply-management/managing-medicine-supply/>.

Pharmac manages supply risks through its contracts with suppliers. Most contracts require suppliers to keep a minimum of two months' stock in New Zealand. This supplier stock is in addition to the four–six weeks' stock held by the combination of wholesalers and pharmacies. Pharmac, through its contract management function, monitors supply. Suppliers must notify Pharmac of any possible shortages. Pharmac can support suppliers to source alternative supply and manage stocks by using levers such as dispensing restrictions to buy time to secure additional stock. However, additional costs in sourcing an alternative supply are borne by the supplier. Pharmac can choose to charge suppliers additional costs incurred by DHBs.

New Zealand's small market and sole supply tendering create single supplier dependency for many products. This has the potential to increase the risk of stock outages and exposure to supply chain disruption, but equally it can improve security of supply by providing greater certainty to suppliers, enabling better stock management.

Disruption caused by supply issues

During the early stages of the Covid-19 pandemic there was major disruption to international medicine supply chains, including (but not limited to) contraceptive pills, hormone replacement therapy, inhalers for asthma and chronic obstructive pulmonary disease, and blood pressure medications. Pharmac restricted the quantities that could be dispensed to patients, suppliers worked to secure supply and pharmacies managed medicine switches with patients. This was an extreme case, but New Zealand was able to come through that disruption without significant impact on access to critical medicines.

However, when pharmacies are directed to limit dispensing (eg, once weekly), people need to make changes in their lives and face increased costs to continue access, placing a disproportionate burden on those already disadvantaged – such as disabled people, Māori, Pasifika, and rural populations. The presence of Covid-19 is expected to continue to put pressure on the supply chain and in turn on those already disadvantaged.

Pre Covid-19, stock outages and potential out-of-stock events that impacted on patient supply were uncommon but disruptive when they occurred. In 2015 and 2016 atorvastatin (a cholesterol-lowering medicine) and metoprolol succinate (blood pressure) were out of stock, causing disruption for prescribers and pharmacies and affecting hundreds of thousands of patients.

Pharmac does not collect data on the number of stock outages that result in disruption to patient supply. However, indemnities sought from pharmaceutical suppliers to recompense for additional costs to DHBs is an indication of the frequency of more serious outage. Pharmac can choose to charge indemnities to recover additional dispensing costs (as a result of more frequent dispensing and medicines changes) during the period of stock disruption. There have been 26 indemnities sought from suppliers over the six years.

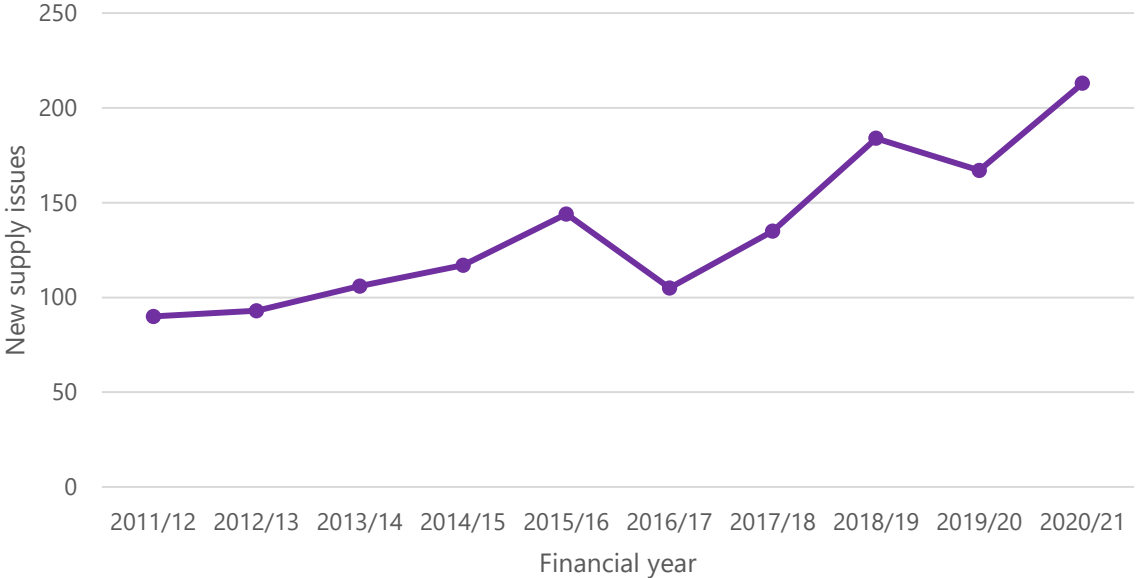
Table 2: Indemnities sought from pharmaceutical companies for out-of-stock/supply issues

Financial year	2014/15	2015/16	2016/17	2017/18	2018/19	2019/20
Indemnities sought	8	1	3	7	1	6

Source: Pharmac OIA¹⁹

Most supply issues, however, are managed without any impact on patients or disruption to the supply chain. Before Covid-19, there were anywhere between 90 and 180 supply issues dealt with each year.

Figure 1: Number of new supply issues by financial year



Source: Pharmac

Pharmac told the review these increases were partly due to better data collected on stock issues. Until recently anything resolved within a week was not recorded.

The issues faced in New Zealand are not unique. In 2016 the World Health Organization recognised increasing global shortages and stock outages of medicines and vaccines.²⁰ In technical documentation on preventing and managing global stockouts published by the WHO,²¹ a range of issues were identified. We highlight two that are relevant to the New Zealand context:

¹⁹ <https://pharmac.govt.nz/news-and-resources/official-information-act/official-information-act-responses/indemnities-sought-from-pharmaceutical-companies-for-out-of-stocksupply-issues/>.

²⁰ https://www.who.int/medicines/areas/access/Meeting_report_October_Shortages.pdf.

²¹ https://www.who.int/medicines/areas/access/Medicines_Shortages.pdf?ua=1.

- Ensure prices paid are sufficient to promote quality products and help to reward the guarantee of a continuous supply.
- Different tender practices can have a significant impact on product availability. For example, finding the balance between short, flexible tenders versus longer-term contract periods or multi-party tenders versus single-source tenders is important in all procurement environments.

The risks of stock disruption are greatest at the start and end of a contract as a supplier is running down stock and another ramping up to supply the market. Pharmac and suppliers work together to minimise the risks during a transition period. Ultimately though, if too much risk is placed on suppliers, they may withdraw product lines or struggle to cover the costs of potential out-of-stock events. In the medium to long term this can impact on competition in the market, affecting prices.

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.

Pharmac needs to manage risk on both sides. By introducing sole supply, it introduces a supply risk it has to manage. It is the review's opinion these arrangements are favourable for securing better pricing, but this needs to be balanced against patient impacts and security of supply. 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.

Enhanced role for Pharmac in supply chain management

The review is conscious New Zealand has a degree of vulnerability in respect of pharmaceutical supply. These include:

- We are a tiny purchaser by global standards.
- We are largely reliant on international manufacturing.
- We are a geographically isolated country, which adds to transactional costs and logistic challenges in getting supply here.

Pharmac tends to favour long-term arrangements, resulting in many 'aged' pharmaceuticals on the schedule. While there are benefits to long-term arrangements, there is also an increased risk to security of supply over time if the international manufacturing volume reduces with reduced international demand.

New Zealand needs to invest in security-of-supply expertise in pharmaceuticals. One of the strong benefits of Pharmac is its expertise on a range of matters relating to pharmaceuticals. As part of its procurement role Pharmac not only already needs to take into account security-of-supply issues, but also has, and needs to keep developing:

- strong intelligence on international practices and trends in the development, manufacturing and supply of pharmaceuticals
- strong working relationships (including information flows) with the domestic wholesalers who play a critical role in the distribution of pharmaceuticals to the front line.

The review thinks there is a case for an enhanced role as an advisor to the Government on supply chain risks. By having it as a separate function of Pharmac, the board would as part of its enhanced role provide confirmation to the Government that it has the right balance between securing better pricing and security of supply (including patient impacts from outages). Our recommendation to make this a specific function provides an indication of the importance of this role.

Pharmac’s ‘new investment’ profile

Pharmac has invested in 384 products over the past decade, including taking budget responsibility for haemophilia products and vaccines. New investments consist of new listings (179), widening access to existing medicines (188) and transfers of budget responsibility (17).

Table 3: Number of new investments made between 1 July 2010 and 30 June 2020

Schedule	New listings	Access widenings	Transferred functions	Total
Community and Hospital Schedule	173	179	0	352
Haemophilia products	2	0	5	7
Vaccines	4	9	12	25
Total	179	188	17	384

Source: Pharmac

The review looked at high-level trends in pharmaceutical expenditure over a decade by reviewing extracts from the pharmaceutical data warehouse taken for the 2009/10, 2014/15 and 2019/20 financial years. This analysis was first undertaken on volumes of pharmaceuticals dispensed, looking at ethnicity, rurality²² and age. Subsequently this data was given to Pharmac, which then attached expenditure, net of rebates. To avoid disclosure of the rebates, Pharmac grouped classes of pharmaceuticals and gave different views of the data so that pharmaceutical supplier confidentiality was preserved. The data is a snapshot in time and measures expenditure in the most recent financial year where information is available on all new listings made over the past 10 years. Investments may be at different stages of maturity and might still be growing in use.

²² Rurality is defined using the geographical Classification for Health (GHC). Whitehead et al 2021.

In the 2019/20 budget for pharmaceuticals, one in five dollars was spent on new listings made over the past decade (\$220 million in expenditure, or 21 percent of 2019/20's pharmaceutical budget of \$1,040 million). Thirty-two percent (\$70 million) was spent on pharmaceuticals prescribed to people with cancer.

Table 4: Expenditure (net of rebates) in 2019/20 on new listings since 1 July 2010 by therapeutic group

Therapeutic group	New listings ²³	Patients ²⁴	Expenditure (\$ millions)	Percent of total expenditure
Cancer treatments ²⁵	25	6,713	\$70	32
Nervous system	20	236,785	\$47	21
Blood and blood forming organs	8	81,118	\$28	13
Respiratory system and allergies	13	85,342	\$21	9
Musculoskeletal system	11	237,855	\$6	3
All other groups	77	471,587	\$48	22
Total²⁶	154	926,449	\$220	100

Source: Sapere analysis with aggregate rebate information provided by Pharmac

The review was not able to look at expansion of eligibility to pharmaceuticals that were already listed. It was too difficult to tell what was newly eligible versus what was already eligible. This deficiency in the analysis means that levels of new investment are understated but we don't know by how much.

New investment across populations

The review examined pharmaceutical spending patterns across population subgroups. We looked at expenditure in total and on a per capita basis to understand which populations Pharmac has invested in over the past 10 years. We observed for the \$220 million spent on new listings in 2019/20:

- 46 percent (\$100 million) was spent on pharmaceuticals consumed by those 65 years or older.
- Expenditure on pharmaceuticals prescribed per capita is less for Māori and Pasifika populations than non-Māori/non-Pasifika (\$35, \$31 and \$47 respectively).

²³ Excludes 19 new listings where usage data was not available.

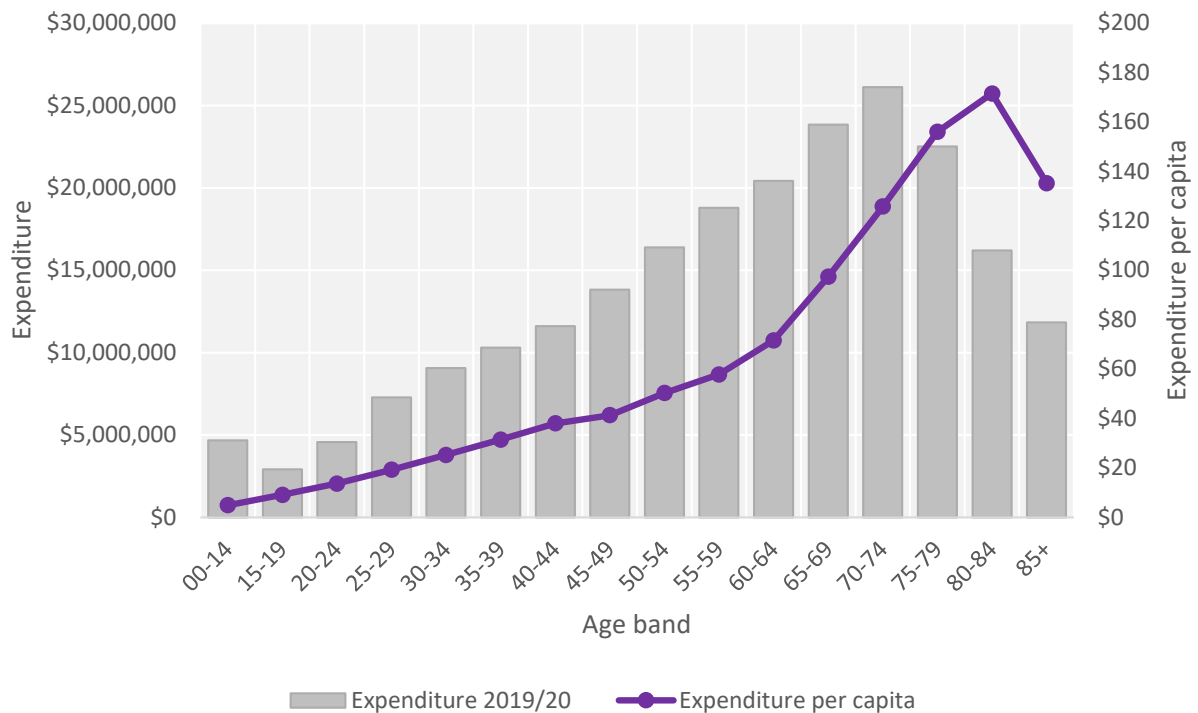
²⁴ Number of patients with at least one dispensing of a new listing in the 2019/20 financial year.

²⁵ There is no therapeutic group for cancer treatments. The category 'Cancer treatments' is a bespoke classification based on Pharmac's description of the conditions new listings are used to treat.

²⁶ Total patients does not equal the sum of the patients column because some people will appear under multiple therapeutic groups.

- Expenditure on pharmaceuticals prescribed per capita was \$48 for males compared to \$39 for females.
- There was no difference between per capita expenditure in rural and urban areas.²⁷

Figure 2: Expenditure in 2019/20 on new listings since 1 July 2010



Source: Sapere analysis, using net drug cost provided by Pharmac

Age standardisation was used to understand how much of the higher non-Māori per capita expenditure was driven by differences in the age structures of the non-Māori and Māori populations. When age standardised,²⁸ per capita expenditure for Māori is higher than for non-Māori. This suggests expenditure is strongly patterned by age with non-Māori receiving greater funding as a result of their older population age structure. Once the impact of age patterning is removed by standardisation, the per capita expenditure becomes more similar between Māori and non-Māori. It is worthwhile noting that, regardless of the effects of age-standardisation, the actual expenditure (ie, per capita) was higher for non-Māori than Māori. No comment can be made as to whether the actual Māori expenditure per capita is commensurate with the greater burden of disease and health needs experienced by the Māori population.

²⁷ Rurality is defined using the geographical Classification for Health (GHC). Whitehead et al 2021.

²⁸ Age standardised using the 2001 census Māori population as the standard population.

Table 5: Per capita expenditure in 2019/20 of new listings since 1 July 2010 by prioritised ethnicity, rurality, sex

Sub population	Expenditure per capita	Age standardised expenditure per capita
Prioritised ethnicity		
Māori	\$35	\$28
non-Māori	\$46	\$22
Rural / Urban		
Rural (R1–3)	\$43	\$19
Urban (U1,2)	\$43	\$23
Sex		
Male	\$48	\$25
Female	\$39	\$21

Source: Sapere analysis, using net drug cost provided by Pharmac

The review is conscious expenditure is not the same as benefit to patients. Higher expenditure does not necessarily mean equally greater benefit. Also, we have not compared expenditure, or use, to health need, which would be a particularly complicated analysis. Despite this, the conclusion we draw from this descriptive piece is that funding Pharmac has invested in new medicines (at least for new listings), has not been equitably distributed over the last decade. In particular, it has benefited older populations and as a consequence of population structure, and a lack of pro-equity strategies, has meant less has been spent per capita on Māori populations.

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.

Future investments

To date, Pharmac's ability to reduce prices on medicines it funds has been effective at freeing up funds for new investments. We are less sure of the future. From what we can see, and from what Pharmac has told us, funding will be tight for the next three to five years. Funding all proposals on Pharmac's options for investment list is estimated to cost in excess of \$400 million per annum. Pharmac's own assessment is that the bottom third of the list has an average cost per quality-adjusted life year (QALY)²⁹ of \$250,000 which in health sector terms is poor value for money but the top third has an average cost per of \$8,850. This top third represents very good value for money and could be better value for money than a number of medicines already listed on the pharmaceutical schedule.

Pricing performance

The review explored whether Pharmac saves as much as it claims. The answer is 'no'. We conclude Pharmac statements on savings are overly optimistic, but even acknowledging this, we think Pharmac's model has delivered significant benefits over New Zealand's historical purchasing model.

Pharmac reports savings of \$6 billion in the quarter century from 1993 to 2018.³⁰ In 2019/20, Pharmac reported it saved New Zealand \$87.4 million that was reinvested into more medicines.³¹ These figures are significant and are an accurate picture of savings relative to historical prices but are not an accurate indication on where Pharmac adds value as they do not consider the counterfactual (what would happen if an entity like Pharmac did not exist). The savings ignore the typical medicine life cycle – patents expire, and generic entry brings about lower prices, regardless of the approach taken by the purchaser. A significant proportion of Pharmac's savings come as the life cycle of a medicine progresses, with Pharmac leveraging generic price competition.

A more useful measure would be an estimate of how much faster New Zealand made savings and how much larger they were as a result of Pharmac's commercial strategies. Requests for proposals and the annual tender processes leverage this competition with the offer of exclusive supply of the subsidised market. This strategy has been very effective at reducing prices. In some instances, pharmaceutical companies offer savings earlier than patent expiry to prevent a medicine being tendered.

Pharmac uses a range of procurement approaches depending on whether there is competition in the market and the stage the medicine is at in its commercial life cycle. Patent pharmaceutical prices are opaque.

²⁹ "A measure of the state of health of a person or group in which the benefits, in terms of length of life, are adjusted to reflect the quality of life. One quality-adjusted life year (QALY) is equal to 1 year of life in perfect health. QALYs are calculated by estimating the years of life remaining for a patient following a particular treatment or intervention and weighting each year with a quality-of-life score (on a 0 to 1 scale). It is often measured in terms of the person's ability to carry out the activities of daily life, and freedom from pain and mental disturbance." <https://www.nice.org.uk/glossary?letter=q>.

³⁰ Pharmac 2018: <https://pharmac.govt.nz/assets/pharmac-25-year-history.pdf>.

³¹ Pharmac 2020: <https://pharmac.govt.nz/assets/Uploads/2020-Year-in-Review.pdf>.

There are two types of prices for pharmaceuticals both in New Zealand and internationally:

- **List prices:** published on the formal schedules such as New Zealand's pharmaceutical schedule. List prices are the public price a manufacturer asks and are the maximum price at which a product can be sold.
- **Net prices:** the actual price paid by Pharmac³² after any discounts or rebates paid to Pharmac by the supplier.

Comparisons are only able to be performed on list prices, as net prices are kept confidential. This means it is difficult to compare costs of medicines across countries. A study of available research using actual prices found only 33 eligible papers from 2007 to 2017 that used actual prices, and of these, only five related to cross-country price comparisons.³³

A comparison of list prices for 16 medications in New Zealand and 16 European countries found that New Zealand's list prices for each type of medication did not deviate significantly from the range seen across the European comparators, even for some countries with significantly larger populations than New Zealand.³⁴ This implies that, at least on the surface, New Zealand is able to achieve reasonable prices despite its relatively small population size and distance from pharmaceutical manufacturing facilities.

The confidential nature of pricing for innovative medicines makes price comparisons very difficult. This challenge is faced by all public purchasers of medications. Some agencies report not being permitted to reveal the size of rebates within their own governments and this is what the review has experienced with Pharmac.³⁵ When it comes to procurement of medications, health systems commonly do not know the price paid by others for individual products due to confidential rebates or discounts.

Price comparison for generics

Pharmac's tender programme for generics is open and transparent. Pharmac's annual tender process requires suppliers to submit tenders where the list price is the true price paid. There is widespread availability of pricing data. Table 6 below summarises the list prices for the top ten medicines by prescription volume for New Zealand, and the price of those same medicines in Australia, Canada, and England (noting prescription volumes vary in each country).

³² In the case of pharmaceuticals, the funder is the district health boards. Pharmac manages the pharmaceutical spend on behalf of the 20 DHBs.

³³ Mardetko et al 2019: <https://www.tandfonline.com/doi/abs/10.1080/14737167.2019.1552137>.

³⁴ Vogler et al 2015: <https://pubmed.ncbi.nlm.nih.gov/26091603/>.

³⁵ See Morgan et al 2016. https://www.commonwealthfund.org/sites/default/files/2018-09/Steven%20Morgan%2C%20PhD_Ten%20Country%20Pharma%20Policy%20Summaries_2016%20Vancouver%20Group%20Meeting.pdf.

Table 6: Comparison of list prices for the top 10 medications by prescription volume

Rank	Medicine	Formulation	Price per 100 (NZD equivalent) ³⁶				NZ % deviation from median price
			New Zealand	Australia	Canada	England	
1	Paracetamol	500 mg tab	2.48	1.60	1.31	4.30	+55.3
2	Atorvastatin	10 mg tab	1.23	4.94	15.36	1.43	-75.1
3	Omeprazole	20 mg cap	2.07	14.33	20.16	3.67	-85.6
4	Aspirin	100 mg tab	1.09	1.59	4.67	<u>0.81</u>	-31.5
5	Amoxicillin	250 mg cap	4.50	7.15	5.92	4.08	-24.0
6	Ibuprofen	200 mg tab	2.14	<u>2.61</u>	4.50	2.28	-18.0
7	Metoprolol succinate	23.75 mg tab	4.83	40.91	N/A	N/A	-88.2
8	Salbutamol	Aerosol inhaler, 100 mcg per dose	380.00	406.97	440.75	277.99	-6.6
9	Cilazapril	2.5 mg tab	5.33	N/A	15.82	N/A	-66.3
10	Cholecalciferol (as alendronate 70 mg + colecalciferol 5,600 units)	1.25 mg cap	37.75	166.18	107.32	93.66	-64.8

Source: Sapere analysis

New Zealand has significantly lower prices than the median for all medicines except paracetamol and salbutamol. Without further analysis we cannot say why but we expect medicines favoured in New Zealand may not be the preferred treatment in other countries. For example, the use of cilazapril has largely been phased out in favour of other ACE inhibitors such as perindopril in Australia and England so there may not be the same motivation to secure lower prices in those countries. Despite this limitation, New Zealand does achieve good prices on its top 10 by volume.

So, does Pharmac do well on pricing? Some analyses show New Zealand performing well on price compared to other countries, though no meaningful comparisons can be made across all medicines.

³⁶ Underlined prices differed in formulation by mass but were scaled up for analysis as they were deemed comparable. Prices marked N/A did not have an equivalent formulation to the medication identified in the Pharmac schedule.

Factors determining pricing effectiveness

In forming its view on Pharmac's pricing performance, the review had to step back from the detail of the price comparisons and, rather, has looked at the factors that may indicate advantage to New Zealand's health sector from Pharmac.

There are areas of advantage that have been earned through their hard work over the years. Pharmac can negotiate prices because:

- there is no other channel to public funding than through Pharmac, strengthening the point of control that the health sector has on pharmaceuticals
- the fixed budget makes it clear what can be spent on pharmaceuticals. This strengthens Pharmac's position in negotiations and provides clear motivation for it to seek the best prices it can, as well as providing accountability that it does so
- Pharmac's systematic approach to investment means all opportunities are weighed against others (though there is a need to address deficiencies, which we identify in the decision-making section). There is an explicit list and ranking of investment preference
- Pharmac is independent of ministers and is not swayed by campaigns or political pressure
- commercial negotiation including multi-medicine deals may mean prices can fall faster even though still on patent
- tenders for off-patent medications generate competitive pressure and achieve lower prices.

Pharmac also faces disadvantages, and these would be broadly true for any New Zealand medicines-buying agency. Being both small and distant means Pharmac:

- has smaller purchase volumes, which limits volume discounts compared to large countries
- freight costs are high in supply of generics
- is unlikely to create greater leverage using cross-industry deals, such as those seen in countries like the UK.

Pharmac exists to counter the power dynamics that exist in the global market for pharmaceuticals. It is designed to create leverage for New Zealand as a buyer in the pharmaceuticals market, where the country only makes up a very small proportion of the overall market. There is little reason to believe that a move away from the existing model would achieve better prices than Pharmac gets.

An indication of the effectiveness of Pharmac's performance is the strong campaign by big pharma launched against the Pharmac model during the Trans Pacific Partnership (TPP) negotiations. The wider adoption of New Zealand's patent protection laws and the Pharmac model was an obvious concern for global pharmaceutical companies.

4 Governance and accountability

In this section, we examine Pharmac's governance and accountability arrangements and their effectiveness in ensuring Pharmac meets its objective and fulfils its various functions. We look particularly at Pharmac's board, its responsibilities and its performance in overseeing Pharmac. We also look at legislation to reform the health sector and Pharmac's place within the new framework. This section also contains a brief discussion of Pharmac's research and communications activities, its clinical committees and its involvement in responding to Covid-19.

In short, we consider there is a need to change the legislation to make equity requirements more explicit in Pharmac's objectives. We consider some of Pharmac's functions would be better performed by other agencies, but that it should have a specific function relating to supply chain oversight enhancing the importance of the work it already undertakes in this area. We consider 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. Pharmac's new performance measures need reasoned analysis and concrete targets. And lastly, we address the importance of a revised medicines strategy to provide a framework for Pharmac to work within.

Background

Pharmac was set up in 1993 as a joint venture owned by the then Regional Health Authorities, and later became a Crown-owned entity in 2001. Crown agents, such as Pharmac, operate at arm's length from core government. The Minister of Health is accountable for money allocated to Pharmac, but operational decisions are left to the board and its delegated authorities.

Pharmac's primary legislative objectives are detailed in the New Zealand Public Health and Disability Act 2020. It also has obligations under the Crown Entities Act 2004 and the Public Services Act 2020. The Public Services Act was introduced to ensure New Zealand had modern, more joined-up and more citizen-focused public services.

Pharmac is governed by a board appointed by the Minister. The role of the board and expectations of the organisation are set by the Minister of Health in both the appointment letters the chair and the board members receive, as well the annual letters of expectations.

Any consideration of whether Pharmac should continue as a Crown-owned entity is outside the review's Terms of Reference and with the scale of change in the health system, the review can see why that is the case. However, this review considers that it is critical Pharmac operates in a more integrated and collaborative way with the health system to deliver the best health outcomes for New Zealand.

Importantly this will require more active oversight by the Pharmac board and in turn by the Ministry. The board needs to ensure concepts such as ‘independence’ and ‘commercial sensitivity’ are appropriately weighted against the corresponding need for decision-making that is grounded in the context of the broader health system. The review does not believe the Crown-owned entity structure, or the important realities of commercial negotiations, prevent a greater level of collaboration and integration. But without clear governance oversight, these concepts risk becoming overstated and impinging unnecessarily on the way Pharmac operates within the system and more broadly with stakeholders.

We also believe the distance the existing structure has put between Pharmac and the rest of the health and disability system has led to the Ministry not being able to be fully effective in its monitoring or to drive integrated system performance.

Pharmac has noted to us that the fragmented nature of the health system to date creates difficulties for effective collaboration. These difficulties are being addressed by the Pae Ora (Healthy Futures) Bill, through the emphasis on a *health system* approach, the significant consolidation of the number of health agencies, new *health system* guiding objectives and principles, and new frameworks (strategies and annual plans) to ensure the key participants in the health system are operating in a more cohesive manner. For Pharmac the task is clear – to operate collaboratively and effectively within the new health system.

Health reform legislation

The Pae Ora (Healthy Futures) Bill, introduced in October 2021, sets out the proposed legislative framework for the new health sector and is scheduled to come into effect in July 2022.³⁷ It will replace the New Zealand Health and Disability Act 2000 in its entirety. As currently drafted, the Bill replicates Pharmac’s existing legislative framework, which is understandable, given it was drafted before the release of this report. We consider the framework should be amended so the Bill’s intent and Pharmac’s role are in alignment.

The Bill strongly emphasises a whole-of-system approach to health. Also, one of its purposes is to achieve equity by reducing health disparities among population groups, in particular Māori.³⁸ This whole-of-system approach and equity purpose both need to be reflected in Pharmac’s purpose. In our view, Pharmac’s objective should state that, in securing the best health outcomes for those needing pharmaceuticals, it should secure equitable health outcomes for Māori and other at-risk population groups and work collaboratively with the Ministry Health NZ and the Māori Health Authority. Both points are elaborated on next.

³⁷ <https://www.futureofhealth.govt.nz/assets/Uploads/Publications/factsheet-pae-ora-bill-oct2021.pdf>.

³⁸ The other two objectives are the protection, promotion and improvement of the health of all New Zealanders, and building towards Pae ora (healthy futures) for all New Zealanders.

Make express reference to securing equitable health outcomes

We do not think the current legislative objectives prevent Pharmac from addressing equity objectives. However, the review is concerned how equity has been addressed by Pharmac. In future Pharmac will be required to interpret its objectives from the legislative text and in the light of the purpose and context of the Pae Ora (Healthy Futures) Bill, once enacted, which provides clear direction on the priority being given to equity.

However, the review feels a stronger link to the overall purpose and principles clause in the Pae Ora (Healthy Futures) Bill is needed. This could be achieved by adding to the Bill a new subsection 61(3): 'In this section, securing best health outcomes includes securing equitable health outcomes for Māori and other populations.' Creating an express link to the purpose and principles of the Pae Ora (Healthy Futures) Bill will also assist stakeholder confidence in Pharmac's legislative framework.

Require Pharmac to work more closely within the new health system

The importance of Pharmac working collaboratively as part of a more integrated health system was raised in our interim report and is re-examined in this report. Pharmaceutical funding decisions aimed at achieving the best equitable health outcomes cannot be made in isolation from the wider health system. And yet the Pae Ora (Healthy Futures) Bill is silent on Pharmac's responsibilities to the new health system – in contrast to new objectives for Health NZ and the Māori Health Authority, which expressly require them to carry out their roles in accordance with the health system's principles, and also to work collaboratively with other agencies to address the determinants of health.³⁹

In our view, Pharmac's objective should be amended to reflect the Bill's intent that agencies adopt a health system approach to their work, guided by the system's new principles. One way to do this could be by adding a new subsection 61(4) that states: 'Pharmac must, in securing the best health outcomes, work collaboratively with the Ministry, Health NZ and the Māori Health Authority, and also in accordance with the health system principles.'

Additionally, the review considers the Bill should not exempt Pharmac from the health system principles, in particular 7(1)(b) regarding engagement with Māori and other population groups and 7(1)(c) regarding decision-making by Māori on matters of importance to them. No other health sector agency is exempt, and we can see no reason why Pharmac should be. The review notes Pharmac itself does not wish to be exempt, and it has written to the Select Committee seeking removal of the exemption.⁴⁰

³⁹ See clause 13 and 18 respectively of the Pae Ora (Healthy Futures) Bill.

⁴⁰ https://www.parliament.nz/resource/enNZ/53SCPOL_EVI_116317_POL2267/d95469ecdcc39db97c10b895e9c1f2ea39419864.

Amendments to Pharmac's functions

Pharmac has a legislative function to promote responsible use of pharmaceuticals.⁴¹ However, to achieve this requires a whole of health system response. The review believes this function should be led by an agency that has, as part of its core role, the responsibility of leading, overseeing and coordinating professions and agencies across the health system. This should be Health NZ and the Māori Health Authority and the legislative function for responsible use should be transferred within the legislation to them. We outline the reasons for this in our section on responsible use. Transferring the function does not change the need for Pharmac to work closely with the health sector on a broad range of pharmaceutical matters, which may, from time to time, include providing information and advice on the optimal use of pharmaceuticals.

Finally, we think there should be an enhanced role for Pharmac as the lead agency around security of supply for pharmaceuticals. To achieve this a new function should be added into clause 61, along the following lines:

‘to keep under review practises and trends in relation to the supply of pharmaceuticals and provide advice to the Minister on matters relating to this’.

Later in this report we also propose to move Pharmac's functions for medical devices and vaccines to other agencies. This does not require a change to the legislation as they have to date been undertaken through written notice from the Minister of Health.⁴²

The primary reason is that the proposed new lead agencies are better placed to advance these functions in the reformed health system. This also has the advantage of freeing up Pharmac to focus more closely on its core role in the assessment and evaluation, purchase, and sustainable supply of pharmaceuticals.

Board's legislative responsibilities

The board's core roles include:

- ensuring Pharmac acts in a manner consistent with its objectives and functions⁴³
- that it performs those functions efficiently and effectively⁴⁴
- in collaboration with other public entities where practicable,⁴⁵ and
- in a manner consistent with the spirit of service to the public.⁴⁶

⁴¹ See section 48(1)(d) New Zealand Public Health and Disability Act 2000.

⁴² See section 48(1)(e) New Zealand Public Health and Disability Act 2000.

⁴³ See section 49 of the Crown Entities Act 2004.

⁴⁴ See section 50(a) of the Crown Entities Act 2004.

⁴⁵ See section 50(c) of the Crown Entities Act 2004.

⁴⁶ See section 50(b) of the Crown Entities Act 2004.

Pharmac's board governance manual, which is drawn from sources including the Crown Entities Act 2004, contains an overview of the board's statutory duties and sets out the governance principles that should guide its performance and conduct. Pharmac management is reviewing the manual and expects to submit a revised version to the board soon.

The board has a responsibility to make and oversee decisions about which medicines should go on the pharmaceutical schedule and receive funding (as well as which medicines already on the schedule should have their use extended). In this, it is supported by the Pharmacology and Therapeutics Advisory Committee, or PTAC, and its specialist advisory committees. The Consumer Advisory Committee, or CAC, which was created under the same section in the legislation, has been excluded from input to this process. Section 5 examines in detail how this decision-making process happens and who is involved in it. There, we also outline our concerns about the quality, transparency and oversight of this decision-making, and how these decisions and certain operational matters are working against Pharmac's stated ambition of making access to, and use of, medicines more equitable.

Delegations

The Crown Entities Act 2004 allows the board to make delegations (and sub-delegations). It can delegate matters to individual board members, employees, office holders and members of Pharmac's advisory committees, crown-owned entity subsidiaries and other people or classes of people approved by the Minister of Health. The board remains legally responsible for all delegated functions and powers.⁴⁷ Pharmac's delegations policy determines delegations by the financial value, risk and level of contention of the matters in question. For funding decisions relating to the pharmaceutical schedule, the board considers only decisions involving expenditure of at least \$10 million over a five-year period, or decisions that are contentious regardless of financial impact.⁴⁸

The review's principal concern in this area, is not about the level of financial delegation per se, but rather how funding decisions are overseen by the board. In the 12 months to 30 June 2021, Pharmac made 32 funding decisions – 13 to fund new medicines and 19 to widen the use of existing medicines. Of these, the chief executive or one of her delegates made 29 decisions, and the board made three.⁴⁹ In the same 12-month period, Pharmac made decisions to switch the brands of 26 medicines. The chief executive or one of her delegates made all 26 decisions.

⁴⁷ See sections 73–76 of the Crown Entities Act.

⁴⁸ Source: Delegations policy and information from Pharmac.

⁴⁹ The board delegated two decisions to the chief executive because a decision was required before the board's next meeting.

Pharmac's investment decisions about which medicines to fund are core to its primary function of maintaining and managing the pharmaceutical schedule. These decisions ultimately determine whether and how it meets its strategic goal of achieving the best health outcomes for everyone, including improving equitable health outcomes for Māori. We note stakeholders have an understandably high level of interest in these decisions, significantly impacting patients' lives and their wider whānau. Given this, we would have expected to see greater oversight by the board.

The review thinks the board should have better processes (and documentation which guides these processes) that demonstrate it is satisfied the delegations are being exercised in an appropriate manner and that overall the functions and strategic goals of Pharmac are being met. Our concerns with the current investment decision-making process and the importance of enhanced board oversight are outlined in more detail in section 5 on decision-making.

Advisory committees' role in governance

Pharmac's two advisory committees (PTAC and CAC) and 21 specialist advisory committees provide advice to the board to help it perform its various functions including clinical and consumer advice. We described the role of these committees, including the changes that were planned to their terms of reference, in the interim report.⁵⁰

Members of PTAC are appointed by the Director General of Health in consultation with the Pharmac board.⁵¹ CAC is required to be in place by the legislation but is appointed by the Pharmac board.

We outlined in the interim report both concerns raised by CAC members at the time, and our own observations, around the limited, ad hoc, and largely reactive role CAC was being asked to undertake by Pharmac. Since our interim report new terms of reference for CAC have been agreed that see a more strategic and proactive role. CAC feedback suggests there have been improvements in the way Pharmac is engaging with it.

Nonetheless the review has heard concerns from a range of consumers and consumer groups around how Pharmac engages with stakeholders and the public, the quality and clarity of its processes and the timeliness and transparency of its decisions. In this report we have also identified key areas for improvement in this area. All this points to the need for a strengthening of the consumer perspective and, more broadly, enhancement of public trust and confidence in the difficult work that Pharmac undertakes.

As a result, we recommend CAC members be appointed by the Minister in consultation with the board and the CAC terms of references 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 interests.

⁵⁰ <https://pharmacreview.health.govt.nz/assets/Pharmac-Review-Interim-report.pdf>.

⁵¹ Section 50(4) New Zealand Public Health and Disability Act 2000.

If this recommendation is accepted, a transition arrangement will need to be provided to maintain the existing CAC members and the TORs now agreed.

Finally, the existing legislative role description for CAC is 'to provide input from a consumer or patient point of view'. We think this is sufficiently flexible to enable CAC to provide support to Pharmac on a wide range of matters. Nonetheless the wording could more closely align with the focus of the Pae Ora (Healthy Futures) Bill as it relates to the work of CAC, with input on the lived experience of population groups as well as consumer and patient voices. An example of how that might be achieved would be to amend the current function of CAC to:

'a consumer advisory committee to provide input from a consumer, or patient point of view, including input on matters that affect population groups'.

The review also notes the minutes of advisory committee meetings are the only part of the medicine's assessment process that Pharmac publishes on its website (apart from information released in response to Official Information Act requests). This means comments made by the committees are often taken out of context by stakeholders who do not have access to other information that might be used in decision-making. Addressing this would improve transparency.

We discuss the role of PTAC and CAC further in section 5 on decision-making, including our observations on how they operate now and the roles that they could have in the future.

Māori Advisory Rōpū

A Māori Advisory Rōpū has recently been established by Pharmac.⁵² The objective is to provide Māori leadership and high-level advice and guidance to the Pharmac board and the senior leadership team in respect to:

- Pharmac's commitment to achieving the best health outcomes for Māori, and
- Pharmac's commitment to upholding the articles and principles of Te Tiriti o Waitangi across their work.

The review sees the establishment of the rōpū as a positive step. We met with members of the rōpū on 28 January to hear their views on their role and the impact that they want to make. In the meeting the rōpū shared concerns about how Pharmac was meeting its equity responsibilities. They also said it was challenging to engage with Pharmac at the right level as they had not met with the board and had had no substantive engagement with the chief executive since they had been established.

⁵² Pharmac Briefing 59: Māori Advisory Rōpū membership September 2021.

Risk and assurance committee

The board's audit and risk subcommittee give advice and assurance on Pharmac's risk, control and compliance framework and its external accountability responsibilities.⁵³ We reviewed the committee terms of reference and agendas and noted the committee receives quarterly risk assessments from Pharmac to review before they go to the board. The risk reports concentrate mostly on organisational matters. Pharmac has advised the committee does provide oversight and assurance of the risks that arise associated with the Combined Pharmaceutical Budget, but this does not appear to the review to be through the risk register.

Governance and equity

Our interim report examined equity matters and the culture of Pharmac in detail, and the key results of that examination bear repeating:

- Pharmac has made various commitments to improving equitable outcomes for Māori, Pasifika and disabled people. However, it is a long way from achieving this goal, just as it is a long way from having a fair representation of Māori, Pasifika and disabled people within its own ranks – or systems, processes and structures that facilitate equitable outcomes.
- Pharmac needs to incorporate equity considerations in all stages of its assessment processes. Equity considerations need to be an everyday part of the work of Pharmac's board, leadership team and various committees.
- Pharmac needs to work more closely with other parts of the health system to better achieve equity outcomes. The health system holds some data on equity and access to medicines, but it is dispersed and needs to be integrated.
- Few Māori, Pasifika or disabled people are on Pharmac's clinical advisory committees. Very few Māori are members of staff. This has been the case throughout its history.
- Pharmac talks about equity engagement and equity outcomes, but there is little evidence of this in practice.

Ministerial appointment on to the Pharmac board is not dictated by any required range of expertise. The review believes the appointment process should follow the same approach adopted in the Pae Ora (Healthy Futures) Bill, which sets out specific guidance for Ministers when appointing members to Health NZ and the Māori Health Authority.⁵⁴ While recognising the skills needed to govern a specialist agency such as Pharmac may differ from those required to govern either of the two new health entities, the skills among those appointed to the board should include expertise in te Tiriti o Waitangi and tikanga Māori and, more generally, lived experience with relevant populations who suffer inequity.

⁵³ Pharmac's audit and risk committee's terms of reference, July 2021.

⁵⁴ See clauses 12 and 22 respectively of the Pae Ora (Healthy Futures) Bill.

Pharmac's strategic planning documents discuss equity and improving outcomes for Māori, but we were unable to determine how the board holds Pharmac to account if and when shortcomings are identified. We note that recent letters of expectations have not been explicit about how Pharmac should improve outcomes for Māori.

For example, Pharmac provided the review with a briefing that identified by 2021 it had completed fewer than half of the actions that had fallen due in its 2017 Pacific Responsiveness Strategy.⁵⁵ Many of the actions that had been completed were either organisationally focused (such as raising staff awareness and building inter-agency relationships) or lacked measurable outcomes (such as 'embedding the need to consider' Pasifika people in activities to promote the responsible use of pharmaceuticals). At the time of our review, responsibility for implementing the Pacific strategy fell largely on the shoulders of one staff member, a distribution of resources that is clearly inadequate given the scale of Pacific health inequities. All of this suggests to us that the board is not holding Pharmac to account for its performance or taking steps to lift that performance. This is an area that warrants further attention.

Strategic direction

In July 2020, Pharmac released its statement of intent for the next three financial years. In that document, it declared its strategic direction as to 'make an even bigger contribution to our health system and to the health of all New Zealanders'. The document also said Pharmac's six strategic priorities were:

- enhance key functions
- medical devices
- equitable access and use
- data and analytics
- public understanding, trust and confidence
- relationships and partnerships.

Pharmac has made progress in some of these six priorities, but not in others for various reasons. Some of these priority areas are wide-reaching, and Pharmac says little about when or how they will be implemented. In our view, Pharmac needs to refine them and also consider how they may need to be adapted to reflect reforms to the health system. In the relationships and partnerships priority area, for example, it will need to look at working more closely with the Ministry of Health, Health NZ and the Māori Health Authority.

⁵⁵ Pacific Responsiveness Papers provided to the review, May 2021.

Measuring accountability

Pharmac's key accountability documents are its statement of intent, statement of performance expectations and annual report. Board members must sign their acceptance of these documents to underline that they are ultimately accountable for the direction and implementation of the first two and the accuracy of the third.

The annual letter of expectations to the board's chair sets out what the Minister expects the organisation to focus on in the year ahead. The statement of performance expectations details how Pharmac intends to give effect to the letter, and the annual report explains how it has achieved what it has committed to deliver. The statement of intent sets out the organisation's goals for the forthcoming three financial years.

Over the years, the number of expectations set out in letters from the Minister has grown, and we note this increase has not been matched by an increase in baseline funding. As a result, Pharmac appears to the review to be stretched and may be trying to achieve too much with its available resources (see more in core functions below). We note two areas of focus typically mentioned in letters of expectations – communication and equity – have been dropped from the latest letter of expectations. We do not take this to mean these are no longer priority areas, but rather that they now constitute part of standard business operations.

The Ministry, as Pharmac's monitoring agency, reviews the agency's accountability documents. It confirmed to us that Pharmac met the expected reporting standards required by these documents, although we consider Pharmac's reporting contains insufficient comment about areas where it has not met performance expectations.

Performance measures

All government agencies are required to explain what they are doing with their funding (outputs) and what benefits New Zealanders derive from this work (impacts). Statements of intent outline planned activities for any three-year period, and the impact and output measures against which those activities will be measured. Pharmac's 2017–20 statement of intent contained four impact measures⁵⁶ and no output measures. By contrast, its latest statement of intent (2021–24) contains three impact measures, three output measures, five Te Whaioranga measures (Māori health-related measures), three organisational measures and six strategic priority measures (one for each of the six priority areas mentioned above) – making a total of 20 performance measures, beneath which sit 23 more specific subsidiary measures, or indicators as Pharmac calls them.⁵⁷ Its three principal output measures are: make choices and manage expenditure and supply; support and inform good decisions and access and use; and influence through policy, research and insights.

⁵⁶ The four impact measures in the 2017–20 statement of intent were: increased access to effective medicines and medical devices; funded medicines and medical devices are available when needed; medicines and medical devices are used optimally; and in a high-performing health system.

⁵⁷ See: <https://pharmac.govt.nz/assets/2020-Statement-of-Intent.pdf>.

This expanded list of performance measures enables Pharmac to report on a broader range of relevant activities and is commendable. Pharmac told us the changes were intended to make its operations more transparent and easier to understand. As this revised approach to reporting is still relatively new it will take time to be of real value. Changes to make the process more transparent have made it difficult to compare the last two years, which means, for a transitional period at least, the changes may result in less transparency rather than more.

A difficulty with the new approach is that in the first annual reporting period 16 of the 25 subsidiary measures were reporting on how the methodology and/or baseline was established rather than describing any progress made in the past year. In effect, Pharmac has set performance goals without knowing how to measure many of them or, indeed, whether it can measure them. Without being able to measure performance, it is hard to see how the board will be able to tell whether Pharmac is making any progress or what impact its activities are having.

In addition, for what should be key performance measures there is a lack of clear targets over and above 'upward' or 'downward' trends and there is limited benchmarking or aspiration. For example:

- measure 4.1 *Timeliness of funding decisions* (time to be ranked which is a stage before any decision is made) is reported as an average time of 16.05 months; and
- 1.3: *Funding decision time* shows the average time taken from an application to a decision is 40.9 months.

The aim is both will trend down (reducing the length of time taken) though there is no measure of an acceptable rate of trending down. It is worthy that Pharmac has shared this information and it will improve transparency but the measures themselves are not measures of performance. The review would expect to see them supported by analysis of the benchmark being aimed at and of what needs to happen for the system to go faster than it does.

The lack of integration within the health system has made it difficult for Pharmac to track and report on the impact of its funding decisions, but the reforms to the health system, including the health strategy and associated health plans, reducing the number of agencies and taking a more patient-centred approach, should help overcome this hurdle.

Monitoring performance

The board monitors Pharmac's performance through material provided by the senior leadership team, in the form of, among other things, financial forecasts, risks analysis and documents detailing progress against key measures. Pharmac staff summarise this performance quarterly and provide it to the board and then to the Ministry (as the monitoring agency) and the Minister. A summarised version of the quarterly reporting is published on the Pharmac website. From what we saw, looking across 12 months of board agendas, minutes, and a selection of papers, it was apparent the senior leadership team's reporting was regular, but often light on detail and had tended to focus on the positive.

The Ministry told us it had moved in 2020 from a compliance-based to partnership-based approach to monitoring, acting as a 'critical friend' to help Pharmac make continuous improvements. Its monitoring approach covers strategic support, accountability and reporting, and relationship-based risk management. This includes:

- providing direction on key areas of focus, including Māori health and equity
- regular meetings with Pharmac's chief executive to discuss any matters of significance
- helping prepare and review the accountability documents mentioned earlier
- when needed, Ministry experts are drawn in to assist with monitoring activities. Members of the Ministry's Māori health directorate can offer assistance on matters relating to Māori health and equity
- supporting a 'no surprises' approach in advice to the Minister. This is particularly important for Pharmac, given the high levels of public and political interest in its work. Pharmac works regularly with the Ministry on a range of topics, enabling free and frank discussions on risks and issues.

All of these are consistent with government expectations and the Public Service Commission's operating expectations framework for Crown entities, which sets out the roles, responsibilities and operating expectations of Ministers, Crown entities and monitoring departments.

The Ministry told us it was generally happy with Pharmac's performance, given its operating environment and original purpose, although it said there were areas where Pharmac needed to improve, including alerting them to matters the Minister may need to be aware of.

Overall, the review has observed the monitoring function could offer a more constructive critique of Pharmac, in particular how it is working with and across the health system in a more active way.

Treasury also monitors Pharmac. Among other things, it considers Pharmac's budget proposals and provides advice to the Minister of Finance. Pharmac's budget arrangements are complex. It currently has immediate control over its operating budget and must report how it is spent and what it has achieved. However, the funding for medicines has been managed in conjunction with the district health boards which hold the appropriation. This is further complicated by the confidentiality arrangements Pharmac makes with the pharmaceutical companies for confidential rebates and discounts against the prices of branded medicines, which can only be shared with the district health boards at an aggregated level.

The Government has decided Pharmac will have its own appropriation from 1 July 2022. This will give greater transparency and accountability requirements, but we are concerned a separate appropriation risks drawing Pharmac away from, rather than closer to, the wider health system. Medicine funding decisions need to be made as part of the revised medicines strategy (see section 5) and the New Zealand Health Plan. In addition, deciding which medicines deserve priority funding needs to be made in consultation with Health NZ and the Māori Health Authority.

A separate appropriation will not necessarily eliminate the difficulties with forecasting actual costs because there will still be a delay between the time when Health NZ (taking over from district health boards: see section 5) buys medicines and when it receives the rebate. We encourage Treasury to monitor how this operates in practice and consider reviewing the arrangement once Health NZ's five-year funding plans have been tested.

Treasury also has a role in ensuring Pharmac's budget is considered as part of the health system budget. We would expect the current level of confidentiality applied by Pharmac needs to be managed so that the Treasury alongside Health NZ and the Māori Health Authority have sight of how public money is being invested and the outcomes Pharmac is achieving.

An updated medicines strategy

The Ministry of Health is responsible for developing health-related policy for the Government. In our view the Ministry needs to develop a replacement medicines strategy to guide the sector, including Pharmac, in its decision-making. Such a strategy could form part of the New Zealand Health Strategy, which will be required once the Pae Ora (Healthy Futures) Bill is enacted.⁵⁸ The Ministry developed the existing medicines strategy in 2007 because it said, 'the medicines system was not serving New Zealanders as well as it could and lacked an overarching strategic direction'.⁵⁹

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. It does not, for example, account for the emergence of treatments for some cancers or costly biological medicines that are individually targeted to patients with rare disorders. Unsurprisingly, given the amount of time that has elapsed since the existing strategy was drafted, four of the five strategies it makes reference to have themselves undergone significant updating. The existing strategy was also developed before the establishment of the Health Quality and Safety Commission, whose role is to coordinate the monitoring and improvement of health and disability services. In 2015, the Ministry published a framework governing the regulation, procurement, management and use of medicines through to 2020, but this, too, needs updating for many of the same reasons the medicines strategy needs updating.⁶⁰

⁵⁸ See clause of 37 of the Pae Ora (Healthy Futures) Bill.

⁵⁹ The strategy is structured around seven areas: making the most of every point of care; enabling shared care through an integrated health care team; optimal use of antimicrobials; empowering individuals and families/whānau to manage their own medicines and health; optimal medicines use for older people and those with long-term conditions; competent and responsive prescribers; and removing barriers to access. See: Medicines New Zealand: Contributing to good health outcomes for all New Zealanders, 2007: <https://www.health.govt.nz/system/files/documents/publications/medicines-nz.pdf>.

⁶⁰ Implementing Medicines New Zealand 2015 to 2020: <https://www.health.govt.nz/publication/implementing-medicines-new-zealand-2015-2020>.

The Ministry told the review the need to prioritise work on the Therapeutic Products and Medicines Bill and respond to Covid-19 meant it was not in a position to update the strategy or framework, and neither was this part of its current work programme. We consider the absence of an up-to-date, principles-based medicines strategy to be a crucial omission, and one that will disrupt Pharmac's integration into the new health system. With health sector reforms not far away, now is an ideal time to update the strategy to define medicines priorities. We consider the Ministry should make updating the medicines strategy a priority. It needs to be developed with Pharmac and the wider health sector, and should involve and consult with stakeholders, priority populations and civil society on its contents over the next 12 months.

Horizon scanning

Our interim report described the lack of time Pharmac spent scanning for emerging trends domestically and overseas that might warrant funding in the future. Actively searching out early signs of potential change, spotting trends before they become issues, and taking early steps to shape desired outcomes are all part of this process. The pace of change in the global pharmaceutical sector makes it essential Pharmac has a proactive approach to identifying priorities, the results of which could potentially affect the make-up of the pharmaceutical schedule. It forms part of the board's role in overseeing management of the pharmaceutical schedule (see section 5).

Pharmac has recently started a formalised process it calls category planning, which consists of taking an in-depth periodic look at each of its therapeutic groups (essentially classes of a disease or condition) and what developments lie ahead.

Other jurisdictions, notably the United Kingdom, have dedicated units devoted to horizon scanning, considering future medicines alongside medical devices and diagnostics, and ensuring patient input to the process.

The future New Zealand health system will be guided by key planning documents:

- **A Policy Statement on Health** issued every three years by the Minister, setting out the Government's overall direction, priorities, and objectives for the health system
- **A New Zealand Health Strategy** establishing a framework for the overall 5–10-year direction of the health system. Hauora Māori, Pacific Health, and Disability Health strategies must also be prepared to sit alongside this; and
- **The New Zealand Health Plan** which will set the operational direction for the system and is to be jointly prepared by Health NZ and the Māori Health Authority.

Each of these strategic documents will need to be informed by comprehensive horizon scanning. As in the UK this should encompass medicines, devices, diagnostics and other non-pharmacological approaches.

While the responsibility for this will primarily reside with various combinations of the Ministry, Health NZ and the Māori Health Authority, Pharmac must be actively involved.

Pharmac should continue to be responsible for the work it is already doing for pharmaceuticals, looking across each of their therapeutic groups. Pharmac should also contribute its research to the broader horizon scanning across the entire health sector.

Research

One of Pharmac's functions is to engage in research to help meet its best health outcomes objective.⁶¹ We consider it should retain this function. Pharmac's research activity is wide-ranging.⁶² Some is operational, and some is strategic. In recent years, more than half of Pharmac's strategic research spending has come from a partnership with the Health Research Council of New Zealand. The two organisations jointly make between \$450,000 and \$650,000 a year available in contestable funding for various projects. This source of funding has been put on hold while the council conducts a review of this partnership. It expects to have a new framework in place in 2022.

Pharmac's individual business units also fund research in support of their individual priorities. Pharmac told us it had not collated the results and so could not give us a comprehensive list of all research funded in this way over the past three years without a substantial amount of work.⁶³ Given all the research it has funded to date, we consider Pharmac has an opportunity here to share its knowledge base more widely within the health sector.

The Ministry has plans to establish its own research and innovation function and to partner with Health NZ, the Māori Health Authority and others to help create a strong, cohesive research and innovation sector that fills knowledge gaps in priority areas and translates research results into policy and practice. This, we note, would fit with the strategic priorities of the New Zealand Health Research Strategy 2017–2027, which are to ensure investments are aligned with areas of need, create a vibrant research environment in the health sector, build and strengthen pathways for translating research findings into policy and practice, and advance innovative ideas and commercial opportunities.⁶⁴ Pharmac needs to be part of this process.

Communications

The review has heard concerns from a wide range of stakeholders that Pharmac's communication approach needs improvement.

The review noted in our interim report the need for better communication with stakeholders, particularly Māori and Pasifika, disabled people and those with rare disorders, to help foster authentic, rather than purely transactional, relationships. We also noted external communication is clumsy and not patient- or consumer-centred.

⁶¹ Section 48 of the New Zealand Public Health and Disability Act 2000.

⁶² These activities include clinical trials, clinical epidemiology, pharmacoepidemiology, literature reviews, evaluations of patients' adherence to medicines, population health programme evaluations, project evaluations and other health services research.

⁶³ Pharmac briefing: Research at Pharmac, October 2021.

⁶⁴ <https://www.health.govt.nz/publication/new-zealand-health-research-strategy-2017-2027>.

We are still of the view that Pharmac's communication style tends to be defensive and to a degree this is not unexpected. Staff are working in a challenging and sensitive area. They are charged with making difficult decisions, within the constraints of a capped budget. Their decisions have significant impacts on people's lives and stakeholders are rightly calling for more transparency to better understand the decisions made. Stakeholders also reasonably told us that they do not want to be criticised by Pharmac for asking questions and challenging the outcomes of decisions Pharmac makes. Maintaining public trust and confidence are key elements to maintaining the agency's social licence, and Pharmac must be in a position to robustly and confidently articulate its rationale for decisions it makes.

Pharmac has implemented some steps to make more information available. It has implemented the website application tracker, which while needing significant updating, does give insights into how an application is progressing. It releases agendas for board meetings and minutes from the Pharmacology and Therapeutic Advisory Committee, the Consumer Advisory Committee and other specialist advisory groups, though the timeliness of their release is a concern. It has also released its options for investment list in alphabetical form showing what has been approved for funding should resources become available.

Any agency, particularly a Crown agency, that delivers a service to the public has a responsibility to make sure information can be accessed by everyone. This warrants further consideration by Pharmac. The formats it uses and the technical nature of the content it produces exclude the general population from getting assurance that their needs and concerns have been considered. Additionally, content must be accessible to provide equal access and equal opportunity to disabled people. Pharmac needs to take rapid steps to enact its accessibility obligations under Article 9 of the UNCRPD, New Zealand Disability Strategy, the Accessibility Charter, and Web Accessibility Standard 1.1.

Overall, the information released to the public is limited and only gives transparency to a small part of the business. Stakeholders have told us in particular that they want more transparency on the decision-making processes, and we address this in section 5.

Covid-19

Pharmac has been conducting negotiations for the supply of medicines to treat the effects of Covid-19, and as part of this work it has been assessing the medicines in collaboration with Medsafe, the Ministry and the Ministry's Covid-19 Therapeutics Technical Advisory Group.⁶⁵

⁶⁵ <https://www.health.govt.nz/about-ministry/leadership-ministry/expert-groups/Covid-19-therapeutics-technical-advisory-group-therapeutics-tag>.

Pharmac has established its own specialist advisory group⁶⁶ on Covid-19 to help assess and negotiate contracts for these medicines, which will be for patients in hospitals or community settings.⁶⁷ Pharmac said the assessment process was comparable to its standard process, although it was completing some steps faster than normal, relying on provisional data and evidence to make decisions because it was dealing with a pandemic situation.

Once health agencies' Covid-19 response moves into a business-as-usual phase, we recommend Pharmac, in conjunction with other agencies, examine how this more collaborative approach and faster evaluation process might be adopted in its regular operations.

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.

⁶⁶ The group is made up of clinicians with expertise in intensive care, infectious diseases, respiratory medicine, critical appraisal, and general practice. It also has four members from the Ministry's Therapeutics Technical Advisory Group, along with an observer from the Consumer Advisory Committee and observers from the Ministry. See: <https://pharmac.govt.nz/about/expert-advice/advisory-groups/>.

⁶⁷ Pharmac briefing: Roles and responsibilities of Pharmac, Pharmac's Covid-19 treatments advisory group, Medsafe and the Ministry of Health technical advisory group, January 2022.

5 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:

- We examined 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. These case studies gave us insights into how the process of assessing applications and determining funding priorities worked in practice.
- We looked at how Pharmac made funding decisions, including who reviews the analysis, what voices were heard in decision-making, and how decisions were communicated to the public.
- We looked at 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.

The path to a decision

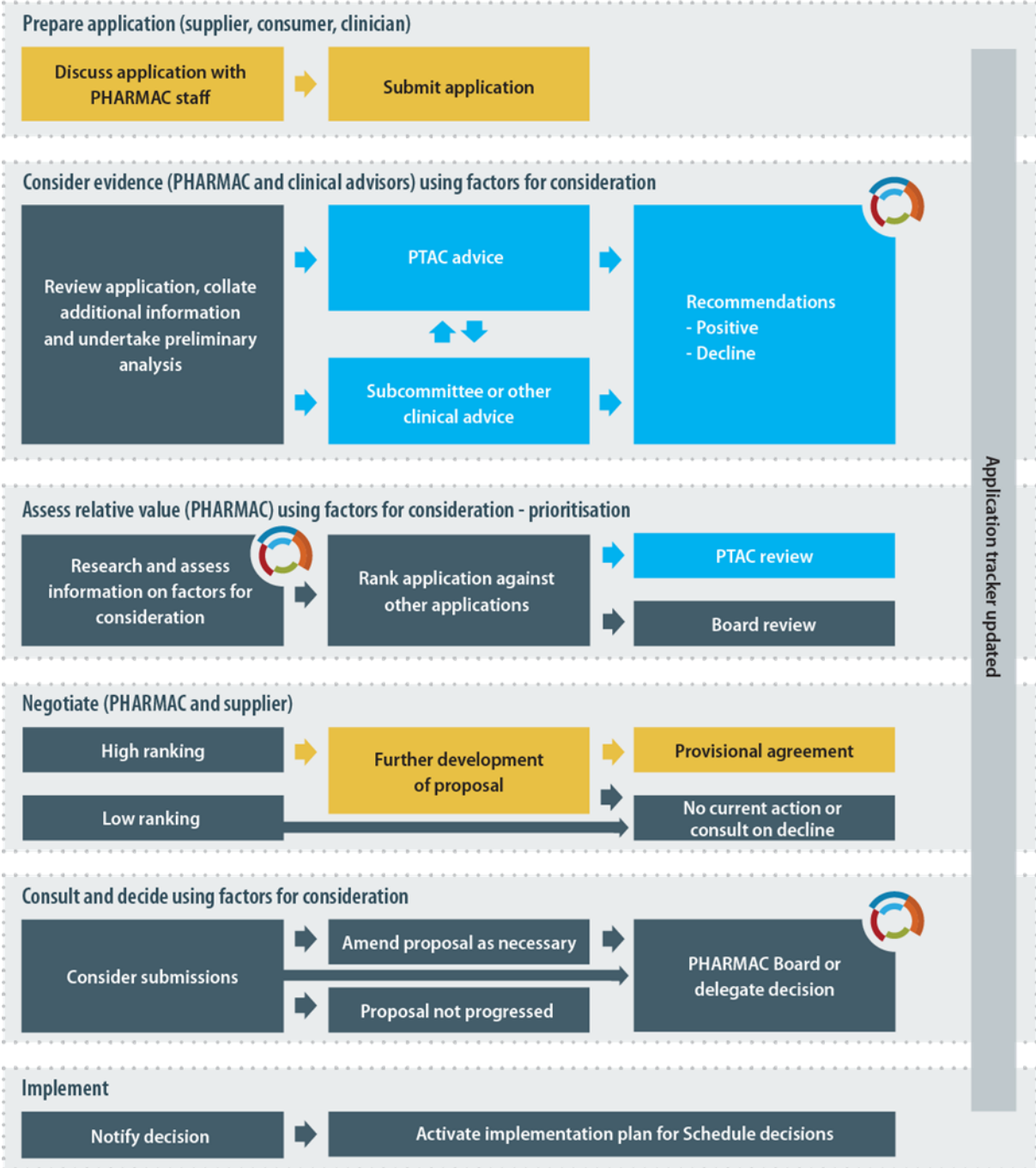
Our interim report contained a detailed description of Pharmac's decision-making process, which can be summarised as:

- clinically evaluate a medicine
- assess its costs and benefits
- compare its relative cost and clinical benefits to other medicines
- assess need
- rank its priority for funding
- negotiate a subsidy with the supplier
- list it on the pharmaceutical schedule.

Figure 3, supplied by Pharmac, illustrates this process. What it does not show is that there are other layers to the process that are not visible to the public. Considerable work happens in the background, usually in the form of papers seldom distributed beyond a small number of staff, that have a bearing on the eventual decision. Once an application has been made, (whether by a pharmaceutical company, an individual or Pharmac itself), staff prepare two policy and technical papers that guide the discussions of the Pharmacology and Therapeutics Advisory Committee (PTAC), a statutorily appointed clinical committee, and the various specialist committees that support it.

All these committees discuss in detail the relevance, importance and soundness of the clinical evidence presented in these internal papers and in the externally provided application documents. This helps Pharmac decide whether to approve a medicine’s listing and what funding priority it should have. Recommendations also set out who should use the medicine and why. In the course of this process, Pharmac must weigh up the medicine’s cost and benefit, budget implications, and other elements of its factors for consideration and pharmacoeconomic frameworks.

Figure 3: The sequence of making a decision



Source: Pharmac.

The two papers that staff produce are a technology assessment report and a statement of the factors for consideration. The factors for consideration paper is broadly based on health need, costs, benefits, savings and suitability of the medicine. The technology assessment paper includes an assessment of the relative value of the medicine, along with cost-utility analysis for a new, innovative pharmaceutical or a cost-effectiveness analysis for a pharmaceutical with the same or similar therapeutic effect as others, using information from clinical advice papers, committee comments and other documents. These two papers are prepared at the same time, while seeking advice from the Pharmacology and Therapeutics Advisory Committee.

If Pharmac deems a medicine potentially suitable to go on the pharmaceutical schedule, it prepares a third document called a prioritisation dossier. This dossier is a summary of the other two papers and may include further information around health need.

Where Pharmac considers a pharmaceutical to be worth considering for public investment, it places it on one of two waiting lists: either the options for investment list or, the cost neutral/produces cost savings list (that is, it would cost the same as or less than a medicine already funded for the same condition).⁶⁸ The options for investment list is publicly available, but Pharmac does not disclose the ranking (where the medicine appears on the list) of the medicines out of concern this information could harm its negotiations with suppliers. If a medicine goes on the options for investment list, Pharmac gives it a priority ranking using its factors for consideration. The list goes from highest-ranked to lowest-ranked, and the medicine remains on this list until it is high enough up the list to get funding. The other list is not ranked on the basis Pharmac will negotiate at any time with any supplier willing to offer the medicine at the same or lower price as that of the already funded equivalent medicine. No deliberations are therefore necessary about determining funding priority for medicines placed on this list.

Setting the priority for funding a medicine typically involves three meetings beforehand:

- a hot topic meeting (not always held) where therapeutic group managers, health economists, medical directors and sometimes other staff present information about the relevant condition, treatment, potential significance and cost-utility analysis
- a pre-prioritisation meeting to which all therapeutic group managers and medical staff are invited, and which all health economists are expected to attend, where a broader discussion of all investment proposals takes place
- a prioritisation meeting open to all staff, including therapeutic managers, medical doctors, the Access Equity team and the Te Whaioranga Team, but compulsory for health economists. A dossier summarising the investment case against all the factors for consideration is presented.⁶⁹

⁶⁸ There is a third list for declined applications, the reasoning being that new information or data could come to hand to warrant reactivating these applications.

⁶⁹ Among those able to attend are members of the Access Equity team, which is responsible for implementing Pharmac's plans to build medicine equity, monitoring and reporting on progress in this regard, establishing partnerships with groups and other areas of the health sector, and providing input into policy and research. Also able to attend is the Te Whaioranga team, which is responsible for implementing the Māori responsiveness strategy. While the Te Whaioranga Team has increased in size over the last two years, the review noted the Te Whaioranga Team was two people – neither of these senior management.

At the prioritisation meeting, staff review and prioritise funding applications and determine where to place them on the options for investment list. Pharmac does not formally decide whether to fund a medicine until it has been through the process just described and the medicine is at a point at which Pharmac could seek a contract for its supply. Pharmac's board manual and its delegation policy state the board must make funding decisions, although it can delegate the task to the chief executive or others below that person. As we note in our governance section, in the year to 30 June 2021, the board delegated responsibility for 32 funding decisions and made only three itself. Senior managers, rather than the board, are therefore making the overwhelming majority of funding decisions.

As new evidence emerges, other new pharmaceuticals are developed, or views of priorities change, the ranking of a medicine can change to reflect this new information.

Pharmaceuticals might be funded if there is a commercial solution, for example by bundling new investments with savings on other pharmaceuticals or structuring contracts to expand access at different prices for different population groups or some other innovative contract arrangement such as further rebating and payments for outcomes.

The description of the approval process is complicated by how Pharmac describes what a decision is and when it is taken. From Pharmac's perspective, a decision only happens at the very end of the process when all its assessment steps (including tanking) have been completed, a suitable price has been agreed and a contract is ready to be entered into. Until that time, which may be never, there has not been a decision. From our perspective, there are a series of intermediate decision points such as the decision to place a pharmaceutical on the Options for Investment list or to not progress with an application.

How Pharmac's decision-making criteria evolved

In our interim report, we said we found it difficult to determine how Pharmac applied its factors for consideration during the decision-making process, and whether it was doing so in a systematic fashion. It is worth noting the factors for consideration were predated by a set of nine criteria, among which were health need, including that of Māori and Pasifika people, availability and suitability of other medicines, clinical costs and benefits, likely therapeutic costs and benefits, and impact on the budget. Over time, however, concerns emerged through public consultation and reviews of the criteria that the financial impact of funding decisions outweighed consideration of other criteria, and there should be an overall set of principles to ensure decisions were fair and reflected community values.

The factors for consideration consist of a decision matrix of four quadrants, each with associated criteria. The quadrants are health need, benefits, costs and savings, and suitability. Each quadrant's criteria look at funding decisions from an individual, community and whole-of-system perspective to try to ensure decisions are fair and reflect community values.

The factors for consideration are wide-ranging, and there is no formal means of evaluating their application. This makes them difficult to apply in the decision-making process, and it is also difficult to assess whether they are being applied consistently across applications and over time. We discuss this in detail below.

Case studies

This section explains how we used the six case studies mentioned earlier to examine how Pharmac applies the decision-making process when assessing medicines and the analytical tools it uses to reach conclusions (see Table 7). We cross-checked our own conclusions against internal guidance and general good practice for funding decision-making and health economic modelling. We commissioned epidemiology, public health and health economic advisers to help us with this work.^{70,71} The results gave us insights into the quality of Pharmac's analysis, and omissions and defects in the decision-making process. We also became clearer about the strengths and limitations of the assessment process and how it could be improved.

The case studies were selected to illustrate how the processes worked across a range of different circumstances, including medicines for a rare disorder, for a cancer and for a high prevalence condition (such as diabetes). We chose some case study medicines because of the diseases' particular relevance to Māori, Pasifika and disabled people. Also, there has been considerable public discussion in New Zealand about the availability of the medicines overseas and how they have performed. Finally, our selection gave us the broadest possible perspective on Pharmac's decision-making process and allowed us to draw the most comprehensive conclusions about that process.⁷²

To help us with our work, Pharmac gave us decision models, internal papers, papers sent to specialist advisory committees and any other relevant decision-making papers, such as board papers. In the case of one medicine, Pharmac arranged for the release of the pharmaceutical company's application, and this allowed us to see the process from start to finish, including how the supplier prepared its application and what it chose to present to Pharmac. (Applications are confidential and ordinarily withheld.)

We were not told the price of the medicines or the potential budgetary impact of funding them. The limited information available to us did not enable us to say whether the analysis Pharmac undertook was proportionate to the potential amount it would have to spend. The medicines selected for analysis are set out in the table below.

⁷⁰ Mcleod M, Harris R. Review of Pharmac cost-utility analysis modelling approaches in relation to Māori health inequity. Pharmac Review, Wellington 2021.

⁷¹ Sapere Research Group (March 2022). Pharmac case studies methodology and source material.

⁷² These cases are as good as any for establishing Pharmac's assessment of equity and how it treats groups who experience disparities within its analysis. They also provide a good insight into the extent to which Pharmac takes note of public views.

Table 7: Six medicines selected to analyse Pharmac’s decision-making process

Medicine	Disease
Ustekinumab, a monoclonal antibody medication	For treatment of Crohn’s disease, a chronic inflammatory bowel disease affecting the digestive tract lining
Nusinersen, in a class of medications called antisense oligonucleotide inhibitors	A novel treatment for spinal muscular atrophy
Pembrolizumab, a humanized antibody used in cancer immunotherapy	As an alternative treatment for non-small cell lung cancer
Pembrolizumab	An additional treatment for metastatic and unresectable melanoma
Empagliflozin, a sodium glucose co-transporter 2 (SGLT2) inhibitor	A treatment to help reduce the risk of cardiovascular and renal complications in people with type 2 diabetes ⁷³
Venlafaxine, an antidepressant medication of the serotonin-norepinephrine reuptake inhibitor class (brand switch)	A treatment for major depressive disorder, generalised anxiety disorder, panic disorder, social phobia and also chronic pain

Comparison with internal guidance documents

We reviewed Pharmac internal guidance – the Prescription for Pharmacoeconomic Analysis that sets out how it conducts economic analysis of pharmaceuticals. Pharmac shared the technical assessment reports, cost-utility analyses, decision tree models, meeting papers and meeting minutes.

We also looked at how Pharmac’s assessments considered equity-related matters, whether there were any material omissions of information, and how Māori health equity considerations could be strengthened in cost-utility modelling. In reaching our conclusions, we took into account that Pharmac has discretion to deviate from its guidance if justified by the context of the funding decision.

We found six instances where there were likely material differences (errors or omissions) from Pharmac’s own guidance and 16 instances where there may have been material differences (see Table 8).⁷⁴

⁷³ Currently licensed in New Zealand for use in people with type 2 diabetes but also used internationally in guidelines for people with heart failure who do not have diabetes, eg, McDonagh TA, Metra M, Adamo M, Gardner RS. 2021. ESC Guidelines for the diagnosis and treatment of acute and chronic heart failure. *Eur Heart J* 2021; [published online 26 August 2021]. doi:10.1093/eurheartj/ehab368.

⁷⁴ ‘Likely’ is a difference that would change the analysis such that a different answer would arise whereas ‘may’ is less certain and it would depend on the wider context of the decision.

There are practical limitations on how Pharmac assesses applications because it is largely dependent on information from pharmaceutical companies, supplemented by input from its special advisory committees. Even so, its guidance stipulates that it must clearly state these deficiencies or assumptions and their impact, yet we found no such statements. Analysis of Māori health needs and issues was particularly flimsy, and there was a disconcerting absence of information on Māori health needs and inequities. The six differences likely to have had material effects on the decisions Pharmac made were all in relation to empagliflozin for type 2 diabetes and pembrolizumab for lung cancer – both conditions that disproportionately affect Māori, Pasifika and socially disadvantaged groups. The 16 differences that may have had a material effect on Pharmac’s decisions, though small in number relative to the 15 criteria for each case study in the Prescription for Pharmacoeconomic Analysis, nonetheless are differences that may have influenced decisions around priorities for funding.⁷⁵

Table 8: Differences from Prescription for Pharmacoeconomic Analysis

	May be material	Likely to be material
Empagliflozin	3	2
Ustekinumab	2	Nil
Nusinersen	5	Nil
Pembrolizumab (lung cancer)	2	4
Pembrolizumab (melanoma)	4	Nil

We also found 25 differences from the 16 criteria in the factors for consideration (see Table 9). Again, the three conditions that disproportionately affect Māori and Pasifika and are all Pharmac Hauora Arotahi Māori health areas of focus – type 2 diabetes, lung cancer and mental health issues – all featured prominently in these differences.

Table 9: Differences from factors for consideration framework

	May be material	Likely to be material
Empagliflozin	1	3
Venlafaxine	1	3
Ustekinumab	4	Nil
Nusinersen	3	Nil
Pembrolizumab (lung cancer)	Nil	5
Pembrolizumab (melanoma)	3	2

⁷⁵ The documents we received contained no formal economic analysis for venlafaxine, given it was a brand-switch decision and Pharmac had already conducted the economic analysis in the initial funding decision, so it was not included in our comparison of documentation against the Prescription for Pharmacoeconomic Analysis.

Examples of errors and omissions

The following selection of examples illustrates the inconsistency of Pharmac's analysis with its own guidance, and also with good practice.

No separate analysis when inequities are likely to have significant impact on analysis results

Both the Prescription for Pharmacoeconomic Analysis and factors for consideration framework recommend separate cost-utility analysis for different population groups if an inequity is likely to cause significant differences in the outcomes of these assessments for the different groups. Pharmac recognises Māori, Pasifika and priority groups are disproportionately affected by diabetes and lung cancer, but it does not recognise this fact in its analyses. Pharmac documents note that lung cancer incidence and mortality rates for Māori are between two and four times higher than those for non-Māori. The documents also note that the average age for the onset of diabetes is significantly lower for Māori than non-Māori, and that Māori have higher rates of diabetes, diabetes-associated complications and cardiovascular disease than non-Māori. Pharmac internal guidance recommends a separate cost-utility analysis be undertaken if there are disparities that are likely to result in significant differences in the benefits of funding a medicine. Based on the above statistics alone, we would have expected such an analysis, but Pharmac did not conduct one.

Failure to consider impact on Māori or known inequities for Māori

To reduce the cost of funding pembrolizumab for lung cancer, Pharmac proposed limiting eligibility to those whose level of the protein PD-L1⁷⁶ exceeded 50 (representing about a quarter of clinical trial populations), based on somewhat inconsistent evidence that such individuals had a higher overall chance of surviving,⁷⁷ as well as a higher chance of progression-free survival.⁷⁸ Pharmac did not investigate PD-L1 levels among Māori, or whether this restriction would have a disproportionate impact on Māori and limit their ability to obtain treatment. Nor did it consider the impact of known inequities for Māori in obtaining quality health care for lung cancer or how the recognised difficulty in accessing PD-L1 testing in the health system would impact Māori.

⁷⁶ PD-L1 helps keep immune cells from attacking non-harmful cells in the body. Cancer cells can often have PD-L1, which allows them to 'trick' the immune system. If cancer cells have a high amount of PD-L1, patients may benefit from immunotherapies.

⁷⁷ Herbst et al 2016: [https://www.thelancet.com/journals/lancet/article/PIIS0140-6736\(15\)01281-7/fulltext](https://www.thelancet.com/journals/lancet/article/PIIS0140-6736(15)01281-7/fulltext); Paz-Ares et al 2018: <https://www.nejm.org/doi/10.1056/NEJMoa1810865>.

⁷⁸ Gandhi et al 2018: <https://www.nejm.org/doi/10.1056/NEJMoa1801005>.

In relation to venlafaxine, Pharmac noted that Māori accounted for about 8 percent of venlafaxine use and that this was about 50 percent below the proportion of Māori in the general population. This simple comparison fails to take the impact of the different age structures of Māori and non-Māori populations into account. Although this was a simple brand switch, the analysis represents a missed opportunity to fully explore the inequities in this area, which include: Māori adults are 60 percent more likely than non-Māori adults to report high or very high probability of having an anxiety or depressive disorder,⁷⁹ Māori are 52 percent less likely than New Zealand European/Other ethnicities to be dispensed venlafaxine,⁸⁰ and dispensing rates for Māori are also lower for all other main antidepressants and anxiolytics, even after taking into account age and seriousness of disease and different levels of access to primary care.⁸¹

Trial populations differed significantly from New Zealand's population

Pharmac applied a trial population in the case of the lung cancer and diabetes applications that had different characteristics to those found in New Zealand and did not acknowledge the implications this would have on modelling. In both cases, Pharmac did not discuss these limitations. For pembrolizumab for lung cancer, the clinical trial population was between 59 percent and 81 percent male which suggests the use of several different trial populations;⁸² whereas 56 percent of all lung cancers in Māori are in females.⁸³ Worse survival rates in New Zealand and for Māori in particular mean the benefits demonstrated in the trials were likely be materially different to those in New Zealand. For empagliflozin for diabetes, the trial population had an average age of 63 years, with 71 percent being male, and the population came from several countries with different population demographics and health care systems to New Zealand's.⁸⁴ This was especially relevant to Māori because there were likely to be significant differences in age and access to health care, which affects diagnosis of cardiovascular disease and access to diabetes medications.

⁷⁹ <https://www.health.govt.nz/our-work/populations/maori-health/tatau-kahukura-maori-health-statistics/nga-mana-hauora-tutohu-health-status-indicators/mental-health>.

⁸⁰ Metcalfe et al 2018: <https://journal.nzma.org.nz/journal-articles/te-wero-tonu-the-challenge-continues-maori-access-to-medicines-2006-07-2012-13-update>.

⁸¹ Ibid.

⁸² Gandhi et al 2018: <https://www.nejm.org/doi/10.1056/NEJMoa1801005>;
Herbst et al 2016: [https://www.thelancet.com/journals/lancet/article/PIIS0140-6736\(15\)01281-7/fulltext](https://www.thelancet.com/journals/lancet/article/PIIS0140-6736(15)01281-7/fulltext);
Paz-Ares et al 2018: <https://www.nejm.org/doi/10.1056/NEJMoa1810865>;
Reck et al 2016: <https://www.nejm.org/doi/10.1056/NEJMoa1606774>.

⁸³ <https://www.health.govt.nz/your-health/conditions-and-treatments/diseases-and-illnesses/lung-cancer>.

⁸⁴ Zinman et al 2016: <https://www.nejm.org/doi/10.1056/NEJMc1600827>.

Use of non-representative population data

Pharmac relied on Māori population estimates gathered by district health board studies in Otago and Canterbury to reach its conclusion about the prevalence of Crohn's disease in the Māori population nationally. However, both regions have relatively small Māori populations compared with the national percentage (7 percent versus 15 percent). This is an important data limitation, but one that was not identified or discussed, suggesting Pharmac either didn't recognise its implications or didn't think it important enough to warrant discussion. (Diagnosis of Crohn's disease among Māori is often delayed because of inequitable access to primary care.)⁸⁵

No adjustment for purchasing power parity

In the case of nusinersen for spinal muscular atrophy, Pharmac used German health care costs in its analysis to estimate equivalent costs in New Zealand. The only adjustments made were for exchange rate and inflation. There was no adjustment for purchasing power parity in the two countries, which have very different economies, or for their different institutional arrangements. Nor was there any explanation given for the omission. Also, Pharmac guidance suggests using New Zealand costs where possible, but Pharmac did not follow this guidance or offer any explanation for why it did not. Although the error had little effect on the funding decision, it highlights the need for Pharmac to learn more from the rest of the health and disability sector about the costs, context and constraints of treating different diseases.

Potential constraints and drawbacks not considered

Pharmac's analysis of pembrolizumab for melanoma (now funded) assumed hospitals' constrained oncology infusion capacity would increase, despite recognising that if it did not, the use of pembrolizumab could overwhelm hospitals' capacity to provide infusion treatments. It made no checks of the likelihood of this capacity increasing and included no discussion of the potential drawbacks to other patients and the health system if capacity did not increase.

In the case of nusinersen, it made some limited qualitative comment about the high rates of adverse reactions when administering the drug that were reported in the clinical trials it relied on. This meant a lifetime of potential risk for those taking nusinersen (because the analysis assumed a lifetime of treatment), and it also meant potential extra health care costs. Neither factor was mentioned in the assessment. Pharmac's guidance requires, and the review would have expected discussion on these matters. Analysts should be transparent on what has been omitted, why, and what the potential impacts are of doing so.

⁸⁵ Mcleod M, Harris R. 2021. Review of Pharmac cost-utility analysis modelling approaches in relation to Māori health inequity. *Pharmac Review*, Wellington.

Findings

We do not know what effect these errors and omissions had as we do not have the context of other pharmaceuticals or other informal discussions that might have happened. We do know, however, that Pharmac does not consistently follow its guidance documents and some of these differences are material in decision-making. We also note there is an inconsistency between the analysis undertaken and Pharmac's stated goal of eliminating medicines inequity by 2025. Specifically:

- **Pharmac fails to consider suitability of information it uses in analysis:** We came across a worrying number of examples in its assessment and decision-making documents where it used information without considering whether there were any limitations on its applicability and relevance, and what implications those limitations might have for the outcome of the analysis.
- **Pharmac poorly considers the distribution of needs and benefits:** The case studies showed time and again how Pharmac did not sufficiently consider the distribution of health needs and benefits for different population groups in New Zealand or the way that those populations access health care.
- **Pharmac omits potentially significant effects that may cause bias in its assessment and decision-making:** Pharmac omits potentially important effects, including costs, that can lead it to understate or overstate the benefits of funding a medicine and potentially result in biased decisions. It sometimes acknowledges these effects qualitatively but does not actively consider the consequences of excluding them from analysis.
- **The factors for consideration are not serving their intended purpose:** Pharmac developed the factors for consideration in response to criticism it was failing to adequately consider fairness, equity and community values. Analysis of the case studies shows the factors for consideration are secondary to the technology assessment. If the factors were, indeed, leading to fairer, more equitable and more representative decisions, we would have expected to see, for example, separate analysis when it knew of significant inequities affecting Māori, Pasifika and other groups.

Errors and omissions of this kind are likely to have a compounding effect on the health of Māori, Pasifika and disabled people, adding to the already well-documented inequities they face in accessing health services and disability support services. In short, the case studies and the focus on utilitarianism suggest Pharmac's decision-making errors and omissions could be increasing inequities.

In addition to the six case studies, we also conducted meetings and interviews with Pharmac and stakeholders, including human rights and consumer groups, and researched what other countries do, to reach further conclusions about Pharmac's decision-making process. These are set out next.

Factors for consideration fail to have substantial impact

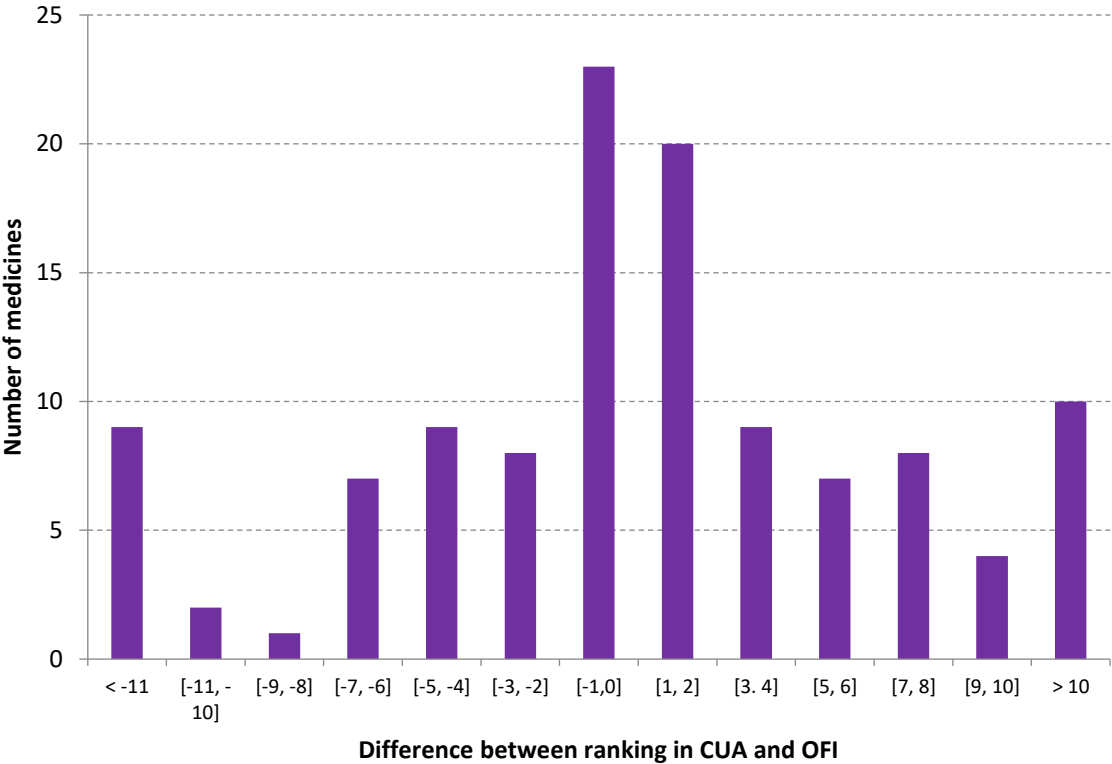
As we noted earlier, the factors for consideration came about with good intentions, but have failed to achieve their purpose. Their wide-reaching domains aim to cover all elements of qualitative assessment, but this makes them challenging to apply meaningfully. The factors' discretionary nature, the lack of documentation about how they are applied in setting funding priorities, and the absence of any evidence they generate meaningful impact suggest the factors have clouded, not aided, the decision-making process, and in so doing may have dented public trust and confidence in Pharmac.

The factors for consideration are intended to be used at each stage of the decision-making process, but this was not evident in the documents we reviewed. To gauge the impact of the factors for consideration on Pharmac's decision-making, we compared the cost-utility analysis ranking of each of 118 medicines with their options for investment list ranking. Pharmac sent us an anonymised snapshot of the rankings medicines received from cost-utility analysis, as well as the rankings those same medicines received on the options for investment list after prioritisation meetings (where staff vote and rank options after cost-utility analysis has been conducted).⁸⁶ In effect, medicines can move up and down rankings on the options for investment list after cost-utility analysis based on staff voting and after applying the factors for consideration, so comparison of the lists shows the impact of the factors other than cost utility.

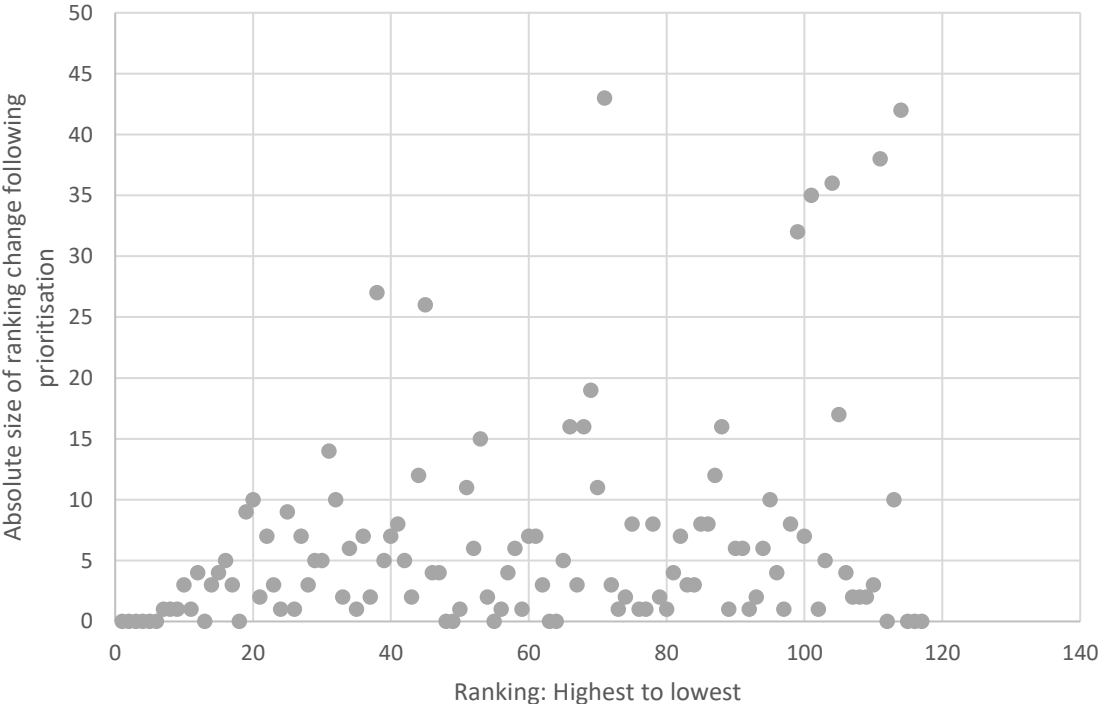
The addition of the factors for consideration did not greatly affect a medicine's ranking. Of the top 10 pharmaceuticals on the list, the first six stayed where they were. Three of the remaining four moved up one place, and the fourth moved down the list three places. Some medicines did change their ranking substantially, but they were too far down the options for investment list for the shift to be likely to change their chance of being funded. We note that a shift of even one rank might be significant if it moves the pharmaceutical over the budget threshold where it gets funded. We don't have the financial threshold information to ascertain whether this would be the case.

⁸⁶ Anonymisation meant we were unable to see the medicine names, although we were able to see the rank each received during cost-utility analysis and once on the options for investment list respectively.

Figure 4: Comparison of cost-utility analysis rankings and options for investment list rankings



In general, we found the highest-ranked medicines (on the left of the graph below) moved the least, and that the range of movement increases for lower-ranked medicines (as you move to the right side of the graph).



This lack of movement is worrying because the factors for consideration (and their application to decisions) are supposed to consider equity explicitly, and because the findings from the case studies highlighted that Pharmac is not considering equity implications well.

Staff have most say in assessing and prioritising medicines

Staff appear to have the most say in what is assessed and prioritised. The prioritisation and ranking of medicines lack governance oversight. The board sees the options for investment list and movement within it through the business reporting process. The board typically appears to see summary information and does not have sight of the detailed analysis that lies behind it.

The board seldom makes investment decisions, rarely looks at spending opportunities and challenges, and does not involve itself in decisions on where medicines sit relative to each other on the options for investment list. Ultimately, staff are responsible for the speed at which medicines are assessed, where medicines end up on the options for investment list, and, more recently, whether medicines end up on the declined applications list.

The prioritisation and ranking of medicines seem to happen within a small group, to which other staff are occasionally invited. Among those whose voices are not heard are members of PTAC, specialist advisory committees, the Consumer Advisory Committee (statutory committees), or people with direct personal experience of conditions or disorders. The Māori Advisory Rōpū has also not been invited but we note they are more recently formed.

Another unsatisfactory aspect of this process is that those developing the papers and advice are the same ones who vote on where to rank medicines. We heard about problems with the ability of staff to contribute meaningfully to the process without being distracted from their primary tasks or overloaded with responsibilities. As noted in our interim report, the number of Māori staff is also very low (no more than 10 despite some recent appointments), and no Māori occupy health economics or therapeutic group manager roles. These issues raise questions about the ability for all staff to be able to contribute meaningfully and about the extent to which Pharmac includes advice and analysis from a Te Ao Māori perspective in the decision-making process.

We would certainly expect the routine involvement of relevant staff in making decisions about the prioritisation and ranking of medicines. However, we would also expect a much greater say by the board, PTAC, CAC, the various specialist committees, patient/carer representatives, and potentially the Māori Advisory Rōpū. We would also expect to see a robust assurance process.

Fragmented documentation

Pharmac's documentation of its decision-making process is fragmented. In addition to PTAC minutes, Pharmac produces three critical documents: a technology assessment report, documentation of factors for consideration and a prioritisation dossier. One cause, in our view, of the errors and omissions uncovered above is that Pharmac develops the first two separately. Another is that the factors for consideration are considered separately from the cost-utility analysis. This has the result that the analysis can fail to include important considerations such as different cost-benefit distributions for people in different circumstances. A third and even more fundamental cause is a general lack of economic methodology, analysis that is not rigorous enough, and too few people with expertise in equity matters.

The fragmentation of documents makes it difficult to understand the assessment and decision-making until Pharmac pulls together the prioritisation dossier. This document, however, is a summary of the other material, making it difficult to determine whether its key assumptions are correct. We would have expected Pharmac to prepare documentation containing an integrated, holistic view of each pharmaceutical application; we would expect one document, not three with different foci. From the evidence we have seen, however, this is unlikely to happen until a decision paper goes to either the board, the chief executive or a delegate of the chief executive, depending on the potential financial cost of the decision. As noted earlier, we are concerned that there are multiple decision points generally happening outside the formal oversight of the board and chief executive.

Lack of transparency

Pharmac has been heavily criticised for its lack of openness about how it arrives at decisions and how long it takes to make decisions, a topic covered in our interim report. Patients, families, whānau, clinicians and the wider community want to know their voices are being heard and that Pharmac is considering medicines to treat diseases in a rigorous, timely fashion. They are entitled to know how Pharmac reached a decision, including which factors it applied and how.

Even as a Minister-appointed inquiry, we sometimes struggled to get the information we needed from Pharmac. It would redact documents or not give them to us at all, citing commercial sensitivity, and this often limited the scope and effectiveness of our analysis. We also struggled to establish whether Pharmac's official decision-making procedures were operationalised in practice because of the fragmented nature of the process, the parallel development of documents and, also, because Pharmac does not consider it has made a decision until a final funding agreement has been reached. A further difficulty was determining what documents were produced, and where and how they were used within each decision made. Staff said some discussions on the application of the factors for consideration took place in meetings or situations that were not recorded, which limited our ability to see how the factors were applied.

Lack of timeliness

Pharmac accepts there are problems with the timeliness and transparency of its decisions. A board memo, dated May 2021, acknowledged the public wanted certainty and timeliness in Pharmac's assessment and decision-making – even if the result was to decline a funding application. Pharmaceuticals can undergo multiple assessments and get stuck in consultation loops with pharmaceutical companies. It is particularly hard to know, even for Pharmac, when the clock starts and stops, particularly when the pharmaceutical comes back for further assessment considering new clinical or economic information.

Pharmac's 2020/21 annual report said it took on average 40.90 months from receipt of an application to making a funding decision. It also said it took 16.05 months on average to rank a medicine on the options for investment list, meaning a medicine spent on average 24.85 months on the list awaiting funding. For both these measures there is no indication of whether this time is appropriate and if not what an appropriate timeframe might be. The only annual reporting measure of improvement is to intend to have a downward trend. Given the budgetary limitations within which Pharmac operates, approving a medicine does not necessarily equate to having funds readily available to pursue that investment, therefore, from the review's perspective, the dimension of time is much less important than the questions of health need, available alternatives, populations affected and cost.

The various stages of the assessment process are unclear to the members of the public who rely on Pharmac's website for information. The website's application tracker provides a summary of the decision-making timeline but does not always provide information about why Pharmac has put an assessment on hold or why it is reassessing an application. The information is also hard to follow because the process can often move back and forth between steps and the language is heavily technical. Furthermore, the only information available to the public on the tracker is the clinical specialist committee minutes.

Pharmac publishes the minutes from the clinical assessment stage of the assessment processes. This both polarises and frustrates stakeholders as there is a great deal of transparency around PTAC but the decision to list or the status on the options for investment list is opaque. There are four scheduled PTAC meetings annually, and minutes are not available on the website until three months afterwards, which we agree is too long. Pharmac will publish some other parts of the assessment process but only if requested under the Official Information Act 1982 and then only if it meets internal guidelines. In our view, Pharmac is taking an unjustifiably narrow view of what it can release, which is reinforcing stakeholder views that the agency is too defensive and secretive.

PTAC members told us the associated reading and time commitment is challenging to manage outside of work commitments. Remuneration is less than that of their usual employment, meaning members face costs should they need to arrange clinical cover. This raises both timeliness and equity issues. We think this issue needs to be considered given the importance of the role of PTAC and the need for a range of skills in PTAC and its committees.

More generally we think PTAC should collaborate with Pharmac to agree on the support it needs to function efficiently. We noted feedback from members about the volume of materials provided to PTAC and wonder whether more could be done through effective triaging of applications and summaries of materials. Fundamentally though, it is PTAC and its committees that are best placed to assess what is needed.

Pharmac told us that to release information in advance of an Official Information Act request would require extra resourcing to ensure redaction of any commercially sensitive material. However, it is a principle under the Official Information Act that information should be made available unless there is a good reason for withholding it.⁸⁷ Pharmac could, for example, regularly release key information in a templated one-page form without overstressing its resources.

One reason the public finds Pharmac's application process so confusing is that historically Pharmac has rarely declined funding applications. Instead, it kept them open but inactive on its declined application list in case new clinical evidence became available or it received a revised application, in which case it could reactivate the assessment without requiring a new formal application. Pharmac's online application system, PharmConnect, doesn't show whether an application is active or inactive, creating uncertainty about its status and how much effort Pharmac is putting into assessing applications.⁸⁸

Pharmac accepts the criticism and is exploring two benchmarking goals, one being a decrease in the average time to rank a medicine on its prioritisation list, and the second being a decrease in the average time to decide whether to fund a medicine. As part of setting the benchmarks, Pharmac proposes closing nearly 400 open but inactive applications to clarify their status and reveal the reasons for declining them. It also proposes clearing the backlog of applications awaiting clinical advice or under assessment by asking the expert clinical network for advice, and also by undertaking economic assessments so it can rank their funding priority.

In addition, Pharmac has set a target of 60 business days (12 weeks) to publish the minutes of PTAC meetings and 70 business days (14 weeks) to publish the minutes of committee meetings. Pharmac states publication times are reportedly that long because the meetings discuss technical and complex matters. The review observes that the time taken is too long and Pharmac should reconsider this.

⁸⁷ Official Information Act principle of availability, Section 5.

⁸⁸ <https://pharmac.govt.nz/medicine-funding-and-supply/make-an-application/pharmconnect-make-a-medicine-funding-application/>.

How to improve decision-making

We now set out changes that would, in our view, lead to a significant improvement in Pharmac's decision-making process.

Develop an integrated analytical framework

Pharmac is doing a great deal of useful work, but it should sit within a unifying analytical framework. Our review of the six case studies leads us to conclude that Pharmac needs to:

- strengthen its analytical workforce
- strengthen the distributional elements of cost-benefit analysis
- make its modelling parameters more consistent to improve comparability
- build its capacity and capability in equity analysis
- improve its ability to connect with real-world, on-the-ground health service delivery
- improve assurance processes.

As noted, the factors for consideration have limited meaning unless there is worthwhile analysis to support them and to explore any issues identified in more depth. To ensure appropriate, high-quality analysis throughout the decision-making process, we recommend Pharmac immediately develop a systematic approach to understanding the epidemiology of health issues for Māori and improve its Māori analytical capability. This systematic approach should:

- use New Zealand data to populate models, and if such data is unavailable, provide a rationale for the data chosen and discuss the strengths, limitations and impact of using the data in a New Zealand context
- incorporate existing data on the burden of disease, inequities in outcomes, diagnosis and treatment
- consider where distributions by specific indicators, such as age, gender, stage and histological type, are important
- where data is unavailable, consider health issues in the broader context of health and health care inequities for Māori.

More generally, our view is that Pharmac should set an organisational expectation it will contribute positively to the elimination of inequities in health for Māori and for other priority populations. The review envisages this will require seeking external expertise in a transition period. Also, it should use the advice of its Consumer Advisory Committee and the newly appointed rōpū and work on a systematic approach to understanding the epidemiology of health issues for Māori, while improving its Māori analytical capability.

There is also room to improve communication, both with the public and with applicants. Pharmac could actively release the conclusion of an assessment to the public, preferably in a one-page format, against standardised headings such as population need, other therapies, incremental effectiveness and health gain, other health system considerations, cost and fiscal impact. There is room to provide more certainty to applicants and the public about the time they can expect to wait for a decision to rank, and on what list, even if this timeframe is indicative.

Revise factors for consideration

We gave considerable thought to whether improvements could be made to the factors for consideration so that they achieve their intended purpose. The four domains, as noted, are broad, discretionary and lack a measurable framework when applied. The four domains should be reviewed to ascertain if they continue to be the most appropriate. A proper framework for the application of the factors, and when and how they are to be used, should be developed. This work could be undertaken with the assistance of Pharmac's advisory groups, including the CAC, PTAC, relevant specialist advisory committee members, the Māori Advisory Rōpū, the chief executive and senior leadership team, and external experts.

Build capacity so factors for consideration framework can achieve its purpose

In line with our interim report findings that the number of Māori staff should increase, we consider this must involve investing in Māori capability and building a Māori workforce to support the continuing need for equity-centred technical analysis. A better organised, more transparent framework sitting behind Pharmac's analysis is likely to improve Pharmac's relationship with key stakeholders and the public. The equity-related capacity of Pharmac's non-Māori workforce should also be a focus for development.

Integrate the factors for consideration document and technology assessment report

These two documents need to come together and be supported by an appropriate analytical framework. The technology assessment report needs to include wider considerations of equity in its analysis, alongside the cost-utility analysis. This may involve taking a more systems perspective of funding decisions, given the numerous outside factors – such as access, uptake, ability of the system to handle treatment, social cost and benefit – that have implications for whether such funding is a good idea.

Academics and health economists around the world are rapidly developing distributional cost-effectiveness analysis and ways of incorporating distributional considerations into health economic assessment. These should be referred to and used appropriately by Pharmac. We would expect Pharmac to have the capability and network to be able to recognise, understand and build on Māori and international best practice and to apply it routinely in its own analysis.

Focus PTAC on clinical matters

Some stakeholders – and some PTAC members – expressed concern about the committee's role in assessing cost. They considered the committee lacked the information or expertise to do this, and this function should not be its role. Pharmac says it takes into account the factors for consideration at all steps of the decision-making process, so PTAC could consider cost. In our view, it would be more appropriate for PTAC to focus on clinical assessments (including where clinical assessments feed into the assumptions of cost-utility analysis), particularly given our concerns over the factors for consideration.

Involve the board more in assessment and prioritisation decisions

Our review of board papers indicates the board is involved only occasionally in funding decisions. We would certainly expect the board to have an overview of the options for investment list, but based on the information we have seen, we do not consider it can assure itself it is maintaining and managing a pharmaceutical schedule in a manner that secures the best health outcomes reasonably achievable from pharmaceutical treatment using the available budget. The board should also be fully aware of health needs Pharmac is not meeting.

To achieve these changes, we consider Pharmac needs to put in place a process that provides quality assurance (including hauora Māori and equity 'quality assurance') and advice on assessing and prioritising medicines. The board will need to develop our recommended integrated assessment approach and subsequent analysis. It also needs to create a mechanism to ensure the analytical frameworks are being correctly applied and regularly reviewed.

The board needs to take responsibility for aligning investment decisions with the strategic direction of the wider health sector as governed by the Ministry, Health NZ and the Māori Health Authority. This would be a major step towards Pharmac finding a better way of talking with others in the health sector about strategic investments in medicines within the wider context of health services.

The approach to achieving this is a matter for the board, though it may want to consider establishing a subcommittee. This could consist of select board members and members of other internal committees or external expertise.

Take equity-centred decisions

Even with all the above steps, we still question whether this would be enough to ensure Pharmac makes decisions that truly take account of and reflect equity considerations for Māori, Pasifika, disabled people and other priority populations. These changes are necessary but not sufficient to ensure Pharmac makes equity-centred decisions. Pharmac, like other health sector institutions and funding mechanisms, has underestimated equity considerations. The issues are systemic, and Pharmac is but one small part of the picture. That said, our review of the case studies and Te Whaioranga, Pharmac's Māori responsiveness strategy, shows Pharmac's funding and operational decisions are undermining its equity ambitions.

Equity considerations need to be at the forefront of every decision. Within the health sector, this commitment is clear in governing legislation (including the proposed Pae Ora legislation) and in accountability documents (such as the Minister's letter of expectation for Pharmac's board (section 4). It is well known the starkest, most consistent inequities are between ethnic groups, particularly between Māori and non-Māori/non-Pasifika groups. That said, inequities affecting Pasifika people, people in lower socioeconomic groups and disabled people (tāngata whaikaha) are also clearly evident. In our view, Pharmac must explicitly take into account the impact of its decisions on these groups. The review of the factors for consideration framework and subsequent improvements must put greater, more obvious focus on equity.

Release outcome of decisions in clear terms

We remain concerned about Pharmac's ability to express in clear terms the basis for its decisions. We suggest it release its decisions once made, 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.

6 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.⁸⁹ 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.⁹⁰ Simply put, Māori are twice as likely to die from cancer as non-Māori.

Scientific advances in cancer medicines mean that more people may be able to be treated. But these new medicines come at extremely high cost, and some may not necessarily be more effective in treating and curing cancer.

There is no doubt New Zealand lags behind 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'. In this section we examine the dilemma this presents for Pharmac – how to make the trade-offs between funding expensive cancer medicines and funding other medicines to treat a growing burden of disease for an ageing population, within its capped budget.

The review observes that Pharmac needs to work more collaboratively with others in the health sector, including Te Aho o Te Kahu (the Cancer Control Agency), to decide whether these new cancer medicines provide sufficient benefit to patients to warrant funding, or whether investment is better spent elsewhere in cancer services, or on other diseases altogether.

New Zealand's Cancer Action Plan 2019–2029 recognises the importance of early access to safe and effective cancer medicines as one of many interventions within a 'whole of health system' approach. The plan is guided by four principles: equity-led, knowledge-driven, outcomes-focused, and person- and whānau-centred equity. We agree with this approach and believe it is important to place Pharmac's response to funding cancer medicines within the broader range of interventions required across the health system, from prevention programmes, early diagnoses, and screening through to surgery and other treatment, survivorship, and end-of-life support. The Government also needs to set priorities that address the cancer burden borne by Māori.

It is important to acknowledge the medicines that Pharmac does fund despite attention typically being directed to the cancer medicines it does not fund, particularly in drawing comparisons with Australia.

⁸⁹ Robson et al 2007: <https://www.otago.ac.nz/wellington/otago067759.pdf>.

⁹⁰ Gurney et al 2019: <https://journal.nzma.org.nz/journal-articles/equity-by-2030-achieving-equity-in-survival-for-maori-cancer-patients>;
Gurney S et al 2020: <https://ascopubs.org/doi/10.1200/GO.20.00028>.

Challenges for all health funders

Clearly, the increasing incidence of cancers and inequitable outcomes for Māori and Pasifika are two key issues for New Zealand's health sector and its funders.

Key international trends that affect New Zealand include:

- the rapid growth in the number of new cancer therapies, increasing the pool of medicines for health funders to consider. This also consumes more of a funder's time doing horizon-scanning and assessing new and emerging medicines
- advanced technologies and the trend towards development of 'personalised medicines'⁹¹ using predominantly biological therapeutics to detect and treat cancers rather than rely on chemotherapy treatments
- regulatory changes in overseas markets mean new cancer medicines are coming to the market faster but with less evidence to support the claimed health outcomes. This makes it harder for funders to establish a timely case for investment (given clinically meaningful outcomes are observed after marketing) that balances public demand for access with medical effectiveness and cost-effectiveness
- rising pharmaceutical prices in the global market, driven by these personalised medicines and biological treatments, raising the cost to New Zealand's pharmaceutical budget.

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 can lead to tension. This is not just with pharmaceutical companies, but also with cancer patients and their whānau who are suffering and who want to see more publicly funded cancer medicines. The mere fact of this tension should not necessarily be viewed as a failure of Pharmac. It is an inevitable by-product of making trade-off decisions about which medicines to fund within a limited budget.

As we noted in the context section, the OECD is concerned about the sustainability of member countries' health budgets in the face of the growing number of new, high-priced cancer medicines. Its report noted spending on cancer medicines had been steadily rising because of higher launch prices and increases in the number of patients being treated for longer periods.⁹²

⁹¹ According to the US National Institute of Health, personalised medicine is 'a form of medicine that uses information about a person's own genes or proteins to prevent, diagnose, or treat disease. In cancer, personalized medicine uses specific information about a person's tumour to help make a diagnosis, plan treatment, find out how well treatment is working, or make a prognosis'.
<https://www.cancer.gov/publications/dictionaries/cancer-terms/def/personalized-medicine>.

⁹² OECD 2020: <https://www.oecd.org/health/health-systems/Addressing-Challenges-in-Access-to-Oncology-Medicines-Analytical-Report.pdf>.

Growth in development of cancer medicines

The global pharmaceutical industry has grown significantly from 2017–2021, with 25 percent more pharmaceuticals under development.⁹³ Cancer medicines alone make up 38 percent of those medicines, and biological medicines are expected to dominate the industry by the end of the decade.⁹⁴ The number of immune-oncology medicines (a form of biological therapy) under development grew to 4,720 globally in 2020, a 233 percent increase since 2017.⁹⁵

Biomarkers are able to identify changes to an individual's genes that signal potential disease risks. For example, in breast cancer, the identification of an inherited breast cancer gene during screening can identify patients who are at risk of developing cancer. This information can help people make early decisions about behaviours and treatments.⁹⁶ Developments in such technologies can help with diagnosing disease and enable more effective and timely targeting of treatments.⁹⁷

Treatments, an increasing number of which are biological, are being developed as treatments. However, they also come at significantly higher prices, as discussed below.

Weaker evidence for new cancer medicines

Regulatory changes within the United States (as the largest pharmaceutical producer globally) have also been a driver of growth in cancer medicine development. New Zealand is a relatively small market, and this has significant impacts on the medicines that can be bought.

As noted in our interim report, the United States Food and Drug Administration (FDA) expedited approval programme speeds up approval for 'breakthrough' treatments. Between 2012 and 2017, 55 of 58 (95 percent) FDA cancer medicine approvals entered an expedited programme, leading to a 5.2-year median time to first FDA approval, compared to a 7.1-year median time to first FDA approval for non-expedited medicine approvals.⁹⁸

⁹³ Intelligence Informa Pharma 2021: https://pharmaintelligence.informa.com/~media/informa-shop-window/pharma/2021/files/infographic/pharmard_whitepaper.pdf.

⁹⁴ Ibid.

⁹⁵ Tang et al 2016: <https://www.nature.com/articles/nrd.2018.167>.

⁹⁶ Jackson & Chester 2015: <https://onlinelibrary.wiley.com/doi/full/10.1002/ijc.28940>.

⁹⁷ McCarthy 2017: <https://link.springer.com/article/10.1007/s00125-017-4210-x>.

⁹⁸ Hwang et al 2018: <https://ascopubs.org/doi/10.1200/JCO.2017.77.1592>.

While faster approval is desirable for patients wanting quicker access, most cancer medicine trials now use a proxy (that is, a lower level of evidence) to measure the health benefits of a treatment given the lower regulatory threshold required for approval.⁹⁹ The degree to which this proxy measure is correlated with important and meaningful clinical outcomes, such as improved survival and quality of life, is variable.¹⁰⁰

Rapidly approved medicines with inferior safety, novelty, and/or efficacy could have significant impacts on health outcomes and budgets should they be funded over other treatments. They could leave patients worse off than if they hadn't received that treatment. Between 2014 and 2019, over half the cancer medicines approved by FDA were not directly compared to existing approved therapies, which is a lower standard of assessment than for other pharmaceuticals.¹⁰¹

Rising prices without rising effectiveness

As already noted, along with growth in cancer pharmaceuticals have come rising prices, without necessarily commensurate increases in effectiveness.¹⁰²

Historically, pharmaceutical pricing has followed a life cycle from innovator medicine, to 'me-too' medicine and finally to generic medicine, with the price typically falling at each stage, significantly when it becomes generic.¹⁰³ However, biological medicines may not fall in price over time in the same way traditional pharmaceutical medicines do because most will not be as easily copied or manufactured to produce biosimilars.¹⁰⁴ Also, pharmaceutical pricing for cancer medicines has increased, with resulting pressure on other pharmaceuticals and health services.

⁹⁹ Del Paggio et al 2021: <https://academic.oup.com/jnci/article-abstract/113/10/1422/6184871?redirectedFrom=fulltext>.

¹⁰⁰ Amir et al 2012: <https://www.sciencedirect.com/science/article/abs/pii/S095980491100863X>;
Arciero et al 2021: <https://jamanetwork.com/journals/jamanetworkopen/fullarticle/2776170>;
Belin et al 2020: <https://www.nature.com/articles/s41416-020-0805-y>;
Gyawali et al 2020: [https://www.thelancet.com/journals/eclinm/article/PIIS2589-5370\(20\)30076-6/fulltext](https://www.thelancet.com/journals/eclinm/article/PIIS2589-5370(20)30076-6/fulltext);
Han et al 2014: <https://academic.oup.com/neuro-oncology/article/16/5/696/1192459>;
Kovic et al 2018: <https://jamanetwork.com/journals/jamainternalmedicine/fullarticle/2705082>;
Tibau et al 2018: <https://academic.oup.com/jnci/article/110/5/486/4735106>.

¹⁰¹ Haslam et al 2021: <https://onlinelibrary.wiley.com/doi/full/10.1002/ijc.33231>.

¹⁰² Vokinger et al 2021: <https://jamanetwork.com/journals/jamaoncology/article-abstract/2781390>.

¹⁰³ 'Me-too' pharmaceuticals are like pre-existing pharmaceuticals but with minor modifications, typically for management of side effects, compliance, cost, and incremental improvements in efficacy. 'Me-too' medicines entering the market do not always cause the price to fall.

¹⁰⁴ Intelligence Informa Pharma 2021: https://pharmaintelligence.informa.com/~media/informa-shop-window/pharma/2021/files/infographic/pharmard_whitepaper.pdf.

Analysis of FDA cancer medicine trials over 1994–2013 showed increasing prices yet decreasing gains in effectiveness for some indications.^{105,106} Other analyses suggest prices of cancer medicines do not always reflect their benefit and that the most expensive medicines are not necessarily the most beneficial.¹⁰⁷

What does this mean for health funders?

The growth in new cancer medicines, weaker evidence for cancer medicines receiving regulatory approval, and rising prices of cancer medicines without commensurate increases in effectiveness pose difficult challenges for health funders.

For Pharmac, this likely means:

- greater horizon-scanning required and an increase in the number of cancer medicines that must be assessed, taking away already constrained resources
- greater impacts of cancer medicines on the national pharmaceutical budget, forcing trade-offs within, and outside of, the cancer therapeutic group
- fewer opportunities to make savings to fund medicine investments, forcing change to long-term decision-making, and potentially foreclosing future investment opportunities
- increased responsibility for managing public expectation of access to new cancer medicines, which takes away from already constrained resources as well as potentially lowering the level of public confidence in Pharmac.

What Pharmac does well is to develop a ranked list of pharmaceutical investments, negotiate commercial solutions to help resolve budgetary issues and maximise value from any investment in cancer pharmaceuticals. It also supports its assessment with independent medical advice.

The importance of ensuring independent clinical advice

The importance of maintaining strong, independent, expert advice from Pharmac's specialist advisory committee on cancer pharmaceuticals increases as clinical judgement is required to sift through different and shifting levels of evidence. Through canvassing the literature, the review identified a number of instances where scrutiny of evidence for cancer pharmaceuticals reveals concerns. For example:

¹⁰⁵ Cressman et al 2015: <https://www.ncbi.nlm.nih.gov/pmc/articles/PMC4492232/>.

¹⁰⁶ Indication here refers to a condition which makes a particular treatment or procedure advisable. Some medicines are effective in treating multiple conditions (indications) and are therefore publicly funded for the multiple conditions (indications).

¹⁰⁷ Del Paggio et al 2017: [https://www.thelancet.com/journals/lanonc/article/PIIS1470-2045\(17\)30415-1/fulltext](https://www.thelancet.com/journals/lanonc/article/PIIS1470-2045(17)30415-1/fulltext); Trotta et al 2019: <https://bmjopen.bmj.com/content/9/12/e033728>.

- In Canada, no or marginal benefits for new cancer medicines. In this cohort study most medicines entered the market without evidence of benefit for survival or quality of life. When there were survival gains over existing treatment options or placebo, they were often marginal.¹⁰⁸
- Technical issues with trial design, conduct, and analysis may lead to overstated benefits. International studies note there is concern that many trials are at high risk of bias because of their design, conduct, or analysis.¹⁰⁹ There is also concern about publication bias. Only those studies with significant findings are published, leading to literature that does not necessarily reflect the true effect of the treatment (ie, there could be a lot of null findings that have not been published, resulting in an overstating of the significance of the effect).
- Potential conflicts of interest in authoring of major cancer medicine trial findings. One study found that editorials in top oncology journals were frequently authored by experts with financial conflicts of interest, including direct financial conflicts.¹¹⁰
- Proxy outcomes used in clinical trials to gain regulatory approval do not always show clinically meaningful information.¹¹¹ As discussed above in remarks on weaker evidence, benefits claimed by trials are not necessarily realised in real life, or significant in a clinical context when considering the health-related gains to the patient.

We also note there is often a lack of ethnic diversity and poor consideration of equity in trial populations, which limits the appropriateness of the trials to inform Pharmac funding decisions, given New Zealand's population mix and needs.¹¹²

Significant investment over the last decade

Pharmac is often criticised in the media for not funding cancer medicines or being slow to fund cancer medicines that other countries, predominantly Australia, fund. Since July 2010, Pharmac has purchased an additional 52 cancer medicines: 27 of these medicines are new to New Zealand, and another 25 have had their eligibility criteria broadened to allow a wider group of patients access. A list of these medicines and the year they were funded is in the table below.

¹⁰⁸ Meyers et al 2021: <https://jamanetwork.com/journals/jamainternalmedicine/fullarticle/2776285>.

¹⁰⁹ Although importantly, some bias is unavoidable due to the inherently complex nature of cancer trials. See Patel et al 2015: [https://www.jclinepi.com/article/S0895-4356\(15\)00277-2/fulltext](https://www.jclinepi.com/article/S0895-4356(15)00277-2/fulltext); Prasad & Booth 2019: [https://www.thelancet.com/journals/lanonc/article/PIIS1470-2045\(19\)30744-2/fulltext](https://www.thelancet.com/journals/lanonc/article/PIIS1470-2045(19)30744-2/fulltext).

¹¹⁰ Sharma et al 2021: <https://jnccn.org/view/journals/jnccn/19/11/article-p1258.xml>.

¹¹¹ Amir et al 2012: <https://www.sciencedirect.com/science/article/abs/pii/S095980491100863X>; Gyawali et al 2020: [https://www.thelancet.com/journals/eclinm/article/PIIS2589-5370\(20\)30076-6/fulltext](https://www.thelancet.com/journals/eclinm/article/PIIS2589-5370(20)30076-6/fulltext); Han et al 2014: <https://academic.oup.com/neuro-oncology/article/16/5/696/1192459>; Kovic et al 2018 <https://jamanetwork.com/journals/jamainternalmedicine/fullarticle/2705082>.

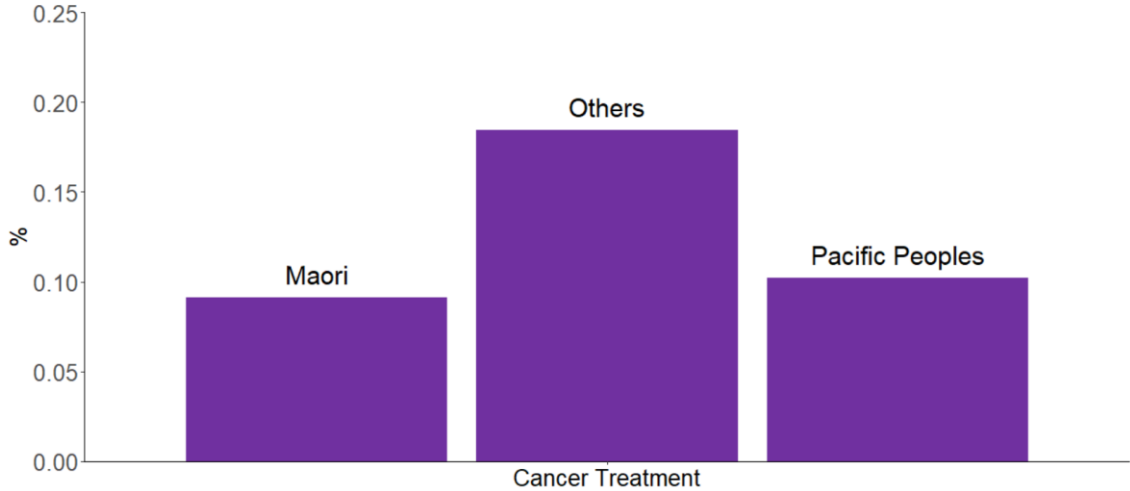
¹¹² The appropriateness of international trials and the implications of their use in Pharmac funding decisions are discussed in depth in the chapter on decision-making.

Table 10: New investment in cancer treatments by Pharmac since 1 July 2010

Financial year	Pharmaceutical (used to treat)
2011	Bortezomib (multiple myeloma), Capecitabine , Erlotinib (metastatic non-squamous non-small cell lung cancer), Gemcitabine , Rituximab , Sunitinib Maleate (Renal cell carcinoma), Temozolomide (glioblastoma), Thalidomide (multiple myeloma)
2012	Docetaxel , Lapatinib (Metastatic breast cancer), Pazopanib (Metastatic renal cell carcinoma), Rituximab
2013	Capecitabine (Metastatic breast cancer, colorectal cancer), Gefitinib (Lung cancer), Gemcitabine Hydrochloride (Metastatic breast cancer, Hodgkin's Disease, soft tissue sarcoma), Irinotecan (Advanced pancreatic cancer, small bowel cancer), Oxaliplatin (Advanced pancreatic cancer, small bowel cancer, advanced oesophagogastric cancer), Pegaspargase (Acute lymphoblastic leukaemia), Sunitinib (Cancer), Vinorelbine (Breast cancer, non-small cell lung cancer)
2015	Abiraterone (Advanced prostate cancer), Azacitidine (Myelodysplastic syndromes [MDS]), Benzydamine hydrochloride (Oral mucositis in Cancer patients), Bicalutamide (Prostate cancer), Everolimus (Sub-ependymal giant cell astrocytoma [a type of Brain tumour]), Lenalidomide (Multiple myeloma), Nilotinib (Chronic myeloid leukaemia), Zoledronic acid (Hypercalcaemia and cancer-related bone metastases)
2017	Nivolumab (Unresectable metastatic melanoma), Obinutuzumab (Chronic lymphocytic leukaemia), Pembrolizumab (Unresectable metastatic melanoma), Pertuzumab (Metastatic breast cancer), Rituximab (Hairy cell leukaemia and re-treatment of chronic lymphocytic leukaemia), Temozolomide (High grade gliomas and well differentiated neuroendocrine tumours)
2018	Bendamustine (Chronic lymphocytic leukaemia (CLL) and indolent non-Hodgkin's lymphoma (iNHL), Cetuximab (Head and neck cancer), Mercaptopurine (Acute lymphoblastic leukaemia), Pemetrexed (Mesothelioma and non-small cell lung cancer), Zoledronic acid (Early breast cancer)
2019	Ruxolitinib (Myelofibrosis)
2020	Alectinib (ALK positive advanced non-small cell lung cancer), Bortezomib (Multiple myeloma and amyloidosis), Fulvestrant (Locally advanced or metastatic oestrogen receptor positive breast cancer), Lenalidomide (Newly diagnosed multiple myeloma post-autologous stem cell transplant), Olaparib (BRCA-mutated relapsed ovarian cancer), Palbociclib (HR-positive, HER2-negative locally advanced or metastatic breast cancer), Rituximab (Cancer, blood and autoimmune conditions), Ruxolitinib (Myelofibrosis, lower-risk [Intermediate-1]), Trastuzumab emtansine (HER-2 positive metastatic breast cancer), Venetoclax (with Rituximab) (Chronic lymphocytic leukaemia)
2021	Bendamustine (Relapsed or refractory Hodgkin's lymphoma), Febuxostat (Prevention of tumour lysis syndrome)

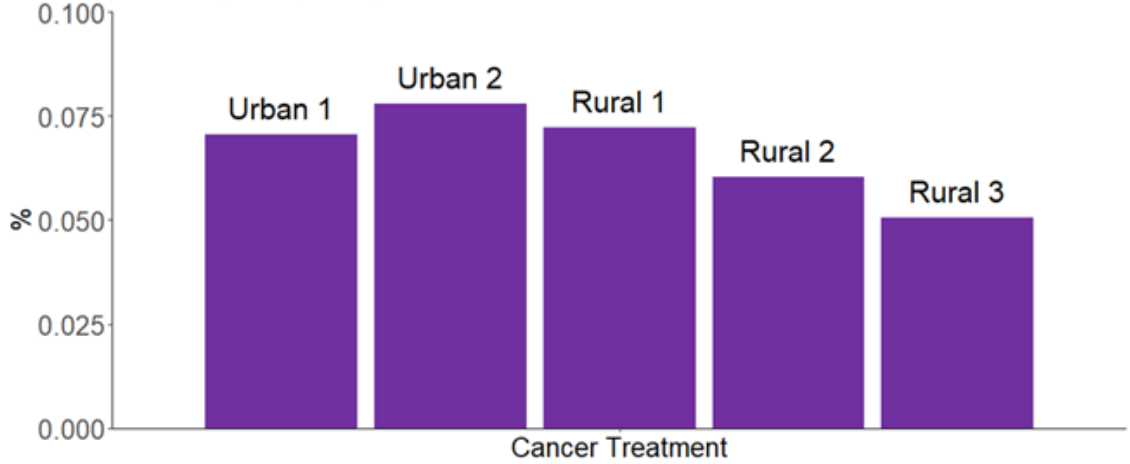
Cancer treatments account for a large proportion of Pharmac’s new investment spend. A snapshot of expenditure on new listings over the past decade shows cancer treatments accounted for 32 percent of expenditure (\$70 million in 2019/20) but less than 1 percent (6,700) of the patients treated.¹¹³ An analysis of the distribution of those dispensed new cancer medicines shows that Māori and Pasifika and those living in rural areas have received new cancer pharmaceuticals at much lower rates than the rest of the population. Although the statistics are stark, the review can’t draw conclusions without knowing more about the different types of cancer and the different types of treatments accessed. On the surface, this needs exploring further.

Figure 5: Age standardised treatment rates for new cancer listings by ethnicity (2019/20 financial year)



Source: Sapere analysis, using Pharmaceutical Claims data

Figure 6: Age standardised treatment rates for new cancer listings by Geographical Classification for Health rurality (2019/20 financial year)



Source: Sapere analysis, using Pharmaceutical Claims and GHC rurality (Whitehead, 2021)

¹¹³ Based on 2019/20 expenditure on new listings made between 1 July 2010 and 30 June 2020.

Pharmac employs the same medicine application assessment framework across all medicines, regardless of the therapeutic area. What does differ is that funding applications for cancer medicines (and rare disorders) can be made to Pharmac at the same time as they are assessed by Medsafe. Normally, medicines would have to be approved by Medsafe first. Pharmac predicts the parallel assessment process will reduce the time it takes for cancer medicines to be considered by Pharmac by 12 to 15 months.¹¹⁴

Submissions and reports from stakeholder groups show there is generally an expectation from consumers and stakeholders that more treatments (particularly those publicly funded in Australia, Canada, and the United Kingdom) will be publicly funded in New Zealand, and also show that there is concern about the level of access New Zealanders have to new medicines. As highlighted, these concerns are often played out in the media.

Does New Zealand lag other countries?

There is no doubt New Zealand lags many other countries in the provision of cancer medicines. Past comparison of funded access to medicines (across all therapeutic groups) between New Zealand and other countries (like Australia) often portrayed a domestic shortfall in terms of the level of medicines being funded and the speed at which the medicines are funded. However, these discussions on access rarely consider health benefits, risks, affordability, and the likely impact on population health outcomes.

A 2021 Merck Sharp & Dohme-funded report published by the Swedish Institute of Health Economics shows New Zealand ranks low compared to other high-income markets in the Asia-Pacific region.¹¹⁵ Of 38 new cancer medicine indications approved by the FDA between 1998 and 2020, New Zealand only reimbursed around 30 percent. This is typically lower than other high-income markets of the Asia-Pacific region.¹¹⁶ Cancer medicine expenditure per capita and cancer medicine expenditure per cancer case in New Zealand in 2019 were also typically lower than other high-income Asia-Pacific markets.

Paying for more cancer medicines will not necessarily benefit New Zealanders. However, it is important for Pharmac to recognise gaps and test them rigorously to ensure options are well considered. Figure 7 below shows the number of cancer medicines publicly funded across Australia and New Zealand in 2016. Of the identified cancer medicines, 89 were available in both countries. Australia had publicly funded access to 35 cancer medicines that New Zealand did not. Of the 35, clinical trial data included survival outcomes for 26.¹¹⁷ New Zealand had publicly funded access to 13 medicines Australia did not.

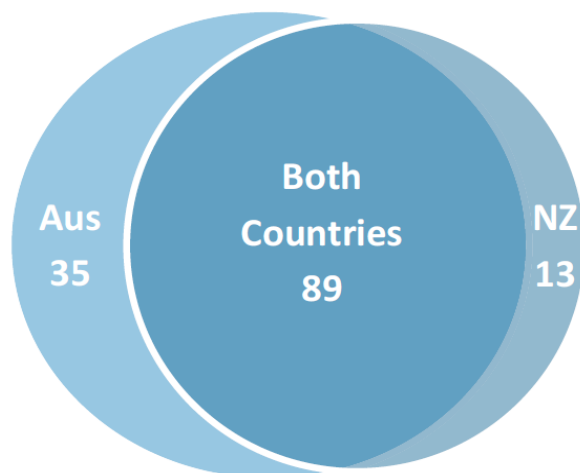
¹¹⁴ Timeliness is a key issue for consumers, drug companies, and the general public with respect to the Pharmac decision making process. As such, it is discussed in more detail in the decision making section. Pharmac's prediction of the reduction in consideration time due to the parallel approval process can be found here: <https://pharmac.govt.nz/medicine-funding-and-supply/the-funding-process/from-application-to-funded-medicine-how-we-fund-a-medicine/cancer-medicine-funding-parallel-assessment/>, accessed 17 February 2022.

¹¹⁵ The Asia-Pacific region refers to 14 countries and locations (called markets), split into two groups based on World Bank classifications. The first group is high-income markets, which includes Australia, Hong Kong, Japan, New Zealand, Singapore, South Korea, and Taiwan. The other group is middle-income markets, which includes China, India, Indonesia, Malaysia, the Philippines, Thailand, and Vietnam.

¹¹⁶ Hofmarcher et al 2021: https://ihe.se/wp-content/uploads/2021/11/IHE-Report-2021_3_.pdf.

¹¹⁷ Evans et al 2016: <https://www.sciencedirect.com/science/article/pii/S0093775416300586?via%3Dihub>.

Figure 7: Number of cancer medicines funded by Australia and New Zealand as of 30 April 2016



Source: Evans et al 2016: <https://www.sciencedirect.com/science/article/pii/S0093775416300586?via%3Dihub>

The authors concluded there were three medicines in Australia that showed clinically relevant improvement in both progression-free and overall survival. At the time of the study (2016), this suggested funding more cancer medicines in New Zealand to achieve parity with Australia may not be cost-effective and in some cases would leave New Zealanders with worse health outcomes. After this analysis Pharmac announced funding for several medicines, including one for treating lung cancer.

More recent work by Te Aho o Te Kahu has also compared New Zealand to Australia.¹¹⁸ This analysis found 71 individual cancer medicines that were funded in both countries. It also found 72 cancer medicines that were funded in Australia and either not funded at all in New Zealand, or not funded for the same specific cancer types. There were 14 cancer medicines identified as funded in New Zealand and not via the PBS in Australia, but at least some of these may be available via other public hospital funding mechanisms. In contrast to the work by Evans et al, Te Aho o Te Kahu identified 19 specific gaps for cancer medicines (just for solid tumours) that are likely to be associated with substantial clinical benefit, and that there may be additional gaps for haematological cancers that require further analysis.

Comparing the work by Evans et al and Te Aho o Te Kahu paints a telling picture of the changing state of cancer medicines over time in New Zealand. While New Zealand may not have previously been missing out by not achieving cancer medicine funding parity with Australia, the gap has widened and New Zealanders are now possibly missing out on important and clinically significant treatments for cancer. The question that remains for Pharmac, given this information, is whether the clinically significant treatments provide sufficient benefit to cancer patients to justify the high and rising cost of investment, or whether the money is best spent elsewhere in cancer services, or elsewhere on other pharmaceuticals.

¹¹⁸ Te Aho o Te Kahu, 2022. Analysis of the availability of cancer medicines in Aotearoa New Zealand (unpublished).

Pharmaceutical companies and cancer patient support groups in their submissions pointed to access around the world. But the review notes approval does not necessarily mean access. Countries like the United States and Canada have significantly inequitable health systems as access means having health insurance, and health insurance generally limits what it will pay, meaning the consumer bears extra costs. This issue has grown to the extent that the term 'financial toxicity' has been coined, as another side effect of cancer treatments. Patients face financial pressure and even bankruptcy to pay for cancer pharmaceuticals,¹¹⁹ experience stress and may be unable to complete the full course of treatment. In New Zealand patients self-funding treatments can have similar experiences. As not every person with cancer has the ability to self-fund or fundraise for treatment, privately funded treatment will increase the inequities in access to new medicines already observed for Māori and Pasifika populations and may introduce new inequities for other population groups.

The review believes access needs to increase and in a way that rebalances to achieve equity. However, it views the number of pharmaceuticals as only one measure, and the focus should be on the small number of material gaps identified by clinicians, patient groups and pharmaceutical companies. Pharmac is aware of those gaps. The review does not know the cost of investing in those medicines as pricing is secret.

A corollary of fewer cancer medicines is that clinical trials in cancer can be more difficult to run in New Zealand as the standard treatment may differ from that of other countries. The review recognises the importance of clinical trials, and would like to see more, including for cancer medicines both old and new. But trials must be relevant to New Zealand. The review also recognises the desire of oncologists to have new medicines and to participate in international clinical trials. Pharmac should consider using post-marketing monitoring of newly listed medicines for a two-year trial period, with firm steps to delist and redirect investment, if they prove to be no more efficacious.

Are more medicines the answer?

In our view it does not make sense for Pharmac or the wider health sector to invest in treatments that have not been proven to provide clinically significant outcomes for patients, particularly when compared with currently funded treatments.

Submissions referenced the National Health Service's Cancer Drugs Fund in the UK as it had historically provided patients with access to the latest cancer treatments through large investment. But multiple studies concluded the Cancer Drug Fund delivered poor value to society at a high cost (before it underwent reform in 2016).¹²⁰

¹¹⁹ Tran & Zafar 2018: <https://pubmed.ncbi.nlm.nih.gov/29911114/>.

¹²⁰ Aggarwal et al 2017: [https://www.annalsofoncology.org/article/S0923-7534\(19\)32125-8/fulltext](https://www.annalsofoncology.org/article/S0923-7534(19)32125-8/fulltext);
Dixon et al 2016: <https://www.sciencedirect.com/science/article/pii/S1098301516300183>.

The Cancer Drug Fund was established in 2011 as an election promise from the Conservative Party to pay for cancer medicines the NHS was not funding at the time.¹²¹ Of 47 Cancer Drug Fund approved indications/uses for medicines, only 9 (18 percent) met American Society of Clinical Oncology and European Society for Medical Oncology criteria for clinically significant benefit.¹²² The National Institute for Health and Care Excellence had previously rejected 26 (55 percent) of the 47 indications because they did not meet cost-effectiveness thresholds.¹²³

University of York researchers estimated that in 2014–2015, the Cancer Drug Fund would cost over five times more than the benefit it would deliver due to the lack of thresholds on the money able to be spent per incremental benefit achieved.¹²⁴

The National Audit Office reported the National Health Service overspent the allocated Cancer Drug Fund budget by 38 percent in the same period of 2014–2015, with the cost of the Fund rising by 241 million GBP.¹²⁵ An Innovative Medicines Fund replaced the Cancer Drug Fund in July 2021, incorporating funding for cancer and rare disorders.

Other countries also have special funding arrangements for oncology medicines.¹²⁶ In Canada, some provincial and territorial governments have cancer agencies with earmarked budgets to deliver cancer treatments. In Italy there is a 500 million EUR annual fund (renewable every three years) for innovative oncology medicines (defined through certain criteria). Chile has established a fund for high-cost treatments and diagnostics and has signalled for cancer to have its own fund. Greece and Malta also have earmarked funding for oncology medicines. It is not obvious how effective these special funding arrangements for oncology medicines are.

¹²¹ UK Government 2010: https://assets.publishing.service.gov.uk/government/uploads/system/uploads/attachment_data/file/78977/coalition_programme_for_government.pdf.

¹²² Aggarwal et al 2017: [https://www.annalsofoncology.org/article/S0923-7534\(19\)32125-8/fulltext](https://www.annalsofoncology.org/article/S0923-7534(19)32125-8/fulltext),

¹²³ Aggarwal et al 2017: [https://www.annalsofoncology.org/article/S0923-7534\(19\)32125-8/fulltext](https://www.annalsofoncology.org/article/S0923-7534(19)32125-8/fulltext).

¹²⁴ This is due to the opportunity cost of investment – by investing in cancer treatments, NHS capacity is taken up and other patients (within, and external to cancer) are closed out of accessing treatments. See Hawkes 2015: <https://www.bmj.com/content/350/bmj.h955> for more.

¹²⁵ National Audit Office 2015: <https://www.nao.org.uk/report/investigation-into-the-cancer-drugs-fund/>.

¹²⁶ OECD 2020: <https://www.oecd.org/health/health-systems/Addressing-Challenges-in-Access-to-Oncology-Medicines-Analytical-Report.pdf>.

A high opportunity cost for other cancer services

Pharmaceuticals are only one type of conventional cancer intervention provided by the health care system. Others include cancer surgery (physical removal of the cancer), radiation therapy and radiofrequency ablation and related focused energy treatments.¹²⁷ There is a clear government priority to improve care through the Faster Cancer Treatment pathways.¹²⁸ Screening programmes, which are very cost-effective, need to be improved. Also, in need of improvement are palliative and other care, as well as access to imaging and other diagnostics. Pharmac 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.

The review explored the literature on societal preferences to help it come to its view. We found:

- Societal preferences for health spending are complex, with studies having mixed results.¹²⁹
- The majority of the literature supports the preference for prioritisation of severity. People consistently give priority to severe illness, while the results for end-of-life preferences are mixed.¹³⁰
- An Australian study found evidence of a societal priority for severity of disease, while finding no compelling evidence for prioritising end-of-life treatments.¹³¹

The question as to whether there are special characteristics of cancer recognised by society is a little clearer. A study of cancer preferences showed people thought cancer was ‘special’ and deserving of prioritisation. However, when presented with the cost of the funding, results were inconsistent.¹³²

This finding had an important policy implication. It is likely the public will receive clear messages about the benefits of cancer pharmaceuticals but opaque messages on their costs (as costs can’t be disclosed), making it difficult for them to weigh the value of any further, expensive investment.

¹²⁷ Radiofrequency ablation is when radiofrequency waves are sent out from a needle-like probe inserted in the body. The waves cause nearby cells to die, and the immune system subsequently removes them. This causes an internal reaction and generally results in shrinkage of the cancer (see Russell: <https://www.hopkinsmedicine.org/health/treatment-tests-and-therapies/radiofrequency-ablation>).

¹²⁸ Of those with cancer who had surgery as a first treatment, 88 percent of patients received their surgery within the 62-day timeframe, and 82 percent of Māori received their surgery within the 62-day timeframe. In 2019/20, through the FCT, 11 percent of all patients had radiation as a first treatment (including concurrent therapy), 71 percent received radiation therapy within the 62-day timeframe, and 80 percent of Maori received radiation therapy within the 62-day timeframe (Te Aho o Te Kahu 2021: [https://teaho.govt.nz/static/reports/state-of-cancer-in-new-zealand-2020%20\(revised%20March%202021\).pdf](https://teaho.govt.nz/static/reports/state-of-cancer-in-new-zealand-2020%20(revised%20March%202021).pdf)).

¹²⁹ Shah et al 2018: <https://eprints.whiterose.ac.uk/128686/5/End%20of%20life%20review%20accepted%20manuscript%20130318%20.pdf> – 8 of 25 studies were consistent with a premium for end-of-life patients and 11 studies were not; the remaining 4 studies were inconclusive.

¹³⁰ Morrell et al 2017: <https://www.ncbi.nlm.nih.gov/pmc/articles/PMC5548817/>.

¹³¹ Chim et al 2017: <https://www.ncbi.nlm.nih.gov/pmc/articles/PMC5332102/>.

¹³² Morrell et al were unable to find consistent support for a preference for health gains to cancer patients when money was included in the discussion and conclude there is a contradiction between findings and the popular view of cancer.

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 that 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 but that 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.

7 Rare disorders

Introduction

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.¹³³ 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.¹³⁴

The effects on individuals' material and social quality of life (and 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 health care and medicines, many people find even getting a diagnosis incredibly difficult. Internationally, we know inequitable access to health care 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.

In this section, we examine how Pharmac approaches assessment and funding of 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. We also consider decision-making processes in light of what we saw in the case studies discussed in the decision-making section. We note the need to involve patients in decision-making and to make the process more transparent.

¹³³ HealthiNZ 2020: https://raredisorders.org.nz/assets/VOICE-OF-RARE-DISORDERS_WhitePaperV5.pdf.

¹³⁴ Pharmac 2019: <https://pharmac.govt.nz/assets/2019-Report-Funding-Medicines-for-Rare-Disorders-PDF-version.pdf>.

A coordinated and inclusive approach

Access to medicines is just one of many challenges that people with rare disorders face. New Zealand does not have a coordinated approach for rare disorders. The review feels this is needed. A cross-agency approach, led by the Ministry, with input from those with lived experience would give formal recognition of the special difficulties patients and their families face.

Australia has such a document, the National Strategic Action Plan for Rare Diseases, as does the United Kingdom with its Strategy for Rare Diseases (see Table 11).¹³⁵

¹³⁵ England, Scotland, Wales and Northern Ireland have all developed plans based on this strategy.

Table 11: Overview of UK and Australian rare disease strategies

	United Kingdom's Strategy for Rare Diseases¹	Australia's National Strategic Action Plan for Rare Diseases²
Knowledge and awareness	<p>Increase awareness of rare disorders among health professionals to support better diagnosis and care</p> <ul style="list-style-type: none"> • increase awareness of rare disorders • increase awareness of treatment needs • increase use of genomic testing and digital tools 	<p>Increase awareness and education</p> <ul style="list-style-type: none"> • increase awareness of rare disorders, including prevention measures • provide access to information to empower those living with rare disorders • develop disorder workforce strategy
Care and support	<p>Help patients get a diagnosis faster</p> <ul style="list-style-type: none"> • support patients with non-genetic rare disorders to reach diagnosis faster • improve diagnosis rates, including using advanced diagnostic technologies and tools <p>Better coordination of care</p> <ul style="list-style-type: none"> • improve coordination of care and support • use advances in technology and digital tools to access services and share information 	<p>Improve integration of care and support</p> <ul style="list-style-type: none"> • provide integrated, person-centred care and support • ensure timely and accurate diagnosis • facilitate increased reproductive confidence • provide equitable access to the best available health technology • integrate mental health, and social and emotional wellbeing, into rare disorder care and support
Research and data	<p>Improve access to specialist care, treatments and pharmaceuticals</p> <ul style="list-style-type: none"> • boost research and innovation into new treatments • review funding processes for treatments 	<p>Develop a national, coordinated and systematic approach to research and data collection</p> <ul style="list-style-type: none"> • enable coordinated and collaborative data collection • develop a national research strategy for rare disorders • ensure research is collaborative and person-centred • translate research and innovation into clinical care
Equity	<p>The UK-wide vision for rare diseases includes a commitment that all four UK countries will 'promote equity of access – allowing everyone with a rare disease to follow a clear, well defined care pathway' and to 'deliver effective interventions... equitably and sustainably'.</p> <p>However, there are no priority populations identified and, in the actions, equity of access is only explicitly mentioned in regard to measuring equity of access to molecular tests to maintain UK Genetic Testing Network diagnostic studies.</p>	<p>Equity of access is one of the three foundations of the action plan. To do this, the plan identifies priority populations to focus actions for access and equity (p 12) including:</p> <ul style="list-style-type: none"> • aboriginal and Torres Strait Islander people • rural and remote populations • people from culturally and linguistically diverse backgrounds • people experiencing socioeconomic disadvantage. <p>As a result of this focus, actions include targeting messages and ensuring culturally safe and appropriate care.</p>

1 UK Department of Health 2021. See https://assets.publishing.service.gov.uk/government/uploads/system/uploads/attachment_data/file/260562/UK_Strategy_for_Rare_Diseases.pdf.

2 Australian Department of Health 2020. See <https://www.health.gov.au/resources/publications/national-strategic-action-plan-for-rare-diseases#:~:text=The%20National%20Strategic%20Action%20Plan%20for%20Rare%20Diseases%20is%20the,plan%20addresses%20this%20common%20ground.>

Rare Disorders New Zealand told the review it had considered the need for a framework and identified seven elements it should contain: faster, more accurate diagnosis; a national rare disorder registry; planned pathways for care; access to appropriate disability and social support services; more accessible rare disorder medicines; more and better targeted research; and greater workforce development. Such a framework would, it argued, require relevant agencies to work together to provide holistic care and support throughout all phases of diagnosis, treatment, and follow-up care.¹³⁶ The review endorses this approach.

No agreed definition

New Zealand has no agreed definition of what rare disorders are, and globally there is no consensus either.

Pharmac uses its own definition: ‘a clinically defined disorder that affects an identifiable and measurable patient population of less than 1 in 50,000 people’, though its Rare Disorders Advisory Committee will consider, by exception, applications outside this threshold. This is higher than the average threshold of one in 2,500 people uncovered in a review of definitions used around the world (see Table 12).¹³⁷ The review identified 296 definitions used by 1,109 organisations in 32 countries.

Severity of disease is also used by some countries. Pharmac’s definition is the same as that used by Australia’s Life Saving Drugs Program and in Scotland’s ultra-rare definition. Rare Disorders New Zealand said it considered the European Union definition of fewer than one in 2,000 people to be the right one to adopt.

We recommend the Ministry develop an official definition of rare disorders when it develops the rare disorders framework mentioned above. This would also give a profile to a group of people who otherwise feel invisible and are marginalised in the health and disability system.

¹³⁶ https://raredisorders.org.nz/assets/HSC-submission-from-Rare-Disorders-NZ_-Sue-Haldane-Petition.pdf

¹³⁷ Richter et al 2015: <https://pubmed.ncbi.nlm.nih.gov/26409619/>.

Table 12: Comparison of definitions used by different jurisdictions

Country/region	Definition (prevalence ¹³⁸ threshold)
Global average ¹	1 in 2,500 people
Pharmac	<1 in 50,000 people
Australia	<5 in 10,000 people (1 in 2,000 people) ≤1 in 50,000 people (Life Saving Drugs Program)
Japan	<50,000 people (about 1 in 2500 people)
Scotland	≤5 in 10,000 people (1 in 2000 people) (rare) ≤1 in 50,000 people (ultra-rare)
United Kingdom	<1 in 2,000 people
European Union	≤1 in 2,000 people
Singapore	<1 in 2,000 people
United States	<200,000 people (about 1 in 1667 people)

1 Source: Richter et al 2015, a systematic review that identified 296 definitions from 1109 organisations across 32 jurisdictions.

Prevalence

We could find no nationally collected data on the prevalence of rare disorders (or any demographic breakdown of the people living with rare disorders for that matter). International prevalence estimates vary considerably (anywhere from 1.5 percent to 6.2 percent of the population) – just as they vary for the proportion that are treatable (2.4 percent to 5.0 percent) and for the number of disorders themselves (between 5,000 and 9,603).¹³⁹

We applied the international percentage ranges for prevalence (1.5–6.2) and for treatability (2.4–5.0) to Statistics New Zealand’s 2021 population estimate of 5.12 million people (Table 13), to estimate the prevalence of rare disorders in this country and the proportion that are treatable. This estimate informed us there could be between 76,839 and 317,601 people living with a rare disorder in New Zealand. Pharmac data provided to the review showed that in 2019–20, 426 people obtained medicines for rare disorders funded by the agency. Even at the lower end, our estimates confirm there is significant unmet need.

The same absence of nationally collected data makes it all but impossible to determine the prevalence of rare disorders among any demographic group, including Māori. Overseas studies suggest Indigenous populations may take longer to be diagnosed with rare disorders, if diagnosed at all, compared to non-Indigenous populations. Reasons include less access to clinical genetic service providers because of lack of referrals, referral bias, remote location, cost barriers, unavailability of culturally safe services, lack of appropriate consent processes, and lack of assurance of Indigenous data sovereignty in the case of genetic testing.

¹³⁸ Prevalence is the proportion of persons in a population who have a particular disease or attribute at a specified point in time or over a specified period of time.

¹³⁹ Prevalence is the proportion of persons in a population who have a particular disease or attribute at a specified point in time or over a specified period of time.

Table 13: Estimated number of New Zealanders with rare disorder and percentage with a treatable disorder

Prevalence	Estimated number of people with a rare disorder ¹⁴⁰	Estimated number of people with a treatable rare disorder (2.4%) ¹⁴¹	Estimated number of people with a treatable rare disorder (5%) ¹⁴²
1.5%	76,839	1,884	3,842
2%	102,452	2,459	5,123
3%	153,678	3,688	7,684
4%	204,904	4,918	10,245
5%	256,130	6,147	12,807
6.2%	317,601	7,622	15,880

Assessing and funding medicines

Pharmac assesses and funds medicines for rare disorders through two mechanisms:

- **Pharmaceutical schedule:** applications for medicines for rare disorders to be considered by Pharmac for assessment and funding are made the same way as all other medicines.
- **Exceptional circumstances framework:** this framework is for individuals who have exceptional clinical circumstances and are seeking medicines not on the pharmaceutical schedule or that are on the schedule but are not listed for their condition or clinical circumstances. Pharmac assesses applications (including those for patients with rare disorders) for medicines against its named patient pharmaceutical assessment policy (NPPA). Only a clinician can make an application – and only for the individual patient in his or her care – and all funded alternative treatments must have been tried.

In 2014 Pharmac piloted an allocated contestable funding pool. Medicines approved from this process continue to be funded, but no new applications are accepted and there are no plans to run another contestable funding process. The contestable fund allocated up to \$5 million from within the Combined Pharmaceutical Budget (existing funding) and was intended to stimulate competition among suppliers of medicines treating rare disorders within Pharmac’s definition. Eight companies submitted 28 funding applications for unfunded rare disorder medicines, 10 of which were approved and nine of which were added to the pharmaceutical schedule. The \$5 million allocated for contestable funding has never been fully utilised; this is in part because approving one more application would have exceeded the \$5 million threshold.

¹⁴⁰ Based on Statistics New Zealand estimated New Zealand population 2021 of 5,122,600 people.

¹⁴¹ Based on Ferreira’s (2019) estimate that 2.4% of rare disorders have a treatment.

¹⁴² Based on the commonly quoted estimate that 5% of rare disorders have a treatment.

In 2017 consultancy Grant Thornton reviewed the pilot for Pharmac. It recommended improvements, namely:

- the establishment of a Rare Disorders Advisory Committee with New Zealand experts including paediatric nephrology, metabolic disorders, blood disorders, neurology, as well as an Australian geneticist who is on the Australian Life Saving Drugs Program
- suppliers to be able to submit their application to Pharmac prior to, or at the same time as, submitting an application to Medsafe; thereby reducing the commercial barriers for suppliers wanting to enter the New Zealand market and speeding up the decision-making process
- a regular call for rare disorders funding applications
- dedicated pre-engagement with new, as well as existing, suppliers prior to the call for new applications
- regular horizon scanning for medicines.¹⁴³

Pharmac does not believe there is a strong case for a separate funding pool. We noted contestable funds lack the flexibility and pragmatism needed to deal with a problem to which there is no easy solution: how to fund very costly medicines for a small number of people from a fixed budget that already has too many demands on it. We understand this was the experience of the pilot.

Submitters and patient groups told us they were confused about whether Pharmac would consider an application for a medicine through both the pharmaceutical schedule and the exceptional circumstances framework at the same time. They cited the scenario of a medicine assessed through the pharmaceutical schedule and placed on the options for investment list, where it might sit indefinitely, but would technically still be under consideration by Pharmac, ruling out its consideration through the named patient pharmaceutical assessment framework process. This restriction currently precludes individuals from accessing unfunded medicines in exceptional circumstances. The review recommends Pharmac revisit this restriction. Pharmac said it had more discretion in such circumstances than many people realised, especially for people with deteriorating diseases.

Rare Disorders Advisory Committee

The Rare Disorders Advisory Committee, a specialist advisory committee of the Pharmacology and Therapeutics Advisory Committee (PTAC), was established as a result of the evaluation of the pilot. As with other specialist advisory committees, it only meets when funding applications require specialist clinical assessment. It has met four times since it was formed in 2018.¹⁴⁴ We consider the committee meets too infrequently, thereby missing an opportunity to look for, and provide advice on, emerging trends in rare disorder medicines, and to regularly call for applications from suppliers.

¹⁴³ Evaluation of Pharmac's commercial approach to fund medicines for rare disorders, 2017: <https://pharmac.govt.nz/assets/2017-06-final-Grant-Thornton-evaluation.pdf>.

¹⁴⁴ November 2018, September 2019, March 2021 and July 2021.

The Rare Disorders Advisory Committee members have expertise working with people receiving pharmaceuticals for which there is less evidence compared to non-rare disorders and are, therefore, more readily able to provide advice for decision-making. While the committee seems to have the appropriate range of skills, including a member of the Australian Live Saving Drugs Program, we heard concern from submitters that they might need additional international expertise from time to time. We agree and support the chair's role in ensuring the right mix of expertise attends relevant meetings.

The review also heard about the approach overseas by comparable committees where people with personal and lived experience of rare disorders have the opportunity to talk to the review. We recommend the committee explore this approach. The review also recommends PTAC give careful consideration to recommendations from the specialist committee, particularly given the lack of evidence there may be for some pharmaceuticals. This is of particular importance due to the lack of clinical trial data. Including relevant patient representatives in the assessment process in addition to patient submissions is something that could be considered and would provide a different and valuable perspective to the assessment. We note that under its revised terms of reference PTAC is able to bring external voices into its assessment process.¹⁴⁵

Case study

In considering the cases studies described in the decision-making chapter of this report, we explored how the factors for consideration were applied to assessing medicines for rare disorders by looking at one medicine, nusinersen, used in the treatment of spinal muscular atrophy (SMA). Submitters felt the dominance of cost utility analysis meant there was a 'one-size fits all' approach that disadvantaged people with rare disorders. Our review could not disprove or prove that hypothesis. Based on the information available to us, we could not definitively determine how the factors for consideration were weighted against cost-effectiveness, and how this interplay altered where the medicine would be ranked on the Options for Investment list relative to all other applications.

Our case study on the application for nusinersen suggests issues in the analysis of the application in both over-stating therapeutic benefit and under-playing other factors. It was immediately apparent that determining long-term benefit of a medicine for a rare and rapidly life-limiting condition was problematic using standard population-wide assessment techniques, and required a distinct, responsive and iterative decision-making process. The severity of the disease was clearly expressed in Pharmac's analysis and therefore we assume was a consideration in where the medicine ended up in the Options for Investment list, although how much so is not clear to us. The price of nusinersen, as with other medicines, is confidential, but likely to be well into the millions over the life of a patient.¹⁴⁶ These are the types of issues Pharmac has to mediate, with the aim of achieving equitable outcomes in the pharmaceutical schedule.

¹⁴⁵ <https://pharmac.govt.nz/assets/ptac-terms-of-reference.pdf>

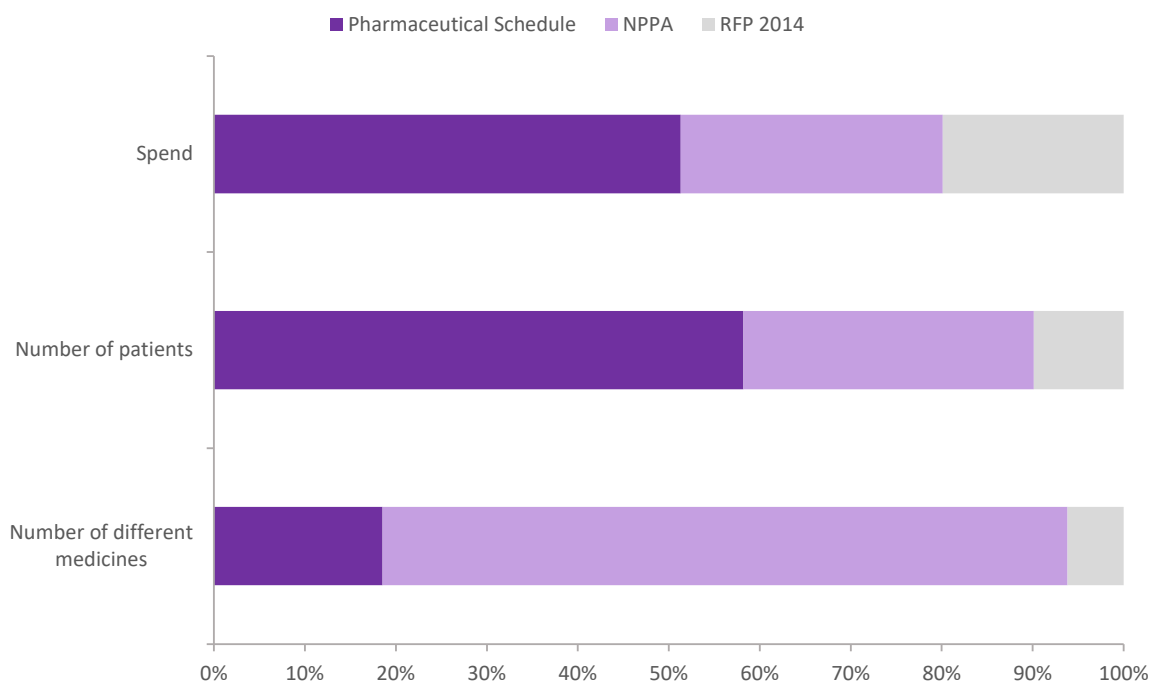
¹⁴⁶ <https://www.theguardian.com/business/2022/mar/05/real-life-hunger-games-lifesaving-drug-costs-2m-dollars>

Spending on medicines

Pharmac’s spending on rare disorders medicines makes up a small proportion of its pharmaceuticals budget. In 2019–20, it amounted to about 0.8 percent of the total (see Figure 8).¹⁴⁷

Figure 8 below shows the proportion of gross spend, number of patients, and the number of different medicines that are attributable to the pharmaceutical schedule, the NPPA process, and the pilot from 2014. Notably, the pharmaceutical schedule accounts for over 50 percent of both gross spend and the number of patients. However, it only accounts for 19 percent of the total number of medicines dispensed. This is expected as each funding mechanism aims to achieve different things. For example, the pharmaceutical schedule is used to fund large quantities of a small number of medicines, while NPPA funding is used for more selective treatments.

Figure 8: Pharmac spending on rare disorders medicines in 2019/20



Source: Pharmac.

Pharmac has also shared its spending and related trends over four years from 2016 to 2020 as Table 14 below. This table specifically shows, in that period:

- The number of medicines dispensed fell 16 percent.
- The number of patients rose 5 percent.
- Gross spending rose 50 percent.
- Average spending on each patient rose 43 percent.

¹⁴⁷ Among the ‘medicines’ funded are special foods, vitamins, amino acids and minerals.

The range of spending per patient is wide, but the average has only increased by about \$6,000 in absolute terms, suggesting there is not much more spending at the top end of the range. In short, these trends indicate Pharmac is not funding many, if any, of the very high-cost pharmaceuticals for treatment of rare disorders for which demand will increase.¹⁴⁸ More detailed analysis showed most of the spending increase between 2016–2020 was primarily due to medicines bought through the 2014 contestable fund pilot rather than the usual funding pathway.

This expenditure does not include spending on medicines for cystic fibrosis such as ivacaftor, for which Pharmac allocates about \$12 million a year for a small subset of cystic fibrosis patients, because Pharmac does not classify it as a rare disorder (although Rare Disorders New Zealand does).

Table 14: Pharmac funding for rare disorder medicines between 2016/17 and 2019/20

	2016/17	2017/18	2018/19	2019/20
Number of medicines dispensed	97	97	82	81
Number of patients	404	410	456	426
Gross spending	\$5.6 million	\$5.9 million	\$7.5 million	\$8.4 million
Average spend per person	\$13,938	\$14,432	\$16,383	\$19,916
Lowest spend per person	\$5	\$3	\$4	\$10
Highest spend per person	\$442,000	\$406,000	\$406,000	\$418,000

Source: Pharmac.

The spend per person information is provided to indicate spending over time and needs be considered with the lowest and highest spend per person. Pharmac advised that a relatively small proportion of people account for a larger proportion of the spend and a smaller proportion at the higher end of the spend range. People that have only received a funded medicine for part of a year are also included.

¹⁴⁸ Advances in biologics and personalised medicines for rare disorders come at very high cost, for example the lifesaving pharmaceutical nusinersen is believed to cost \$1 million per annum per patient.

What other countries do

Other countries also have challenges in designing efficient, effective and equitable funding systems for rare disorder medicines.¹⁴⁹

- Across most of those countries the key decision-making elements (application requirements, committees, decision-making criteria) remain the same for rare and non-rare disorder medicines.
- Some countries, notably Scotland and Australia, have developed specific programmes for *ultra-rare* (what New Zealand currently defines as rare) disorders.
- Only one country utilises a separate process for rare disorder medicines from the outset based on specific criteria.
- Most countries required economic evaluations of budget impact analysis (typically cost utility).
- It was not clear the extent to which patient involvement through the stages (initiation of review, scoping and evidence review, economic models, review committee meetings and managing uncertainty in the contracting process) made any material impact on the investment decision in any country.
- All countries had contractual mechanisms to manage uncertainty. This might involve payers (eg, government and/or patient) providing coverage by paying for a fixed period while suppliers collect more data to address specific evidence gaps. Where additional data is required, the cost falls to the manufacturer.

We analysed the impact of the different reimbursement and pricing processes on decision-making for a number of case studies overseas. We identified a useful article which worked through seven case studies. The medicines chosen by the authors are set out in the table below.¹⁵⁰

Table 15: Medicines analysed

Generic name	Rare disorder
Asfotase alfa	Paediatric onset hypophosphatasia
Burosumab	X-Linked hypophosphatemia
Cerliponase alfa	Neuronal ceroid lipofuscinosis
Elosulfase alfa	Mucopolysaccharidosis
Nusinersen	Spinal muscular atrophy
Lumacaftor/ ivacaftor	Cystic fibrosis – F508del in CFTR gene
Tolvaptan	Autosomal dominant polycystic kidney disease

Source: adapted from Stafinski T. 2021. Health technology assessment decision-making for drugs and rare diseases. University of Alberta.

¹⁴⁹ Stafinski T 2021. Health technology assessment decision-making for drugs and rare diseases. University of Alberta. The 14 were: Australia, Canada, Catalonia (Spain), England, France, Germany, Italy, New Zealand, Ontario (Canada), Scotland, Spain, Sweden, the Netherlands and Wales.

¹⁵⁰ Note that lumacaftor and ivacaftor would be considered by PTAC's respiratory sub-committee, as cystic fibrosis is not a rare disorder under New Zealand's definition.

We concluded there was no clear correlation between the frameworks and processes used, and a positive reimbursement decision. In other words, assessing the rare disorder medicines using a separate framework or process did not increase the likelihood the medicine would be approved. Instead, the review found positive reimbursement decisions for most pharmaceuticals were tied to either outcome based on contracting or real-world evidence generation after a fixed period.¹⁵¹

Despite the international review finding patient and carer involvement in the decision-making process made no apparent difference to the outcome, we consider procedural fairness and human rights obligations demand their involvement, particularly as a useful source of supplementary information when there is little clinical trial evidence.

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 health care 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 the RFP 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

¹⁵¹ Stafinski T. 2021. Health technology assessment decision-making for drugs and rare diseases. University of Alberta.

- 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.

8 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- to four-year cycle. New vaccines are assessed by Pharmac and compete with pharmaceuticals for funding. In this section, we look at whether Pharmac's approach to procuring vaccines fits with New Zealand's public health priorities. In our view, it does not.

Background

Immunisation saves millions of lives around the world each year by protecting against harmful infections. The World Health Organization emphasises the importance of immunisation as one of the most effective interventions in public health and access to immunisation as a key step towards access to health and universal health coverage¹⁵² and equitable health outcomes.¹⁵³ Immunisation programmes have the potential to reduce the risk of disease among our most vulnerable communities and individuals. New Zealand's national immunisation schedule lists vaccines offered free to babies, children, adolescents and adults. It also lists vaccines targeting specific health needs, such as infants at increased risk of tuberculosis.

The national immunisation schedule has been part of Pharmac's pharmaceutical schedule since 2012. Before that date, the Ministry was responsible for funding, prioritising, buying and managing vaccines on the schedule (but not the purchasing of influenza vaccines, which Pharmac has managed since 2004). The Ministry reviewed the schedule every three years, and the Minister of Health approved any changes. The Ministry contracted the Institute of Environmental Science and Research to tender for and manage vaccine supply contracts with pharmaceutical companies. The institute was also funded to manage storage and distribution of vaccines which require cold storage.

In 2010, Cabinet's social policy committee directed the Ministry to look at how it could get better value for money from vaccines. To this end, Cabinet agreed in 2012 to the transfer of the Ministry's vaccine-buying responsibility to Pharmac, as described in Table 18. Cabinet said the Ministry could continue to manage the national immunisation programme, and Pharmac could manage contracts to supply vaccines for personal and public health need, including vaccination programmes and outbreaks. It was noted that vaccine procurement for outbreaks would depend on their scale. Larger outbreaks, such as pandemics, would continue to be managed by the Ministry.¹⁵⁴

¹⁵² https://www.who.int/health-topics/vaccines-and-immunization#tab=tab_1

¹⁵³ <https://www.euro.who.int/en/health-topics/disease-prevention/vaccines-and-immunization/activities/immunization-systems/equity-in-immunization>

¹⁵⁴ Cabinet social policy committee (12) 45/1.

The review found it hard to obtain comprehensive information on New Zealand's end-to-end immunisation supply chain, from forecasting and purchasing vaccine supply through to administering vaccines. As a result, this section relies heavily on interviews with Ministry and Pharmac staff and such cited documents as we could source.

Roles and responsibilities

Pharmac is responsible for considering any changes to listed vaccines (including eligibility criteria), new vaccines, and managing supply for local and national programmes and most outbreaks. The Ministry is responsible for managing the national immunisation programme. District health boards are responsible for funding vaccines (using the same process as funding medicines) once Pharmac lists them. In 2014, Pharmac, the Ministry and district health boards signed a memorandum of understanding setting out their respective roles and responsibilities for purchasing, storing and distributing vaccines (see Appendix 1). The document outlined that each agency would work together to ensure good decision-making on procurement processes (tenders or otherwise), changes to the vaccines on the pharmaceutical schedule, and other decisions required to prevent or manage vaccine-preventable diseases. It detailed a no-surprises policy and a commitment to monitor and review implementation of the agreement. It also set out an escalation process for any disagreements or disputes and required Pharmac and the Ministry to meet regularly to review its operation. The Ministry transferred the necessary funds to Pharmac via a baseline adjustment from 2012/13. The Ministry told us this left them with no or very little operating budget to continue its roles as assigned in the memorandum of understanding.

As best we could determine from discussions with Ministry officials, Cabinet's original intention was for the Ministry to retain say over which vaccines to buy, including their eligibility criteria, and Pharmac would buy vaccines on the Ministry's behalf. This, however, is at odds with Cabinet's final position, which was that 'within annually agreed parameters, Pharmac will make decisions over the prioritisation of vaccines'.¹⁵⁵ Cabinet noted that Pharmac's negotiating position would be weakened if priorities were set for it, and this would reduce savings. It concluded that Pharmac would need to become the formal decision-maker for changes to the national immunisation schedule, as with other pharmaceuticals.

At that time, Pharmac clearly felt it could bundle contracts across pharmaceuticals and vaccines. This has subsequently not proven so and Pharmac operates the tender for vaccines separate from any pharmaceutical tenders or contracts.

Cabinet said the usual accountability measures between Ministers and their agencies would ensure Pharmac's prioritisation model remained appropriate for vaccines. It noted that the Ministry could make formal applications, with the Minister of Health's approval, to add vaccines to the national immunisation schedule. To our knowledge, this has never happened.

¹⁵⁵ Cabinet social policy committee (12) 45/3.

The technical advisory group to the Ministry was disbanded when the vaccine procurement functions transferred to Pharmac. Pharmac established a new specialist Immunisation Advisory Committee to PTAC and invited former technical advisory group members to join.¹⁵⁶ Ministry officials attend Immunisation Advisory Committee meetings as observers.

Vaccines compared to pharmaceuticals

Vaccines differ from pharmaceuticals as they are both a personal health and a public health intervention, seeking to provide protection to both individuals and population groups, whereas medicines are usually focused on improving the health and wellbeing of individuals. However, vaccines are used for individual personal health reasons also. From the perspective of a purchaser, vaccines and pharmaceuticals differ in other ways too, for example:

- vaccines tend to have a longer production time (sometimes up to 18 months) and a shorter shelf life than pharmaceuticals
- vaccines, as biological products, are more fragile and less stable than chemical-based pharmaceuticals and need very careful handling
- vaccines demand more quality controls during manufacturing than pharmaceuticals because of the complexity of the manufacturing process and the fact they are more susceptible to quality-control problems and delays¹⁵⁷
- vaccines must be kept at a controlled temperature (called cold chain management), whereas pharmaceuticals usually need not
- vaccines are purchased in bulk through a contract for delivery of volume/time, whereas pharmaceuticals are demand-driven by prescribers
- vaccine supplies can be subject to delays and competition from other countries during emergencies, whereas pharmaceutical supplies are more certain.

In short, buying, supplying and managing stocks of vaccines is more complex than for pharmaceuticals. This complexity means that a well-functioning vaccine programme demands clear delineation of roles and responsibilities and sound communication and supply management.

¹⁵⁶ Immunisation Technical Forum.

¹⁵⁷ Morrow & Felcone 2004: <https://www.ncbi.nlm.nih.gov/pmc/articles/PMC3564302/>.

Influenza vaccines and the supply chain

Procuring and managing influenza vaccines differs from other vaccines in several ways, one being the greater difficulty in forecasting influenza vaccines and the different supply chain required.

There is a publicly funded influenza programme (free for groups at the highest risk of complications from influenza) and a private market.¹⁵⁸ As a review in 2020 of the influenza vaccine supply chain noted, during heightened demand there is a risk that without careful coordination and oversight, vaccine stocks can be directed to the private market, leaving inadequate supply for the publicly funded market.

Forecasting volumes for most vaccines is relatively straightforward because they target a defined population, usually an age group. Pharmac provides a two-year rolling forecast to suppliers and buys vaccines up to 18 months ahead of needing them because it takes this long to make and supply them. Forecasting the influenza vaccine is more difficult due to a number of factors such as being able to determine features of the northern hemisphere influenza season, including what strains are circulating. The influenza season is short and the potential risks of undersupplying or oversupplying vaccines are greater than for other vaccinations.¹⁵⁹ Suppliers undertake their own forecasting for both the publicly funded and private markets and have input from Pharmac and the Ministry on publicly funded influenza vaccines. If Pharmac believes the supplier is proposing too few doses, then it shares some of the risk with the vaccine supplier by, for instance, underwriting some of the stock.

Problems with the supply and distribution of influenza vaccines during 2020 prompted the Ministry to contract consultancy firm PwC to review supply and distribution arrangements. PwC noted that forecasting had significant risks for suppliers and providers, and that the long lead times meant manufacturers could not make more vaccines quickly if demand exceeded forecasts.

Suppliers are responsible for storing and distributing influenza vaccines, and Pharmac is responsible for storing and distributing all other vaccines (which it does through a national vaccine store at Healthcare Logistics in Auckland and then through six regional branches of the wholesaler ProPharma). One known issue is that Pharmac currently cannot view stocks in the supply chain, meaning it cannot easily check where stock is distributed to and where it should be redistributed if there is an outbreak or heightened demand.

¹⁵⁸ This includes people 65 and over, pregnant women, children four or under who have been hospitalised for respiratory illness or have a significant history of respiratory illness, and people under 65 years with heart disease, chronic respiratory diseases, diabetes, chronic renal disease, cancer and other conditions (autoimmune, immune suppression, HIV, transplant recipient, neuromuscular or CNS disorder, haemoglobinopathy, children on long-term aspirin therapy, cochlear implant, metabolism errors, pre-/post-splenectomy and Down syndrome).

¹⁵⁹ Pharmac presentation 2021; PwC 2020, Influenza vaccine supply chain report.

Pharmac also cannot track vaccines once they leave the warehouses; it is up to the Ministry and district health boards to ensure they are distributed to where they need to go, given that those administering vaccines (for example, district health boards, general practices, pharmacies, Māori health providers, Pasifika health providers, occupational health providers) order the influenza vaccine from the supplier or its distributor directly. We consider there needs to be greater control of the whole supply chain. The Ministry told us that the new Covid-19 national immunisation register has a built-in system to hold information on the location and expiry date of vaccines, and there are plans for it to eventually replace the national immunisation register and to be used for all vaccines. We support the adoption of this new system for publicly funded vaccines.

We consider the supply of vaccines to be unnecessarily complex. Different organisations and providers each take responsibility for separate parts of the supply chain, and there is little oversight of the supply chain from start to finish – matters which were raised during two independent reviews (see next section).

Reviews highlight need to clarify roles and responsibilities

Two independent reviews undertaken in 2020, the PwC review just mentioned and a health sector response to the 2019 measles outbreak, made findings and recommendations relevant to Pharmac, although only one specifically mentioned Pharmac.¹⁶⁰ Both reviews found the respective roles and responsibilities of the Ministry, Pharmac, district health boards and providers to be unclear. Findings about the influenza vaccine supply chain relevant to our review (in addition to the lack of clear roles and responsibilities) were:

- **Ambiguous policy settings:** There was an obvious lack of alignment between the influenza vaccine programme's aim, the more common understanding of what constituted an at-risk community, and the more specific definition in the pharmaceutical schedule.
- **Limited planning to manage demand, including seasonality:** Responsibility for estimating demand sat with the supplier, which meant that, along with providers, they carried the commercial risk of being left with unused stock which contributed to a limit on supply.¹⁶¹

¹⁶⁰ Dr Gerard Sonder and Dr Debbie Ryan, July 2020: [https://www.moh.govt.nz/notebook/nbbooks.nsf/0/FBD9839C84AA1090CC25860A00098E66/\\$file/health_sect_or_response_to_the_2019_measles_outbreaks_1_july_2020.pdf](https://www.moh.govt.nz/notebook/nbbooks.nsf/0/FBD9839C84AA1090CC25860A00098E66/$file/health_sect_or_response_to_the_2019_measles_outbreaks_1_july_2020.pdf).

¹⁶¹ PwC, June 2020, Influenza vaccine supply chain report.

The measles outbreak review made 13 recommendations, two of which are relevant to our review. One was to clarify and/or officially mandate the roles and responsibilities of the organisations (Pharmac, the Immunisation Advisory Centre, Healthline and the Institute of Environmental Science and Research) managing regional and national outbreaks. The other was to consider whether Pharmac and the Ministry should share decision-making about whether, which and how many vaccines should be kept in stock for emergencies, and how to prepare for outbreaks.¹⁶²

In response, the Ministry set up a working group to plan for the 2021 influenza season, although this has yet to happen for the 2022 season. Pharmac and the Ministry have worked more closely, holding regular meetings and conducting more collaborative forecasting, but it is fair to say we heard differing views on how successful these meetings were in reaching a shared understanding on vaccine supply.

How other countries list and buy vaccines

In the four countries we examined, decision-making sits with either public health agencies or responsible Ministers, who take advice from public health experts. New Zealand, by contrast, leaves decisions to Pharmac.

¹⁶² Dr Gerard Sonder and Dr Debbie Ryan, July 2020: [https://www.moh.govt.nz/notebook/nbbooks.nsf/0/FBD9839C84AA1090CC25860A00098E66/\\$file/health_sect_or_response_to_the_2019_measles_outbreaks_1_july_2020.pdf](https://www.moh.govt.nz/notebook/nbbooks.nsf/0/FBD9839C84AA1090CC25860A00098E66/$file/health_sect_or_response_to_the_2019_measles_outbreaks_1_july_2020.pdf).

Table 16: Comparison of decision-making

Country	Selection and eligibility criteria	Purchase	Storage and distribution
New Zealand	Pharmac's Immunisation Advisory Committee provides clinical advice. The Ministry has no formal input into listing decisions.	Pharmac runs a tender every three to four years.	Wholesaler contracted by Pharmac
Australia	Pharmaceutical Benefits Advisory Committee, an independent body appointed by the Government. It advises the Minister on which vaccinations to include in the National Immunisation Program. ¹⁶³ The committee is advised by the Australian Technical Advisory Group on Immunisations. ¹⁶⁴ State and territory health departments can also fund additional vaccines.	Population Health Division seeks approval from Federal Government to fund the vaccine and conducts tenders to supply vaccines. ³	
United Kingdom	Joint Committee for Vaccination and Immunisation, an independent advisory committee that advises the United Kingdom's health departments (statutory role in England and Wales only) on immunisation, making recommendations on the vaccination schedule and vaccine safety.	Public Health England (children), general practitioners and pharmacists.	Public Health England (children), general practitioners and pharmacists
Norway	Norwegian Institute of Public Health (supported by National Immunisation Technical Advisory Group) and Norwegian Medicines Agency provide advice to the Ministry and Care Services.	Norwegian Institute of Public Health buys centrally using a national public tender process.	Norwegian Institute of Public Health
Canada	National Advisory Committee on Immunization, a national advisory committee, provides recommendations to the Government. The committee reports to the vice-president of the Infectious Diseases Prevention and Control Branch of Canada's Public Health Agency. Provinces and Territories determine the best schedule for their region.	Public Services and Procurement Canada has contracts with manufacturers. Inventory is held centrally and allocated to provinces in line with requirements.	Public Health Agency of Canada

¹⁶³ <https://www.directory.gov.au/portfolios/health/department-health/pharmaceutical-benefits-advisory-committee#:~:text=The%20Pharmaceutical%20Benefits%20Advisory%20Committee,on%20the%20National%20Immunisation%20Program>

¹⁶⁴ <https://www.directory.gov.au/portfolios/health/department-health/australian-technical-advisory-group-immunisation>.

Vaccines funded by Pharmac

Vaccines on the vaccination schedule are put out to tender every three years. The number of vaccine suppliers is relatively small. Pharmac tenders for several vaccines at once to encourage bundle deals. Many suppliers don't offer pharmaceuticals, so bundle deals include vaccines only.

Pharmac assesses new vaccines or changes in eligibility for publicly funded vaccines in the same way as other pharmaceuticals, prioritising them against other medicines. If there are savings on vaccines listings, these are not specifically tagged to purchasing other vaccines or widening existing vaccine eligibility.

Pharmac takes advice from its Immunisation Advisory Committee on comparability of health benefits between different brands of vaccine for the same disease. The advisory committee meets once or twice a year.

Five suppliers provide the 22 vaccines on the national immunisation schedule at a cost of \$120 million a year. The suppliers have \$50 million of vaccines in stock at any one time. Since taking over the national immunisation schedule, Pharmac has added three new vaccines and widened access for 12 vaccines. No data is available for us to assess the size of any benefits or costs from these changes.

Equity considerations

When considering vaccines, we cannot avoid commenting on the sharp decline in childhood immunisation in general and for Māori in particular. The decline, along with increasing inequities since 2016 and the impact of Covid-19, leaves Māori disproportionately vulnerable to disease and death from vaccine-preventable diseases. Many systemic factors have contributed to this decline, and Pharmac, the Ministry, district health boards and providers have all expressed concern about this trend.

From what we could see, Pharmac has given little consideration to equity in determining what goes on the national immunisation schedule. For example, independent expert advice on the Covid-19 vaccination programme argued that chronological age set at 65 when used to determine eligibility would embed and increase inequities because proportionately the Māori population is younger than the non-Māori, non-Pasifika population and less likely to benefit. It was also argued the rationale for setting the age at 65 for non-Māori (such as increased risks from infection) applied to Māori about 10 years earlier.¹⁶⁵ We found no information to suggest Pharmac sought expert advice of this nature for influenza vaccines. However, on 8 February 2022 the Minister of Health announced that extra doses of the influenza vaccine would be purchased for 2022, and that the eligibility criteria for free flu vaccinations would be amended to reduce the eligibility age for at-risk groups and potentially to include a wider range of young people.¹⁶⁶

¹⁶⁵ Waitangi Tribunal, 2021: Haumaru <https://waitangitribunal.govt.nz/assets/Documents/Publications/Covid-Priority-W.pdf>.

¹⁶⁶ <https://www.beehive.govt.nz/release/free-flu-vaccines-more-new-zealanders>

In considering equity within its vaccination programme, Australia has two national immunisation schedules for publicly funded vaccines – one for non-Indigenous people and another for all Indigenous people. The key differences are:

- Meningococcal B is funded for Indigenous children from two months, but not for non-Indigenous adults.
- Pneumococcal (both the 13 antigen and the 23 antigen) is funded for Indigenous adults from 50 years of age compared with 70 years of age for non-Indigenous adults. Also, a third dose of PCV13 is funded for Indigenous children living in certain areas and for all children with specified medical risk conditions for pneumococcal disease.
- The annual influenza immunisation vaccine is funded for all Indigenous people over six months, but for non-Indigenous people it is funded for children between six months and five years; people six months and over with specified health problems; people aged 65 and over; and pregnant women as in New Zealand.¹⁶⁷

As with pharmaceuticals in general, we strongly urge Pharmac to ensure that equity is factored into all vaccine policy and procurement decisions, drawing on relevant equity expertise, and that this analysis is documented transparently.

Question mark over savings

In advice to Cabinet in 2012, on moving vaccines, it was estimated Pharmac could fund \$43 million of the cost of vaccine purchases over three years from savings on other pharmaceuticals as a result of ongoing negotiations. These savings were projected to come from doing better deals with suppliers through negotiating across a range of products including bundling (where a supplier agrees a lower price in exchange for benefits such as the listing of a new product or a wider use for an existing product).¹⁶⁸ In a media release two years later, Pharmac said district health boards would save more than \$100 million over five years as a result of the expansion of its role to include vaccines as well as medicines.¹⁶⁹ However, Pharmac told the review it was hard to determine whether these savings had, indeed, happened. It said direct comparisons were difficult. Bundling in the most recent request for proposal (in 2018) had produced savings of ‘several million dollars’ over the next best offer. However, the recent unbundling of a vaccine from other products had resulted in its price doubling.¹⁷⁰ It would seem to us that Pharmac’s attempts to bundle vaccines in the same way it bundled pharmaceuticals did not produce the savings it anticipated because the scope to bundle vaccines is inherently more limited.

Despite the question mark over the extent of any savings, Pharmac is properly equipped to continue to negotiate contracts with vaccine suppliers and obtain competitive prices.

¹⁶⁷ <https://www.health.gov.au/health-topics/immunisation/when-to-get-vaccinated/national-immunisation-program-schedule>

¹⁶⁸ Cabinet social policy committee, SOC Min 12 (10/4), 2012.

¹⁶⁹ <https://pharmac.govt.nz/news-and-resources/news/savings-from-extended-roles-exceed-100m/>

¹⁷⁰ Meeting with Pharmac, 2021.

The issues we identified

We identified the following 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, as noted, 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).
- The memorandum of understanding is inadequate and limits the Ministry's ability to influence Pharmac's decisions. It requires that 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.

Options

We looked at three potential ways to improve arrangements:

- **Improve the status quo:** This could be done by including Ministry officials on the Immunisation Advisory Committee and on PTAC when it is making vaccine decisions; implementing the changes we suggested to decision-making; making equity considerations central to all decisions; revising the memorandum of understanding and conducting more regular, formal reviews of it; the final decision on listing vaccines would remain with Pharmac.

- **Shift decision-making on vaccines and their purchase to the newly formed Interim Public Health Agency:** This would require the Immunisation Advisory Committee to be re-established, and the vaccine budget to be transferred to its baseline funding. The Interim Public Health Agency would then make decisions on what vaccines it supplies and would take the risk on over- or under-supply of the influenza vaccine as well as decisions on listing new vaccines or extending eligibility. Responsibility for the vaccination budget would need to sit with the decision-maker.
- **Establish a formal partnership:** The Interim Public Health Agency would become the principal decision-maker on vaccine choice and eligibility criteria for the national immunisation schedule, and Pharmac would assist by undertaking the commercial negotiations. A contract would formalise this partnership between the Ministry and Pharmac, and there would be regular review dates.

We favour the final option. In our view, the Interim Public Health Agency needs to take back control of the vaccine programme, including which vaccines are listed on the immunisation schedule. The Interim Public Health Agency would be the logical agency to have this function. Pharmac should act as the agent in negotiations. Pharmac told us it would be hesitant to take on this more limited role, saying it was not a 'procurement agency' and that tendering on a predetermined product in this way would make it a 'price taker' rather than a 'price maker'. Despite Pharmac's view, we consider such an arrangement would still leave room for it to obtain competitive prices for vaccines. Pharmac buys vaccines primarily through a tender process, which is an effective way to secure the best price.

As for improving oversight of the vaccine supply chain, we would go further than the two previous reviews' call for greater precision about each agency's role and responsibilities. In our view, a single national agency needs to oversee the entire supply chain.

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.

9 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 health care 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.

Despite this good work, the review considers that under the reformed health system Pharmac is no longer the most appropriate agency to lead this function. 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.

Range of devices

Medical devices span a wide range of equipment – everything from swabs, bandages and surgical gowns through to stents, orthopaedic joint kits and respirators. These devices make up a very small proportion of the health system’s operational spending (between about 2 percent and 5 percent) but a much bigger share of hospitals’ costs (between 10 percent and 20 percent).¹⁷¹ They are critical to successful health care, and there are systems to purchase and warehouse them and distribute them to sites across the health system including outpatient clinics, wards and theatres.

Pharmac’s definition of a medical device is broadly consistent with the description in section 3A of the Medicines Act 1981. It includes products and equipment used on, in or by a person for a diagnostic or therapeutic purpose. This ranges from simple dressings to complex clinical equipment, supportive devices with therapeutic/safety considerations (beds and mattresses, disability support devices) and devices hospitals provide to community patients. It does not include equipment used by health providers in primary and community care or private hospitals.

¹⁷¹ Medical Technology Association of New Zealand (2010). Medical Technology – A guide to market access in New Zealand.

A major category of equipment is devices for disability and rehabilitation. New Zealand has duties to recognise the right of people with disabilities to enjoy the highest attainable standard of health and take measures to support their full inclusion and participation in all aspects of life, in accordance with Articles 25 and 26 of the United Nations Convention on the Rights of Persons with Disabilities. These duties include promoting the availability, knowledge and use of assistive devices and technologies designed for people with disabilities, as they relate to habilitation and rehabilitation. The Government intends to provide \$832.5 million over the next five years for disability support services, and this money includes funding for rehabilitation equipment.¹⁷² Health NZ, the Accident Compensation Corporation and the new Ministry for Disabled People will all manage medical devices relating to rehabilitation and disability.

Medical devices catalogue

Pharmac discovered early on that successful contracting of medical devices started with a catalogue of those devices.

Pharmac has about 20 staff working on medical devices, including compiling the catalogue, organising contracts with suppliers, and considering the assessment process needed for new medical devices. The Excel-based catalogue runs to more than 150,000 line items from more than 100 suppliers, and Pharmac says it has about 100,000 more to go.¹⁷³ It told us it had catalogued 62 percent of possible items by value (\$430 million) and hoped to reach 75 percent by the end of June 2022. Some devices have more than one line item. A respirator, for example, may have separate line items for component parts such as face masks and tubes. The task of keeping the catalogue up to date is considerable because some products stop being used, are replaced, or are no longer available. Up to 9,000 items a year can require such updating. District health boards have operated different systems, and the lack of a common inventory management system has hampered the sector's ability to get the best out of Pharmac's cataloguing work.

Savings are elusive

Cabinet had expected Pharmac's savings to more than offset the money spent setting up and operating the cataloguing function, most of which came from district health boards. In 2015, the Government took over funding the operating costs of this function. Savings have proved much harder to make than expected. Pharmac thought it could extract savings in the same way it did with pharmaceuticals but found it could not. Early on, for example, it identified the reagent market for laboratory analysers as an area where it could generate healthy savings, but it soon realised district health boards had largely outsourced laboratory operations, achieving savings through a competitive process for wider service delivery rather than just one laboratory process. Pharmac has, however, made some savings by negotiating market share contracts to preferred suppliers that give it bigger discounts as volumes increase.

¹⁷² Wellbeing Budget 2020: <https://treasury.govt.nz/sites/default/files/2020-05/b20-wellbeing-budget.pdf>

¹⁷³ As at October 2021.

Pharmac's first real attempt to use its buying power was in 2016, in a request for proposals for wound care products. The review understands modest but acceptable savings were achieved. In 2018, there was a further request for proposals for permanent coronary drug-eluting stents, with 65 percent of the market being awarded to two products from one supplier. This preferred supplier approach is still very tentative, and savings are equally modest as the catalogue is not restricted and, if it were, clinicians would simply source product from another supply channel. Clinician support is vital when making any catalogue changes that may reduce user choice.

Buying from the catalogue is not compulsory, and hospitals and health services can – and often do – order off their own catalogues. Clinicians may get devices directly from suppliers, undercutting the usefulness of both Pharmac's and the district health board's catalogues. We were told there is substantial warehousing in Auckland for clinicians to call on, which operates outside of the district health board supply chain. Other reasons for the limited savings are suppliers' reluctance to sign contracts with Pharmac because they are reluctant to cede control, the health sector's mixed results in trying to digitise the supply chain, and Pharmac's need to develop relationships with clinicians while adding additional product categories to the catalogue.

In our view Pharmac operates too remotely from clinical and operational matters in hospitals and health services to be able to oversee the supply chain and deal adequately with difficulties as they arise. Supply chains require expertise in a host of practical logistical matters, such as warehousing and supply, sourcing replacements when stocks are low, and seeking advice from equipment users, such as ward nurses, medical technicians and medical officers. In contrast, suppliers have strong, direct relationships with district health boards and their clinicians, as well as arrangements in place to supply them with the medical devices they need. Under the health reforms it is likely that these relationships will most easily transfer with the clinicians to Health NZ.

Pharmac has become aware it needs to build relationships with clinicians, but stakeholders still feel Pharmac is ill-placed to coordinate the work of standardising clinical processes and too distant from clinicians to standardise clinical equipment or support clinician-run research (such as outcomes research on hip transplants). Pharmac has unquestionably done good work in developing the catalogue, but we think medical device supply chain management is not its strength, and Health NZ would be better placed to carry out these contracting and cataloguing functions as part of its wider procurement remit.

Pharmac's limitations, and those of the health systems supply chain, came into sharp focus in the initial struggle to secure basic medical items in the early stages of the Covid-19 pandemic. Pharmac told the review the pandemic highlighted its difficulty in telling with any certainty what district health boards were purchasing and therefore what stocks were held where, by whom. Those stocks could not be reallocated and ran out in some areas, with other areas holding stocks. Those managing the Covid response needed information quickly about the location and number of medical devices, and Pharmac could not supply that information.

The review considers the cost of medical devices needs to be funded from Health NZ's clinical and hospital budgets.

Health NZ systems review

The interim Health NZ organisation is reviewing systems for purchasing and supplying medicines, and as part of that it has been looking at medical devices. It has found that the health system's supply chain, including medical devices, needs a stronger, more coordinated approach.

The supply chain review (which at the time of writing this report was ongoing) identifies district health boards' culture and organisation as the main impediments to a more effective supply chain for medical devices. The interim Health NZ is in the middle of bringing the supply chain together with a national approach to purchasing and supplying medical devices. In the past, district health boards have found it difficult to work with each other, and there has been a concerted effort, with over 60 interviews and several workshops, to bring those disparate district health board supply chains together as one integrated system. The health systems supply chain will be led from the centre even if some of its activities such as warehouses are necessarily regional.

The interim Health NZ has identified the following principles¹⁷⁴ as the basis for its supply chain design work:

- The purchase and supply of medical devices will be nationally based except when it makes no sense to do so.
- Considering the system in its entirety at a national level will ensure the best outcomes.
- Supply chain efficiency is best achieved by considering the system at a national level.
- Achieving the best overall national cost is the priority, even if regional or local costs are sometimes higher.
- Decisions balance achieving the best price with meeting local population needs and taking into account equity considerations.

Pharmac's catalogue and associated systems would be a useful foundation for Health NZ in setting up its supply chain management operations.

Our recommendation – to transfer responsibility for the procurement of medical devices to Health NZ – should be subject to Health NZ transition requirements. There may be a case for Health NZ to gradually absorb Pharmac's medical devices operations rather than establish the necessary infrastructure from scratch. A service-level agreement would allow Pharmac to act as Health NZ's agent during the transition period.

¹⁷⁴ Presentation to supply chain conference, Enclosure 1 Central Model – Procurement and Supply Chain power point presentation, Roger Jarrold, 23 January 2022.

Medical devices health technology assessments

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.

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.

10 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, and nor does any other legislation or policy statement. According to its definition, Pharmac currently spends about \$2 million a year on promoting the responsible use of pharmaceuticals. 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 that responsible use of pharmaceuticals is 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.¹⁷⁵

Our conclusion in this report is that Pharmac’s performance in responsible use is lacking. To make real inroads into achieving equity, what we think of as responsible use, should be broader than Pharmac’s current approach. A broader approach to optimal medicines use, which incorporates responsible use with a strong focus on equity, needs to be led by an agency that has, as part of its core role, the responsibility of leading, overseeing and coordinating 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’s performance

Pharmac is the only health entity with a statutory obligation to promote the responsible use of medicines.¹⁷⁶ Pharmac has told us that it aimed to ensure ‘existing funded medicines aren’t overused, underused or misused’.¹⁷⁷ Looking across its more than two-decade history, Pharmac has done this by:

¹⁷⁵ Pharmac Review Interim report, p 20.

¹⁷⁶ This includes medical devices, although Pharmac has yet to spell out how it will promote their responsible use.

¹⁷⁷ Pharmac briefing to the review, 2021.

- supporting best guidance prescribing and providing information on the place of medicines in condition management in primary health care
- providing information, through external expertise, when there are new medicine subsidies and/or brand changes
- developing population health programmes to respond to particular medicines-related issues, such as inappropriate prescribing of antibiotics in winter.¹⁷⁸

Pharmac has also indicated that it has recently enhanced its responsible use function by making it a 'whole-of-Pharmac' responsibility. This is discussed further below, but in summary its newest iteration of responsible use activity includes things like the application of special authorities to ensure equitable access to specific medications, the provision of additional clinical advice, enhanced use of data and insights, and managing the pharmaceutical budget 'responsibly'.¹⁷⁹ It is not yet clear what Pharmac's role is in terms of responsible use of hospital medical devices.¹⁸⁰

Information and support for prescribers

Pharmac began encouraging the responsible use of medicines in its present form in the late 1990s when pharmaceutical companies began scaling back their education and information-sharing activities. It has done so through outsourcing, at first through four providers already offering such services through contracts with the Health Funding Authority, and then from 2003 through the Best Practice Advocacy Centre (usually referred to as BPAC), an amalgamation of these providers,¹⁸¹ and the Goodfellow Unit at the University of Auckland.¹⁸² Both agencies prepared and disseminated articles and produced reports on primary care providers' prescription patterns (although we understand the production of these reports was irregular). These reports were distributed in print form to prescribers (who at that time were solely general practitioners, but more recently expanded to nurse practitioners and prescribing pharmacists) and now by electronic distribution. BPAC determined topics in response to questions from prescribers and suggestions by an external clinical advisory group (typically related to funding decisions Pharmac was making or new data or analysis). Pharmac said health care professionals used and highly valued this material, and stakeholder feedback to us confirmed this assessment. BPAC also enabled prescribers to conduct self-assessments of their prescribing patterns. The Goodfellow Unit provided (and continues to do so) webinars, podcasts, gems, and an annual conference. Finally, Pharmac also facilitated education seminars on a range of topics chosen by independent advisors with external experts contracted to present the topics.

¹⁷⁸ Pharmac briefing to the review, 2021.

¹⁷⁹ Medicines Optimisation presentation to secretariat and advisors, November 2021.

¹⁸⁰ Pharmac briefing to the review, 2021.

¹⁸¹ For more information on the centre, see: <https://bpac.org.nz/about.aspx>.

¹⁸² For more information on the Goodfellow Unit, see: <https://www.goodfellowunit.org/>.

Apart from the seminars, these education and information activities continue today albeit in a modified form (with an aim of assisting equitable access to medicines) and through a new provider, Matui Ltd, following a tender in 2019. Matui runs an equity-focused education programme called He Ako Hiringa for general practitioners and other clinicians offering primary care. The programme is largely web-based and offers various resources, including articles, short practice points, webinars, videos and podcasts to help health care professionals with clinical decision-making. Prescribers are given a personalised dashboard of their prescribing history (based on their pharmaceutical claims) to help stimulate behavioural change from an equity perspective.

Given that the programme has been operating for only about two years, and Pharmac is still awaiting more information about its results, it is difficult to assess whether Matui is having more impact than its predecessors in turning around medicines access inequities. Matui has also outsourced expertise, including individuals with considerable equity, Māori health and Pasifika health backgrounds. The use of data and analytics at a clinician level is an encouraging step, but we are not convinced Pharmac should be responsible for this work, a task it seems to have earned by default. In our view, such activities should be more fully integrated into a whole-of-system approach to primary health care, as discussed below. The total cost of all such contracted information and education activities since 1999 has been about \$30 million.¹⁸³

Special authority criteria

Pharmac has said that its core function of funding medicines has incorporated strong elements of responsible use, including the use of special authority criteria (usually referred to as ‘special authorities’).¹⁸⁴ Special authorities are the mechanism by which access to some subsidised medicines is limited to specific people who meet criteria. Pharmac explains that the rationale behind special authorities is to ensure supply is for those deemed to benefit the most from that treatment.¹⁸⁵ We note that as special authorities only apply to expensive medicines (and not generic medicines), they are clearly also about reducing costs to the pharmaceutical budget.

Until recently, special authorities have been used to restrict access on specific clinical grounds, for example, Sacubitril with valsartan, a medicine for heart failure. For a person to be eligible for the subsidy for this medicine, they have to have a diagnosis of heart failure and receive other heart failure medicines.

In February 2021, Pharmac for the first time used Māori and Pacific ethnicity as a special authority criterion for two medications used in type 2 diabetes (empagliflozin and dulaglutide), on the basis of the inequitable impacts of type 2 diabetes on these population groups. These medicines help manage type 2 diabetes-related complications like kidney and heart disease in people who are at high risk of these complications as well as managing blood sugar levels. The funding through the special authority significantly reduces the financial barriers to accessing the medicine, reducing the price patients pay to \$5 per prescription.

¹⁸³ Pharmac presentation to secretariat and advisors, November 2021.

¹⁸⁴ Pharmac presentation to secretariat and advisors, November 2021.

¹⁸⁵ Pharmac ‘Special Authority Applications’, website information, accessed 17 February 2022 (<https://pharmac.govt.nz/medicine-funding-and-supply/make-an-application/special-authority-forms/>).

During the review's stakeholder engagement, we heard that this use of a special authority was an example of Pharmac taking pro-equity steps to improve access to medicines. While this is clearly a positive step, it is important to note the frustration shared by some stakeholders that Pharmac was only able to create the special authority because of external Māori health expertise, which provided substantial evidence on the grounds for creating this special authority, to Pharmac. This lack of equity capability within Pharmac was also noted in the review's analysis of decision-making (section 5) and in our interim report.

Pharmac has used the public awareness campaign for empagliflozin and dulaglutide as an opportunity to test different approaches that work best when sharing medicine information with Māori audiences. The resulting 'You are a Priority' campaign was, according to the material we were provided, developed by a public relations company with specific Māori communications experience with a very light touch from Pharmac. The campaign finished in December 2021. The specific campaign results notwithstanding, this seems to reinforce that Māori-focused communications could be better led from somewhere else in the health system.

Social marketing campaigns

Pharmac has run a relatively small number of campaigns over the past two decades, aimed at improving consumer knowledge around specific medicines. These include the long-running Wise Use of Antibiotics campaign, which was seen as having some success in raising awareness about inappropriate overuse of antibiotics. However, New Zealand's antibiotic prescribing rates remained high overall, but with lower than expected rates for Māori in rural areas with high levels of acute rheumatic fever.¹⁸⁶ This raises questions of how well Pharmac has been able to apply an equity and te Tiriti o Waitangi lens to all of its population health programmes.

Pharmac cited three programmes with a Māori health and equity focus: One Heart Many Lives, He Rongoā Pai He Oranga Whānau¹⁸⁷ and Space to Breathe/He Tapu Te Hā. We were able to take a closer look at the first of these.¹⁸⁸ One Heart Many Lives began with the aim of encouraging Māori and Pasifika men to get their hearts checked and eventually transformed into a programme promoting appropriate use of medicines, and better lifestyle choices such as eating well, being more physically active and stopping smoking. The programme helped Māori and Pasifika men to get a cardiovascular risk assessment through primary care and at community events with their whole whānau.

¹⁸⁶ Norris et al 2011: <https://academic.oup.com/jac/article/66/8/1921/673653>.

¹⁸⁷ Pharmac still offers this programme, which features a wānanga approach to building knowledge of medicines among Māori, but has been forced to put it temporarily on hold because of a lack of wānanga facilitators: <https://pharmac.govt.nz/te-tiriti-o-waitangi/programmes-to-support-maori-health/he-rongoa-pai-he-oranga-whanau/>.

¹⁸⁸ Space to Breathe/He Tapu Te Ha was a pilot programme and then evaluative trial on childhood asthma that ran from 2009 until 2013.

Pharmac ended funding for the programme in 2014. An evaluation in Porirua (one of the first One Heart Many Lives sites) found limited community recall of the programme and varying degrees of knowledge of the programme in primary health care practices. This suggests to us that some of the biggest barriers to conducting cardiovascular risk assessments and appropriately prescribing statins¹⁸⁹ are outside Pharmac's control, most notably the cost for people accessing primary health care and the limited capacity of primary health care to take on extra health promotion work.¹⁹⁰ Although we note the evaluation report is based on small numbers, with methodological limitations, it confirms what we heard from stakeholders that Pharmac is not well placed to run these kinds of primary health care programmes. It further suggests the need to move some of Pharmac's responsible use functions to other agencies with more direct involvement in primary health care. Stakeholder feedback also suggested the programme might have had better success if it had been fully resourced (at its height, as a national programme between 2010 and 2012, annual funding was about \$800,000) and if it had been run by a national Māori organisation that had stronger links to the community and primary health care providers and had a track record in health promotion.

Equity

Pharmac first published research on the variation of medicines use by ethnicity during the period 2006–07.¹⁹¹ This evidenced that Māori were missing out on more than a million prescriptions a year. Despite this alarming gap, and a Māori strategic framework, a further analysis found no improvement over the following six years, and in fact the situation had become nominally worse.¹⁹²

In 2017 Pharmac set a bold goal of achieving equity in medicines access by 2025. This goal drove the development of a theory of change.¹⁹³

Pharmac's theory of change identifies the main drivers of inequity as being medicines availability, medicines accessibility, medicines affordability, medicines acceptability and medicines appropriateness. All five of these are part of what could be considered responsible use of medicines. But as Pharmac says itself in the theory of change document, its only direct role is in the area of medicines availability by ensuring appropriate decision-making for investment in medicines and imposing funding restrictions and schedules rules (like adopting special authorities for some types of medicines to increase access for Māori and Pasifika populations).

¹⁸⁹ A group of medicines used to lower cholesterol (or lipids) in blood: <https://www.healthnavigator.org.nz/medicines/s/statins/>.

¹⁹⁰ Leow et al 2011. One Heart Many Lives Evaluation Report. Public Health Project: The University of Otago.

¹⁹¹ Metcalfe et al 2013: <https://journal.nzma.org.nz/journal-articles/variation-in-the-use-of-medicines-by-ethnicity-during-2006-07-in-new-zealand-a-preliminary-analysis>.

¹⁹² Pharmac analysis of prescription medicines showed a shortfall of 1,126,300 pharmaceutical treatments for Māori for 2012/13 and there is nothing to suggest any change in this shortfall during succeeding years. Metcalfe et al 2018: <https://www.nzdoctor.co.nz/sites/default/files/2018-11/Pharmac%20equity.pdf>,

¹⁹³ Pharmac. 2019. Achieving Medicines Access Equity in Aotearoa New Zealand. Available here: <https://pharmac.govt.nz/assets/achieving-medicine-access-equity-in-aotearoa-new-zealand-towards-a-theory-of-change.pdf>.

Pharmac has told the review that along with Te Whaioranga and its Pacific Responsiveness Strategy, this approach to equity has been part of enhancing its responsible use function using three key principles:

- making it a responsibility of the entire Pharmac organisation to promote the responsible use of medicines
- modifying its own behaviour and that of its partners, not just that of clinicians and patients, to help bring about this outcome
- introducing systemic solutions to a systemic problem.¹⁹⁴

One of Pharmac's initiatives is to produce analytical papers suggesting ways to achieve equitable health outcomes. The first of these, released in November 2021, examined gout,¹⁹⁵ a known area of inequity¹⁹⁶ that disproportionately affects Māori and Pasifika people.¹⁹⁷ The paper found, among other things, that about 10,400 more Māori need preventive gout medicine each year than are currently prescribed it, that Māori are about twice as likely to receive gout medicine compared with non-Māori and non-Pasifika people, that Māori are nearly seven times more likely to be hospitalised for gout compared with non-Māori and non-Pasifika people, that 60 percent of Māori hospitalised for gout were not receiving preventive gout medication in the six months prior to hospitalisation, and that a quarter of Māori hospitalised for gout did not receive a preventive gout medication after hospitalisation. None of these findings, however, adds anything novel or different to what is already known on the subject. Furthermore, some experts we spoke to questioned the paper's results.

The paper focuses on recommendations for practitioners on the diagnosis of gout, the harm from long-term use of non-steroidal anti-inflammatory pharmaceuticals, the prescribing of preventive gout medication for young Māori, and the genetic predisposition to gout among Māori whānau. But it fails to mention access to primary health care, the cost of prescription co-payments, regular monitoring of urate levels, and preventive medicine dose titration – all significant issues highlighted by academic literature and all essential to any data insights aiming to improve equitable access to medicines.¹⁹⁸ Despite these shortcomings, the stronger focus on equity is encouraging.

¹⁹⁴ Presentation to secretariat and advisors, November 2021.

¹⁹⁵ Pharmac 2021: <https://pharmac.govt.nz/assets/Gout-insights-Impact-on-Maori-December-2021.pdf>.

¹⁹⁶ Dalbeth et al 2018: <https://journal.nzma.org.nz/journal-articles/gout-in-aotearoa-new-zealand-the-equity-crisis-continues-in-plain-sight>.

¹⁹⁷ Te Karu 2021: https://www.publish.csiro.au/hc/HCV13n2_ED2.

¹⁹⁸ Dalbeth et al 2018: <https://journal.nzma.org.nz/journal-articles/gout-in-aotearoa-new-zealand-the-equity-crisis-continues-in-plain-sight>.

Limited influence and collaboration

We heard a lot of feedback from stakeholders that Pharmac goes about its business in a way that undermines any influence it may have over prescriber and provider behaviour. We also found the way Pharmac interacts with consumers and communities remains underdeveloped. Pharmac has attempted to be more systematic in the way it promotes the responsible use of medicines, but a truly systematic approach would require it to bring along partner organisations in the wider health sector and, based on our observations and stakeholder feedback, it is much too insular for the task. Equally importantly, most changes that need to happen are outside Pharmac's direct influence or area of legislative responsibility, making it unreasonable to expect Pharmac to perform the task alone.

A natural partner for Pharmac – at least until recently – would have been the country's 20 district health boards, which strive to improve health outcomes, eliminate inequities and offer the most effective, efficient delivery of health services, including through collaboration with relevant organisations. However, stakeholders said Pharmac tended to go it alone when promoting the responsible use of medicines.¹⁹⁹ They cited examples of Pharmac presenting position statements and analysis as finished products, without discussion about how to collaborate on the matters under discussion. We see little sign of any change in outlook in Pharmac's latest attempt at promoting the responsible use of medicines more effectively.

Partnering with Māori and reaching communities

Outside of formal health system structures, we have been told, by Māori stakeholders in particular, that Pharmac doesn't '*partner well with anyone*'.²⁰⁰ Māori stakeholders have also highlighted concerns about a siloed approach to the responsible use of medicines and have argued for a cohesive, strategic approach.

[The way things are run now] currently Pharmac only has a limited role, but if you had a system-wide approach then you could think about much more effective ways of improving quality.²⁰¹

Similarly, other stakeholders told us they felt Pharmac's profile was too low for it to have impact within Pasifika communities, suggesting that Pharmac needed to spend time building trusting relationships and '*inform the Pasifika community what they do, how communities can engage and have their say*'.²⁰²

¹⁹⁹ Stakeholder discussion, December 2021.

²⁰⁰ Key informant interview, December 2021.

²⁰¹ Key informant interview, November 2021.

²⁰² Written submission (email).

Optimising medicine use

The overuse, underuse and misuse of medicines is a worldwide problem. According to the World Health Organization, more than half of all medicines are prescribed, dispensed or sold inappropriately, and half of all patients fail to take medicines correctly. It says the responsible use of medicines includes the following elements:

- The patient's condition is diagnosed correctly.
- The patient is prescribed the most appropriate medicine in the right dose and formulation.
- The patient (and the medical system) can afford the medicine.
- The patient is well informed about the medicine and takes it as required and for the prescribed time.
- Prescribers are competent and ensure the proper use of medicines, using evidence to determine the best therapy choices.²⁰³

In more recent times, the term 'optimal use of medicines', or 'medicines optimisation', has come to be used alongside or instead of the term 'responsible use of medicines' because of the growing awareness that prescribing the right medicines to the right patients at the right time and in the right way requires a systems-wide approach by all participants in a health sector.²⁰⁴ It also recognises that the patient is not a mere passive recipient but rather an active participant with clinicians in the process of regaining health. It places more focus on cultural dimensions and on partnering with patients and their whānau. As one group of researchers noted, the optimal use of medicines is about 'more than the provision of "understandable" information, founded on clinical competence' and, for Māori in particular, requires 'genuine relationships that are connected to culture and underpinned by trust and collaboration'.²⁰⁵ As illustrated in the cancer section, decisions on funding medicines and access to appropriate medicines also rely on robust drug trials, conducted with representative populations, so we consider this has a role to play in optimal medicines use too.

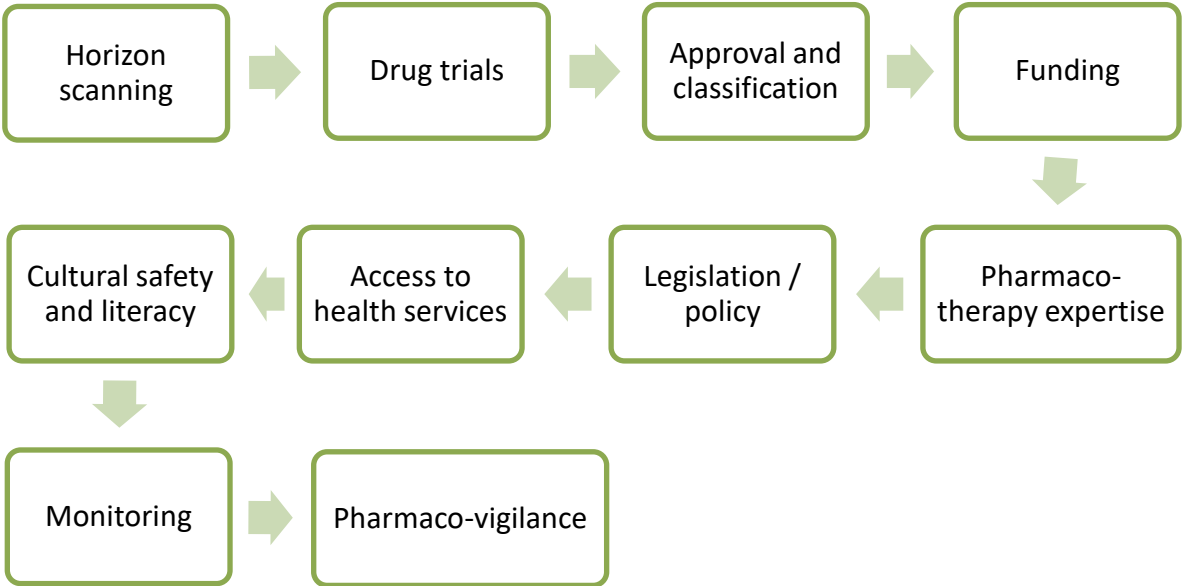
The various activities that make up optimal use of medicines require the involvement of a broad range of government agencies. Figure 9 below illustrates the extent of those activities (not all of which, we note, are within the direct control or influence of government agencies). We favour this more comprehensive approach and note that Pharmac is resourced to perform only a limited number of these activities.

²⁰³ See: <https://www.euro.who.int/en/health-topics/Health-systems/health-technologies-and-medicines/policy-areas/responsible-use-of-medicines>.

²⁰⁴ It can even extend beyond the health system. Antibiotic resistance, for example, is an issue for primary industries and animal health, too. Ministry for Primary Industries, 2017. Antibiotic Resistance Information Sheet: <https://www.mpi.govt.nz/dmsdocument/26341-Antibiotic-resistance-information-sheet>.

²⁰⁵ Te Karu et al 2018: <https://www.publish.csiro.au/hc/hc17067>.

Figure 9: Stages of a medicine’s optimisation approach



Source: Leanne Te Karu²⁰⁶

This system-wide approach would align with the Ministry’s medicines strategy, released in 2007, which envisages all organisations in the health and disability system (from the Ministry itself to Medsafe, district health boards, ACC, Pharmac and prescribers, dispensers, and other kaimahi) having some responsibility for ensuring the optimal use of medicines.²⁰⁷ Although this strategy is now in need of updating, we think this systematic approach is both sensible and necessary if all New Zealanders are to benefit equitably and appropriately from the country’s investment in medicines, and it should be a feature of any future medicines strategy.

While medicines optimisation is most clearly in the ambit of the health and disability systems, it is also important to note other sectors have a part to play too. For example, antibiotic resistance (and the concerns that existing antibiotics could become ineffective in treating bacterial infection) is an issue for primary industries and animal health, just as it is for the health system.²⁰⁸

Table 17 below provides a high-level summary of the agencies currently undertaking activities that can be classed as helping to ensure the optimal use of medicines.²⁰⁹

²⁰⁶ Derived from Te Karu. 2021: https://www.publish.csiro.au/hc/fulltext/hcv13n2_ed2#R16 (and discussion).

²⁰⁷ Ministry of Health 2007. Medicines New Zealand: Contributing to good health outcomes for all New Zealanders. Ministry of Health: Wellington.

²⁰⁸ Ministry for Primary Industries, 2017. Antibiotic Resistance Information Sheet. Available online <https://www.mpi.govt.nz/dmsdocument/26341-Antibiotic-resistance-information-sheet>.

²⁰⁹ The table is non-exhaustive, focusing on the various roles of government health organisations, although the review notes many organisations outside of government have a role.

Table 17: Agencies involved in helping ensure optimal use of medicines as at February 2022

Role	Agency	Commentary
Horizon scanning – Scanning for emerging trends	<ul style="list-style-type: none"> • Pharmac • Te Aho o Te Kahu (for cancer-related matters) • Ministry of Health 	<p>Scanning takes place in a piecemeal fashion, and no agency has explicit responsibility for such work. We have noted other jurisdictions with a dedicated unit have a continual and focused approach.</p>
Drug trials	<ul style="list-style-type: none"> • Ministry of Health • Medsafe • Health Research Council of New Zealand • Health and Disability Ethics Committees 	<p>Trials in New Zealand must be approved by the Director-General of Health, on advice of the Health Research Council of New Zealand (Medicines Act 1981).</p> <p>Medsafe, a business unit of the Ministry, runs the application process for clinical trials.</p> <p>HDEC administer the ethics approval system, which applies to all clinical trials conducted in New Zealand.</p>
Approval and classification of medicines	<ul style="list-style-type: none"> • Medsafe • Ministry of Health 	<p>New medicines cannot be marketed in New Zealand without the consent of the Minister of Health. Changes to use of medicines require consent of the Director-General of Health. Data that satisfactorily establishes the quality, safety and efficacy of a product must be submitted for evaluation before consent can be granted (Medicines Act 1981).</p>
Funding of medicines	<ul style="list-style-type: none"> • Pharmac • Ministry of Health • ACC 	<p>Pharmac is primarily responsible for funding and buying medicines (New Zealand Public Health and Disability Act 2000), although ACC can, in some circumstances, fund medicines not on the pharmaceutical schedule.</p> <p>Some medicines listed in the pharmaceutical schedule have conditions, determined by Pharmac, that must be met before funding will be granted.</p> <p>Pharmac manages the negotiation and purchase of subsidised medicines.</p> <p>Patients and their whānau may pay for some medicines directly. Medicines not appearing on the schedule require full payment by patients. For medicines that are partially funded by Pharmac, the patient pays the shortfall. Additionally, pharmacies can charge for extras such as out of hours dispensing or blister packing.</p> <p>Costs may also include prescription co-payments, which are currently set at \$5 for most subsidised medicines. There is also a prescription subsidy scheme available for people and families who have more than 20 prescriptions per year.</p>

Role	Agency	Commentary
Pharmacotherapy expertise	<ul style="list-style-type: none"> • Pharmac 	<p>Pharmac has contracted out this function to specialist providers since the 1990s.</p> <p>Other groups outside of government provide support to health professionals too, such as:</p> <ul style="list-style-type: none"> • The Goodfellow Unit delivers continuing professional development for primary health care professionals through multiple mechanisms • NZ Formulary, an independent resource for health professionals providing clinical validated medicines information and guidance on best practice in order to support prescribers to select safe and effective medicines for each of their patients. • Best Practice Advocacy Centre (BPAC) which continues to provide articles and prescribing tools • Matui Ltd, which provides He Ako Hiringa (discussed above) • Clinical Advisory Pharmacists Association (who provide advice to the above and also write regular columns for NZDr).
Legislation and policy	<ul style="list-style-type: none"> • Ministry of Health 	<p>The Ministry is the primary policy agency and is responsible for health-related legislation and associated strategies.</p>
Access to health services	<ul style="list-style-type: none"> • Ministry of Health • District health boards • Health providers 	<p>The New Zealand Public Health and Disability Act 2000 sets out the personal health, public health and disability services available to New Zealanders and establishes district health boards with functions to ensure provision of services for their populations and the reduction in health disparities.</p> <p>Subsequent policies, such as the Primary Health Care Strategy and He Korowai Oranga (the Māori Health Strategy), have set policy directions for access to services generally.</p>

Role	Agency	Commentary
Cultural safety and literacy	<ul style="list-style-type: none"> • Ministry of Health • District health boards • Health providers • Health professional responsible authorities • Health professional bodies • Health Promotion Agency • Medsafe 	<p>Ministry of Health has provided frameworks and guidance to district health boards and health providers on health literacy and communication.</p> <p>Responsible authorities are required to set out competency standards under the Health Practitioner Competence Assurance Act 2003, including cultural competence, which includes cultural safety.²¹⁰</p> <p>Ministry of Health has also provided information specifically around medicines in residential services (disability, mental health and addiction services). Other targeted messaging, focused on promoting health and wellbeing, can fall within the functions of the Health Promotion Agency (New Zealand Public Health and Disability Act 2000). Pharmac has in the past also worked on building health literacy around medicines – contracting this out to third-party providers.</p> <p>Medsafe provide detailed consumer medicine information factsheets (often available from pharmacies or prescribers and online).</p> <p>The Health Navigator Charitable Trust also runs a website (www.healthnavigator.org.nz) that provides a range of health information to New Zealanders, including about prescription medications.</p>
Monitoring	<ul style="list-style-type: none"> • Ministry of Health (date collection) • Medsafe • Medicines Control • ESR • Pharmac • Health Quality and Safety Commission • Providers • Health professionals 	<p>Monitoring happens at different levels and can range from monitoring the effectiveness of medicines, licencing of pharmacies, to monitoring access to services and prescriber behaviour.</p> <p>In some instances, the Ministry may work with ESR to monitor specific medications, such as vaccines. Pharmac reviews prescription patterns. The Health Quality and Safety Commission runs the Atlas of Healthcare Variation, which looks at variations across a range of clinical domains, including medicines for asthma, contraception, diabetes, gout and mental health. It also looks at opioid use, antibiotic use and polypharmacy.</p>

²¹⁰ See, for example, the Medical Council of New Zealand’s cultural safety standards: <https://www.mcnz.org.nz/our-standards/current-standards/cultural-safety>.

Role	Agency	Commentary
Pharmacovigilance	<ul style="list-style-type: none"> • Medsafe • The New Zealand Pharmacovigilance Centre 	<p>Medsafe undertakes post-marketing surveillance with the New Zealand Pharmacovigilance Centre, which umbrellas the Centre for Adverse Reactions Monitoring.</p> <p>This includes:</p> <ul style="list-style-type: none"> • monitoring adverse reactions to medicines used in New Zealand and monitoring international literature and other information sources • testing marketed medicines against product quality standards • handling complaints and investigations • auditing and licensing medicine manufacturers. <p>The Independent Safety Monitoring board monitors the safety of Covid-19 vaccines.</p>

Our impression is that a lot of this activity is often disjointed and inefficient, and that what coordination does take place between agencies is as much a matter of chance as design. The absence of a systemic approach also misses opportunities to eliminate barriers to equitable health outcomes through the provision of medicines, especially to priority population groups – Māori, Pasifika communities and disabled people.

For example, ensuring medicines are appropriate and accessible for disabled people requires not only competence from prescribers but for medicines information to be available in a range of different formats, including easy to read, and for online material to follow web content accessibility guidelines. Disabled people might also need different medications or for medicines to be dispensed in different ways, different formulations, or for carers or support people to be provided with extra information or support around the use of medicines.

It is well documented in literature,²¹¹ and was shared by a range of stakeholders,²¹² that eliminating inequities in many health conditions is dependent on equitable access to primary health care and that there is a range of barriers (such as cost and accessibility) for Māori and Pasifika communities and disabled people. This was also shown in the One Heart Many Lives programme evaluation, which highlighted the pressure on general practices, particularly Māori-owned providers, to provide wrap-around patient and whānau support within limited funding. While we are not convinced that prescribing the right medicines is an additional role for general practice – in fact it is core business – we are persuaded that improvements in primary health care overall are a necessary part of achieving medicines access equity, and responsibility for this rests with a wide range of agencies.

²¹¹ Te Karu et al 2018, available here <https://www.publish.csiro.au/hc/pdf/HC17067>.

²¹² Raised in particular by Māori stakeholders who saw the need for a joined up strategy around medicines, beyond just Pharmac *‘[the way things are run now] currently Pharmac only has a limited role, but if you had a system wide approach then you could think about much more effective ways of improving quality’*.

This provides a rationale, which is compelling to us, to ensure that strengthening the whole-of-system approaches to medicines optimisation is part of providing effective and comprehensive primary health care. We also think that having Pharmac as the only part of the health system with explicit obligations around 'responsible use' has meant that it has taken on activities that, while necessary, do not allow Pharmac to play to its strengths and that add to health system fragmentation.

A more cohesive approach is clearly required, but the question is what form exactly this new approach should take. In light of pending reforms to the health and disability system, our view is that overall responsibility for optimising the use of medicines should be a shared function of Health NZ and the Māori Health Authority and should be included in the New Zealand Health Plan, which is already a joint responsibility of these agencies.²¹³ Both agencies would be able to commission work to further the optimisation of medicines. This function needs to be woven into the entire health and disability system and should adopt a partnership approach and have links to the communities most likely to benefit from more equitable health outcomes.

We do not suggest Health NZ and the Māori Health Authority undertake all elements of optimisation work. On the contrary, we envisage other agencies in the sector, with health professionals, providers, communities and consumers, playing an active and at times leading role in aspects of this work. But Health NZ and the Māori Health Authority should set the strategy and plan, oversee its progress, ensure agencies are working in their areas of responsibility to advance optimisation, and ultimately be held accountable for the success of the strategy. As for Pharmac's place in this new arrangement, we consider it should concentrate on ensuring equity is a core part of technical assessments, funding decision-making and negotiations.

We also note the data and insights work has focused on Māori and Pasifika populations, and we see that as critically important. However, in the near future we expect Pharmac, as well as other health sector organisations, to be in a position to undertake deeper analysis of medicines optimisation for disabled people, and the intersection between disability and ethnicity. It is also essential this analysis move beyond identifying solutions through the narrow lens of what prescribers can do where there is good reason to suspect that this will not lead to the level of gains required to shift deeply rooted inequities. Ensuring analysis is acted on will require stronger partnerships and more collaboration throughout the health and disability system, as well as with communities and consumers.

Lastly, a new medicines strategy needs to be developed to replace the existing strategy if the goal of optimising medicine use is to be achieved. We cannot overstate the importance of this step. The strategy should be systems-based, contain a set of principles on the health and disability system's obligations under te Tiriti o Waitangi, have an unapologetic commitment to equity, apply the enabling good lives principles, and have a commitment to working in partnership with communities and consumers.

²¹³ Pae Ora (Healthy Futures) Bill, clause 14(1)(a).

Recommendations

Most of the recommended action in relation to responsible use can be found in the governance and decision-making sections of the report. In summary, they recommend the Minister:

- direct the Ministry to develop an updated medicines strategy and to consult stakeholders (including Māori, Pasifika, disabled people) on its contents over the next 12 months
- 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.

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.

11 Summary of recommendations

The review has provided 33 recommendations to the Minister of Health. If implemented, these recommendations will result in changes to the way Pharmac operates and makes decisions. They will also support Pharmac to work more closely within the health system as a whole. These recommendations are categorised below.

Governance and accountability

We examine Pharmac's governance and accountability arrangements and their effectiveness in ensuring Pharmac meets its objective and fulfils its various functions. We look particularly at Pharmac's board, its responsibilities and its performance in overseeing Pharmac. We also look at legislation to reform the health sector and Pharmac's place within the new framework. This section also contains brief discussion of Pharmac's research and communications activities, its clinical committees and its involvement in responding to Covid-19.

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 should apply to Pharmac
- amend Pharmac's functions:
 - transfer responsible use of medicines to Health NZ and Māori Health Authority
 - enhance its role as the lead advisory agency in security of supply for pharmaceuticals
- agree that 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 improve the transparency and accessibility of its systems, processes, resources, and communications to allow disabled people to participate and contribute on an equal basis
- require Pharmac to ensure its contractual obligations do not preclude sharing of commercially sensitive information with key monitoring agencies such as Health NZ, the Māori Health Authority and the Treasury
- 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.

Decision-making

There are improvements to be made in Pharmac's decision-making processes. These recommendations, if implemented, should result in more equitable access to medicines while retaining the successful elements of Pharmac's current work.

The review recommends the Minister direct 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 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

Pharmac continues to find itself in an unenviable position, needing to determine how it trades off expensive cancer medicines with other medicines that are also required to treat a growing burden of disease for an ageing population – all within an annual pharmaceutical budget.

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 but believed that 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

We examined how Pharmac approached assessment and funding of rare disorder medicines. We note the absence of a high-level strategy or formal definition for rare disorders which has wider impacts than Pharmac, and we also note the need to make improvements to the way the Rare Disorders Advisory Committee works. We also consider decision-making processes in light of the case studies discussed in the decision-making section.

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 health care 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 the RFP 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 (although the Ministry still has some input into the process). Pharmac follows the same approach as it does for pharmaceuticals. In this section, we look at whether Pharmac's approach is working. In our view, it is not. We looked at whether Pharmac's approach to procuring vaccines fits with New Zealand's public health priorities. In our view, it does not.

The review recommends the Minister:

- transition 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 they adopt
- Pharmac should continue to negotiate the price, supply and terms of conditions of supply, but should no longer 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 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

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

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. This transition should happen at the speed Health NZ determines
- direct Pharmac to work with Health NZ to complete the work to design the health technology assessment process
- any ongoing role for Pharmac in medical devices (for example in technical evaluation or as a purchasing agent) is a matter for Health NZ to consider and agree with Pharmac.

Responsible use

Pharmac is required to promote responsible use of pharmaceuticals and currently spends about \$2 million a year doing this. The review has examined whether this work is effective and if Pharmac is the right agency to be performing it. We concluded it is not, and we make recommendations in the section on Governance and Accountability to move the function to Health NZ and the Māori Health Authority.

The review 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.

12 Appendix

Table 18: Responsibilities under the memorandum of understanding

Ministry of Health	Pharmac	DHBs
<ul style="list-style-type: none"> • Continue to manage the national immunisation programme • Manage the national immunisation register • Ongoing surveillance of vaccine-preventable diseases, informing Pharmac where there are regulatory or public health changes • Seek via Pharmac advice from the Immunisation Advisory Committee on the national immunisation programme • Monitor the Government's immunisation health targets and notify Pharmac of emerging targets/government priorities • Accountable for international obligations and goals for vaccine-preventable disease management • Ensure adverse events are monitored and managed • Maintain cold chain management guidelines, work with district health boards to ensure best practice and provision of training • As required, make formal applications with the Minister of Health's approval to add vaccines to the national immunisation schedule • Publish the immunisation handbook that describes the policies and recommendations for use of vaccines 	<ul style="list-style-type: none"> • List vaccines on the pharmaceutical schedule, including eligibility criteria for personal and public health uses • Obtain data on the demand for/prescribing of vaccines • Manage all future vaccine funding applications and procurement of vaccines • Manage activities related to the supply of vaccines (including discussing with the Ministry and affected district health boards) and any proposed vaccine response to outbreaks and pre-pandemic preparedness, and vaccine supply and distribution, including cold chain management • Co-ordinate the provision of advice from the Immunisation Advisory Committee and/or PTAC to the Ministry about the national immunisation programme 	<ul style="list-style-type: none"> • Fund the vaccines on the pharmaceutical schedule under the Combined Pharmaceutical Budget • Fund the supply of vaccines required for local outbreak of diseases • Monitor and manage the cold chain practices of immunisation providers • Achieve the Government's immunisation targets

Source: Memorandum of understanding on vaccine funding arrangements, 2012.