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NEW ZEALAND INSTITUTE OF ECONOMIC RESEARCH

A new generation medicines policy

Improved access and equity for New Zealanders

NZIER report to Medicines New Zealand

May 2020

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Executive Summary

The purpose of this discussion paper is to help stakeholders understand and consider the issues when developing a new generation medicines policy, so that it is fit for the future.

What's the problem?

The Ministry of Health's *Implementing Medicines New Zealand 2015-2020* plan expires in 2020 (Ministry of Health, 2015). The plan was based on a medicines strategy dating back to 2007 (Ministry of Health, 2007).

The *New Zealand Health and Disability System Review* has excluded access to medicines. Now is the critical time to re-think medicines policy because medicines are integral to a high-performing health system. The COVID-19 pandemic has brought this into sharp relief.

The risk is that without a modern medicines policy that addresses the strategic issues, the access and equity gaps discussed in this paper will widen even further for New Zealanders in comparison, not just with other countries, but amongst ourselves.

What's been happening?

Access to medicines in New Zealand is falling behind comparator OECD countries due to a combination of reasons including:

- Medicines funding not keeping pace with health spending
- Social costs and benefits being systematically undercounted in funding decisions
- Decision rights on budget setting being unclear and not conforming with good public financial management
- The emergence of effective new medicines that are not accommodated by current funding appraisal processes. The speed of access can be slower in New Zealand.

What improvements might we expect?

New Zealanders have a fair expectation that the health system be on par with other OECD countries in terms of value, equity and quality in pursuit of good health outcomes.

Medicines policy objectives (and performance measurements) are the same as overall health system objectives, aimed at maximising value, equity and quality. Speedy access to medicines is core to public trust and confidence in the health system.

Based on the World Health Organization's health system building blocks, Table 1 sets out a summary of potential solutions and a set of key questions for consideration in a strategically pitched new generation medicines policy.

The recommended improvements in this paper draw largely on:

- *Pharmaceutical Innovation and Access to Medicines* commissioned by OECD Health Ministers in 2018.
- NZIER reports analysing:

- community pharmaceutical spending (to clarify actual medicines spending)
- the establishment of a medicines appropriations (to track, forecast and allocate)
- rapid access schemes (to efficiently assess and provide patient certainty).

- Other published literature and special reports on recent world-wide experience.

Table 1 Design considerations for a new generation medicines policy

Design element	Potential improvements and solutions	Questions for strategic policy design
Leadership and governance	Stakeholder collaboration to design a strategy that serves triple aim objectives for all New Zealanders	What are the advantages of an updated medicines strategy?
Leadership and governance	Publish forecasts based on analysis of science and expected innovations	Are there benefits in a transparent long-term assessment medicines funding path?
Leadership and governance	Health Minister (with Research, Science and Innovation Minister) set the budget	What is the best way to decide the medicines budget?
Leadership and governance	Legislative change to explicitly broaden the definition of health outcomes	Can governance and oversight arrangements be improved to focus on improved outcomes?
Funding	Establish target for medicines access in line with OECD countries	Should New Zealand's medicines budget be aligned with comparator countries?
Service delivery	Timelines for medicines approval and/or referral to rapid access scheme	Is there an optimal time limit for funding appraisal? Is there a case for rapid access?
Service delivery	Nation-wide standardisation of assessment of access to technology/medicines to reduce service variation via clinical networks	Does medicines and health technology assessment need to be better integrated to services and practice? What kinds of partnerships are needed to support this?
Information	Require Pharmac to include social costs and benefits in appraisals Design a Multiple Criteria Decision Analysis process to increase the quality of decision making.	How should cost-utility/benefit appraisal methods be updated?
Information	Improve authoritative data for dialogue	Do stakeholders need better information?
Workforce	Consolidate and strengthen capability	Is there capability to assess innovation?
Access	Regular reporting on population access Incentivise Māori and Pacific peoples-based trials 'Real world evidence' programmes	How can medicines policy support improved equity for disadvantaged populations?
Other considerations		What else can be done to support triple aim objectives for quality, equity and effectiveness?

Source: NZIER, OECD, WHO

What needs to happen next?

Medicines policy cannot be isolated from consideration of the report of the *New Zealand Health and Disability Services Review* to be completed in March 2020, because medicines are part of an integrated health system. The OECD recommends open stakeholder dialogue as the best way to address the challenges. This paper is intended to support that dialogue.

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1 What is the case for a new generation medicines policy?

1.1 The current strategy and plan expire in 2020

The New Zealand medicines action plan entitled *Implementing Medicines New Zealand 2015 to 2020* expires this year (Ministry of Health, 2015). The current action plan is based on *Medicines New Zealand: contributing to good health outcomes for all New Zealanders* dating back to 2007 (Ministry of Health, 2007). The government's strategy was based on three outcomes:

- quality, safety and efficacy
- access
- optimal use.

The 2007 strategy and 2015 plan focused more on *operational* policy (e.g. medicines adherence, prescribing practices) than on *strategic* policy.

New Zealand's previous strategy was *not* pitched to address:

- scientific advances
- funding
- equity
- counting social and economic benefits.

New Zealand is not alone in the need for an updated strategy. OECD Health Ministers commissioned a review in 2018 to better understand the advances and challenges in medicines policy (OECD, 2018).

The Australian *National Medicines Policy 2000* is up for review in 2020, having lagged other countries, with disruptive innovation, precision medicine and other factors as part of the rationale (Shaw and Chisholm, 2019). The Australian review will include multiple stakeholders (Hunt, 2019).

In accord with the OECD review, a 2013 review of national medicines policies from around the world concluded that good process involving stakeholders is important for a collective sense of ownership (Hobert et al., 2013). These relationships are especially important when systems are under extra strain, such as during the COVID-19 pandemic.

1.2 Social and patient perspectives appear to be systematically undercounted

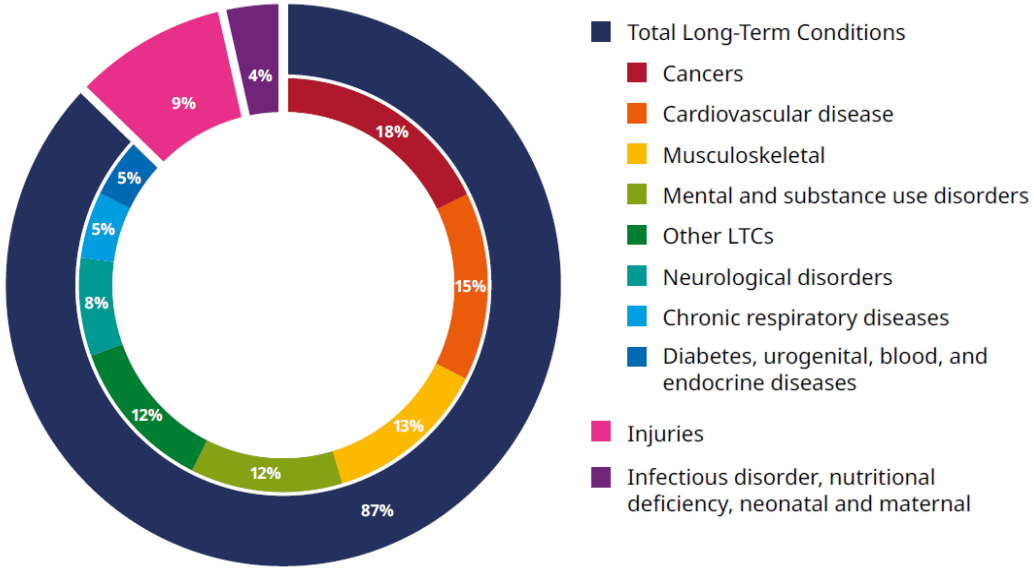
The report of the *Second Panel on Cost-effectiveness in Health and Medicine* (Saunders et al., 2016) concluded that health economic assessments too often are based on a health care sector perspective alone but presented as also including a social perspective when in fact they have not. This has resulted in systematically undercounting of wider benefits in funding decisions. Social benefits and patient reported outcomes are valid perspectives that are considered inconsistently in decision-making.

Pharmac's guide for medicines assessment, *Prescription for pharmacoeconomic analysis: methods for cost-utility analysis* (Pharmac, 2015b) focuses on a health care sector

perspective. The wider social and economic perspective is not taken fully into account when assessing and making trade-offs. New Zealand is in a unique position with Stats NZ's Integrated Data Infrastructure to connect health services and outcomes to social and economic outcomes and take a global leadership position in value-based funding.

The social and patient perspective becomes increasingly important when looking at the burden of disease and the extent to which people are now, and in the future, will be living with long term conditions as illustrated in Figure 1 below.

Figure 1 Leading causes of health loss in the New Zealand population



Source: Ministry of Health, 2018

The International Society for Pharmacoeconomics and Outcomes Research Taskforce has identified emerging good practice in multi-criteria decision analysis (MCDA) for the health sector (Thokala et al., 2016). This approach adds other perspectives (e.g. Te Ao Māori, patient reported outcomes) explicitly to decision making. MCDA is used in a range of sectors but only slowly gaining application in health. The Taskforce recommends that decision-makers work with stakeholders and provide support and structure to the decision-making process. Thokala et al. explain that:

> *MCDA techniques provide clarity on which criteria are relevant, the importance attached to each, and how to use the information in a framework for assessing the available alternatives. By doing so, they can help to increase the consistency, transparency and legitimacy of decisions.* (Thokala et al., 2016)

MCDA processes can be more complex but at the same time are more legitimate because the weighting of patient reported outcomes, social benefits, economic benefits and any other factors in consideration must be made explicit. This process is consistent with the OECD recommendations to improve stakeholder engagement in decision processes.

1.3 The equity gap grows when wider considerations are neglected

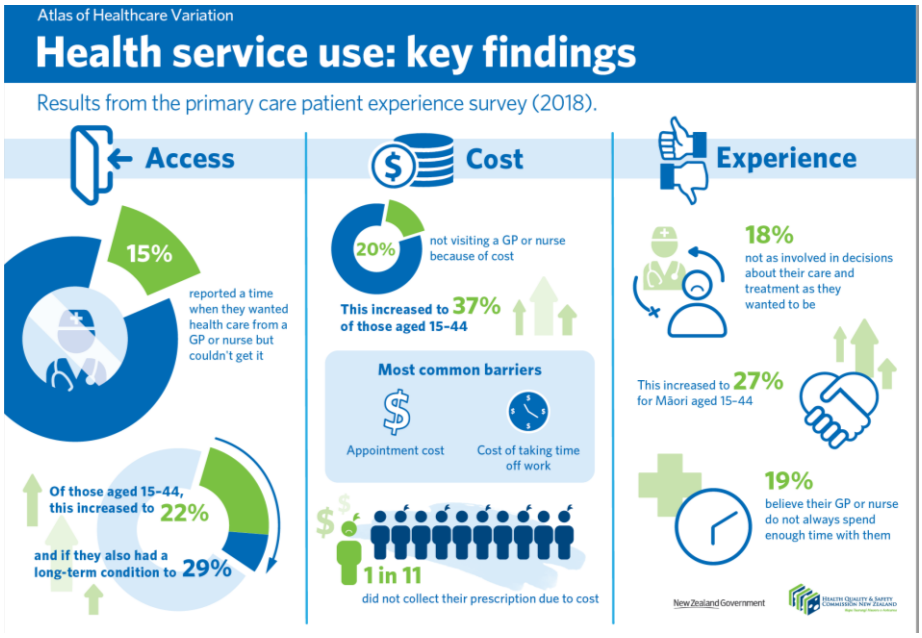
Improved equity is an enduring objective in health policy and a high priority for the government. Those left behind due to inability to pay are often those already disadvantaged. The Health Quality and Safety Commission (HQSC) stated in a July 2019 data release of the *Atlas of Healthcare Variation* (HQSC, 2019a) that:

> *One in five Māori and Pacific peoples reported not collecting a medicine due to cost in the past year. At all ages, people who reported a long-term condition were more likely to report not collecting a prescription due to cost*. (HQSC, 2019b)

This figure just applies to the part-charge for already subsidised medicines. The risk is that New Zealanders find, without system changes, the access gap and equity gap to modern medicines widens even further, because most patients cannot afford to pay out of pocket. This situation can erode public trust and confidence in the publicly funded health system.

Figure 2 below sets out some of the multiple sources of service variation in primary care due to access, part charges and experience. With these reported access barriers for funded medicines, the gap for access to unfunded medicines is likely to be far greater.

Figure 2 Patients face hurdles to services and medicines



Source: HQSC, 2019b

1.4 Budget decisions are opaque

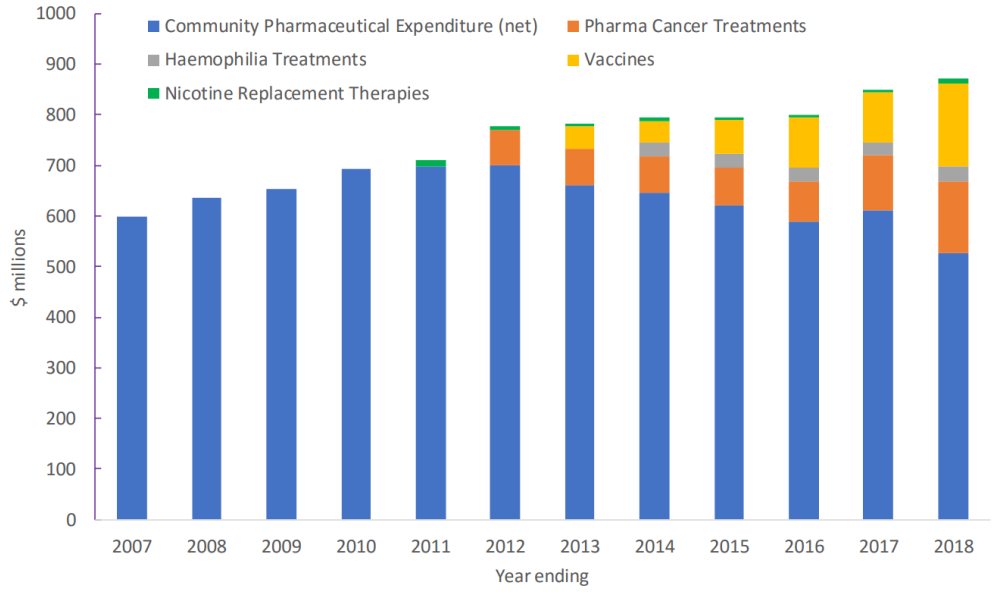
Pharmac is funded annually by District Health Boards (DHBs) to a budget agreed with the Minister of Health.

Figure 3, assembled from a range of sources, shows how from 2011, the year in which overall pharmaceutical budgets become 'combined' with vaccines, some medical devices, and some specialist treatments, community medicines expenditure dropped. Difficulty in understanding occurs when non-homogenous items (e.g. medical devices mixed in with medicines) are put together.

These changes have not been clear to the public because there is no requirement nor accountability to report. In fact, a false impression can be given when the scope of responsibility increases to include new items in what is essentially a budget transfer.

DHBs, who currently hold the decision right on medicines funding, have weak incentives to fund community medicines because of funding pressures on the DHB provider-arm. DHB (ownership) deficits can take priority over optimised service expenditure (DHB as a funder) for their populations. Making funds available for investments in prevention and chronic conditions management which would be expected to reduce acute demand in the future is challenging in an environment where current acute demand requires prioritisation for scarce resources.

Figure 3 Pharmaceutical expenditure by type, 2007–2018



Source: NZIER

Medicines meet the New Zealand Treasury's criteria for a separate appropriation (NZIER, 2019a). A separate medicines appropriation voted by Parliament would increase transparency so that medicines funding can be optimised across the entirety of the Health Budget.

1.5 New modern medicines can have high enduring benefits and higher costs

Pharmac's capacity to approve new medicines is limited by two factors:

- Rapid growth in 'supply', e.g. at 30 June 2019, it had 105 applications for new listings with positive recommendations from the Pharmacology and Therapeutics Advisory Committee (PTAC) (Della Barca, 2019)
- A budget that is not keeping pace with overall health expenditure (NZIER, 2018).

Science is creating new medicines in a way that has not been possible previously (NZIER, 2019b). This has become an allocation issue for the health system as a whole not just Pharmac, who are working to their currently defined role. Pharmac's statutory role,

breadth and weighting of evaluation criteria, timing of benefits, who captures those benefits (e.g. wider health system) needs review for new medicines that are high-cost, highly effective for a small group of individuals.

Current institutions and processes are not well placed to evaluate and fund new classes of medicines that are very effective because they come with high per patient costs and fall outside Pharmac's current ability to pay. These high cost treatments, while very cost-effective, are often so costly that they would crowd out too many other important investments within Pharmac's fixed budget.

What is not able to be fully accounted for in Pharmac's focus on value within the fixed budget resource, is the potential for new medicines to provide long term patient/social outcomes and health system savings.

An IQVIA Report (2019) concluded that up to 10 times more modern medicines (new molecular entities) are funded among 20 OECD countries, selected for being comparable to New Zealand. The New Zealand funding approval process for modern medicines was twice as lengthy. An examination of the way the New Zealand medicines budget is managed in comparison to Australia shows that New Zealand's access is much more limited for new medicines (Taylor and Wonder, 2015).

2 What are the relevant objectives and building blocks?

2.1 Taking aim with a framework

In pursuit of health outcomes, many health systems, including New Zealand draw on the triple aim objectives when designing and evaluating health policies. HQSC, for example, uses the triple aim when it reviews and evaluates health services in New Zealand.

Figure 4 Triple aim objectives for health policies

Source: HQSC

The triple aim matches health economics concepts of efficiency, equity and quality. A well-performing medicines policy is one that is designed to maximise the triple aim.

2.2 Building blocks of health policy applicable to medicines policy

The World Health Organization's building blocks of a well-designed health policy include:

- leadership and governance
- funding
- service delivery
- information systems
- workforce capability
- access to medicines and technology.

These building blocks should underpin the design of a medicines policy.

3 What are the triple aim considerations for each building block in a new generation medicines policy?

This section discusses triple aim's value, equity and quality dimensions of each of the six health system building blocks so that a new generation medicines policy is strategically integrated into the system as a whole and not separate from the system.

3.1 Leadership and governance

3.1.1 Understanding innovation for a modern health service

A well performing health system has the ability to understand the wider environment of science and technology. This means being able to assess innovation and improvements that can be adopted to increase value, equity and quality in use of the resources to produce services. The benefits of innovation are more services/medicines at the same cost or freed resources to deploy on new activities. Currently professional colleges, Pharmac and several DHBs undertake some limited technology and innovation assessment.

The system should be able to produce an outlook of potential future improvements to inform decision making at all levels of the system. This is a core strategic function that could be carried out by the Ministry of Health (led by the Chief Science Advisor) or another central body like the HQSC.

3.1.2 Getting away from narrow definitions of health outcomes

In the New Zealand Public Health and Disability Services Act 2000, the objectives of Pharmac are:

> *(a) 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; and*
>
> *(b) any other objectives it is given by or under any enactment, or authorised to perform by the Minister by written notice to the board of Pharmac after consultation with it.*

Operationalising the definition of "best health outcomes" is potentially limiting if it focuses solely on clinical outcomes. Good assessment looks at total costs and total benefits – including currently 'undercounted' social and economic benefits for the patient, health system and society. The determination of what constitutes a 'best health outcome' depends on perspective about value, equity and quality. Whānau, community and patient defined outcomes are all valid. Patient reported outcomes, for example, are important measures of quality that count and can be collected in well-designed trials and at implementation. A holistic view of 'best health outcomes' includes other aspects of health such as mental wellbeing and indirect contributions through social and economic participation, such as primary care giving, return to work and other activities of daily living.

Unless this wider perspective is made explicit in objective (a), the risk is that decisions on access to medicines can be sub-optimal due to underestimation of benefits relative to cost.

3.1.3 Improve forecasting and horizon scanning

A clear understanding of science, innovation in medicines and the funding path help health sector actors and institutions to plan and invest. This includes preparedness for unforeseen events such as pandemics that sit high on international and domestic risk registers.

The 2019 OECD report on *Improving Forecasting of Pharmaceutical Spending – Insights from 23 OECD and EU countries* concludes, inter alia, that:

> *… short-term projections of pharmaceutical expenditures can be used to support the determination of needed resources, the setting of budgets, or in the context of a hard budget constraint, to estimate the available 'headroom' for the addition of new medicines to a national formulary.*
>
> *A foundational element is effective horizon scanning, to identify late stage products in global industry pipelines.*
>
> *Above all, repeated comparison of actual trends to projected estimates is important for adjusting assumptions and improving both the confidence in, and the predictive value of these heavily parameter driven models, particularly if they are to be used to estimate the potential effects of proposals for policy reforms. This will also inform an assessment of the need for trade-offs between resource intensity and forecasting precision.* (OECD, 2019)

For New Zealand, this means the capability to produce short term out-year forecasts (4-years) and the capability to understand innovations including medicines that are consistent with triple aim objectives in the health system.

3.2 Funding

3.2.1 Establish a medicines appropriation and bring budget transparency up to standard

The medicines budget is not visible to the public because it is left to DHBs to decide with input from Pharmac and the Minister of Health. Creating a medicines appropriation means that Parliament decides as part of the government annual budget package instead of a negotiation between 20 DHBs, Pharmac and the Minister of Health. This supports triple aims objectives for value, equity and quality by recognising that medicines innovation is growing and should be compared to other health expenditure choices, free of DHB incentives/pressures to fund the services they own (NZIER, 2019a).

The annual medicines budget managed by Pharmac is presently determined by DHBs collectively and not part of the Parliamentary budget process. The Minister of Health is able to 'top up' this Budget through the budget process. Presently, it is possible to see the annual increment for each DHB without knowing the base medicines funding. Pharmac has some administrative discretion on how much is spent. In all, this governance situation is not transparent, and accountability is attenuated (NZIER, 2019a).

A specific medicines appropriation means that discussion on what the 'optimal' allocation of the health budget to medicines budget should be. This change would be in line with the New Zealand Treasury *Guide to Appropriations* and supports effective administration of the Public Finance Act (New Zealand Treasury, 2013).

An appropriation will increase the transparency of current and future budgets. Strategic allocation and technology assessment are not a focus or function of the 20 geographically based DHBs as service funders and providers. A medicines appropriation can be informed by improved horizon scanning and forecasting.

Importantly, an appropriation allows transparent consideration of how large the medicines budget should be in relation to other strategic health expenditure choices. Over time, transparency helps with decisions to optimise health expenditure and assist with public trust and confidence that New Zealanders have access to a fair and modern health service.

3.2.2 Reinstate expenditure trend reporting so resource allocation can be tracked

Reporting expenditure against the OECD health accounts framework is necessary for historical and inter-country comparison. While there is some data on pharmaceutical sales, it is not possible to accurately compare historical domestic trends or current international health expenditure trends including pharmaceutical spending in the government funded health system (Ministry of Health, 2012). This reporting is also important for understanding the public/private mix in funding, important for understanding overall access and equity. The last report was to 2010 published in 2012 (Ministry of Health, 2012).

The reporting is important because it allows for examination and optimisation of the high-level choices in the allocation of health resources (e.g. prevention, primary care, medicines, disability).

3.2.3 Match medicines expenditure with New Zealand's ability to pay and to benefit

New Zealand sits in the middle of the OECD in terms of wealth and development. Based on improved forecasting and horizon scanning, more insightful decisions can be made about how much should be spent on access to medicines. This will become an increasingly pressing issue as new medicines, producing good outcomes become available. The combination of a new appropriation, regular forecasts and reinstatement of expenditure reporting will help to determine a level of expenditure that is right for New Zealand.

3.3 Service delivery

3.3.1 Put time limits on assessment of new medicines to improve timely access

Placing a time limit on new medicines funding applications means that professionals and patients have a clear idea of what is, and what will be, accessible. This is an important matter of fairness, so patients and professionals know where they stand with treatment

options. 'Knowing where you stand' is an especially important aspect of the triple aim quality dimension of patient experience with the health system.

Rapid access schemes work alongside existing medicines and funding approvals to provide new classes of medicines to people who have high unmet medical needs, limited treatment options and may otherwise face lengthy waiting times for Pharmac-type approval processes.

In some cases, a time limit may be difficult to meet due to information needs. One way to support a final decision, while providing greater professional and patient certainty, is to place the medicine in a rapid access scheme where/while the missing information can be collected.

3.3.2 Link health technology and medicines assessment to service requirements

The New Zealand Productivity Commission (2019) noted in *New Zealand, technology and productivity* that:

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the main problem facing New Zealand today isn't too much technology, it's not enough. New Zealand needs to embrace technology, not treat it as a threat.

Presently health technology assessment is dispersed and uncoordinated across DHBs, professional colleges, universities and specialised agencies such as Pharmac. There is no systematic mechanism for the uptake of clinical guidelines, best practice etc. The National Institute of Clinical Excellence (NICE) plays this role in the English National Health Service (Cowles et al., 2017).

3.4 Information systems for better decision making

3.4.1 Update pharmacoeconomic appraisal to include patient and social outcomes

Pharmac's (2015b) guide for medicines assessment, *Prescription for pharmacoeconomic analysis: methods for cost-utility analysis,* needs to be updated in line with the report of the *Second Panel on Cost-effectiveness in Health and Medicine* (Saunders et al., 2016), if Pharmac is to retain good health economic assessment practices.

While this 'upgrade' applies across all health services, the wider social perspective in medicines appraisal will improve Pharmac decisions. It will also assist with comparing pharmaceutical-based interventions with radiological, surgical and medical interventions in clinical departments (e.g. cancer services) as evaluation in those areas improve. A review of the application of health economics evaluation in New Zealand points out that there is significant need for more health economics evaluation at treatment level to support cost-effectiveness and prioritisation beyond medicines (Cumming, 2015).

The Health Quality and Safety Commission conduct patient experience surveys but other countries have gone further with introduction of patient reported outcome measures (PROMS). Patient reported outcomes 'could help transform health care' because:

1 Most healthcare aims to reduce symptoms, minimise disability, and improve quality of life—these are aspects that only patients can assess

2 Patients welcome being involved, and this may have health benefits in itself

3 Patients' response rates are invariably better than clinicians' (a patient only has to complete one questionnaire whereas a clinician has to do it for every patient)

4 The measure avoids observer bias (inevitable if asking clinicians to assess their own practice)

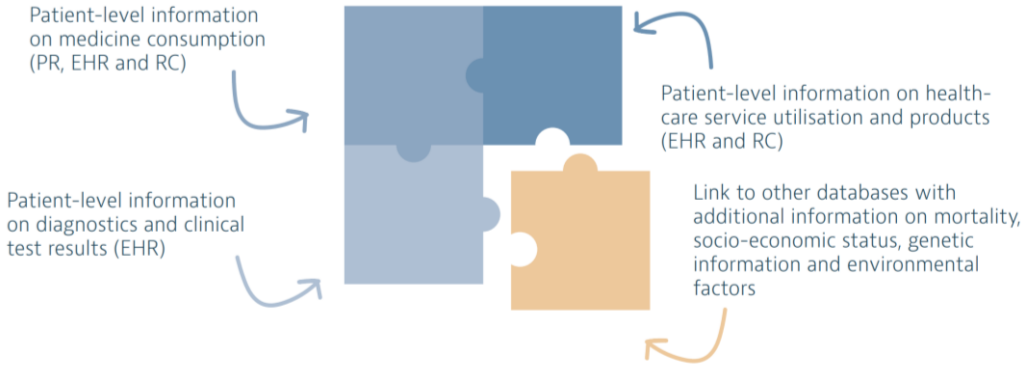
5 Considering patients' views increases public accountability of health services and healthcare professionals.

(Black, 2013)

3.4.2 Establish an information platform for collaboration

Having a common, trusted source of information assists with stakeholder engagement and is the basis of monitoring effectiveness, safety and the cost-effectiveness of medicines. The Pharmac stakeholder review report (Pharmac, 2015a) cites opportunities to strengthen communications with stakeholders. In particular, is the opportunity to use multiple channels (see Figure 5).

Figure 5 Main sources of routinely collected data



Note: PR: Pharmacy records, EHR: Electronic Health Records, RC: Reimbursement claims and billing information.

Source: OECD

A shared information network is the basis for dialogue. New Zealand is not pursuing a single electronic record. The National Health Information Platform (nHIP) will be the basis for a common platform (Macdonald, 2018). This approach can be helpful for medicines stakeholders looking to contribute.

3.5 Workforce

3.5.1 Develop skills to undertake health economic appraisals across the health system

The capacity and capability to undertake health economic appraisal in New Zealand is limited and 'stagnating' (Holmes et al., 2014). Innovation maturity can be assessed, benchmarked and a plan put in place to improve innovation capability maturity. New

Zealand universities graduate small numbers of economists, public health medicine specialists, and Master of Public Health graduates each year with expertise in epidemiology and biostatistics that can be deployed in health and technology assessment.

3.6 Access to medicines – especially for disadvantaged populations

3.6.1 Improve understanding of 'who gets what and why' in access

Medicines access can be limited by the overall size of the funding pool, and within the pool, a particular challenge for disadvantaged groups. Distributional effects – who gets what and why – is a central consideration in health policy. Improved equity is a centrepiece of government policy. Pharmac has set a goal to eliminate inequities in access to medicines by 2025 but cannot do this on its own. Pharmac has framed improved equity along the following five dimensions:

- availability – how PHARMAC makes and implements funding decisions so that everyone who is eligible can access funded medicines
- affordability – reducing cost barriers for priority populations so that people can afford funded medicines
- accessibility – ensuring people don't face challenges getting to see a prescriber or to the pharmacy
- acceptability – the ability of health services to create trust, so patients are informed and engaged enough to accept the medicines they've been prescribed
- appropriateness – the adequacy and quality of prescribing to ensure equitable health outcomes.

Pharmac will draw on their own research programme and the measures from Health Quality and Safety Commission to identify evidence of improved equity (Pharmac, 2019).

3.6.2 Improve the pace of access

Rapid access schemes (for medicines with government safety regulatory body approval processes fully completed) have emerged in response to promising medicines that are demonstrated as safe and efficacious but face delays in funding consideration. These schemes allow patients access when there are few if any options and allow for local data to be captured ahead of final funding consideration (NZIER, 2019b). Rapid access schemes can support triple aim's value and equity objectives when they provide real world evidence from New Zealand.

Under the Ministry of Health's Cancer Action Plan (2019-2029), access to modern medicines is currently being investigated and the Plan states that:

> *As a priority, the Government has agreed to a work programme allowing the Ministry to work with PHARMAC to explore options and provide advice on how to address the complex challenges of enabling earlier access to new cancer medicines" (Ministry of Health, 2019).*

In the past, early access but not rapid access has been afforded, in part, through Phase III trials (when medicines are introduced in therapeutic settings). As noted above, access at this stage is considered early access as the medicine is still at pre-approval stage in which

Medsafe has not approved the medicine. The number of Phase III trials for modern pharmaceuticals has dropped away in New Zealand from 72 in 2013 to 54 in 2018.

During this same period there have been no more than 6 Phase IV trials (of approved and registered medicines that are already in use in the New Zealand health system) to follow long term outcomes (NZIER, 2020). Boosting opportunities for these Phase IV trials is part of understanding 'real world' use in New Zealand. Late stage trials can have additional benefits such as attracting and keeping high calibre clinicians engaged in New Zealand.

3.6.3 Design clinical trials to support improved outcomes for Māori and Pasifika

One way to support triple aim's equity objective and Pharmac's goal to eliminate inequities by 2025 is through improved support for the involvement of Māori and Pacific peoples in research trials. If New Zealand does not undertake research on the mix of genomics and environment in the development of new medicines as they relate to Māori and Pacific peoples, it is unlikely to occur because the benefits are New Zealand specific.

*The Future of Precision Medicine in Australia (*Williamson et al., 2018) for the Australian Council of Learned Academies states that:

> *Twenty-first century biomedicine has started to unravel the complex interactions between genomic and environmental factors that underlie all biological functions. An enhanced understanding of genomics has allowed for better prediction, detection and treatment of certain cancers, rare diseases and many other conditions.* (Williamson et al., 2018)

Indigenous populations are underrepresented in pharmaceutical trials and genome studies with 80% of participants globally classed as European and 0.05% as 'indigenous' (Popejoy and Fullerton, 2016). There are international standards for diversity in clinical trials that may assist in working specifically with Māori and Pacific peoples to design protocols that serve these populations (Knepper and McLeod, 2018) Specific attention needs to be given to Treaty of Waitangi obligations.

If improving equity is an important triple aim objective, then research proposals that investigate Māori and Pacific peoples should be prioritised. This comes with costs of added study recruitment, greater attention to research ethics, Treaty of Waitangi obligations and data sovereignty. It may therefore take financial incentives to increase diversity in studies.

In any event, science progress in precision medicine technologies is likely to force the ethical issues faster than the ability of society and the regulatory regime to keep pace. National leadership will be required to manage the ethical, economic, social and cultural dimensions and maintain public trust and confidence.

4 How are other countries responding to the big challenges?

4.1 United States: partnering with leading pharmaceutical firms to accelerate COVID-19 vaccine and treatment options

The National Institutes of Health is leading a public-private partnership that brings together US health agencies and the European Medicines Agency to:

develop an international strategy for a coordinated research response to the COVID-19 pandemic. The planned Accelerating COVID-19 Therapeutic Interventions and Vaccines (ACTIV) partnership will develop a collaborative framework for prioritizing vaccine and drug candidates, streamlining clinical trials, coordinating regulatory processes and/or leveraging assets among all partners to rapidly respond to the COVID-19 and future pandemic. (NIH, 2020)

The programme, working with 16 industry partners, centres on four fast-track focus areas that will:

- standardise and share preclinical evaluation methods in an open forum that allows for comparison and validation
- prioritise and accelerate clinical evaluation of therapeutic candidates with near-term potential
- maximise clinical trial capacity and effectiveness by connecting existing networks of clinical trials to build capacity and capabilities
- advance vaccine development by creating a collaborative framework to share insights.

This approach recognises that the effort required to address COVID-19 is larger than any one country, institution or industry.

4.2 Australia: modernising medicines policy

The Government of Australia has announced a review of their 20-year-old National Medicines Policy. The policy was framed around access, safety, quality and innovation. Shaw and Chisholm (2019) have set out a rationale for review including changes in technology, personalised medicines, prescribing practices, information needs and industry policy.

Access to new medicines and ease of doing business are part of the review. The review terms of reference are expected to include a range of industry, patient, professional and government stakeholders (Hunt, 2019).

4.3 England: responsive appraisal of new medicines

The National Health Service (NHS) in England has established an Accelerated Access Collaborative (AAC) to bring health services and innovators from industry together to accelerate the introduction of cost-effective technologies and medicines, in pursuit of benefits:

> ***For patients:*** *this means access to new technologies and treatments faster than ever.*
>
> ***For clinicians:*** *you will have access to cutting-edge innovations that will help you deliver excellent care.*
>
> ***For industry:*** *we will help you understand the needs of patients and clinicians, and champion great solutions.*
>
> ***For investors:*** *innovations that meet NHS needs will receive greater support, from proof of concept to national commissioning.* (NHS, n.d.)

The AAC scheme helps to manage the 'pipeline' by accelerating promising technologies and medicines unto the assessment process (managed by NICE) and the support for faster adoption. These efforts are part of a wider health innovation ecosystem.

4.4 France: supporting innovation and paying for added value

Strengthening innovation capacity and culture

The French National Authority for Health has established an action plan for granting conditional approvals for 'high potential' new medicines, but for which long term data is not yet available (Haute Autorité De Santé, 2020 and Rodwin, 2019). This allows patients to benefit while the data is gathered on long term effectiveness. The action plan includes:

- Conditional approvals, allowing time for uncertainties to be resolved
- Tracking medicines in patients in real time against initial promises:
  - Prioritised by potential value
  - Early engagement with clinical developments
  - Promoting fast-track assessment
  - Streamlining assessment and approval processes
- Systematically involving patients
- Increased transparency
- Strengthen cooperation in shared knowledge.

Paying for the value increment

The French health system appraises pharmaceuticals on a 5-point added value scale. The subsidy/prices paid for coverage under the French national health insurance is a percentage of the total medicine cost based on the additional valued over the existing medicines. This pay for value incentivises the introduction of new and better medicines.

4.5 Germany: transparency for all

Transparent with incentives to agree a price

Germany is unique among pharmaceutical purchasers in that the negotiated net prices for a medicine is transparent, not the manufacturer's list price. German health insurers and the manufacturer are incentivised to agree a price. Failure to agree results in a referral to an independent board for binding arbitration. Both the insurer and manufacturer are bound by the arbitrated price. Therefore, there is an incentive to reach agreement.

5 Can we clarify institutional roles to meet the challenges?

5.1 Improve Parliamentary transparency in budgeting for medicines spending

Parliamentary scrutiny can improve understanding of how New Zealanders' access compares to other countries of similar levels of development. Parliament can help meet the challenges of a modern medicines policy by improved tracking of trends over time and between country performance for access and outcomes of medicines spending. The starting

point for this is a new medicines appropriation and holding executive government to account for decisions.

5.2 Strengthen support for the Minister of Health's role with medicines policy and procurement

As responsible Minister under the Crown Entities Act 2004, the Minister of Health is responsible for Pharmac's board appointments, strategic direction, performance expectations and review of overall performance.

To do this, the Minister needs strong support for good decision-making from advisors, including the Ministry of Health . This requires exercise of the public service stewardship function as set out in the State Sector Act 1988. Section 32 of the State Sector Act 1988 describes the stewardship responsibility of the chief executives to include:

- organisational capability and capacity to offer free and frank advice to successive governments
- stewardship of assets and liabilities on behalf of the Crown and
- the legislation administered by a department or departmental agency.

Effective stewardship encompasses strategic policy advice and monitoring from the Ministry of Health and the New Zealand Treasury. Other public sector entities such as MedSafe and the Health Quality and Safety Commission have important roles to play in monitoring service and clinical level aspects of Pharmac decisions.

5.3 Provide central leadership for innovation capability and capacity

Creating and maintaining a culture of innovation is important for a modern health system because it supports the triple aim objectives for value, equity and quality.

As a next step, and building on the work of the Productivity Commission, an innovation capability stocktake is a way to assess how mature the health system currently is and to identify the stretch needed to be reach full maturity. Innovation capacity and capability models can help identify the characteristics of a high-performing system (Essmann and du Preez, 2009). This is a leadership role, a combination of the Ministry of Business, Innovation and Employment, the Ministry of Health and the New Zealand Treasury who collectively have responsibility.

There are already partial elements of support for innovation to build on. The New Zealand Health Innovation Hub is owned by the Canterbury DHB with funding support from the Ministry of Business, Innovation and Employment. This hub focuses on commercialisation rather than uptake in the New Zealand health system, but it is an example of collaboration between industry and the health sector that can be expanded and made purposeful in supporting health sector performance. Example of overseas innovation partnerships are covered below.

5.4 Adopt a 'best practice' approach to pharmacoeconomic assessment

Updating Pharmac's pharmacoeconomic assessment guidelines and practice is a priority if Pharmac is to be up-to-date with best practice on health economic assessment.

MCDA and social and economic cost perspectives will improve the quality of decision-making because they examine total costs and benefits to people.

5.5 Provide research support for equity of outcomes in Aotearoa

As part of accelerated access, there is an opportunity to learn how medicines access is impacted in the real world where a range of barriers cam limit access and outcomes for disadvantaged groups before final, long term funding decisions are made. *Kaneke*, is an example of a Māori-responsiveness strategy aimed at supporting research and improved outcomes under a Māori kaupapa (Johnson and Johnson, 2018).

Improved access and equity starts with clinical research efforts because different patient groups may respond differently to a particular medicine. Improved participation by Māori and Pacific peoples in clinical trials provides data to support improved equity of access and outcomes. This includes sample sizes that allow for evidence to form around efficacy and effectiveness of medicines for Māori , Pacific peoples and any other groups that are unique in a New Zealand context. The medicines industry and the Health Research Council have an opportunity to work with Māori and Pacific peoples to improve our understanding of what works. New Zealand's Integrated Data Infrastructure can be used to research outcomes for Māori and Pacific peoples.

5.6 Strengthen collaboration through partnerships

Collaboration between academics, government, health professionals and the medicines industry is a way to accelerate development and introduction of medicines and technologies. This can support improved health, social and economic outcomes. Collaborations can be wide ranging and designed to ensure industry has 'skin in the game' in support of national priorities.

The examples in Appendix 1 have not been evaluated in this report but are provided to indicate the kinds of partnerships that can be formed to support a range of shared objectives.

6 Policy design questions: what needs to be considered?

This NZIER report examines key policy design issues for a new generation medicines policy for New Zealand. The design focuses on the 'big picture' policy issues for a modern health system.

The OECD Report to Health Ministers recommended improved stakeholder engagement and communication between patients, professionals, payers, policymakers and pharmaceutical manufacturers. This report concludes with a set of questions, examined in this report, as a contribution to improved engagement among these stakeholders.

Table 2 Design questions for a new generation medicines policy

Policy design questions for patients, professionals, policy-makers and medicines industry
1. What are the advantages of an updated medicines strategy?
2. Are there benefits in a transparent long-term assessment and funding path for medicines?
3. What is the best way to decide the medicines budget?
4. Can governance and oversight arrangements be improved to focus on improved outcomes?
5. How can New Zealand's medicines budget be aligned with comparator countries?
6. Is there an optimal time limit for funding appraisal?
7. Does medicines and health technology assessment need to be better integrated with services and practice? What kinds of partnerships are needed to support this?
8. How should cost-utility/benefit appraisal methods be updated?
9. Do stakeholders need better information?
10. Is there capability to assess and deploy health innovation?
11. How can medicines policy support improved equity for disadvantaged populations?
12. What else can be done to support triple aim objectives for quality, equity and effectiveness?

Source: NZIER

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Appendix A Examples of strengthening collaboration through partnerships

One-stop shop industry policy and regulatory partnership

The Medicines Manufacturing Industry Partnership (MMIP) established by government in the United Kingdom and industry in 2013 works closely with the Medicines and Healthcare Products Regulatory Authority (MHRA), UK Trade and Investment (UKTI), Innovate UK and the Office for Life Sciences (OLS), to sustain the United Kingdom's global position in medicines manufacturing technologies, production and digitalisation (The Association of the British Pharmaceutical Industry, n.d.).

The workstreams cover a broad set of policy and regulatory issues inherent in fostering a thriving industry:

- the technology and innovation landscape
- the fiscal environment
- the regulatory environment
- the skills environment
- advanced therapies manufacturing.

Access to global science networks for universities

In the Australian state of Victoria, Johnson and Johnson Innovation have partnered with Monash University, the Walter & Eliza Hall Institute, the University of Melbourne, RMIT and St Vincent's Institute to upskill the Victorian life science community and to develop programmes to foster greater connectivity at both a state and global level (Monash University, 2018). Medical technology and pharmaceuticals is one of eight growth priorities for Victoria.

Supporting global public health priorities

Johnson and Johnson has committed US$500m in support for research & development and delivery programmes over 2020–2024 to accelerate global efforts to eliminate HIV and tuberculosis (TB) by 2030 (Linnane, 2019). This partnership is in support of UN Sustainable Development Goals and works with international aid and development programmes to complement investments being made by governments.

Diversity and inclusion

The member companies of Medicines Australia have grouped together to support gender equity in the industry. The Pharma Australia Gender Equity Group supports Australian Government goals for gender equity through a commitment to pay equity, flexible working hours including part time, job-sharing and parental leave for everyone. Medicines Australia reported on industry performance in 2019 (Medicines Australia, 2019).

Community Impact with NGOs at local level across the globe

In New Zealand, the Salvation Army have partnered with Johnson and Johnson Medical to provide financial support to the Life Skills for Women programmes being run in Auckland, Rotorua, Wellington and Christchurch. The Programme last year helped more than 120 women complete a 10-week group-based programme designed to develop self-awareness, skills and confidence (Salvation Army, n.d.).

Integrating research trials, industry development and rapid access to innovation
The NHS England, Novartis, Oxford University and National Institute of Health Research have banded together to establish an innovation model for working fast and at scale. The collaboration centres on providing access to a cholesterol-lowering therapy with the potential to improve the outlook for people with heart disease (Nuffield Department of Population Health, 2020).

Building capacity for the future of personalised health care
The Australian government and Roche have formed a partnership to generate 'real-world' data about the potential impact and value of genomic profiling. In the context of profiling newly diagnosed lung cancer cases, the research skills developed increase capability to support government objectives to advance genomics and precision medicine (Australia. Department of Health, 2020).

Big data for application in the real world
Janssen (n.d.) has established *Real World Evidence Partnerships* with universities and the governments of Alberta, Newfoundland and Quebec in Canada. These long-term partnerships analyse large datasets to generate insights into how well medicines perform outside the clinical setting. This helps to inform decisions in health care delivery and policy in a way that is beneficial to the patients, professions and pharmaceutical firms.

Get Real is a similar programme in Europe with contributions from the major pharmaceutical firms, universities, government agencies, patient groups and small and medium sized businesses. *Get Real* stakeholders work together to develop consensus on best practice for use in regulatory and reimbursement decision-making.