PHARMAC

Pharmaceutical Management Agency

BRIEFING TO THE INCOMING MINISTER OF HEALTH

Date **8 November 2017**

To **Hon Dr David Clark (Minister of Health)**

Copies to

PHARMAC Board

DHB spokesperson on pharmaceutical issues

Director General of Health

Manager Governance & Crown Entities

Recommendations

It is recommended you:

- **note** the contents of this report

Contact(s)

Steffan Crausaz, Chief Executive
Jude Urlich, Director of Engagement and Implementation

[redacted]

Purpose

This briefing provides an overview of PHARMAC's work and signals some key issues for PHARMAC. We would be pleased to provide more detailed information on any specific area or issue.

An integral part of the NZ health system

We aspire to be a critical part of the health system delivering better health for New Zealanders

PHARMAC is a Crown agent governed by a Board. PHARMAC was established in 1993 and became a Crown entity under the NZ Public Health and Disability Act (2000).

Initially PHARMAC's role was to manage Government funding on medicines used in the community – those dispensed in community pharmacies. As PHARMAC established a successful track record in this area it was progressively tasked with more responsibilities. In 2001 the Minister of Health, Hon Annette King, directed PHARMAC to perform an additional function to manage the purchasing of hospital pharmaceuticals. Together, these responsibilities now see PHARMAC fully or partly managing public spending of approximately $2 billion, out of the total $16.7 billion spent on health.

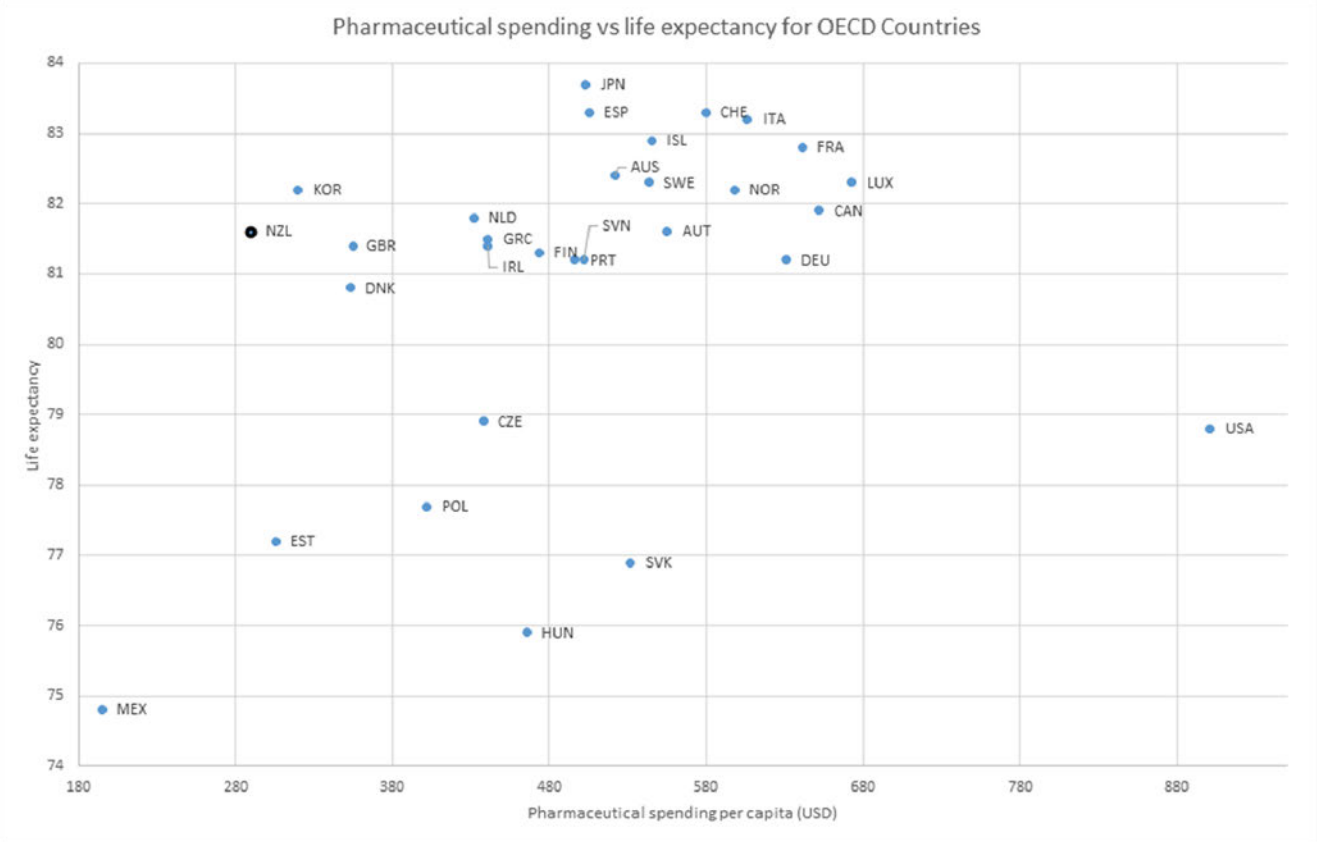
PHARMAC's growing responsibilities

1993: community medicines
2002: off-patent hospital medicines and cancer basket
2004: influenza vaccine
2011: hospital cancer medicines funding
2012: vaccines
2013: hospital medicines
2013: haemophilia treatments
2014: first contracts for hospital medical devices

PHARMAC's role, as defined in the NZPHD Act, 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."***

While there are a range of factors affecting life expectancy, New Zealand has a high life expectancy relative to the amount of money spent on pharmaceuticals, in particular when compared with the UK, Canada and the USA.



One of the underlying principles of PHARMAC's work is nationally consistent access to pharmaceuticals used either in the community, or in hospitals. Primarily, this is effected through PHARMAC's management of the Pharmaceutical Schedule: the list of medicines, medical devices and related products that are funded by the Government. DHBs are required to comply with the Pharmaceutical Schedule, and this generally occurs consistently across New Zealand, avoiding the phenomenon of 'postcode prescribing' which has been an issue in New Zealand and other countries.

PHARMAC manages the Combined Pharmaceutical Budget (CPB), funding set by the Minister of Health and provided by DHBs for community medicines, hospital cancer medicines, vaccines and haemophilia treatments.

Over time, PHARMAC intends to move the following additional responsibilities into a similar level of budget management:

- managing the list of **hospital medicines** that DHBs can use including generating savings and deciding on new investments
- negotiating national contracts for **hospital medical devices** that DHBs already buy, building our knowledge and offering early savings for DHBs.

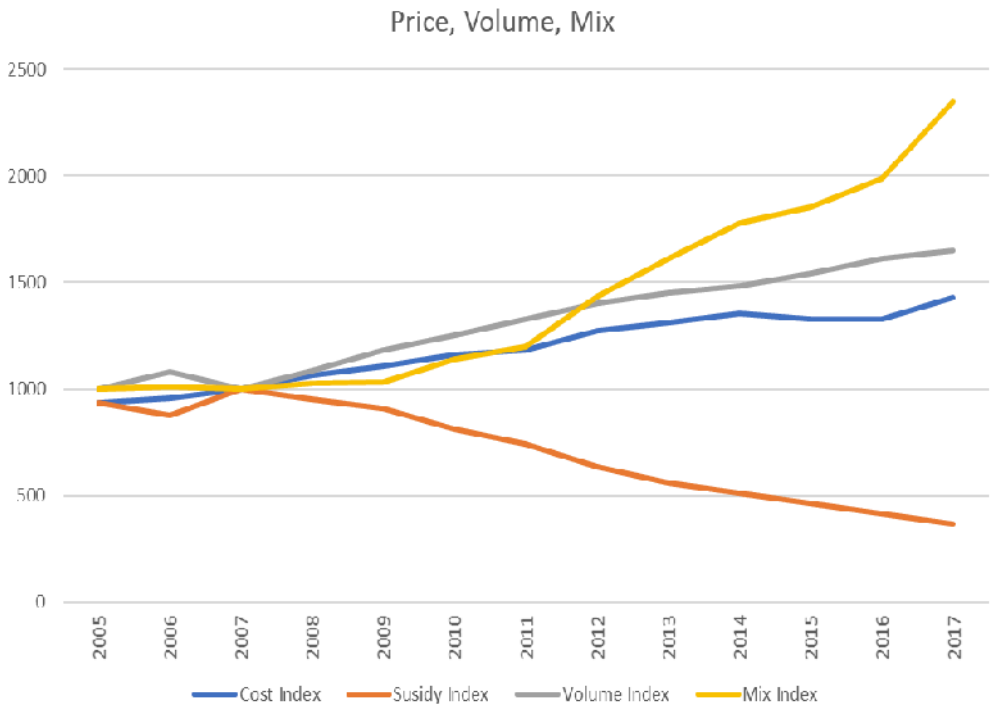
Regardless of whether a budget applies, PHARMAC takes a similar approach to all its work. Commercial strategies promote competition amongst suppliers, leading to long-term and sustainable falls in the cost of products. PHARMAC is able to free up funding to create additional "headroom", to supplement any budget increases approved by the Minister. This means that, over time, the cost of medicines is falling and PHARMAC's value to the system is rising. At the same time, despite being just 0.1% of the global pharmaceutical market, we contract for medicines supply and maintain active supply chain vigilance to ensure minimal impacts to New Zealanders from global stock supply issues.

We engage widely with stakeholders, listen to their concerns and modify our proposals where this helps us achieve our goal of best health outcomes. Alongside our regular management activity, we are committed to eliminating medicines access inequity, delivering $1 billion savings in hospital medical devices, and creating systems to make the best investment choices across all PHARMAC activity.

Our achievements

PHARMAC consistently manages DHBs' spending on medicines within budget and broadens the range of medicines available to New Zealanders, demonstrating the value of the PHARMAC model.

The Price, Volume, Mix chart shows that within the allocated budget (cost) the number of funded prescription items is increasing (volume), changes in the type of funded medicines rise each year (mix), but their individual prices (subsidy) are falling overall. This shows PHARMAC's management is creating greater efficiencies in funding while improving the range of options available.



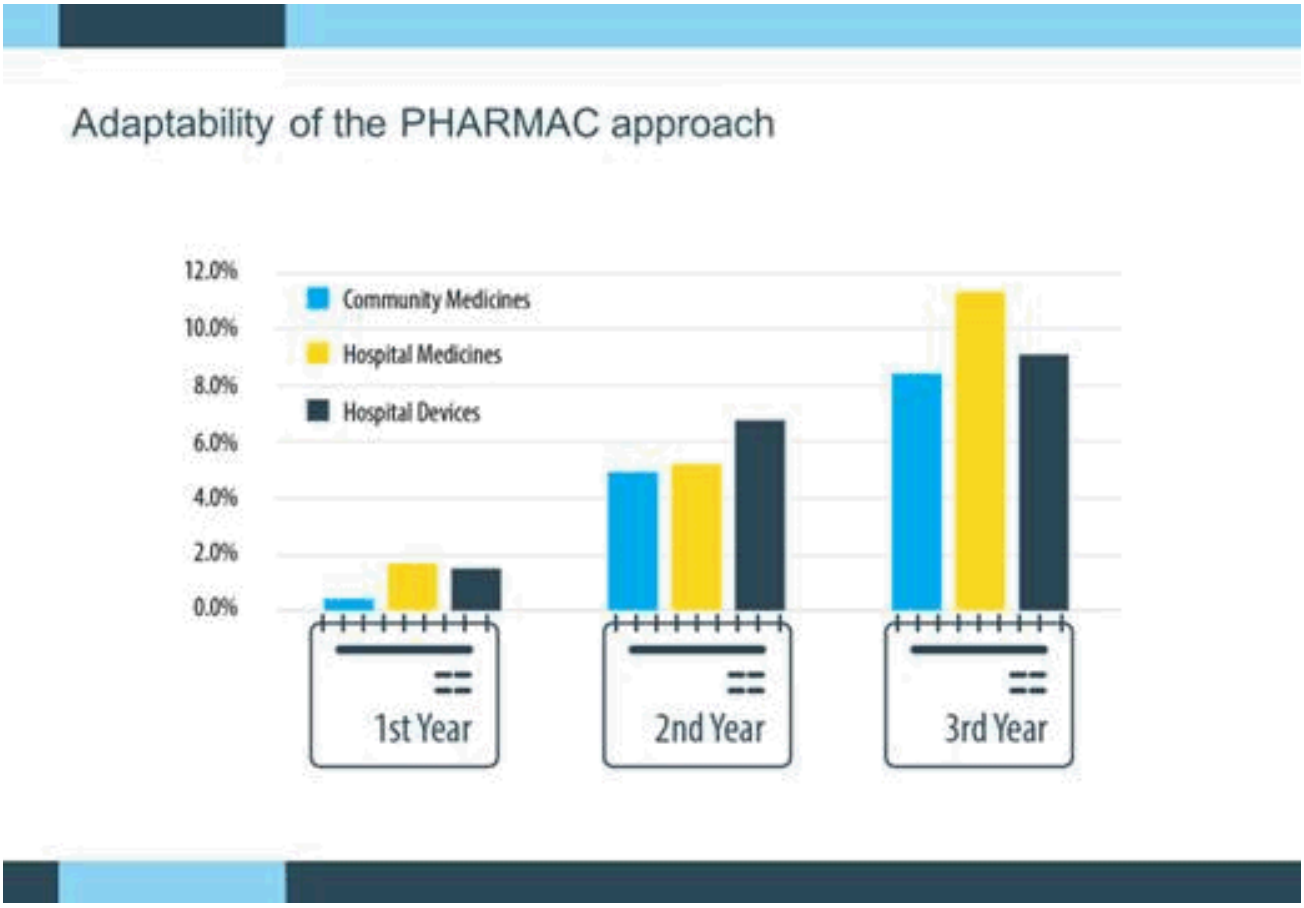
Without PHARMAC managing the CPB, over 10 years DHBs would have needed to find an additional $6 billion to fund existing health services. The five year cumulative savings to the health system of all hospital medicine and medical device decisions since PHARMAC took on this management in 2013 is an additional $251 million.

PHARMAC creates considerable efficiencies for the investment from Government. In 2016/17 the operating cost of PHARMAC was $24 million, while cumulative savings in the year were $1.5 billion in the CPB. Savings in hospital medicines and medical devices were a further $48 million over five years. This means that for every dollar spent by Government each year, PHARMAC creates long term benefits which over 10 years are worth at least $50 in savings to the system.

In recent years, PHARMAC's size has increased to enable effective management of its expanded role in hospital medical devices. The work began in 2012, and first national contracts were reached in 2014. By 1 November 2017, PHARMAC had national contracts covering more than $155 million of DHB spending on hospital medical devices, with plans for continued expansion.

Our work in hospital medical devices is tracking well. In the first three years of activity, savings on hospital medical devices are following a similar pattern to what PHARMAC saw in the early years of its management of both community and hospital medicines. This gives us confidence that the PHARMAC model is applicable to, and can have long-term benefits for, DHB spending on hospital medical devices.

Comparison of savings on medicines and hospital medical devices



PHARMAC's strategy

PHARMAC's Statement of Intent 2017/18-2020/21 includes three 'bold goals' to achieve its vision of being critical to the health system delivering better health for New Zealanders. Central to these is a goal to tackle inequities in access to funded medicines. Common to achieving all our goals is a requirement to work effectively with our health sector partners, as some of the enablers to achievement of these goals will involve cross-sector activities, or changes to systems managed by other entities.

Eliminating inequities may be about initiating programmes to ensure the medicines that are currently funded are better used, or used more equitably. Equity issues are at the heart of two strategies we currently have: Te Whaioranga, our Māori responsiveness strategy; and our Pacific Responsiveness Strategy. Achieving both involves close community engagement and support.

Our bold goals to achieve by 2025 are:

1. Eliminate inequities in access to medicines
2. Generate $1 billion of savings from medical device management to reinvest in health outcomes for New Zealanders
3. Create systems that enable the best investment choices to be implemented consistently across all PHARMAC activities.

These are ambitious targets which may not be achieved, and as such they may set up PHARMAC for criticism as having not met targets. However, our view is that setting ambitious targets and falling short can still take an organisation further than setting modest targets that are achieved.

Getting better health gains and more value from our wider activities

Vaccines

PHARMAC began managing the national immunisation schedule in 2012. Since then, PHARMAC has added five new vaccines and given New Zealanders greater access to 13 funded vaccines.

We have achieved these results while managing the impact on our budget - while the gross expenditure on vaccines has increased, implementing the PHARMAC model has meant that actual expenditure, via confidential rebates, is well managed. Highlights of our management of vaccines have included:

- Listing of **rotavirus vaccine** for all children, from 1 July 2014. Rotavirus is a significant source of gastric illness in young children, and the listing of the vaccine was linked with a 75% reduction in children up to 2 years being admitted to Auckland hospitals.
- Listing of **varicella (chickenpox) vaccine**. This was first listed in 2013 for children with compromised immune systems. In 2017 access was widened to all eligible children, when chickenpox vaccine was included in the national immunisation schedule.
- Funding **HPV vaccine** for males up to age 26. As well as causing cervical cancer in women, human papilloma virus (HPV) is associated with other cancers such as throat, and head and neck cancers.
- In 2013, pregnant women were able to obtain the funded **vaccine against diphtheria, tetanus and pertussis**, ensuring an estimated 30,000 women and their new-born babies being protected against these diseases.
- The awarding of a sole supplier, Mylan, to provide **influenza vaccine** in 2017-19 and enabling pharmacists to administer the flu vaccine to people aged 65 and over, and pregnant women, meant more people could access the flu vaccine.
- The **shingles (zoster) vaccine** for people aged 65 will be available from 1 April 2018. A two-year catch-up programme for people aged 66-80 will also be funded in combination with the annual influenza vaccines, so 600,000 New Zealanders can benefit.

The number of New Zealanders who have access to funded vaccines grew from 1.75 million in 2013 to an anticipated 2.05 million in 2018. At the same time, the net price paid for vaccines is falling.

Distribution efficiencies

PHARMAC also streamlined vaccine distribution systems from 2014, and signed new agreements for national and regional storage and distribution. This has resulted in improved stock management, reduced wastage and improved reporting to enable greater oversight by PHARMAC. As well as driving more savings in our operational budget through these agreements, PHARMAC is better placed to respond to supply issues, or issues that may occur in the environment.

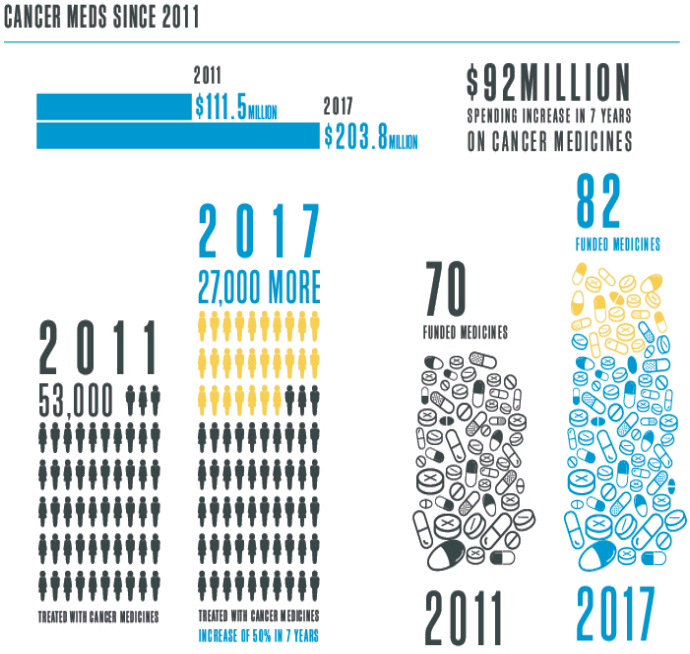
The vaccines story demonstrates the power of the PHARMAC model - improving access to vaccines, listing more vaccines and streamlining distribution, all while containing increases in cost, so that more New Zealanders can live longer and healthier lives.

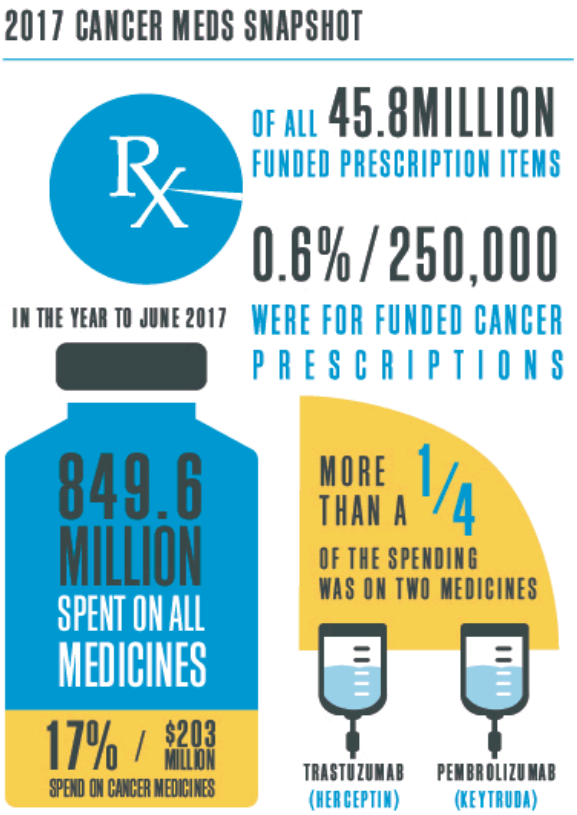
Cancer medicines – more spending, increased access

Historically, PHARMAC's role with funded cancer medicines had been to manage those dispensed in the community, while hospital cancer treatments were funded by DHB hospitals, up to an agreed amount in a separate fund.

In 2001, the New Zealand Cancer Treatments Working Party (NZCTWP) initiated by Minister of Health, the Hon Annette King, recommended all new pharmaceuticals for treatments of cancer be assessed by PHARMAC. The hospital-funded cancer treatments list (the cancer treatments "basket") was established as a list of medicines to be funded and available consistently across New Zealand.

PHARMAC was later directed by Minister King to take responsibility, on behalf of DHBs, for expenditure on pharmaceutical cancer treatments used in hospitals for in-patients and out-patients. This meant that as PHARMAC obtained savings on hospital cancer medicines, they could be reinvested into new cancer medicines or widening access to existing cancer medicines – much the same system as now in place for the remainder of all other hospital medicines.





Pharmaceutical cancer funding was eventually transferred to PHARMAC and became part of the combined pharmaceutical budget from 2011/12.

Since PHARMAC took on the funding of all cancer medicines within the CPB, there has been considerable expansion in access to cancer medicines in New Zealand. About 50% more New Zealanders are treated with funded cancer medicines now, compared to 2011.

We've funded 12 new cancer medicines since 2011, and enabled 12 others to be funded for more types of cancer. This has widened the choice of cancer medicines available for clinicians to treat their patients.

At the same time, PHARMAC has helped to influence the model of care for cancer patients by introducing new types of cancer medicines that can be given in the community, mainly oral treatments like tablets. This means that many people can now take their cancer treatments at home, reducing costs and freeing up capacity in hospitals, so that more people can be treated overall. And receiving cancer treatments at home can be less disruptive and stressful for patients.

New cancer medicines continue to be expensive. In the year to June 2017 total spend on cancer medicines was $203 million, or 17% of total spending on all medicines. However, prescriptions for cancer medicines represent just 0.6% of the total. This reflects the very high cost of new cancer treatments. More than a quarter of the spending was on only two (but high-profile) medicines, – trastuzumab (Herceptin), and pembrolizumab (Keytruda).

Managing this expenditure within larger budget means that PHARMAC is able to negotiate bundled product deals across a supplier's portfolio of medicines. This enables agreements to list more cancer medicines overall and improve access to existing medicines, while managing pricing and access to other important medicines. We work closely with DHBs so they can plan for and manage the associated hospital service impacts such as increases in specialist diagnostics or infusion services.

What makes PHARMAC different?

Most developed countries have a pharmaceutical technology assessment agency, separate from the regulator. In New Zealand the regulator is Medsafe, part of the Ministry of Health, and the technology assessment and funding agency is PHARMAC.

New Zealand is unique in creating a management agency that combines clinical, economic and commercial aspects, and decision-making within a fixed budget for pharmaceuticals.

PHARMAC has a focus on the best health outcomes it can achieve within available funding (be it the fixed value of the CPB or the amount of savings generated in hospital medicines transactions).

PHARMAC's core competency is our ability to distil a wide range of disparate information, and apply that knowledge and expertise to make funding decisions that achieve the best health outcomes for New Zealanders in the long run. We do that by:

- distilling clinical evidence and critically appraising evidence;
- having a deep understanding of the health system, and how it works, including the supply chain and the impacts of our decisions on the system;
- appreciating and understanding patient-level impacts of diseases and treatments and the impacts on each opportunity;
- using commercial nous and analytical rigour in our supplier negotiations;
- communicating and engaging with stakeholders to receive thorough advice via consultation; and
- implementing the decisions so they land safely in the market, and evaluating our results.

PHARMAC is an evidence-based decision-maker and this approach means that New Zealanders can be reassured that the medicines funded are those that offer the best health outcomes for patients, and that medicines funding is being used wisely.

PHARMAC in action – the hepatitis C story

New generation antiviral drugs to treat half the total hepatitis C patient population are now funded – but their high cost was an initial barrier. At an international market price of about $1000 per pill per day for three months, funding direct-acting antivirals for everyone with hepatitis C could have cost in excess of $1 billion.

PHARMAC combined clinical advice and commercial negotiation, targeting the drugs to specific groups of patients while negotiating confidential discounts.

Now half the total patient group are getting fully funded access to these new drugs at a sustainable cost, with expert clinicians reporting high cure rates above 90%. At the same time as improving people's quality of life this can also reduce demand for liver transplants, and the incidence of liver cancer.

Factors for Consideration

Another unique aspect is the Factors for Consideration, a holistic decision-making framework that PHARMAC developed in 2014-15 in consultation with the New Zealand public, to better reflect New Zealanders' views of what is important for PHARMAC to think about in making its funding decisions.

The Factors for Consideration came into effect in July 2016. An advancement on the nine Decision Criteria, this new framework was extensively consulted on, both in face-to-face meetings with the general public, suppliers and clinicians, as well as online. The Factors for Consideration provide stakeholders with improved transparency around our decision-making, and take into account key issues such as health system costs and impacts on patients, their families and whānau. The Factors for Consideration also give PHARMAC more flexibility to deal with complex commercial environments and future proof our decision-making processes for the longer term.

The Factors for Consideration allow us to consider evidence across four dimensions when making funding decisions (need, health benefits, costs and savings, and suitability), and three levels of impact (to the person; the person's family, whānau and wider society; and to the broader health system).

We take current government health priorities into account under two of our Factors for Consideration and we keep these up-to-date in consultation with the Ministry of Health:

1. **The impact on government health priorities** - this factor asks whether the disease, condition, or illness is a Government health priority.
2. **Consequences for the health system** - PHARMAC's decisions can have flow-on impacts for the rest of the health system. This factor considers the potential consequences of a decision for the wider health system (for example, the funding of a pharmaceutical that can be delivered in the community may free up resources in hospitals which could lead to greater efficiencies to the health system). Considering the government's strategic intentions for the health system under this factor ensures alignment across the health system.

Commercially astute

PHARMAC's approach goes well-beyond procurement, seeking to actively manage markets for pharmaceuticals so it can seek out opportunities for savings. This includes a wide range of commercial strategies to ensure that New Zealanders get the best health outcomes.

Evidence-based

All decisions are underpinned by clinical evidence, with objective expert advice provided by the Pharmacology and Therapeutics Advisory Committee, and its 20 subcommittees in speciality areas such as cancer, diabetes and mental health. Altogether these committees provide a network of about 140 highly skilled New Zealand health professionals providing expert advice to PHARMAC – a considerable resource. For decisions affecting individual patients, expert clinical advice is sought to inform funding decisions. For hospital medical devices, two specialist advisory groups of clinicians have been established so far.

Approachable and sincere

PHARMAC recognises the impact of our decisions on New Zealanders and their families. Sometimes those decisions may not always be popular, and we understand that this can be difficult for some affected people and their families. We are sincere in our efforts to do everything we can to fund the medicines that New Zealanders need, and we work tirelessly to lift the quality of our engagement with New Zealanders and those most closely affected by our decisions. This has included regular community forums, and topic-specific forums and meetings. We have a Māori Responsiveness Strategy, Te Whaioranga and a Pacific Responsiveness Strategy, both developed in partnership with those communities. Our Consumer Advisory Committee is a statutory Committee providing advice to the PHARMAC from a consumer or patient point of view. We survey our stakeholders and continuously respond to feedback on ways to improve our communication.

Challenges for PHARMAC and the wider system

We recognise the importance of improving how the health system works together. The New Zealand Health Strategy provides welcome direction and the health system's Medicines New Zealand Action Plan and Pharmacy Action Plan are also relevant to our work. PHARMAC works with a wide range of stakeholders, particularly clinicians, colleges and professional associations, DHBs and their agent New Zealand Health Partnerships Ltd, pharmaceutical suppliers, the Ministry of Health, pharmacists and consumer groups. We constantly seek to improve our communications and engagement, to work well with others and improve understanding of PHARMAC's work.

A recent area of focus has been improving engagement with the Ministry of Health and DHBs on impacts from our decisions for other health sector expenditure (both now and in the future). This work is a collaborative effort, consistent with the New Zealand Health Strategy's expectation that health entities actively pursue a better-integrated and consumer-focused system.

Pharmaceutical suppliers continue to push the boundaries of evidence-based decision-making, a challenge in an environment of continued high public expectation for access to new medicines. Our view is that the industry has a continuing obligation to provide high-quality evidence to support their funding applications. PHARMAC applies a range of factors to guide our decision-making which include the benefits, risks and costs and savings of treatments. High-quality evidence is important for understanding who is likely to benefit the most from the available funding and where the risks might lie — particularly the risk that new and significant side-effects will emerge from on-going treatment not discovered in an under-powered or poorly-designed trial[1].

Important components that build system integrity

Supporting factors

The pre-defined level of the CPB is a key strength, enabling PHARMAC to make responsible choices whilst understanding the relative value across other funding opportunities the sector might have. A discipline regarding the need to create savings before making investments in hospital medicines is particularly important for hospital medicines investments, as PHARMAC is able to determine the next best spend regardless of setting.

A Memorandum of Understanding with 20 DHBs, which they are required to adhere to under the Crown's Operating Policy Framework.

Cabinet decisions – on the strength of the 2001 Ministerial Direction authorising PHARMAC to manage hospital pharmaceuticals, Cabinet required PHARMAC to take on making decisions about vaccines, remaining hospital medicines and all hospital medical devices. This provides a clear pathway towards greater gain for the health system as PHARMAC works towards full budget management in all of these areas.

Crown agent – the fact that PHARMAC is the Crown's agent enables a clear focus on achievement of what is in the long-term national interest. It ensures PHARMAC exerts stewardship over this area of Government investment.

The Budget setting process involves an annual review of expected pharmaceutical expenditure. If PHARMAC did not undertake transactions to lower costs, each year between $60-$90 million new funding would be required from Government every year to maintain growth in usage of current products. However, PHARMAC ensures that this does not need to occur. Most years, PHARMAC is able to secure new medicines and widen access to existing medicines for new groups of patients from within those savings. This is confirmed with the Minister of Health, and in agreement with DHBs, including any advice for whether new Vote funding is desired.

[1] http://archive.jsonline.com/watchdog/watchdogreports/fda-repeatedly-approved-cancer-drug-afinitor-without-proof-it-extended-life-b99628814z1-361607291.html/

System challenges

Hospital data systems – these are variable in scope and quality and not nationally-consistent. To obtain the best health outcomes from applying a national lens, PHARMAC requires an effective hospital management system to ensure ordering and supply is linked to products within the Pharmaceutical Schedule. This is a particular issue for PHARMAC's long-term aim of securing even more significant savings from hospital medical devices. At this stage, the National Oracle Solution commissioned by the 20 DHBs is likely to be available for PHARMAC's use from 2023, at which point securing accelerated benefits from national management will be possible.

Sector operations – the Ministry of Health provides a claiming system for community pharmacies that relies on PHARMAC providing the Pharmaceutical Schedule in a suitable format. This system enables pharmacies to claim the service, distribution and pharmaceutical cost for each dispensing from the relevant DHB. PHARMAC relies on the data produced from these claims to manage the CPB. There is a risk in that the IT product is 20 years old and out of service. Future-proofing this essential service is necessary. In the interim, PHARMAC has signalled its willingness to provide the claims validation component of the system, to feed into the claims payment service offered by the Ministry of Health.

Funder incentives – under current policy settings DHBs pay for pharmaceuticals, including the distribution and services in the community. Currently this is negotiated between the 20 DHBs and pharmacy owners. The cost of doing so largely reflects the bricks and mortar approach of pharmacies and the variability in business process efficiencies across this part of the sector. As a consequence of approach, DHBs pay pharmacy owners around $400 million to dispense and provide advice to consumers on pharmaceuticals. Last year all pharmaceuticals cost DHBs $849.6 million to purchase. The most expensive or complex medicines are managed by PHARMAC through direct distribution arrangements, DHB hospitals or, in the case of vaccines, through other primary care claiming mechanisms.

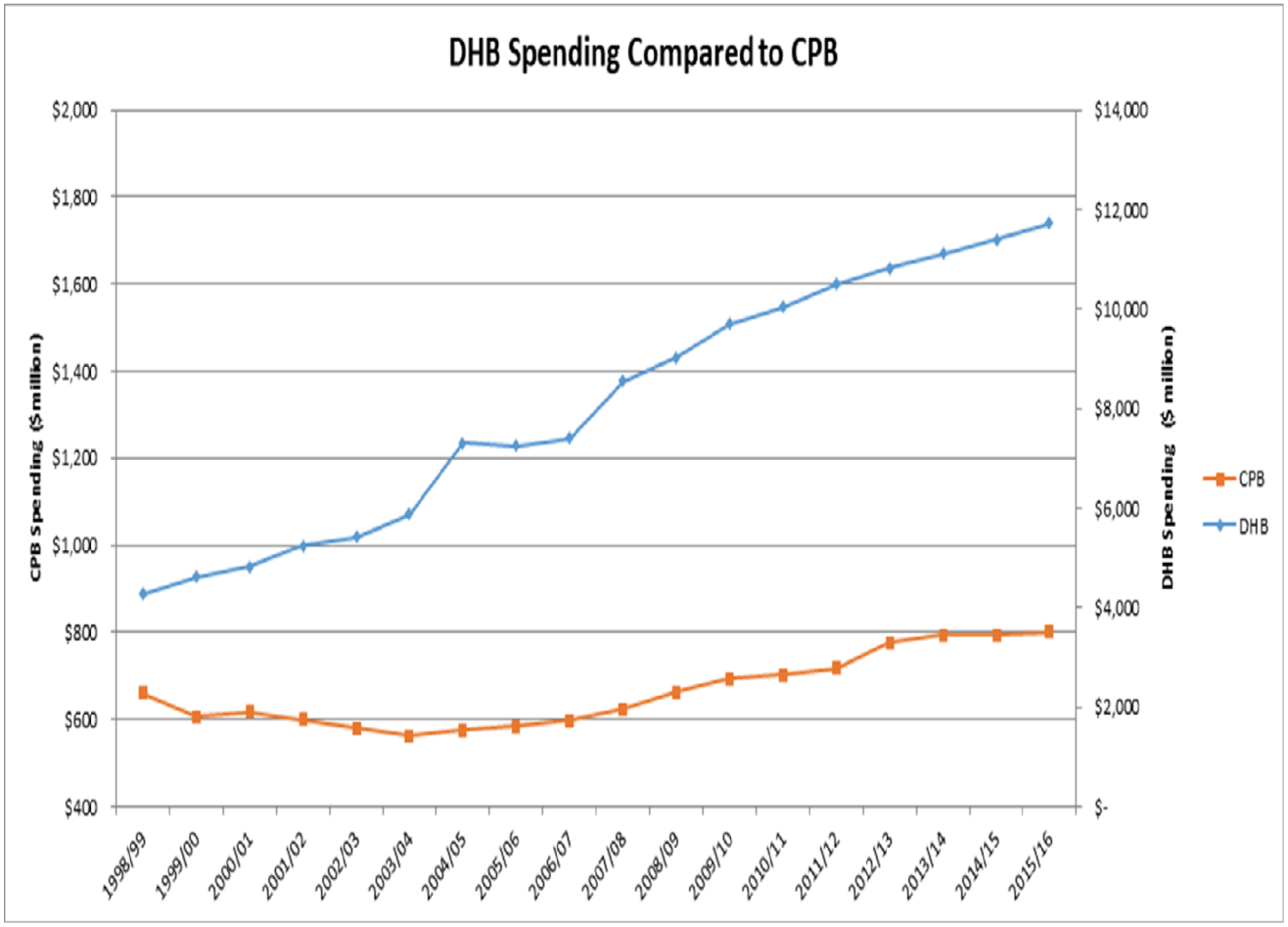
Every year, PHARMAC works with DHBs to forecast growth and anticipate the demand for the portfolio of medicines included in the CPB, while taking into consideration the value of savings PHARMAC expects in advance to offset this. Where an increased amount is identified, this is typically well-within the general funding increase DHBs receive from Government. The funding arrangements and financial flows are complex, however we believe there are some opportunities to further improve value in the system.

Co-payment policies - co-payment policies are set by the Ministry of Health with the pharmaceutical co-payment activated through the Pharmaceutical Schedule. The policy as it stands currently may lead to unintended consequences particularly for less socio-economically advantaged New Zealanders. While the rules around the use of co-payments in public hospitals are well-defined, they are not so in the community. User charges for services as they move into the community are increasingly evident, for example infusions provided in primary care have out of pocket costs upward of $100 per person. Such costs are likely to raise considerable barriers to access and equity. PHARMAC sees value in reviewing the current co-payment settings, particularly in respect to pharmaceutical co-payments.

Current activity and issues

Ongoing demands for medicines – recent examples have included immunotherapy or PD-1 inhibitors (pembrolizumab/nivolumab) for advanced lung cancer; earlier access to HIV medicines including PReP; medicines for rare disorders; medicines for breast cancer; funding for Medsafe-regulated pharmaceuticals derived from cannabinols (NB: not medicinal cannabis). Public advocacy for funding medicines can be vigorous and sometimes supported and driven by pharmaceutical suppliers. Increasing the level of medicines funding does not 'solve' this issue as it simply shifts the focus to the next medicine for a particular disease. It is appropriate that people should feel able to enthusiastically participate in public processes and have their voices heard. However, there are important questions of equity raised when relatively poorly-resourced and under-represented groups are not similarly able to participate in raising access to medicines issues. PHARMAC's job is to make the best choices from the available funding, and often the medicines subject to topical debate are not at that time the next best spend, having regard to all of the factors for consideration.

The funding level of the CPB – often commented on as 'adequacy of funding'. It is important to note that while the value of the CPB has risen over time, so too has its scope. From its origins in community medicines and a limited range of medical devices and related products, it now includes hospital cancer medicines, vaccines, and haemophilia treatments. This enables PHARMAC to manage access to these products as well as managing all costs associated with growth in uptake. Over time more products used in hospitals are expected to be added to full budget management under the CPB. This portfolio approach enables the widest possible benefits to be obtained for New Zealand. PHARMAC's ability to secure multi-product bundled deals with pharmaceutical suppliers across a wider range of community and hospital products allows for more new medicines and wider access to existing medicines to become available.



System changes arising from the TPP – PHARMAC is continuing to progress work required for New Zealand to implement the TPP, under Ministerial direction. If implemented, PHARMAC would be required to make certain medicine funding application decisions within 30 months, and to offer an internal review to those which are declined.

Community-based access to medicines – for more than a decade, New Zealand has had a system goal of shifting care closer to home, with the person at the centre of our service delivery. In the case of pharmaceutical funding this is relatively easy to achieve and PHARMAC has succeeded in transferring some, but not all, of certain hospital outpatient services to community access. Service planning is not advanced enough to ensure that people receive this care at no charge at all DHBs. Over time, the service capacity of DHB hospitals is likely to be exceeded due, in part, to the development pipeline for new pharmaceuticals being dominated by biologics (which are infusions requiring in-patient or out-patient infusion services). While PHARMAC intends to continue to consider increasing access to funded pharmaceuticals delivered outside DHB hospitals, the question of removing private sector service payments is likely to develop into an issue of access equity.

Stock supply continuity – PHARMAC actively manages pharmaceutical stocks with suppliers, and sometimes needs to take action to enable New Zealanders to continue to receive their medicine or medical device. Typically we are managing potential stock issues on around 40 products at any time. However, overall New Zealand has fewer medicine supply issues than other countries due to its approach to managing supply, despite being a small player in the global pharmaceutical market.

Personalised medicines - medical practice has always been evolving in terms of the ability to target illness or disease more accurately, efficiently and/or safely. Personalised or precision medicine is a further evolution in this trend, an attempt to harness breakthroughs in science and technology to customise or tailor pharmaceutical treatments to individual patient characteristics, including genetic make-up and biomarkers of disease. Personalised medicines account for around 40% of all medicines under development, including three quarters of cancer medicines under development[2].

Personalised medicine is a term that needs to be used carefully given its use as a marketing technique. New medicines marketing tends to involve launch of a high-priced product targeted for a specific use. After funding is secured it is successively extended to include more groups of patients as further trials are completed. New immunotherapy agents such as PD-1 inhibitors were initially marketed as personalised medicine because of their ability to target T-cells directly for specific types of cancer. In New Zealand PD-1 product launches have conformed to this practice. However, one product was recently approved in the US for all cancers based on a common biomarker, regardless of tumour type[3]. This transforms the product from a personalised one, to one able to be marketed for a broad market.

While personalised medicines present exciting opportunities, there are also challenges including pricing, impacts of supplier marketing, regulatory and privacy issues. PHARMAC already assesses personalised medicines for funding and is well-placed for future assessments as the trend of increased tailoring of medicines continues. A well-established process for named patient pharmaceutical assessment (NPPA) for patients in exceptional circumstances is working well.

Comparisons with Australia - naturally being New Zealand's closest geographical neighbour, comparisons of treatment availability in Australia compared with New Zealand is a recurring theme brought up by advocacy groups, such as Medicines New Zealand. As with all medicines, our approach is to look for best value investment in medicines that give therapeutic benefit and rely on the evidence, and good commercial positioning when making our funding decisions. Our recent analysis entitled 'Mind the Gap' demonstrates that the medicines Australia funds for treatment of an array of cancers often do not demonstrate sufficient progression-free or overall survival gains, and that some may be harmful. PHARMAC's evidence-based approach to funding cancer medicines has been endorsed in *Seminars in Oncology*[4] as an example for other jurisdictions to follow. This is not a unique finding – other publications in peer reviewed journals have reported similar issues recently[5].

A contestable funding pilot for **medicines for rare disorders** commenced in 2014/15 and closed in early 2017. PHARMAC was able to fund 10 medicines from the pilot and after an external review published in June 2017, PHARMAC decided to introduce a set of dedicated initiatives to consider medicines for rare disorders in the future. A standing PTAC expert committee for rare disorders is being established, and PHARMAC will regularly call for rare disorder funding applications, and undertake pre-engagement with new and existing suppliers to encourage more applications. We have adjusted our policy settings so that rare disorders are defined more clearly and we have waived the requirement for Medsafe registration to participate in the process (but we would still need this for listing of a treatment). We will run a contestable RFP process for a portfolio of rare disorders treatment and use alternative commercial arrangements, depending on the circumstances.

[2] Personalized Medicine Coalition (2017) The personalized medicine report: 2017 – opportunities, challenges, and the future (PMC: Washington DC; USA).

[3] https://www.fda.gov/newsevents/newsroom/pressannouncements/ucm560167.htm

[4] https://www.fda.gov/newsevents/newsroom/pressannouncements/ucm560167.htm

[5] https://www.ncbi.nlm.nih.gov/pubmed/28453615

Special Ring-Fenced Funds - PHARMAC has always been open to funding promising new medicines with emerging evidence. Our expert clinical advisers critically appraise key trials including study design and the data generated. Important connections are made to New Zealand clinical practice and adjustments for the pharmaceutical comparators used, where these are not the standard of care in our health system. Where data are incomplete yet show high health gain, we look for answers given that results of first trials presenting large treatment effects often dissipate as new evidence accrues.

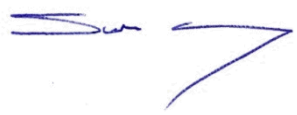
The medicines industry has an incentive to secure funding for new medicines while evidence is still emerging, in some cases prior to regulatory approval and commercial launch. This is because studies of both US and European regulatory approvals show these early approvals often fail to deliver on early promise. In the US of 36 cancer drugs approved by the FDA between 2008 and 2012 on the basis of surrogate outcome measures, only 5 were shown to improve overall survival by 2014[6]. Analysis from the EMA's approvals for cancer drugs between 2009-13 showed most entered the market without evidence of benefit or survival gain. More than three years afterwards, there was still no conclusive evidence that lives were extended or improved for most cancer indications, and when they did, these were often marginal[7].

The experience of the UK Early Access to Medicines Scheme[8] and separate Cancer Drugs Fund have meant we have proceeded cautiously, particularly given the PHARMAC model is so different to other systems. In New Zealand we would need to consider the impact of separate funds on PHARMAC's ability to negotiate complex multi-product deals across a portfolio of medicines. It would also make unpicking existing arrangements challenging. However, there is likely to be a uniquely-New Zealand solution to management of separate funding initiatives, potentially along the lines of the novel approach successfully developed to improve access to medicines for rare disorders funded within the CPB. PHARMAC is constantly keeping track of trends and opportunities in medicine development and is happy to provide more advice on emerging trends and how we might consider these.

Mental health - is a priority area for the new Government, and PHARMAC has a critical part to play to ensure people can access the right pharmaceutical treatments for mental health conditions. We carefully consider any funding decisions that will impact on this vulnerable population group and consult with patients, advocacy groups and health professionals when making decisions that are likely to have an impact on them. Brand changes are an example of decisions that can have a disproportionate impact on people with mental illness. In such circumstances, we work closely with health professionals and patients to provide support so that people are smoothly transitioned to the different brand. PHARMAC also has a mechanism through our decision-making framework (factors for consideration) that ensure we take into account government health priorities in our pharmaceutical funding decisions.

Closing comment

We take a 'no surprises' approach with open communications with you and your office staff. We look forward to meeting with you to discuss our role; key opportunities and challenges ahead; and what reporting and meeting arrangements would best work for you.



Steffan Crausaz
Chief Executive

[6] https://jamanetwork.com/journals/jamainternalmedicine/fullarticle/2463590
[7] http://www.bmj.com/content/359/bmj.j4530
[8] https://academic.oup.com/annonc/article-lookup/doi/10.1093/annonc/mdx110