PHARMAC
TE PĀTAKA WHAIORANGA

Level 9, 40 Mercer Street, Wellington
PO Box 10254, Wellington 6143, New Zealand
P: +64 4 460 4990 | F: +64 4 460 4995
www.pharmac.govt.nz

21 October 2019

Ms Louisa Wall
Chairperson
Health Select Committee

Via email: Health@parliament.govt.nz

Dear Ms Wall

Re: Questions from the Health Select Committee ahead of PHARMAC's appearance on the 23rd October 2019

Thank you for the opportunity to provide more information about PHARMAC's assessment and decision-making processes. We understand that you are particularly interested in the progress of the medicines listed in recent petitions to the Committee. Attached (refer to Appendix One) is an update on the medicines since last presented to the Committee in May 2019.

Increasing transparency and improving public trust, confidence and understanding of PHARMAC remains an organisational propriety for PHARMAC. An update on PHARMAC's work programme is also attached (refer to Appendix Two).

Below are PHARMAC's responses to the specific questions you have raised. We look forward to discussing these with you further on Wednesday 23 October.

Yours sincerely

Sarah Fitt

Sarah Fitt
Chief Executive
PHARMAC

How does PHARMAC consider international best practice guidelines when considering funding new medicines, particularly for rare disorders?

Health funders around the world are grappling with the challenges of meeting the often-high health needs of people with rare disorders. PHARMAC has been wrestling with the same challenges. Common challenges experienced around the world for the assessment of medicines for rare disorders include:

- lack of good quality clinical data, including real-world data, and long-term outcomes data;
- poor cost-effectiveness of these medicines;
- high budgetary impact; and
- difficulty in monitoring treatment efficacy.

PHARMAC routinely considers international best practice guidelines when assessing and considering medicine funding applications. PHARMAC's clinical advisory committees assess the strength and quality of the clinical evidence submitted by applicants. In making their recommendations to PHARMAC, they also take into account international treatment guidelines and recommendations by key advice or funding medicines bodies in similar countries (Australia, England/Wales, Scotland, Canada) – particularly when giving advice to PHARMAC on how a new medicine would be used in New Zealand were it to be funded. This is the same for all health conditions, including rare disorders.

In 2018, PHARMAC established a Rare Disorders Subcommittee of expert clinical advisors to provide us with advice on medicines for people with rare disorders. Given the limited expert pool of clinicians in New Zealand that treat people with rare disorders, one of the members that PHARMAC appointed to this Subcommittee is from Australia; a clinical geneticist and metabolic physician. This enables direct access to best practice knowledge from Australia.

How does PHARMAC's cost/benefit analysis for medicines that treat rare conditions and have fewer patients, differ from the analysis for medicines that treat more common conditions?

PHARMAC considers all medicine funding applications, including rare disorders, through its decision-making framework, the Factors for Consideration[1]. The Factors for Consideration require PHARMAC to contemplate a broad spread of issues, including the health needs of patients and their whānau, the benefits and suitability of a potential treatment and the costs and savings associated with the treatment.

The Factors that are particularly relevant for people with rare disorders include:

- *The health need of the person* – rare disorders can often be debilitating and severe, so individuals with a rare disorder are often considered to have a high health need (in terms of high case morbidity and fatality, and thus loss of life potential through dying early and/or beforehand suffering or being disabled with poorer quality of life).

[1] www.pharmac.govt.nz/medicines/how-medicines-are-funded/factors-for-consideration/

- *The availability and suitability of existing medicines, medical devices and treatments* – people with rare disorders often have limited alternative treatment options available that would treat the disorder itself (as opposed to the symptoms or side effects of their illness).
- *The health needs of others* – caring for a person with a rare disorder can affect the health of those with this responsibility.

The economic analysis that feeds into the Factors for Consideration is based on a Cost Utility Analysis (CUA)[2]. This analysis is the same methodology applied to all medicine applications to PHARMAC.

A PHARMAC staff member recently attended a one-day workshop on rare disorders titled '*Improved methods and actionable tools for enhancing Health Technology Assessment'* at the 2019 annual conference of Health Technology Assessment International (HTAi)[3] in Cologne. The preliminary results of a study of 32 developed countries on their assessment processes for rare disorders was presented at this conference. Two-thirds of countries, including New Zealand, were identified as having the same economic analysis process for rare disorders as for other health conditions. Other countries had different standards including additional appraisal criteria for rare disorders, different requirements for economic evaluation, and higher willingness to pay thresholds[4] compared with non-rare health conditions.

PHARMAC revised its application criteria for rare disorders medicines in 2018, to encourage suppliers to make more applications and potentially lead to improved access to medicines for people with rare disorders.

Unlike other medicines currently, suppliers of rare disorders medicines are not required to have gained Medsafe approval for the medicine before it can be considered by PHARMAC for funding. This has removed a potential barrier to suppliers making applications, due to the cost of registration. Previously suppliers were sometimes hesitant to pay the cost of registration when there was uncertainty about the chances of funding (given the small patient numbers and high expenditure). Medsafe approval is, however, required before PHARMAC will list the medicine on the Pharmaceutical Schedule.

Why is the treatment of lung cancer not considered an equity issue, given that it is the single biggest killer of Māori men?

Equity is an important factor when considering the needs of people with lung cancer in New Zealand. We know that lung cancer is a significant area of health disparity for Māori. Lung cancer registration and mortality rates are consistently higher for Māori when

[2] www.pharmac.govt.nz/medicines/how-medicines-are-funded/economic-analysis/

[3] HTAi is an international not-for-profit society that promotes health technology assessment globally. Its membership includes some of the most prominent health technology assessment agencies internationally - including the UK National Institute for Health and Care Excellence (NICE), the Canadian Agency for Drugs and Technologies in Health (CADTH), and the Australian Pharmaceutical Benefits Advisory Committee (PBAC), along with academics, researchers and health service providers.

[4] England, Scotland, Slovakia, Norway, Romania & Sweden had higher WTP thresholds. New Zealand does not operate with a WTP threshold.

compared with non-Māori. Incidence and mortality rates are 2 - 3 times higher in Māori males and 3 - 4 times higher in Māori females, compared with non-Māori.

If a medicine is for a health condition that disproportionately affects priority population groups, including Māori and Pacific people, this is an important consideration in the priority PHARMAC attaches to funding it.

This is because, under the Factors for Consideration decision-making framework, PHARMAC specifically considers:

- The impact on health outcomes for population groups experiencing health disparities; and
- The impact on Māori health areas of focus and Māori health outcomes.

These two factors are considered both by PHARMAC's clinical advisors and in PHARMAC's decisions.

Enabling equitable access to medicines is an organisational priority for PHARMAC. PHARMAC has committed strategically to eliminating inequity in medicine access by year 2025. Right now, our work is focussed on understanding why people don't, or can't, access medicines that are already funded. However, in parallel, we are also examining our decision-making processes and systems, to make sure our funding decisions work to achieve equity, and do not contribute to further inequities for priority populations.

Why is Keytruda not funded for lung cancer?

PHARMAC has assessed pembrolizumab (Keytruda) for the treatment of lung cancer, informed by the advice of its clinical advice committees and the Factors for Consideration, and compared and ranked this application against other medicine funding applications.

PHARMAC's clinical advisory committees have given positive recommendations for funding pembrolizumab[5]. However, they have noted that, at the price that the supplier is currently asking for, pembrolizumab has poor cost-effectiveness and funding it at that price would have a significant budget impact.

As the number and mix of medicines we have for funding consideration is dynamic and ever changing, PHARMAC is continually reviewing, comparing and making judgements about which ones should have the highest priority for new funding.

PHARMAC is responsible for making difficult choices about which new medicines will provide the best overall health outcomes for the New Zealand public from available funding. When funds become available to invest in new medicines, we work through our priority list.

[5] https://connect.pharmac.govt.nz/apptracker/s/application-public/a0R2P000000Lmaa
https://connect.pharmac.govt.nz/apptracker/s/application-public/a0R2P000000Lmr4
https://connect.pharmac.govt.nz/apptracker/s/application-public/a0R2P000000Lpmy

Is there a bias towards funding drugs that have more active and engaged advocacy groups?

Consumer engagement is very important to PHARMAC. Understanding the health needs of patients and their family is one of the things we consider when making judgements about which medicines should have the highest priority for new funding.

People share their stories and give us feedback in a range of ways, and this helps us gain a rich understanding of the health needs of patients living with specific health conditions.

PHARMAC makes decisions about medicines funding using an evidence-based approach and our Factors for Consideration framework. While patient stories are an important input into this process, PHARMAC's medicines funding decisions are not influenced by the number of submissions or frequency of consumer advocacy associated with a specific health condition. What is important is the issues raised, and we do our best to ensure that all advocacy groups are informed and can engage with us on the work we do.

Why are the parameters around the delivery and administering of medicines not factored into their price?

PHARMAC manages the Combined Pharmaceutical Budget (CPB), which is solely for the funding of pharmaceuticals. PHARMAC's supplier contracts therefore specify the cost of the medicine or medical device. Any associated costs of delivery and administration of medicines are funded from other budget streams, usually managed by DHBs. If the costs of delivery and administration were included in the CPB, this would mean less money available to spend on medicines.

PHARMAC's economic analysis of the value for money of new medicines takes into account both the cost of the treatment itself and any other costs or savings that might be incurred by other parties in the health sector that may occur as a result of funding the new treatment[6]. This includes things like service delivery (time and resource impacts for health professionals), additional testing or diagnostic procedures, and the way the treatment might change services (i.e. it might result in shorter hospital stays or could be delivered in a community setting rather than in hospital). PHARMAC actively engages with DHBs on proposals when there are significant additional associated costs of delivery or administration.

Why has it taken so long to fund the range of drugs presented to us by petitioners at the Health Select Committee, especially in cases where they've been used effectively and without side effects internationally, often for several years?

PHARMAC's role is to make more medicines available to more New Zealanders to help them live healthy lives. As the number and mix of medicines we have before us for funding consideration is dynamic and ever changing, PHARMAC is continually reviewing, comparing and making judgements about which ones should have the highest priority for

[6] See https://www.pharmac.govt.nz/assets/cost-utility-analysis-explained-2015-08.pdf for more information about how we assess costs, savings and benefits

new funding. When funds become available to invest in new medicines, we work through our priority list.

A simple comparison of medicines available internationally won't demonstrate how effective a health system is. It is about overall health outcomes and having access to medicines and treatments that work – that is, getting the best health outcomes available within the funds we are given. Other countries have other ways of deciding, and different budget arrangements.

How many medical professionals does PHARMAC pay on a part- or full-time or unpaid volunteers basis to provide advice or feedback on drug purchasing and investigation of which drugs to fund?

PHARMAC draws on the expertise of medical professionals through several mechanisms:

- The Pharmacological and Therapeutics Advisory Committee (PTAC) is PHARMAC's primary clinical advisory committee[7]. PTAC meets regularly to provide objective clinical advice to PHARMAC. The committee is made up of senior health practitioners from a range of professions and specialities. There are currently 13 PTAC members, who are appointed by the Director-General of Health, in consultation with the PHARMAC Board.
- PTAC has a number of expert subcommittees[8] who meet as required to discuss issues referred to them by PTAC or PHARMAC staff. There are currently 21 different subcommittees. They range in size from 7 - 13 members (all senior health practitioners generally nominated by relevant Colleges or DHBs).
- PHARMAC has from time to time convened advisory groups on other matters, including two advisory groups on specific medical devices, each made up of 8 - 10 relevant health professionals[9].
- To help ensure medicines are appropriately targeted, PHARMAC manages several panels[10] of expert clinicians to provide advice on funding for specific patients. This includes patients seeking funding in exceptional clinical circumstances (Named Patient Pharmaceutical Assessment), as well as patients with complex clinical cases, where a set of simple eligibility criteria is not adequate to determine which patients can access treatments. These panels range in size from 4 - 11 members.

All committee and advisory members are paid fees for their services at rates established in accordance with the State Services Commission Fees Framework and Cabinet approvals.

[7] www.pharmac.govt.nz/about/advice/ptac/
[8] www.pharmac.govt.nz/about/advice/ptac-subcommittees/
[9] www.pharmac.govt.nz/about/advice/groups/
[10] www.pharmac.govt.nz/about/advice/panels/

In addition:

- PHARMAC has a Medical Directorate, which includes five staff who are registered medical practitioners.
- PHARMAC has several other staff, working in various roles across the organisation, who are registered health care professionals (including pharmacists, nurses and doctors).

Who and which group and/or individuals decide on specific drug purchasing and procurement? How regularly do they meet, and where?

All medicine funding decisions are made by the PHARMAC Board or by PHARMAC staff under delegated authority from the Board.

The PHARMAC Board meets regularly through the year (generally monthly) at PHARMAC's offices in Wellington.

- *What level of secretariat assistance is required and who provides it?*

A large proportion of PHARMAC staff activity is dedicated to progressing new funding applications and preparing relevant information for the Board or its delegate(s) to make medicines funding decisions (including in relation to changes to funding arrangements for existing funded medicines).

This includes co-ordinating, facilitating and recording clinical advice, undertaking economic and public health assessment and analysis, negotiating commercial deals, contracting with suppliers, as well as consulting and engaging with interested stakeholders.

- *Does the Ministry of Health provide any support or assistance during evaluation process?*

No. The Ministry of Health is not involved in assessing new medicine funding applications or making any funding decisions on individual medicines.

PHARMAC does sometimes, if the information is not available via our expert clinical advisory committees or requires clarification, consult with the Ministry of Health or DHBs to ensure we understand and can analyse the wider health sector impacts (including costs and savings) that may result from the funding of a new medicine.