PHARMAC REVIEW
Te Arotake i Te Pātaka Whaioranga

New Zealand Government

# Submission form

To help us to consider your submission we are asking that you focus on the following questions. There is the opportunity to provide additional feedback at the end. We expect to get a high response and ask that, where you can, you are concise. Once you have completed your submission please send it to: pharmacreview@health.govt.nz

**Note that submissions are subject to the Official Information Act and may, therefore, be released in part or full.**

If your submission contains any confidential information please state this within submission, and set out clearly which parts you consider should be withheld and the grounds under the Official Information Act 1982 that you believe apply. We will consult with submitters when responding to requests under the Official Information Act.

## Submission questions

### Tell us about your current experience with PHARMAC and how it functions

1. What is your understanding of what PHARMAC does?

PHARMAC's functions are as described on its website and in its publicly available documentation. Our understanding is also informed by our direct interactions with PHARMAC since 2005. BCAC (Breast Cancer Aotearoa Coalition) is a non-profit umbrella group representing NZ breast cancer patients. BCAC responds to consultations from PHARMAC, meets and corresponds with PHARMAC staff, and has made several applications to PHARMAC for listing of breast cancer medicines.

In particular, our understanding of the steps involved in listing a medicine on the Pharmaceutical Schedule (i.e. government funds or subsidises cost to patient) are as follows:

- Application to PHARMAC (for each particular use of a particular medicine, both clinical and financial applications are required)
- Medsafe approval (also known as 'registration') will be required as well, and this step is usually undertaken before application to PHARMAC, although this is not always the case. Medsafe ensures that particular medicines meet safety, quality and performance standards' and perform as claimed by the manufacturer
- Receipt of application acknowledged by PHARMAC
- Referral to the Pharmacology and Therapeutics Advisory Committee (PTAC) or one of its sub-committees - in the case of breast cancer, the Cancer Treatments Subcommittee (CaTSoP). CaTSoP comprises clinicians representing various cancers, bringing their own expertise to the room, but they are not required to

consult more widely with others in their field if further expertise is required, or with patients or their representatives. There is one breast cancer specialist on CaTSoP at present. The committee is appointed on the basis of their clinical expertise and ability to appraise scientific evidence from clinical trials, but when making their recommendations they often also take financial cost into account, despite the fact that they have no particular expertise in economic assessment (e.g. CaTSoP minutes September 2018, published December 2018; https://www.pharmac.govt.nz/assets/ptac-cancer-treatment-subcommittee-minutes-2018-09.pdf).

- PTAC or CaTsoP minutes are produced, giving the committee's recommendation, with a priority ranking (low, moderate, high or decline). Note that these minutes may take months to be made available in the public domain.
- Referral back to PTAC, if the recommendation was from CaTSoP.
- PTAC minutes produced, giving the committee's recommendation. PTAC meets 4 times per year.
- If the recommendation is positive, then the medicine (for the use ('indication') described in the application) goes onto a list to await prioritisation by PHARMAC staff.
- The prioritisation process takes place at an unspecified time, involving unspecified PHARMAC staff, and decsions, according to PHARMAC, are made using their decision-making framework. This process includes measuring each recommended medicine against the Factors for Consideration, except that PHARMAC has stated that 'The Factors are not weighted or applied rigidly, and not every factor is relevant for every funding decision PHARMAC makes. …. the context within which decisions are made is constantly changing.' (https://www.pharmac.govt.nz/medicines/how-medicines-are-funded/factors-for-consideration/). These meetings occur 3 or 4 times per year.
- There are no published minutes of these prioritisation meetings.
- When PHARMAC reaches a deal with a pharmaceutical company to fund a medicine, PHARMAC issues a consultation on a proposal to list the medicine on the Pharmaceutical Schedule and seeks feedback from the public (about 2-3 weeks are allowed for input). Minor changes are sometimes made at this stage, e.g. to the eligibility criteria for funded access.
- The PHARMAC Board (the Chair of PTAC attends as an observer) makes the final decision on listing the medicine (unless it has delegated this to the CEO of PHARMAC). https://www.pharmac.govt.nz/assets/pharmac-board-governance-manual.pdf
- Decision to list is notified

2. What has been your experience of working with PHARMAC?

Since 2005, BCAC has used PHARMAC's consultation process to provide feedback on any proposals to fund medicines for breast cancer and has contacted and met with PHARMAC staff at regular intervals to discuss the need for particular medicines.

BCAC has formally applied to PHARMAC for four breast cancer medicines to be listed. Two of these, for palbociclib (later line metastatic) and fulvestrant, have been successful and two, for everolimus and nab-paclitaxel, have not.

BCAC suggested some changes to PHARMAC's initial proposal to fund palbociclib, to which PHARMAC responded positively.

BCAC and BCFNZ wrote jointly to PHARMAC asking that patients already self-funding pertuzumab be given funded access along with those newly diagnosed.

BCAC has participated at every opportunity as consumers commenting on PHARMAC's policies and processes (e.g. 2008 Terms of Reference and Appointment Protocols for PHARMAC's Pharmaceuticals and Therapeutics Advisory Committee, 2009 Consumer Engagement consultation, 2010 consultation on Consumer Advisory Committee Terms of Reference, 2011 consultation on PHARMAC's Exceptional Circumstances funding, 2012 PHARMAC's Operating Policies and Procedures, 2016 Trans-Pacific Partnership consultation, 2018 Consumer Voice review, 2019 Access to DHB Hospital Medical Devices, 2020 Trastuzumab biosimilars, 2021 PHARMAC's Advisory Committees, including the Consumer Advisory Committee).

We note that from a consumer perspective very little has changed as a result of the above consultations.

One of our members served on PHARMAC's Consumer Advisory Committee between 2010 and 2013, but there are currently no members of this committee with direct experience of breast or any other cancer. Nor does this committee have meaningful input into decision-making at PHARMAC.

3. What are the challenges with PHARMAC's functions for funding medicines and devices?

**Current model outdated**

PHARMAC's current model of a fixed budget that combines assessment and purchasing functions within one organisation, which has '*within our means*' as one of its abiding values, is not fit for purpose in today's world. Advances in medical technologies and medicines continue to be made and innovation provides patients with a better health, improved quality and increased length of life. The PHARMAC model was established in 1993 with the aim of securing medicines for the lowest possible price, within a fixed budget. This has remained the enduring underlying aim of PHARMAC. The legislation defining PHARMAC's role and the resulting institutional processes and culture have prevented any adaptation of the model to achieve improved performance and outcomes.

Repeated petitions from NZ patients, often supported by their expert clinicians, asking for access to medicines, show that this model has some serious shortcomings that impact on the lives of New Zealanders. The PHARMAC model requires New Zealanders to carry the cost of having a sub-par medicines assessment and procurement system, resulting in New Zealanders who are able to pay for medicines having better health.

Even though the size of PHARMAC's budget is out of scope for this review, its fixed nature is a key driver of PHARMAC's inability to deliver the health benefits that could be gained from faster access to a greater choice of medicines. Of all the categories of public expenditure on health, only PHARMAC's budget is fixed.

The current value of medicines approved but not yet funded by PHARMAC stands at $417,670,000, demonstrating that this fixed budget is insufficient for the current needs of New Zealanders for medicines that have been shown by PHARMAC's own process to have benefits. This amount reflects only the medicines that companies have made the effort to register in New Zealand (with Medsafe) and seek funding from PHARMAC for, and which have been referred to PHARMAC's committees, been considered and recommended for funding. There are many more medicines that companies have not bothered to seek registration or funding for in NZ because of the low likelihood of success.
Other medicines have been applied for and not even referred to committees for consideration. For example, BCAC applied to PHARMAC for nab-paclitaxel, a form of chemotherapy with reduced toxicity, in February 2018 and this has finally been referred to CaTSoP for consideration in June 2021. Nab-paclitaxel was first Medsafe registered in NZ in 2010, and the company also applied for PHARMAC funding that year and again in 2013. BCAC applied for everolimus in July 2018 and this medicine has still not been referred to CaTSoP as of June 2021. Although this medicine is recommended for use in international guidelines and has been funded in Australia since 2014, the company has not applied for its use in breast cancer in New Zealand.

We believe that the fixed budget has created a rationing mindset within PHARMAC, leading PHARMAC to emphasise reasons to reject funding applications rather than the possibilities for better health outcomes and cost savings elsewhere in the health system. For example sub-cutaneous trastuzumab, which is administered by rapid injection, is not funded even though administration costs would be significantly lower and chemotherapy infusion chairs freed up for other patients, reducing treatment waiting times.

This rationing mindset can produce perverse and unfortunate outcomes, such as the recent example of PHARMAC ignoring the advice of Medsafe on brand switching of epilepsy medicines. In spite of the coroner's conclusion that there was insufficient evidence for a link between deaths and the brand change, this decision certainly falls short of PHARMAC's stated aim to make decisions "in line with societal preferences".

Neither were societal preferences met by PHARMAC's recent proposal to cease funding child cancer medicines to create "equity at the lowest common denominator" with children denied access to spinraza for spinal muscular atrophy.

This rationing mindset, and the fact that PHARMAC combines clinical assessment and purchasing functions, has also led to misleading statements about the efficacy of medicines, which are used to justify decisions driven primarily by monetary considerations. For example, we were disappointed to see the following statement in PHARMAC's Briefing to the Incoming Minister of Health: "*New cancer medicines often come with a significant cost and limited evidence of effectiveness".* This was then repeated in the very brief section on new medicines in Te Aho o Te Kahu's recent report on the State of Cancer in New Zealand: "*These treatments can also be very expensive, often with limited information available about their effectiveness".* These statements demonstrate a lack of acknowledgement that New Zealanders are missing out on a raft of effective treatments recommended in international guidelines and funded across the developed world. They also express a lack of aspiration to do better.

Furthermore, the way in which PHARMAC conducts its role is very much "reactive" rather than "proactive". It waits until applications arrive and then eventually seeks to fund them (on a one-by-one basis) from within existing funds rather than horizon scanning for new technological advances and forward planning for new technologies that could transform outcomes for particular therapeutic areas. This would require a much more forward thinking attitude to both acquisition of new technology and budget setting.

To ensure the integrity of the process of assessing clinical efficacy, this should be clearly separated from PHARMAC's purchasing role, especially while this is constrained by legislation that dictates a fixed budget.

One possibility could be to shift the clinical assessment process to Medsafe, New Zealand's regulatory body that determines whether medicines meet the required standards of safety and efficacy to be approved for use. BCAC has not considered this possibility in any depth, but this could potentially provide a framework for better processes. Under such a scenario expert committees such as CaTSoP would still be needed for more detailed assessment, but this function could potentially be housed within Medsafe, which is seen as an independent body that bases its decisions purely on evidence. This independence would provide the public with certainty that determinations and recommendations were not contaminated by budgetary influences. It would, in fact, be more efficient to create a single trans-Tasman medicines regulatory authority by combining Medsafe with Australia's Therapeutic Goods Authority, thus avoiding duplication of assessment processes. Again, BCAC has not considered this in depth. We offer the suggestions in this paragraph simply as food for thought for the Review Committee.

**Lack of transparency**

The quality of PHARMAC's public communications has improved over the last year. The website is easier to navigate and the most recent Statement of Intent and Annual Report are both very detailed documents. PHARMAC has recognised that the public want greater transparency and these communications go some way to providing that. However, there are still critical elements of the PHARMAC process that remain opaque and not open to meaningful scrutiny.

PHARMAC's Factors for Consideration are described on the website and it is stated[1] that they are 'used throughout' 'the journey of a funding application'[1], but 'Not every factor may be relevant to every funding decision PHARMAC makes' (website link). How they are in fact applied is not made clear. There seems to be no published methodology for their use, nor any record of how they have been applied to particular decisions. Under the subheading "Decision", the Application Tracker simply gives a standard statement in every case. There is no transparency or accountability in this part of the process.

Another opaque area arises between the time of a decision being made and the actual funding of a medicine. PHARMAC often negotiates deals for 'bundles of medicines' with suppliers, and this can lead to medicines that were given a range of different individual priority rankings being funded all at once. Because the total budget is fixed, this will inevitably lead to a shifting of medicines on the priority list with some that were of higher priority missing out to others within the 'bundle'. Recently, diabetes specialists were astounded that an old medicine they did not seek to use was funded while other more

effective modern treatments remained unfunded. This was the result of a bundling deal with a pharmaceutical company. We would ask that PHARMAC be more transparent about how this and other 'deal-making' factors come into play before a medicine is actually funded.

Pharmaceutical companies and the public are not given any access to PHARMAC's medicines priority list, leaving patients worrying about the progress of funding for a medicine they need and preventing transparent negotiation with the company. It needs to be stressed that for a patient making critical choices about potentially life extending therapy, a decision by PHARMAC to wait until they have budget available, is for the consumer a negative decision with respect to their treatment choice. They do not have access except by paying for therapy in the private market.

The UK's NICE will negotiate openly, letting pharma companies know if a proposal does not meet the value required. Patients can see and understand this process. Unless the company offers a price that NICE thinks fair the medicine will remain unfunded, but PHARMAC's policy of secrecy leaves companies in the dark as to where their offer stands and this slows progress towards a positive outcome.

**Consultation too little, too late**

Despite PHARMAC's references to consultation with various stakeholders, consumers have the opportunity to comment on funding decisions only at the very last stage, when a funding agreement has already been reached with a pharmaceutical company and the decision has already been drafted. PHARMAC's latest Statement of Intent refers to a desire to 'review and improve the way we publicly consult on our decisions'. More value could be obtained if input was sought from consumers at the start of and throughout the decision-making process. Patients and their representatives have valuable insights into their needs that could contribute directly to better decision-making on applications for medicines if such input was an integral part of the consideration process.

The current Consumer Advisory Committee does not fulfil this role as it has no input into funding applications or decisions; their membership is limited and fixed, and there is no requirement for them to have expertise or experience or knowledge of any particular patient group or therapy area. BCAC has provided a response to the recent consultation on the role and composition of PHARMAC's Committees, including the Consumer Advisory Committee, suggesting that expert patient and clinician input be sought on each consideration. We look forward to seeing detail of PHARMAC's plans to 'strengthen the role of CAC'.

Note, we have appended BCAC's recent submission to PHARMAC's consultation on its Committees to the email to the PHARMAC Review Committee with the present submission.

**No aim to obtain better medicines or more of them**

Among PHARMAC's current 'enduring impacts'[1], there is no mention of an intention to obtain better medicines that would be more effective than current offerings or treat currently untreatable conditions.

In contrast to PHARMAC's 2017/18 impact statement – *Increased access to effective medicines and medical devices,* the 2020/21 version is – *Our investment choices enhance wellbeing*. This represents a considerable watering down of intention and, combined with a stated value of '*Within our means'*, does not bode well for any improvement in our current situation (i.e. patients having to fund raise and present petitions to Parliament to gain access to the medicines their doctors say they should have).

Two of the three measures for this impact statement ('health outcomes' and 'uptake of medicines') have yet to be developed, with 'average time from application to funding decision' being the only current measurement available. We note that one method PHARMAC has employed to improve this measure has been to eliminate a number of medicines awaiting a decision from the list. And as we are compiling this submission we note that on 29th June 2021 PHARMAC has proposed to decline 97 further applications, including three for breast cancer medicines. This includes fulvestrant used as a first line metastatic treatment, but fulvestrant is used both as a single agent and as an important endocrine partner for palbociclib, first and later line in metastatic breast cancer. The decline list also includes neoadjuvant pertuzumab following a 2018 comment by CaTSoP that there was "insufficient evidence at this time". This medicine is recommended in ESMO guidelines for adjuvant or neoadjuvant use in early breast cancer. PHARMAC should await the presentation of further evidence instead of declining outright. A decline means any further evidence could only be added through a completely new application which involves far greater effort than addition of new evidence to be considered. Lapatinib is also proposed for decline, but is recommended for later line metastatic use in ESMO guidelines for advanced breast cancer. How this proposal to decline 97 applications for medicines contributes to wellbeing is hard to fathom. It seems that they are just, somewhat cynically, trying to make their statistics look better.

PHARMAC and the Ministry of Health seem to be at pains to resist international benchmarking with respect to medicines access. We are hopeful that Te Aho o Te Kahu will undertake such benchmarking and that this will be done independently of PHARMAC's influence. PHARMAC wrote a notorious paper, 'Mind the Gap', defending its record of refusing to fund for cancer medicines that are funded in Australia. That paper's viewpoint and analysis were was widely discredited by clinicians and patients alike.

The uniqueness of the PHARMAC model is cited repeatedly, but New Zealanders' needs for medicines are not unique. In comparison to PHARMAC, the UK's NICE aspires to get modern medicines to people faster. NICE provides an example of a model that works better than New Zealand's and we expand on this in Section 6.

**What do you know about PHARMAC's processes and how they work?**

4. What do you think works well with the processes PHARMAC uses to assess the funding of medicines and medical devices?

It is difficult to identify elements that work well when the experience of breast cancer patients is that modern medicines that are shown to be effective in clinical trials, are recommended in international guidelines and are funded across the developed world

remain unfunded in New Zealand and can only be accessed through personal, family or community fundraising. Every now and again a key medicine is funded, bringing huge relief to patients, but this is often after petitions to Parliament and years of advocacy. As part of our role in supporting patients, BCAC actively supports those who are missing out on the health and longevity benefits of unfunded medicines and we understand how difficult this is for these women and their families. The slowness of the assessment and funding processes, the uncertainty and lack of transparency, the absence of opportunity for expert patient/representative input and the misleading denial of strength of evidence all create frustration and discontent for patients and whānau. The mental and financial burdens that this creates are immense. BCAC believes that New Zealand's citizens deserve better and that as a nation we can and must be more aspirational.

PHARMAC's processes can be flexible when the need arises, and this is seen as positive.

The decision to promote early access to cancer medicines by accepting submissions before MEDSAFE approval was also a positive step, although, it has not resulted in earlier access to any new medicine that we are aware of.

5. What do you think are the barriers to accessing medicines and devices?

**Insufficient public investment**

PHARMAC is not resourced sufficiently for New Zealanders to have access to the medicines that other OECD governments subsidise. Personal fundraising for medicines by patients and petitions to Parliament seeking increased funding for PHARMAC are evidence of this, as are our poorer health outcomes. For example, in New Zealand the median survival for those with advanced breast cancer is 16 months, compared to 29.4 months in the Netherlands, 36.8 months in Germany, 25 - 54 months in the USA, 23.1 months in France, and 33 months in Sweden (3). Overseas expert, Dr Fatima Cardoso, Chair of the ABC Global Alliance and co-author, of ESMO guidelines for treatment of advanced breast cancer (4), confirmed our fears about New Zealand's poor outcomes for breast cancer patients by referring to the 'worrisome case of New Zealand' and stating that the outcomes were 'not like those of a developed country' (Cardoso presentations, Auckland, 29 January 2019 and Wellington 'Cancer Care at a Crossroads' conference, 1 February 2019). NZ research has also shown better outcomes for those with breast cancer who are cared for in the private sector, i.e. wealthier patients have better survival.

This creates and magnifies ethnic and socioeconomic barriers to accessing medicines and medical devices.

**Sponsors not willing to deal with NZ**

BCAC is concerned that one significant impact of limited funding over time has been a trend in reduction in medicines choice because sponsors are not willing to 'navigate the New Zealand system' because of the low probability of success and protracted delays to funding. Unless the system changes, we are destined to fall further behind the world's best practice. BCAC has taken a role in filling the gap left by pharmaceutical companies by preparing formal applications to PHARMAC to fund vital breast cancer medicines. We are able to do this because one of our founding committee members is a professional pharmacoeconomist who is able to compile and present the evidence required in such an application. Most patient groups do not have this capacity. Similar applications have

been submitted by clinicians who are familiar with the literature and are concerned that their patients are missing out on vital medicines.

**Lack of access to clinical trials**

BCAC is also concerned that New Zealanders' participation in clinical trials is being stifled. Such trials are an important means for some patients to gain access to new medicines and to contribute to research, and for treating clinicians to experience new medicines by providing them to their patients. Without the treatments that are the current international standard of care, we are excluded from trials aimed at testing the addition of a new treatment (i.e., we cannot participate in the control arm of the study let alone the experimental one). For example, our lack of access to fulvestrant, first applied for in 2006, prevented New Zealand patients from participating in clinical trials of the CDK4/6 inhibitor palbociclib.

6. Is there any other country that does it better? What is it that it does better and would any of those systems apply here?

Yes, the UK does it better through their National Institute for Care and Health Excellence (NICE) and this provides a model for New Zealand to follow.

**National Institute for Care and Health Excellence, UK**

At a 2019 Parliamentary Dinner, Sir Andrew Dillon noted that pharmaceutical companies and health systems share the challenge of funding modern medicines and that they need to work together to restructure their relationships and work collaboratively so we can reap the benefits of the extraordinary innovations that are emerging. NICE asks whether the incremental health benefit is worth what they are being asked to pay. NICE evolved from an organisation that only dealt with pharmaceuticals to now dealing with clinical, technical and social elements of health, evaluating healthcare practices. Clearly defined, transparent cost-effectiveness thresholds are applied across all of NICE's operations. It holds itself out as consultative and responsive to reasoned argument. "We will say no if the value proposition does not stand up however we are there to provide faster access to modern medicines".

The role of NICE in relation to medicines is to understand the lifetime benefits and lifetime cost and determine whether they are worth the price. Sir Andrew described features of NICE and we contrast these here with the current PHARMAC model (italicised observations BCAC):

- NICE provides guidance on medicines and devices to the National Health Service (NHS), through a process of technology appraisal. The NHS has a broad indicative target on pharmaceutical expenditure which is not constrained by being capped. The drugs and other treatments recommended by NICE in its technology appraisals must be funded, by law, through a 'funding directive'. Normally, when the funding directive is applied, the NHS has 90 days in which to make the treatment available. *PHARMAC's clinical advisory committee and its sub-committees evaluate medicines and provide recommendations to decline or fund with low, medium or high priority. There is no guarantee or directive that medicines recommended for funding, even as a high priority, will ever be funded. The NZ medicines budget is capped at an extremely low level compared to other OECD countries, leaving many prioritised medicines unfunded.*
- NICE staff pull together the evidence base for a medicine or technology and provide this to an advisory committee which undertakes a 'technology appraisal'. Committees comprise patients, health economists, health professionals, epidemiologists etc, who have

evaluative skills. Chairs of committees have been trained to ensure all voices are heard. Advisory committees vary in size, with around 20 members appointed for each evaluation. The advisory committee writes the recommendation. NICE publishes this with no interference or overwriting of the recommendation by NICE staff. This is seen as important for the credibility of the process, as is the independence of advisory committees. Once a recommendation has been published there is genuine consultation with stakeholders. This can reveal any flaws, mistakes or anything that has been overlooked. There is no shame in altering an initial recommendation, in fact it is seen as good if the document is amended following consultation. This process produces a guidance document indicating which medicines and technologies the NHS must provide for patients. *PHARMAC has a single Pharmacology and Therapeutics Advisory Committee (PTAC) with fixed membership. It has a number of expert sub-committees, also with fixed membership. The only area of expertise included on these committees is clinical. Patients and their representatives have no opportunity to participate in evaluations or the development of recommendations. The narrow expertise included in committee composition limits the ability to fully evaluate the impact and value of medicines and technologies. PHARMAC staff write all minutes of meetings, including recommendations, which compromises the independence of outcomes. There is no mandate to fund medicines that the PHARMAC committees recommend for funding. Instead the recommendations go to a 'waiting list' of 'fundable' medicines that are then separately evaluated at secret meetings of PHARMAC staff. The lack of transparency of these funding meetings compromises public understanding of and trust in the process and its outcomes.*

- Stakeholders, such as clinical experts, patients and their representatives and carers, healthcare providers, pharmaceutical companies and manufacturers, are invited to submit written information and evidence on a medicine or technology at the beginning of the NICE appraisal process. This information will form part of the evidence used by the appraisal committee. *PHARMAC only allows input on a consultation only when it has completed its own internal appraisal process including pharmacoeconomic evaluation, provided a recommendation, held a meeting at which a medicine has been prioritised and selected for funding, held a Board meeting at which this has been approved, negotiated with a pharmaceutical company to reach an agreement for supply and published its funding criteria. Input at that stage can only achieve minor alterations to PHARMAC's proposal. In effect, stakeholders other than the company submitting the proposal are shut out of the appraisal process.*
- If a medicine meets NICE's transparent, defined cost-effectiveness threshold it is recommended for funding. This cost-effectiveness model applies across all of NICE's activities; it has been refined over time and is now being used in other parts of the UK health system as well. *PHARMAC has no defined criteria for funding or threshold over which a medicine will be funded, only a set of loose criteria which are applied as PHARMAC sees fit in any consideration. Medicines recommended by PHARMAC's committees go onto a waiting list of fundable medicines which may or may not ever be prioritised for funding at periodic secret PHARMAC staff meetings.*
- NICE was set up originally to provide "faster access to modern treatments". NICE finds 82% of the medicines they appraise to be good value propositions. *PHARMAC was established to control the costs of pharmaceuticals. New Zealand's small, capped medicines budget results in many of the exceptional medicines prioritised by PHARMAC's committees remaining unfunded.*
- 90 days after a European licence is granted for a drug, NICE must provide their advice on whether to fund. *PHARMAC operates under no timeframes for considerations or decisions.*
- Transparency was mandated as an essential element of how NICE works from its inception. This culture of transparency around medicines funding is inherent in the interactions among NICE, clinicians, the NHS, pharma companies and patient groups. NICE works hard with companies to be able to say 'yes' to funding, with the aim of getting

modern medicines to people as soon as possible. *PHARMAC has stated that secrecy is a key element of controlling the process and outcomes of interactions with pharma companies. PHARMAC does not provide information to pharma companies to help them understand what they would need to offer to make a drug fundable, or how far from the top or bottom of recommended but unfunded products their medicine sits. PHARMAC processes very often involve delays of several years and the process appears to be geared to saying 'no' i.e. to denying the benefits of medicines and refusing to fund.*

- NICE believes that companies need to understand the market they are in, act accordingly, implement sustainable business models and seek realistic prices for innovative medicines that health systems are able to tolerate. NICE works to dismantle barriers to access. Companies interact with the appropriate elements of the health system as soon as possible so that impacts of funding can be understood and managed. New approaches must be taken so companies and health systems can share the risks of uncertainties. *Companies are forced by PHARMAC to operate in an opaque environment, preventing productive discussions about how risk could be shared while allowing early access for patients. PHARMAC tends to provide a negative view of pharma companies in the media, presumably to convince the public that 'greedy' pharma companies are to blame for making medicines unfundable. This adversarial approach does nothing to help patients in need of treatment.*
- Cancer has long been a priority for NICE because modern medicines can make such a dramatic difference to patients' lives. An increasing number of cancers are now becoming chronic diseases because of the provision of effective treatments. NICE applies higher QALY values for cancer medicines and medicines for rare disorders to allow these medicines to be funded. A standard QALY (2019) is £20,000, although this can reach £30,000. Medicines that extend life such as cancer medicines are more often £45,000 to £50,000, and cancer treatment is seen as a priority. Medicines for rare disorders can have QALYs of £100,000 to £300,000. *In contrast, PHARMAC says it wants long-term gains from investments and cancer, especially when it is terminal, is absolutely not prioritised. Quality and extension of life for cancer patients doesn't stack up well in PHARMAC's QALY evaluations.*
- Investments by NICE are monitored to ensure the health gain is real and ongoing. *There is no measurement of health gains (or losses) resulting from PHARMAC's decisions.*
- All stakeholders including patient advocacy groups have an opportunity for input while a drug is being considered for funding by NICE. Sir Andrew stated that there is no shame in NICE listening to input and changing their view about funding. He stated that NICE will respond to 'reasoned argument'. *In New Zealand stakeholders only get to comment during a 2 – 3 week window once a deal has been struck with a pharmaceutical company and a proposal has been released for consultation – only minor tweaks are possible at that stage.*
- NICE is committed to continuing to evolve and learn from other evaluation systems around the world. *PHARMAC appears to be locked into the outdated, harsh, adversarial model developed 28 years ago that is enshrined in legislation.*
- NICE delivers on the aspirations of the health system. Sir Andrew Dillon stated that this would not work without a supportive Government and a responsive health system. *Successive New Zealand Governments have, presumably on PHARMAC's advice, been convinced that the current system works well. PHARMAC's briefing to the incoming Minister of Health in 2017 stated that with the exception of Mexico, New Zealand has the lowest per capita spend of all 36 OECD countries. As a country we spend only 5% of NZ's health budget on medicines, compared with the OECD average of 14%, representing 0.34% of GDP compared with the OECD average of 1.4%. This does not mean that PHARMAC gets great deals that allow New Zealanders to get the same access to medicines as comparable companies. Between 2011 and 2018 New Zealand spent 5% of its health budget to acquire 24 innovative medicines. In the same period Australia invested 15% of its health budget*

*and accessed 106 modern medicines, Finland invested 15% for 154 such medicines and the UK 12 % for 204 innovative medicines. Multiple patient groups have petitioned Parliament in recent months to fund vital medicines that are funded across the developed world. New Zealanders deserve better.*

**NICE 5 year strategy launch 2021**

NICE recently released a 5 year strategy for improving UK healthcare. During their online strategy release the senior managers of NICE presented their aspirational approach. New Zealand patients and clinicians alike are desperate to see PHARMAC take a similar approach as a connected element of our health system.

NICE articulated their commitment to be "dynamic, collaborative, excellent, innovative, agile and flexible"; to undertake "rapid, robust, responsive technology assessment"; to be "world-leading in technologies and medicines, to implement innovation fast, with innovation at the heart of patient care"; to "make more treatments available, with most assessments of medicines and technologies leading to a positive outcome"; to be "focused on patient benefit"; "to adopt gene therapies and CAR-T for cancer"; to produce "dynamic, living guideline recommendations with uptake to provide maximum benefit"; "to provide guidance on drugs, diagnostics, digital health, machine-learning enabled devices and ensure rapid access to effective, innovative medicines".

NICE sees itself as an enabler of market access, a broker of the price for medicines and technologies via the UK's Cancer Drugs Fund and Innovative Medicines Fund. They see collaboration with industry as a key element and are open to offers to amend value propositions to get medicines and technologies over the line faster. They aim to have significant user input on guidelines, to ensure these are developed through a robust, open, transparent process and available on different platforms and to fit into pathways of care, useful as tools for individuals. They want their guidance "to be used, not to sit on shelves gathering dust". In contrast, it is typical for guidelines in New Zealand to do just that, e.g. the NZ Guidelines for Early Breast Cancer (2009) and our Standards of Care for NZ Breast Cancer Patients (2014). These Guidelines and Standards were developed through excellent processes where expert clinicians across the relevant disciplines from around New Zealand workshopped with consumers, using the latest available evidence. Sadly, neither the Guidelines nor the Standards were ever implemented. As of 2021 QPIs are being developed, seemingly more as a tool for measuring performance of DHBs (or their replacements) than as useful tools for clinicians and patients that would enable best practice care to be understood and implemented.

Returning to NICE, we heard at the strategy launch that the NICE Accelerated Access Collaborative has funding and a mandate to ensure uptake of guidance. They plan to tackle inequalities "front and centre" and aim to determine where the biggest impacts can be achieved (public health, clinical, social needs etc). They will use more "real world" evidence, not just randomised controlled clinical trials to find evidence of small differences for small populations. They will "ensure patients are embedded in conversations, on committees, and will be engaged and listening". The aim to be "trusted, respected and responsive to meet local, national and global need".

There couldn't be more contrast with PHARMAC's goals of "*…enhancing wellbeing… within our means*" while stating in their Ministerial Briefing that "*New cancer medicines often come with a significant cost and limited evidence of effectiveness*". It is not surprising that patients see

PHARMAC as advocating against their need for access to medicines rather than supporting them to receive optimal care and better health outcomes.

## What should PHARMAC's role include in the future?

7. How might PHARMAC look in the future? And what needs to change for this to happen?

PHARMAC could look more like the UK's NICE, with a focus on innovation, responsiveness, transparency and connectedness to stakeholders and the rest of the health system, to achieve better health outcomes for our people. This could be achieved through a significant redesign of the system for assessing medicines and technologies.

- Review and reform the legislation governing PHARMAC and its purposes.
- Separate the clinical assessment function from the purchasing function. Ensuring these are undertaken in separate organisations would enable clinical assessment to be undertaken independently, on the basis of evidence and patient need, and not contaminated or undermined by purchasing.
- Adopt aspirational, patient-focused goals.
- Measure the ongoing health impacts of decisions.
- Remove the cap from the pharmaceuticals budget to allow pharmaceutical purchasing to be responsive to opportunities for innovation and better health outcomes.
- Evolve beyond the rationing mindset and the outdated, patronising attitude that patients don't know what's good for them.
- Include patients/representatives and clinicians with expertise in the relevant health areas in all committees and steps of assessments.
- Establish timeframes for assessments; hold more frequent assessment meetings.
- Establish timeframes for funding of medicines and technologies recommended for adoption.
- Take PHARMAC out of its separated "black box" and connect it with the rest of the health system.
- Ensure transparency in all its activities.

8. Are there additional or different things that PHARMAC should be doing?

Horizon-scanning to identify innovations that could benefit patients, seeking resource to acquire these and helping other elements of the health system prepare for innovation by being transparent in this activity and in progress towards adopting new treatments.

Working in proper partnership with experienced, informed consumers/patient representatives at every step of the assessment process. Seeking and using input from consumers and clinicians who are experts in the affected health conditions. This will lead to better decision-making and help to fulfil PHARMAC's newly stated goal to make decisions "in line with societal preferences".

Stop assuming that New Zealanders accept that their country is too poor and small for them to have funded access to medicines available in other countries.

Stop assuming that we don't want to be a bother and are OK dying earlier than those in other countries.

Stop assuming that Māori health will improve only if Māori get equal access to current medicines. Māori deserve better and will benefit as much if not more than non-Māori from better access to modern medicines; precision medicine based on genetic tests could be a potent force for eliminating ethnic inequities in health outcomes.

Stop demonising pharmaceutical companies and start working with them in partnership to provide faster access to innovation for New Zealanders.

9. What do the wider changes to the Health and Disability system mean for PHARMAC?

Unknown – PHARMAC was specifically excluded from the Health and Disability system review. The changes could well affect PHARMAC's operations, and PHARMAC's current way of operating, as an entity separate from the rest of the public healthcare system, could well affect the chances of the review's recommendations being successfully implemented. However, a systematic, expert review (rather than this public forum) is probably needed to ascertain what these effects might be and what could be done to ameliorate them.

## How should PHARMAC address the need for greater equity in the decisions it takes, in particular for Māori, Pacific and disabled people?

10. How well does PHARMAC reflect the principles of Te Tiriti o Waitangi?

PHARMAC's inability and seeming lack of aspiration to provide access to effective modern medicines has the greatest impact on those who do not have comprehensive private health insurance or the ability to pay for their own treatment in a private setting. Impacts are likely to be greatest on Māori, Pasifika and socioeconomically deprived populations. We have seen deep anger among our Māori members at PHARMAC's adoption of a Māori name, as this was seen as lip service to equity and Te Tiriti.

It remains to be seen if the high-level plans outlined in the very recent strategy document (Te Rautaki o Te Whaioranga, Māori Responsiveness Strategy - August 2020) will be implemented and if they will be sufficient to make real change in Māori health outcomes. We hold the view that deeper structural reform will be needed to achieve positive change.

11. How can PHARMAC achieve more equitable outcomes?
Facilitate greater funded access to modern, more effective, more precisely targeted medicines, especially for diseases and conditions which currently disproportionately negatively affect Māori and Pasifika (i.e. virtually everything except melanoma). Simply aiming for equity of medicines access across the motu will not be sufficient, when even

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| those New Zealand groups with the best current access do not have access to the medicines, or the resultant health outcomes, that those in other OECD countries enjoy. |

## Additional feedback

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| Is there anything else that you think the Review Panel should consider?<br><br>If necessary, consider seeking an extension of time so that the stakeholder feedback received in the consultation can be properly considered and reflected in the final report, which we note is due by the end of this year.<br><br>Do whatever it takes to open the Government's eyes to the depth of the problems with the current model and their real impacts on the health and wellbeing of New Zealanders and their whānau. It would take a significant shift of political attitude, systems and funding to achieve meaningful reform so please take the input of affected patients and their representatives seriously and do your best to convince the Government that deep change is needed. This review provides a rare opportunity to shine a light on a broken system. Kia kaha in your mahi, panel members. |

# Contact information

Your feedback is important to us. If you are comfortable for us to get in touch if we have any questions or points of clarification regarding your feedback, please provide your name and contact email address below.

| | |
|---|---|
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If you do not want your personal details to be shared for any other purpose (for example if we receive a request for information under the Official Information Act) please signal this using the box below.

☐ I do not want my personal details to be shared for any purpose other than this review.

Thank you for providing your feedback.

Tēnā koe mō tō tuku urupare mai.