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 assesses the cost utility of medicines, ranks and prioritises them, decides on which should be publicly funded and when, negotiates prices with suppliers, and procures the chosen medicines. Its objective in carrying out these functions is to secure the best health outcomes from within a fixed budget.

We understand that Pharmac's budget is set and/or negotiated collectively by DHBs, Pharmac and the Minister of Health. But as it is not reported on publicly, or subject to Parliamentary or public scrutiny, we do not know the relative size of medicines spending or the health outcomes it achieves compared with other major areas of health spending. We understand that NZ spends the least on medicines as a proportion of health spending of all OECD countries (to the extent that it is possible to calculate that, given the lack of published information).

What has been your experience of working with PHARMAC?

Our experience with Pharmac has been deeply frustrating.

Background: CLL and who we are

Chronic Lymphocyctic Leukaemia (CLL) is a life-threatening blood, lymph node and bone marrow disease that usually progresses over time. Around 2000 New Zealanders live with CLL, of whom around 80% are aged over 60.

CLL Advocates NZ (CLLANZ) clladvocates.nz was set up specifically to advocate for treatments for CLL patients with high unmet needs, treatments that are standard of care throughout the developed world. While effective treatment remains unfunded, NZ patients in desperate need have the choice of self-funding, seeking public charity, getting onto a clinical trial, moving countries, or facing death or a greatly diminished quality of life.

As CLL patients tend to be older they are often less able to advocate actively for themselves and be heard by the decision makers. We therefore also seek to ensure patients have comprehensive, up-to-date information about their disease and its treatment, to give them greater confidence in advocating for themselves.

CLLANZ is a member of an international network CLL Advocates Network (CLLAN) that works together and shares resources. This enables us to closely follow and report to our patient and carer network on international developments in the treatment of CLL.

We are a small but passionate group of volunteers committed to raising awareness of this cancer and the unmet and largely unheard needs of those who live with it.

Focus of our submission

We advocate for all available, effective CLL treatments in their needed applications, and for reforms of treatment pathways in New Zealand. See details on page 24 in our patient booklet here.

But for the purposes of this submission we have chosen to focus on ibrutinib, the longest awaited and most sorely needed treatment for NZ CLL patients. It is the recommended first-line therapy in current international guidelines for older CLL patients (70+), recurrent or refractory disease, and 17p deletion. Ibrutinib (a daily oral medicine) is a B-cell inhibitor that blocks a protein called Bruton's Tyrosine Kinase (BTK). See here for more information about ibrutinib.

Our experience with Pharmac in trying to get this treatment funded is in our view a valuable case study and a graphic illustration of all that is wrong with Pharmac and its processes. Please see Appendix 1 of this submission document for a table of the history of Pharmac's reviews of this medicine.

Our experience of Pharmac: a case study

In the 5+ years since ibrutinib was assessed by Pharmac and ranked as a priority (of varying degrees) for funding, our experience of Pharmac's processes has been: obfuscation; continuous inexplicable delay; an absolute lack of transparency and clarity in its dealings with stakeholders and in the processes it employs; a disregard for the views of patients and their advocates and lack of opportunity for them to be heard; an apparent unwillingness to accept international treatment guidelines and the vast body of international evidence on the efficacy of this medicine; and even claims of lack of evidence.

Our experience with Pharmac also suggests that, no doubt due to their strict, budget-driven mandate, they do not factor into their processes the speed, scale and significance

of the advances going on in medicines discovery and development, and the many more potentially transformative treatments on the horizon that New Zealand needs to prepare for.

Nor do they appear to take account of the growing importance of precision medicine in diseases such as CLL and other blood cancers, with clinicians increasingly able to tailor treatments to an individual's disease, if they have the range of treatments needed to do so. Instead Pharmac frequently makes choices that disregard clinicians' advice and recommendations, including the advice of its own specialist committees, severely limiting clinicians' flexibility in how they treat patients.

Ibrutinib, when it first came onto the market, was a breakthrough medicine that radically changed the course of CLL patients' lives around the world. Today it is standard-of-care internationally, available and funded in all OECD countries except New Zealand. New Zealand clinicians treating CLL patients regard funding it here as an absolute 'no brainer'.

We have had meetings with Pharmac executives (initiated by us) which have always been polite and friendly, with undertakings that another meeting on this medication is coming up soon, but nothing comes of it.

We are told that a factor in the slowness of processes is the limited availability of clinical advisory committees, who have a set number of annual meetings, but this would appear to be a self-imposed budget-related constraint.

We do not know what if anything is being negotiated behind closed doors with suppliers, in the world of 'bundled' deals and rebates. Secrecy around price and dealings with suppliers appears to be yet another factor inherent in Pharmac's zealous, budget-driven approach.

Over six years of PTAC and CaTSoP committee meetings on ibrutinib there have been 20 widely vacillating outcomes, ranging from decline, medium, low, high, no decision and defer.

At the end of all this we still have no idea when or if ibrutinib is ever going to be funded.

We can only conclude that the delay in funding it is all about price, basically tactical 'rationing by delay', waiting for the medication to come off patent and for generics or cheaper versions to come onto the market.

Clearly this overall approach of 'cheaper ahead of better health outcomes' cannot and does not produce best health outcomes for those in need of this treatment.

Out of the blue in 2019 Pharmac announced it was funding another CLL treatment we had been advocating, venetoclax. See details on page 22 in our patient booklet here. This is a different class of treatment, a BCL-2 inhibitor, that is also needed but is not interchangeable with ibrutinib, is not the recommended international first-line therapy, is unproven in one of the applications it is funded for, does not meet the most serious unmet needs, involves some serious risks, including hospitalisation and relapse, and requires hospital-based infusion. But it is a fixed duration therapy (two years).

The literature suggests that about 30% of those going onto venetoclax (and in New Zealand these will be the sickest, most severe CLL cases) won't get into a prolonged remission. This is because 5% of people don't have the receptor required to allow venetoclax to work, and 25% will either get intolerable side effects, or the CLL won't respond, or will come back after the 2 years of therapy is completed. In New Zealand that group will usually have no funded further option (except, perhaps, an allogenic stem cell transplant, a treatment almost obsolete in other OECD countries). Outside New Zealand this group would get ibrutinib funded, which would almost certainly work and work well, and save the lives of many in this group.

But in New Zealand, to add to the inconsistency, as our table in Appendix 1 records, CaTSoP accorded high priority in 2020 and 2021 to ibrutinib as therapy for CLL patients who have failed or relapsed after venetoclax, but it is still unfunded for this application.

Looking at the wider equity and economic considerations, it is well known that BTK inhibitors such as ibrutinib are very well suited to outpatient therapy, including in remote regions or those underserved by local specialists (via telephone/remote consultation). We would have expected that factors such as time off work, travel to infusion facilities, potential need for a carer, and/or the ability to remain in or return to work and a normal productive life should have been considered. But it appears the decision took no account of these potential downstream social and economic costs or savings.

The decision to fund venetoclax was of course welcome. But again, in the absence of any explanation, we have to conclude this treatment was chosen because it is a fixed duration therapy, and therefore likely to be cheaper in the short term.

Opaque processes and decisions like this do nothing to promote public trust and confidence in Pharmac.

Recently through a media OIA we learned for the first time that there are 73 medicines Pharmac would like to fund if it had the budget to do so. The cost would be $417.7m. The actual list is not published (why not?), but it is broken down into how long each has been waiting. Although we have no way of knowing if or where ibrutinib sits on the list, we assume it will be one of the 14 that have been waiting the longest, i.e., for 6+ years.

Among the many concerns this revelation raised is that the Minister confirmed to media that any additional funding for Pharmac (in the then upcoming Budget) would be a political decision, i.e., there is no relationship between the medications identified as needed as a matter of priority, and decisions about Pharmac's funding. We do not know what if any additional budget request was put up in this year's Budget. It was also revealed that in 2020 Pharmac did not ask for any more budget at all. So who is advocating for funding to match the highest priority needs of NZ patients?

To pursue our mission, as well as actively engaging with Pharmac, we have used every available channel within our means and our democratic system to be heard: through petitions, submissions, engaging with media, marching on Parliament, and appearances at select committees. But Pharmac seems deaf to these efforts by patients to be heard. They say they are improving stakeholder engagement, but there is no evidence of this. We think

it is outrageous that people who are sick and in some cases terminally ill feel they have no choice but to lie down on the concrete in the rain outside Parliament to express their desperation. But what other options do they have?

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

Budget fixing/forced savings bias

We understand that the fixed nature of the pharmaceutical budget is out of the scope of this review. However we believe the single greatest challenge with the current Pharmac model is the constraint of operating within a fixed budget that is unrelated to NZ patients' medication needs, is not publicly reported on, and is out of line with relative medications funding levels in comparable countries. We believe this is an insuperable challenge to Pharmac's ability to perform its core function effectively.

While health funds will always be scarce and heavily contested, Pharmac's heavy institutional bias towards making savings, needing to disinvest in an older medicine before it can fund a new one, disregarding wider social and economic impacts, choosing the cheapest rather than most effective medicines, and tactically delaying funding innovative, very expensive but in some cases curative treatments, does not produce best health outcomes.

A further cost-saving tactic used by Pharmac that is inconsistent with other countries is to tightly limit the applications a new treatment can be used for, heavily restricting the number of patients who can access it.

Fitness for purpose of the Pharmac model

Much has been said about how well the model's focus on savings worked in the early years after Pharmac was established in 1993, and how successful the agency was in taking on the pharmaceutical companies and bringing cheap, or free mass-use medicines to New Zealanders.

But the model is now an anachronism:

- The world of medicines development has changed exponentially. Highly sophisticated, new generation medicines and technologies that are transforming the management of diseases and health outcomes are flowing onto the market.

- Pharmac's mandate does not permit it to be proactive or forward looking. Our medicines assessing and funding agency should not only be advising the government of the day on what is on the horizon and how to plan for it, but also the size of the gap between what medicines need to be funded to bring New Zealand into line with the rest of the developed world and the cost of closing that over time.

- Pharmac's current institutionally reactive attitude to assessing new medicines, and its long, drawn-out, 'behind closed doors' processes make Pharmac quite unfit for assessing and ranking these new medicines in an effective and timely manner.

- Pharmac's model is also out of sync with the wider wellbeing approach to health. With its mandate limited to health considerations, and its narrow cost utility assessment approach, Pharmac's decisions do not take account of the social costs and benefits of medicines and medical treatments on people's lives. These include savings made through early treatment, and the costs of not treating people at all. They include the costs of medicines compared with other interventions. They include the impact on patients of being able to return to or remain in work, or have home-based treatment, the impact on primary care givers and whanau, barriers to equity of access such as travel costs, and the difficulties experienced by Māori, the elderly, disabled and rural people in accessing treatment.

- Adding medical devices to Pharmac's responsibilities and the need to be abreast of technological developments can only exacerbate these challenges, stretching timelines and already limited resources, and further clouding accountabilities.

- Another impact of Pharmac's long delays in funding modern treatments that are international standard of care is that they prevent New Zealand patients and their clinicians from accessing international clinical trials of these treatments.

- Speed of access to new medicines to New Zealanders is not part of Pharmac's mandate. NZ is 19th out of 20 OECD countries for access to new medicines, and unlike funding systems in comparable jurisdictions, Pharmac has no 'rapid access' scheme to efficiently evaluate and fast track access to new targeted medicines for people with high or urgent unmet needs.

The net result of these issues is that NZ patients are missing out on life-saving treatments, and we now have a two-tier system, where the wealthy can self-fund and enjoy a good quality of life, while the poor have a reduced quality of life or die.

A further challenge to Pharmac is a growing sense among the general public that Pharmac is not serving us well, as patients increasingly do their own research, collaborate on social media, and resort to public demonstrations.

For all of these reasons we believe the Pharmac model is no longer fit for purpose.

Overlap and lack of transparency between functions

We believe the overlap between Pharmac's various roles, specifically between assessment and procurement, adds significantly to the time taken by both processes, and blurs the 'waiting room' area between them, where stakeholders have no idea whether or not or when even a high priority treatment is going to be funded.

Once the DHBs are rolled into Health NZ, procurement will become one of its core functions, and Pharmac's role in procuring medicines for the DHBs would likely be absorbed into that, consolidating Health NZ's procurement capability and capacity.

This would be an opportunity to separate Pharmac's two main roles (and resources) and remove the inherent conflict between the two. In line with international best practice a

standalone assessment agency could be created, able to build capacity and expertise in assessing health technology as well as medicines. NICE in the UK is widely regarded as the gold standard for such an agency and there may be potential to access their resources. PBAC in Australia would also be a valuable source of support, and there may even be a case for merging with them.

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

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

Given the outcome of our efforts, our only experience of Pharmac's processes has been negative. We have heard that their commercial negotiators are very skilled, but we have not had sufficient engagement or information to identify any other strengths.

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

We have covered these in our discussion (in 1. above) of our experience with Pharmac.

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

Judging by the medicines they fund and make available to their citizens, and the proportion of their health budgets they spend on medicines, we believe every other country in the OECD does it better than we do. Indeed 44 countries including many outside the OECD fund the CLL treatment ibrutinib, along with many other medicines that are not funded for New Zealanders.

As noted above, many OECD countries have or are developing rapid access schemes and all are faster in funding new medicines than NZ. We could learn from them.

As also noted, we are aware that NICE in the UK is regarded as a standout in its field. We trust the Review Panel will be reviewing NICE's latest 5 year strategy as a model NZ could potentially aspire to, no doubt along with other international models such as Australia's PBAC and CADTH in Canada.

The NICE model in particular is well understood in New Zealand and has been widely discussed in health and medical circles. We hope the Panel will take the opportunity of this review to be bold, and put forward a proposal to the Government on adopting and creating a new, fit for purpose model based on international best practice, such as the NICE one.

We do not of course have the resources to review the literature on successful systems from around the world, but we also commend to you this OECD study commissioned by OECD health ministers that provides an evidence-based assessment of the current performance of the pharmaceutical innovation system, and presents a critical analysis of policy options for reforms to promote access and sustainability'.

What should PHARMAC's role include in the future?

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

We believe Pharmac is rapidly losing the trust and confidence of the public, for all the reasons outlined above. These issues – lack of transparency, decisions on medicines bogged down in years of unexplained delays, failure to engage honestly with stakeholders, failure to keep pace with the rest of the world, etc – are symptoms of significant failings in the Pharmac model that require radical change, including changes to Pharmac's statutory objective and functions, as follows.

We believe:

- The NZ medicines budget should be determined by the Minister of Health and/or Health NZ as an appropriation in Vote: Health. This would allow proper scrutiny and transparent review of how resources are allocated among health services.

- The NZ medicines budget (which currently appears to be around 5% of the total health budget) should at least be set at the average level of comparable OECD countries (in the range of 10 – 15%).

- Pharmac's functions should be split, with procurement taken over by Health NZ in co-governance with the Māori Health authority, and a new 'Pharmac' being responsible for assessment of medicines and health technology, potentially utilising expertise and resources from international agencies.

- The reformed Pharmac should be modelled on NICE's vision, structure and new 5 year plan.

- In addition to addressing the key shortcomings in the current model, a new 'Pharmac' should be required to publish a regular horizon scan of discoveries and innovations in medicine, therapies and health technologies that are in the pipeline. It should also advise the government of the day on the cost of closing the medicines access gap with other comparable countries (see 'Challenges' above).

- Wider and downstream social and economic costs and benefits should form part of its assessment criteria, together with reducing inequities and inequalities in health access and outcomes.

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

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

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

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

We do not have sufficient information to judge this, but as noted in our submission, Pharmac's current narrow limitation to health considerations rather than the wider social and economic impacts of decisions on medicines, will be impacting more heavily on people who are already disadvantaged in the health system, including Māori, Pasifika and disabled people.

9. How can PHARMAC achieve more equitable outcomes?

Faster, better access to effective medicines, achieved through more transparent, fairer processes that consider wider aspects of health and wellbeing, overseen by a forward-looking, dynamic agency that operates in line with international best practice will benefit all New Zealanders and reduce current inequities.

Additional feedback

Is there anything else that you think the Review Panel should consider?

We are concerned about the rushed timeline and clearly limited resources the Panel appears to have been given to carry out this very important review. We urge you to ask for an extension to allow you sufficient time to thoroughly consider and do justice to the large volume of feedback you've received. This is a once in a generation opportunity to achieve much-needed reform in an area that profoundly impacts the wellbeing of New Zealanders' and will do so for years to come.

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.

Name	Dr Neil Graham
Email address	neil@clladvocates.nz
Organisation	Chronic Lymphocytic Leukaemia Advocates NZ (CLLANZ)

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.

PLEASE NOTE OUR TABLE APPENDED TO THIS SUBMISSION

Appendix 1

Table 1: New Zealand registration dates and funding status for ibrutinib and venetoclax

Targeted treatment	Year (of registration)	Funding application received	Indication/ line of therapy	Clinical review meeting outcomes	Date completed	Status
Ibrutinib	2015	Aug 2015	Relapsed/ refractory CLL del17p	Nov 2015: PTAC - Decline May 2016: CaTSoP – Medium priority Aug 2016: PTAC -Low priority Sept 2016: CaTSoP – Medium priority Nov 2016: PTAC - Defer Feb 2017: PTAC – Medium priority	N/A	Unfunded
Ibrutinib		Aug 2015	1st line del17p CLL	Nov 2015: PTAC - Decline May 2016: CaTSoP – Medium priority Aug 2016: PTAC - Low priority Sept 2016: CaTSoP – Medium priority Nov 2016: PTAC - Defer Feb 2017: PTAC – Medium priority	N/A	Unfunded
Ibrutinib		Aug 2015	Relapsed/ refractory CLL	Nov 2015: PTAC - Decline May 2016: CaTSoP – Medium priority Aug 2016: PTAC – Low priority	N/A	Unfunded
Ibrutinib		Mar 2018	1st line CLL for unfit patients	Mar 2018: CaTSoP – No formal recommendation Sept 2020: CaTSoP – Low priority	N/A	Unfunded
Ibrutinib		Aug 2015	Ibrutinib for CLL as a subsequent line of therapy (RR or intolerance) to venetoclax containing regimens	Sep 2020: CaTSoP – High priority Feb 2021: CaTSoP – High priority	N/A	Unfunded
Ibrutinib		Aug 2015	1st line or RR as alternative to venetoclax where ibrutinib is a more appropriate option, within context of	Sep 2020: CaTSoP – Medium priority	N/A	Unfunded

			treatment malignancy			
Venetoclax	2017	Nov 2017	Relapsed/refractory CLL, del17p	Sept 2018: CaTSoP – High priority	Dec 2019	Funded
		Nov 2017	1st line CLL, del17p or TP53mut	Feb 2018: PTAC – Defer Sept 2018: CaTSoP – High priority	Dec 2019	Funded
Venetoclax (plus rituximab)		Nov 2017	Relapsed/ refractory CLL	Apr 2019: CaTSoP – High priority	Dec 2019	Funded
Venetoclax (plus obinutuzumab)		Feb 2020	1st line CLL patients for whom fludarabine-based chemoimmunotherapy is inappropriate and who are IGHV unmutated	Jul 2020: CaTSoP – Low priority	N/A	Unfunded