Researched Medicines Industry Association of New Zealand Incorporated

High Cost Medicines Panel Final Submission

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A. **Background**

PHARMAC and the High Cost Medicines (HCM) Panel have identified that there are some medicines that would offer substantial therapeutic benefit that are not yet funded, but the Panel have not provided practical methods of ensuring New Zealanders gain access to these medicines. In the preliminary report, the Panel appears to endorse the approach taken by PHARMAC in most areas. This endorsement is appropriate where PHARMAC is succeeding in its mandate - i.e. to contain costs within its budget. In the area of high cost medicines however, where PHARMAC is not successfully providing optimal access for patients, the Panel should provide specific recommendations to improve this access.

The New Zealand Institute of Economic Research (NZIER) has performed an independent review of the HCM Panel's preliminary report; and some of the submissions made to the HCM panel (Annex 2). The NZIER review considers it possible that a reallocation of funding within the health system, in favour of medicines, would generate an improvement in health outcomes for the same financial outlay. The NZIER report also expresses concern that the HCM Panel's preliminary report does not attempt to offer a solution for managing future demand for high cost medicines. The NZIER have not provided detailed economic solutions for improving access to high cost medicines, largely as reliable information on which to base their recommendations is not readily available.

This lack of publicly available information is reflective of the lack of transparency of the PHARMAC and DHB processes.

Although the NZIER report and this RMI submission overlap in some areas, they approach the issue from different perspectives and should be read independently of one another.

B. **High Cost Medicines Definition**

The range of medicines identified as high cost and/or highly specialised, as described in the HCM preliminary report, appears to span the entire range of medicines not presently funded by PHARMAC. To focus the possible interventions on manageable areas, with different practical interventions; the short term solution should focus only on the high cost and highly specialised medicines; while the longer term focus should be on improving access to medicines in general.

The major foci of the two solutions are:

- improving the Exceptional Circumstances (EC) Scheme; and
- improving the budget setting process.

This submission will first discuss those problems, relating to high cost medicines, not yet adequately addressed by the HCM Panel's preliminary report; before moving onto the solutions outlined above.

C. Current Problems

The lack of a nationally driven prioritisation framework is linked to the problems impeding optimal access to medicines in New Zealand.

There are a number of discreet problems with the current medicine procurement processes that also negatively impact patients' access to High Cost Medicines. Addressing these problems would have the effect of improving access to high cost medicines in New Zealand. The HCM Preliminary report does not adequately address the following problems:

1. **Health Outcomes**
   The current approach encourages a lack of focus on patient outcomes. PHARMAC is not held accountable for monitoring health outcomes influenced by pharmaceutical procurement practices. The New Zealand Public Health and Disability Act 2000 sets out PHARMAC's objective as: to secure for eligible people in need of pharmaceuticals, *the best health outcomes* that are reasonably achievable from pharmaceutical treatment and from within the amount of funding provided. Currently PHARMAC's operations show a bias towards fiscal restraint, with little, if any resource spent on identifying the impact that medicines funding decisions have on patient outcomes.

   It is entirely feasible that long term restrictions on medicine funding have resulted in strain on other areas of the health system. The accelerated growth of spending in other areas of health relative to medicines may indicate that this is actually occurring.

   For PHARMAC to efficiently discharge its responsibility, if it is tasked with ensuring access to hospital medicines and devices, it will need to better gauge health outcomes from its funding decisions. Although we recognise the challenges this poses, it should be considered within the wider reforms being embarked on in the health system. Based on this information PHARMAC will be in a better position to determine clinical need for medicines, including new medicines into the future. It will not be adequate to project future expenditure from past spending, simply by adding an allowance for volume growth. The risk is that in the absence of adequate planning, hospitals will either overrun their budgets, or face shortages of these hospital treatments and devices.

   An effective system of assessing clinical need would prevent the need for people to lobby for funding for a specific medicine, with the accompanying drain on resources and political risk evident in the present system.

   We recommend that PHARMAC, the Ministry of Health, clinician groups and the pharmaceutical industry work together on identifying future optimum and affordable treatments for New Zealand, based on clinical need.

2. **"Post Code" Medicine Access**

Patients do not have equal access to medicines across New Zealand.

DHB's are currently able to reduce their medicines budget to fund other areas; and there are a number of DHB's that limit access to hospital medicines depending on their financial positions. One needs to accept that population based funding formula is not a perfect tool and patients in need of treatment should have the system accommodate their needs. Patients should not have to work around a rigid system by moving to another area for the sole reason of accessing a funded medicine. If (as described verbally by the HCM panel), PHARMAC is involved in establishing the cost effectiveness of a medicine and procuring medicines, but DHB's retain responsibility for restricting patients' access to hospital medicines based solely on DHB budgetary constraints; the "post-code" prescribing currently seen in many facets of health care in New Zealand will continue.

We submit that all pharmaceuticals that PHARMAC considers worthy of funding should be listed on the Schedule and access to these medicines should be guaranteed for all patients for whom they are clinically appropriate. In order to prevent over-prescribing, a special authority system of clinical criteria should be used. The comprehensive pharmaceutical schedule should be underpinned by a single medicines budget for PHARMAC as described in the "Budgetary Process" recommendations below.

3. **Inefficient Resource Allocation**

With no prioritisation framework, how do we know value for money is being achieved?

Where different agencies are involved in assessing health technologies, unless they have an explicit method of comparing analyses, they will risk allocating resources inefficiently. This problem is evident in the current system, where DHB's, Ministry of Health and PHARMAC are making decisions based on different considerations of what constitutes value for money (QALYs per dollar spent). For the New Zealand Health system to make more efficient spending decisions as envisaged by the Ministerial Review Group, the agencies involved need to have a method of sharing decision benchmarks. We continue to consider that publishing a cost-per-QALY range, considered current value for money, would be the most efficient benchmark.

As the HCM Panel has indicated strongly that it will not be recommending publishing a cost-per-QALY considered value-for-money, a second best alternative would be for PHARMAC to publish a manual of standard unit costs to be taken into account when preparing Cost Utility Analyses. PHARMAC have in the past undertaken to prepare this manual, but have given this work a low priority and have not yet produced it.

The HCM panel should recommend that PHARMAC publish a manual of standard unit costs as a matter of urgency, both to facilitate access to high cost medicines and to further drive efficient purchasing as envisaged by the Ministerial Review Group.

4. **PTAC Independence and Transparency**

For a medicines funding system to effectively balance the tensions between clinical advice and financial imperatives, the two bodies (PTAC and PHARMAC board) should be independent of each other.

The HCM Panel has identified that PTAC is perceived as not receiving independent advice. PHARMAC currently changes some of the financial parameters of sponsors' submissions, prior to submission to PTAC. Sponsors subsequently have no way to ensure that any changes made by PHARMAC accurately reflect the cost benefit of their applications. This lack of transparency brings into question the independence of PTAC decisions.

There are also instances of the PHARMAC board prioritising medicines investments contrary to PTAC recommendations. We consider that the fiscal bias shown by PHARMAC over clinical recommendations poses a risk to optimal patient outcomes.

We would like to see independence and transparency improved for sponsors by:

- **being given an opportunity to present to PTAC;**
- **being able to review the changes that PHARMAC make to submissions; and**
- **receiving firm guarantees on timelines for funding decisions for new medicines.**

5. **Disinvestments**

Future pressure on health budgets will increase, this requires PHARMAC to focus its spending. Although PHARMAC report that they use a Programme Budgeting Marginal Analysis (PBMA) approach to medicines investments, they do not appear to seriously consider the opportunity cost of continuing to spend money on historic funding decisions. Continuing to subsidise medicines at the current co-payment level of $3 per prescription does not foster equal access to medicines according to affordability. Although making changes to this area of funding may be contentious; if PHARMAC are truly to follow a utilitarian system, it must be considered.

We recommend that the High Cost Medicines Review Panel or the Minister of Health use pharmaco-economic principles to investigate the potential benefits of increasing medicine co-payments, to release funding for high cost medicines.

6. **Accountability**

PHARMAC is currently perceived to be accountable to the Minister of Health. We contend that as a democratic institution, PHARMAC should ultimately be accountable to the population of New Zealand.

We recommend that consumer representatives be included in PTAC and on the PHARMAC Board in order to ensure that PHARMAC becomes accountable to the public.

D. **Proposed Solutions**

We would like to propose the following two broad approaches to improving high cost medicine access in New Zealand:

1. <u>Exceptional Circumstances Scheme</u>

For this option, the definition of High Cost medicines should be narrowed down to those medicines which are both highly specialised and high cost as shown in the shaded portion of the table below.

Costliness		
	High Cost and Not Highly Specialised	**High Cost and Highly Specialised**
	Low-Cost and Not Highly-Specialised	Low Cost and Highly Specialised

Degree of Specialisation

The HCM Panel has recommended that the three exceptional circumstances schemes be amalgamated into one. We agree with this proposal and consider that it may improve clarity for prescribers, as long as other efficiencies are incorporated into the process.

Eligibility for medicines for relatively rare indications would need their assessments to be more heavily weighted in favour of the PHARMAC criteria other than cost-effectiveness. In this way, issues such as the lack of alternative treatments, risks of other treatments, and the severity of people's health problems could be taken into account as envisaged by the nine PHARMAC decision criteria. Although this approach is not utilitarian, it parallels the current PHARMAC approach of spending on lower priorities where the sole reason for the spending appears to be that the overall budget impact is low and thus fits within the remaining annual medicines budget.

We propose that applications be made for indications and conditions for which high cost, highly specialised medicines exist; rather than full applications being made for each patient. These applications should be made by prescribers, patient groups, sponsors or individual patients and then the scheme can be administered by means of special authority-type applications. Where an application has been made for an individual patient, the application should be seen as a precedent for the indication concerned. (Please see Annex 1 for proposed details of how the new Exceptional Circumstances Scheme should be administered.)

Any numerical limit of patient numbers should be flexible, with the proposed number being 100, but this could be varied on the basis of epidemiological data and budget. Clearly an overall EC budget would still be required, the extent of which would be a political decision.

The pharmaceutical industry is prepared to negotiate terms of risk sharing in order to allow patients to access high cost medicines. The pharmaceutical industry already assists PHARMAC in providing improved access to medicines outside of the EC scheme, using risk sharing arrangements. If an indication is funded based on epidemiological data suggesting that 100 patients would benefit from the medicine per year, and further patients are subsequently prescribed the medicine, the industry may bear part or all of that cost. Risk sharing would be reliant on a minimum number of patients being funded publicly.

Any exceptional circumstances scheme should not pose an unreasonable administrative burden, as the cost of health practitioner's time represents a substantial transaction cost to the health system. An unwieldy system, such as the current one, also impedes reasonable access to medicines based on administrative burden rather than clinical or financial grounds.

2. Budgetary process

Over the longer term, access to high cost medicines requires a more substantial change to medicines procurement policies.

There is evidence that, even when PHARMAC considers a medicine to be cost effective, the delay to funding this on the Pharmaceutical Schedule can be a number of years (See PHARMAC response to Dr Ken Whyte[1]). We contend that PHARMAC should be more cognisant of its legislated objective of providing the best health outcomes that are reasonably achievable for New Zealanders.

The High Cost Medicines Review should take into account the broader context of the MRG findings with the likely outcome of PHARMAC's procurement responsibility extending to medical devices (possibly on a phased basis). The MRG also recommends that health interventions be assessed on a similar basis but by the National Health Committee (NHC). In order to accommodate the increasing number of highly specialised medicines in the pharmaceutical pipeline, which often are appropriate for small numbers of people, the High Cost Medicines Panel should recommend a practical process of forward budget setting.

[1] Metcalfe S, Rasiah D, Dougherty S, (2005); PHARMAC responds on treatments for pulmonary arterial hypertension; *Journal of the New Zealand Medical Association* 118(1227) https://www.nzma.org.nz/journal/118-1227/1805/

Proposed budget setting process:

- PTAC should continue to set a flexible priority list based on current applications and advice about what medicines are likely to exit development in a specified timeframe. (generating the research areas for step 2)

- Research epidemiological evidence of disease burden for each potential indication, together with downstream costs to the health system and society. This should be researched by medicine sponsors, PHARMAC and DHBs (or Public Health departments). There is already a substantial database of relevant information in New Zealand, and this would support the Government's objective of New Zealand becoming a knowledge based economy, as the resulting information would be pertinent internationally.

- All treatments (devices and pharmaceuticals) should then be compared according to similar economic benchmarks, taking into account benefits and costs, including downstream cost savings from changed health outcomes. (generating a refined priority list)

- The refined priority list should be put forward for allocation of a dedicated nominal procurement budget from Vote Health. This budget would receive consideration alongside a NHC priority list for health investments, and would need to make provision for growth in funding due to underlying volume growth, as well as funding for new initiatives or medicines. (NHC and PHARMAC must produce comparable assessments).

The process of integrated budget setting across the health system is the only method to guarantee that funds will be used efficiently and not on health delivery projects that offer poor cost-effectiveness. By assessing all health interventions according to similar benchmarks, it is likely to become apparent how favourable pharmaceutical cost-effectiveness is relative to other interventions. Once an integrated budget setting process is established, any enforcement of that budget becomes more legitimate and is likely to have public and health professional buy-in.

This system should then use a single nominal medicines budget (as opposed to separate community and hospital budgets) from which to purchase medicines and guarantee national access to medicines considered cost-effective.

The medicines budget should cover an extended comprehensive pharmaceutical schedule to include all hospital administered medicines in addition to the current pharmaceutical schedule, with the requirement that DHB's provide all products on the schedule. The major benefit of this is that NZ patients will receive a national standard of care. If a product has been assessed as being cost-effective, a DHB cannot then decide not to fund it. The control of overprescribing can be achieved through special authority administration which would be based on clinical criteria being appropriate. Any attempt to limit usage according to budget is an infringement on a patient's right to optimal treatment, as this treatment has already been decided to be affordable at a national level.

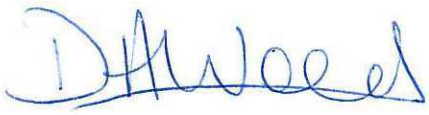
When developing the comprehensive schedule, consideration will need to be given to automatically incorporating medicines already in common use in hospitals, but which do not appear on any schedule.

Where DHB's are in a budget deficit situation, it becomes even more important to focus their spending on medical interventions that have been shown to be cost-effective. If a product is funded by PHARMAC, it is by definition considered cost-effective. If the DHB's have embarked on the negotiations with PHARMAC about the annual pharmaceuticals budget in a meaningful manner, they will not have any potential investments that would be more cost-effective.

Conclusion

Any meaningful attempt to improve health outcomes by improving access to high cost medicines would need to address the underlying systemic problems currently impeding this access.

The High Cost Medicines Panel Preliminary report has identified some of these problems. The Panel should now provide recommendations for practical steps to be implemented, rather than simply recommending that the current stakeholders hold the answers and should be left to implement them.



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Annex 1

Proposed Exceptional Circumstances Scheme:

Due to the nature of people and their health problems being diverse, there are always going to be some people with conditions that are not catered for in the standard system. Any humane health system focusing on optimal health outcomes should make a reasonable attempt to accommodate these individuals; and this principle of enabling people to access medicines, devices and services is proscribed in the New Zealand Public Health and Disability Act 2000 as "the right to the best care or support for those in need of services".

To progress effective access to care for some of those people most disadvantaged by their health conditions, we recommend changing the current Exceptional Circumstances Schemes to include the following principles and processes:

1. It should be a single national program, incorporating what are now called the Community, Cancer and Hospital EC Schemes.

2. Eligibility for the program be decided on the basis of a disease (or medication indication) being considered appropriate for funding rather than on applications for specific patients. Decisions about eligibility for exceptional circumstances funding by condition should be made by a group of clinicians, possibly a subcommittee of PTAC. Disease states may be proposed for inclusion by prescribers, patients, families, patient groups or technology suppliers.

3. Specific patients should be enrolled using criteria and application processes similar to the current Special Authority system, this would reduce the substantial burden to prescribers which the current system poses.

4. Epidemiological data should be used to identify a limited number of patients who would be expected to have the condition and need treatment for it in any one year. The current limit of nine patients is unreasonably restrictive and should be increased, possibly to the order of 100. This number may need to be set according to a budgetary limit, but should not cause inequity by being applied too rigidly.

5. Medicine Sponsors will consider risk sharing for costs incurred due to inaccuracies around epidemiological estimates of disease incidence or prevalence. This may be discussed in advance of specific conditions being included in the new Exceptional Circumstances Scheme.

6. For life saving medicines, the program would cover access to medicines while PHARMAC are assessing an application.

Annex 2 (See PDF attached to email)

Response to the Preliminary Report: Review of access to high-cost, highly specialised medicines in New Zealand
Report to the Researched Medicines Industry