Medicines New Zealand

Medicines New Zealand Submission:

PHARMAC Contestable fund for medicines for rare disorders consultation and draft RFP

July 2014

Thank you for the opportunity to submit the views of our member companies on the Contestable fund for medicines for rare disorders proposal and associated draft RFP.

Our members are pleased that the proposal shows meaningful progress towards a system that provides better access to medicines for rare disorders than the current approach, but have some concerns that the limited budget will result in this access only improving gradually. At the current budget, the time to provide access comparable to that in Scotland for example may take 5 years or longer based on estimates by the patient group NZORD.

We would like to see PHARMAC commit to actually using the budget for this group of patients, as the current proposal advises that one potential outcome of the process would be to not fund any of the proposals put to them. We consider this would be a missed opportunity for substantially improved access for patients who have in many instances been waiting for access for a number of years, with consequent permanent negative impacts on their health.

The approach to move medicines that are funded under the RFP process to the schedule in subsequent years is essential to providing new access each year under a limited budget and as such we strongly support this aspect of the proposal.

Rare disorders subcommittee

We note that there are no specific terms of reference for the proposed subcommittee and it appears that the subcommittee may operate in a manner somewhat different from the usual PTAC subcommittee arrangement. We would recommend that specific terms of reference be developed and consulted on for the subcommittee and that its functions and relationship with PTAC be clearly defined.

We consider that it is essential for the subcommittee to be comprised at least in part of clinicians who are actively treating the rare disorders for which the subcommittee is responsible. It would not be sufficient to have clinicians without this expertise as the sole makeup of the subcommittee.

Meeting Prerequisites and expressions of interest

The ability of PHARMAC to pre-screen for clinical evidence and the ability of the application dossier to meet the prerequisites is important. We would like to have clarity about how this expression of interest and pre-screening would work in practice and if there would be a set process with timelines within which companies and other stakeholders may submit information.

Clinical and Patient input

It appears that insufficient consideration has been given to the need for patient input to the process, we recommend that the Subcommittee have a transparent and robust process to gain the patient and clinician input prior to making decisions about funding.

The process of the RFP appears to envisage the analysis and recommendation to the Board being done by the PHARMAC secretariat and use of the PTAC or its Rare Disorders Subcommittee only in certain instances. This would make it even more important to include a meaningful opportunity for input from patients and clinicians to ensure decisions are based on the optimal amount of information.

Timeframes for the process

We are concerned that, as with PHARMAC's usual application process, there are no timeframes to which PHARMAC is committing to in which to make a decision or close-off the process, other than a date by which applications would need to be submitted. For a process that would run annually and have an annual budget it is important for the RFP process to have a close-off date by which funding decisions should have been made at the very latest. It would not be acceptable for a company to be held to conditions submitted in one RFP round to be competing with a subsequently submitted application under different criteria or circumstances. We believe that a 6 month timeframe from the closing date for proposals should be an adequate time in which to process the proposals and make decisions.

The need for a timeframe is further highlighted by the requirement set by PHARMAC that a company may not withdraw a proposal "*while the RFP process is continuing*" (page 4 of draft RFP). It is not feasible for proposals to remain open indefinitely or at the sole discretion of PHARMAC.

In addition, a process with timeframes for the "expressions of interest" that may be made outside of the PTAC process should be provided.

Implications for NPPA

We consider that the interpretation set out by PHARMAC about how medicines or patients are eligible for consideration under NPPA and how this relates to the Proposed RPF process and usual Schedule listing process is appropriate.

Consultation Questions

1. **What do you think of the proposed rarity definition?**

Prerequisites one and six – rarity

We support the clear definition that is likely to include the appropriate group of patients.

We would like to highlight however that rare disorders can and do include diseases that are not inborn errors of metabolism, and recommend that provision should be made to be able to compare benefits between other types of conditions such as rare cancers and these conditions in order to gain most health benefits for people with rare diseases.

We support the ability to identify subgroups of people who would benefit most from receiving access to the medicine in question as a way to provide the best health outcomes for the limited budget.

For prerequisite 6:

The medicine is not registered for the treatment of another, non-rare disorder, or if it is, the cumulative prevalence across all the indications still falls within the definition of rare.

We would be concerned if this precluded some medicines from applying, for which the initial indication is rare, but for which larger indications are in development. These subsequent indications sometimes take a number of years to further develop evidence in large trials. Examples may include specific rare types of cancer for which a treatment may be used, but for which medicine, other indications later become recognised. It would be inappropriate to completely exclude patient access for a number of years based on a possibility of subsequent wider indications being developed.

2. **Do you think that the RFP should be limited to medicines that treat disorders that cause a significant reduction in either life expectancy or quality of life? Why or why not?**

Prerequisite 2 – disease severity

We support the reservation of the fund being to treat disorders that cause substantial negative impacts on health, of which life expectancy and quality of life are reasonable measures.

We are concerned however at the limited ability of QALY's to reflect all of the impacts on people's health and although there is good reason to use QALY's when they are available, we recommend that PHARMAC and the subcommittee be prepared to consider other aspects and measures of health as also important in the decision making.

3. **Do you think prerequisite 3, which limits eligible bids to those that have either been approved by Medsafe or an international regulator recognised by Medsafe, is appropriate? Why or why not?**

Prerequisite 3 – registration

We support the requirement for medicines to be registered in New Zealand or internationally in order to be considered eligible for the RFP.

4. **Do you think prerequisites 4 and 5, relating to the effectiveness of the medicine, are appropriate? Why or why not?**

Prerequisite 4 and 5 – effectiveness

We recognise the need for applicants to have an opportunity to share evidence of effectiveness with PHARMAC outside of the RFP process and would encourage PHARMAC to provide more clear guidance about the process for doing this.

We recommend that PHARMAC explicitly recognise the challenges in developing evidence for efficacy in small populations of people with rare disorders and that this limitation will not be the sole reason for excluding medicines for funding. We believe that with robust entry and exit criteria the appropriate clinical treatment of these people can be achieved.

5. **Do you think prerequisites 7 and 8, relating to the availability of suitable alternatives, are appropriate? Why or why not?**

Criteria 7 and 8 – alternative treatments

We consider the exclusion of products for which there is a therapeutic alternative funded is appropriate as long as what is considered therapeutically equivalent is a robust analysis of the benefits and safety profile. Medicines that provide equivalent efficacy but improve safety profile substantially should not be excluded from the RFP.

6. **Do you consider it appropriate to state in the RFP that the existence of proposals is not confidential? Do you consider it appropriate for PHARMAC to secure the ability to make the name of the suppliers submitting proposals public?**

PHARMAC proposes making an amendment to expressly state that the following information is not "Confidential Information":

1) The existence of a proposal; and/or

2) The name of the supplier who submitted a proposal.

We consider the above changes to enable the sharing of the existence of the proposal and the name of the submitter is acceptable. This should not extend to other information within the proposals that would still need to receive the appropriate level of confidentiality.

Medicines New Zealand appreciates the opportunity to submit on this important step forward in providing access to medicines for rare disorders.

For further information or queries, please contact:

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