25 July 2014

Contestable fund for medicines for rare disorders.

This feedback is from Lysosomal Diseases New Zealand (LDNZ). We are a charitable trust set up in 2000 to improve information, support research and ensure families have access to health and disability services including access to medicines where there is a therapy.

After more than a decade of reviews, consultations, meetings with Pharmac, meeting with Politicians, a 2008 election pledge by National to fund specialised medicines and a complaint to the Ombudsman we finally get to see Pharmac establish a contestable fund for medicines for rare disorders**.** LDNZ thanks Pharamc for listening and responding to the criticisms we have made over the years.

However; in reading all the documents Pharmac has put out for this consultation process we have identified several areas that concerns LDNZ greatly.

1. LDNZ is very concerned over the small size of the budget in relation to the number of orphan disease patients likely to be indicated for treatment with medicines currently available but not funded here. We state this has particular risk in relation to moral hazards and adverse selection, unless those risks are identified and carefully managed. We continue to state that an estimated $20 to $25 million would be needed to treat all patients who are currently waiting to gain access to their particular therapy and provide a much fairer system for patients with rare diseases.

2. It appears that the $5million fund is a **one off**, seeing the therapies accepted for funding moving onto the schedule with no further funding being made available for new treatments. Pharmac states it will provide 25 million over 5 years but we don't believe Pharmac is providing any further new money to continue to fund orphan drugs beyond this first $5 million. It seems clear that reference to $25 million relates to the ongoing costs over 5 years of any drugs funded by this fund.

    - ***This will not solve the on-going problem of how New Zealand will provide access to medicines for those with rare diseases in the long term, given the number of drugs and numbers of patients who are waiting.***

3. **Prerequisite 5** – "Substantially improved" This sets the threshold far too high for treatments such as the Lysosomal enzymes. Pharmac has previously stated in declined Lysosomal applications that one would expect to see ***modest improvement and stabilisation of disease***. Our Lysosomal patients consider modest improvement and stabilisation of their disease to be a great result from treatment.

    - If Pharmac insists on using such strong wording it will continue to set rare diseases up to be disadvantaged due to the lack of long-term clinical evidence and the lack of detailed

natural history data and a presumed lack of cost effectiveness that would arise from these factors.

- LDNZ would like to see Pharmac readdress this wording and change it to **Stabilisation and improvement in health status** as the prerequisite.

4. **PTAC subcommittee Paragraph 1.9.** PTAC state "for all the treatments that have been recommended for decline previously, to be considered under the new scheme must either present, new information, changes in pricing or clinical data indicating better efficacy, safety and cost effectiveness".

    - It appears to us that Pharmac and PTAC has some inherent contradictions in attempting to address the needs of these ultra-orphan diseases and seems to be insisting on clinging to an expectation of cost-effectiveness and comparing these treatments with the costs of medicines for common diseases. **If PTAC continue along this line we will never see these treatments funded. As stated in other submissions there needs to be a softer approach in assessing orphan medicines. These therapies should not be assessed against drugs for common diseases. They need their own set of assessment criteria and fair entry and exit criteria.**

5. **PTAC subcommittee Paragraph 1.10** states "If different evidentiary thresholds conceptually for accepting efficacy were applied to medicines for the treatment of high need rare diseases then this could lead to unfair funding decisions across all Pharmac funding setting".

    - We struggle to see why PTAC would think like this. Different evidentiary thresholds for rare diseases **do NOT lead** to equity problems and potentially unfair decisions across all Pharmac funding settings. They would redress the inherent inequities of rarity, just as countervailing priorities for ethnic or socio-economic deprivation factors that are well established in our health system (and other social policies) to try and achieve equity of outcomes. ***We suggest that the PTAC subcommittee should reconsider this statement very carefully.***

It is extremely difficult to have confidence that Pharmac and the PTAC subcommittee will actually do the right thing in relation to fairness and equity of outcomes for the Lysosomal Enzymes.

LDNZ hopes that Pharmac will consider the points above very carefully as it seems to us that Pharmac is setting this scheme up to fail.

Jenny Noble
Field Officer
Lysosomal Diseases New Zealand