

## Exceptional Circumstances review

On behalf of Muscular Dystrophy Association I am writing to urge you to reconsider the current proposed exceptional circumstances scheme so that it enables New Zealanders with Pompe disease to receive treatment as well as providing for access to medicine for other rare disorders where the treatment is likely to be expensive and for the life-duration of the patient.

The Exceptional Circumstances review proposes a single exceptional circumstances scheme called Named Patient Pharmaceutical Assessment (NPPA). NPPA would consider funding for individuals for treatments prior to their assessment for Schedule listing for the relevant indication. Within NPPA there would be three distinct pathways by which individual patients can apply for funding for pharmaceuticals not listed on the Pharmaceutical Schedule. However the eligibility of patients with rare disorders, where the treatment is likely to be expensive and required long-term, is too easily challenged. Patients requiring long-term enzyme replacement therapy, for example patients with Pompe disease, will not be eligible for funding for treatment with Myozyme in any of the three categories.

Under category a. Unique Clinical Circumstances, Pompe patients applying for funded treatment with Myozyme would be seeking the standard care for their condition. Hence this would not be a unique set of clinical circumstances to that particular patient.

Under category b. Urgent Assessment, the patient applying for treatment with myozyme would likely be declined given that usually once a product is registered the pharmaceutical company applies to Pharmac for funding. The application is reviewed by PTAC (Pharmaceutical Therapeutic Advisory Committee). In 2008 Pharmac decided to review all enzyme replacement therapies (ERTs). The review can be found on the Pharmac website under PTAC minutes. Myozyme was assessed along with all other ERTs at their meeting 19<sup>th</sup>, 20<sup>th</sup> February 2009 and "The committee therefore recommended that the listing of the six enzyme replacement therapies be declined". As Myozyme has already been declined by PTAC "there would be no justification for considering funding for additional individuals through the NPPA scheme (unless unique clinical circumstances applied)."

Under category c Hospital pharmaceuticals in the community, the patient applying for treatment with Myozyme would likely be declined given that enzyme replacement therapy is longer than 3 months and the cost would be more expensive than palliative care.

New Zealanders living with Pompe disease and other rare disorders deserve the same access to treatment as their counterparts in other first-world countries and we suggest that category b Urgent Assessment is amended to provide for such individuals.

Sincerely,

Miriam Rodrigues  
Membership Services Manager  
Muscular Dystrophy Association of NZ

### **a. Unique Clinical Circumstances**

Some patients experience such a unique clinical condition or set of clinical circumstances that we would be unlikely to have considered these when deciding whether to list a pharmaceutical on the Schedule (a process which is designed for population groups). In these situations we would not expect there to be a patient population (e.g. this patient's clinical circumstances would differ from all others who are seeking this particular treatment). In order to meet our statutory obligations we need to assess whether we should fund treatments for individuals in this position. The Schedule process is not the most appropriate process for those patients who differ significantly to the population group(s) seeking the treatment. We therefore consider it would be more appropriate to assess funding for these individuals through the NPPA scheme. A number of stakeholders who responded to our discussion document commented that EC should provide for conditions that are 'rare' or 'out of the ordinary'. Unique Clinical Circumstances (UCC) provides for truly exceptional cases; although we recognise our proposal is not aligned with the views of those people who wanted to see the number limit on 'rarity' increased from 10. We consider the Urgent Assessment pathway (described in the next section) is the most appropriate avenue for considering the circumstances of those people who are part of a patient population (albeit a small one in some cases).

### **b. Urgent Assessment**

There are some situations in which a patient with a serious clinical condition may experience a significant deterioration in health or lose the opportunity for a significant improvement in quality of life before assessment for listing treatment(s) not listed on the Schedule for the relevant indication is undertaken. This patient may be part of a group of patients with similar conditions. In our view, until the treatment(s) being sought has been subject to a full Schedule assessment for the relevant indication, it is fair to consider funding the treatment for individuals at risk of significant irreversible damage. This proposed change would be significant as it would see the balance shifted in favour of potentially providing funded access to treatments prior to full Schedule assessment. The circumstances in which we would consider an application under the Urgent Assessment pathway of the NPPA scheme include where the patient's condition may be, but is not necessarily, 'rare' and where provision of the treatment is potentially life saving. These are situations that stakeholders who responded to the discussion document suggested an EC process should provide for. In addition, some medicines likely to be sought by people eligible for consideration under Urgent Assessment could be described as 'high cost' and 'highly specialised'. Improving access to these types of medicines has been a focus for some stakeholders, and this theme was also reflected in submissions. Under the proposed Urgent Assessment pathway, once a Schedule assessment is completed and the pharmaceutical is either included on PHARMAC's prioritisation list or declined for

funding, there would be no justification for considering funding for additional individuals through the NPPA scheme (unless unique clinical circumstances applied). These individuals would be in the same position as others awaiting the outcome of PHARMAC's Schedule funding process. Named patients who had received funded treatments prior to Schedule assessment would continue to receive funding following assessment, even if the pharmaceutical was not listed on the Schedule for the population, provided they continue to benefit from treatment and meet any conditions of funding at the time of approval. This is discussed further in section 4(c).

### **c. Hospital Pharmaceuticals in the Community**

There are some circumstances in which it would be cheaper for a District Health Board (DHB) to fund an otherwise unfunded pharmaceutical for use in the community for an individual patient under its care, than to pay for the most likely alternative treatment or outcome. DHBs cannot do this without PHARMAC approval as the NZPHD Act requires DHBs to act consistently with the Pharmaceutical Schedule. We consider it fair that a process exists for DHBs to seek PHARMAC approval to be able to supply, and fund from their own budgets, otherwise unfunded treatments. The Hospital Exceptional Circumstances scheme currently provides this process. The proposed name change to 'Hospital Pharmaceuticals in the Community' (HPC) aims to better reflect the purpose of this proposed pathway and to clarify that under this pathway it is the provision of funding for treatments outside the Schedule that is exceptional, not the clinical circumstances of the individual applicant. This clarification was sought by a number of stakeholders who responded to our discussion document