20th August 2015

Australian Pompe's Association

Dear Committee,

Re: PBAC Review from the Australian Pompe's Association (APA)

The Australian Pompe's Association (APA) is grateful for the opportunity to provide input to the Department of Health's *Post-Market Review of the PBAC guidelines.*

The APA represents the interests of the 37 Australians known to be living with extremely rare, debilitating and life threatening Pompe disease and has intimate knowledge of the impacts the PBAC process has on people impacted by life threatening conditions.

There are two core areas that I would like to address in relation to this review:

1) the input of patients and patient groups – and the consideration accorded these key stakeholders; and
2) the way in which the guidelines can further support the review of treatments for rare diseases.

The APA has been involved with the PBAC in regard to approval of treatment for our members since 2007. While treatment was approved on the Life Saving Drugs Programme for those diagnosed as infants relatively quickly, treatment has only recently been approved for the broader patient population this year.

The APA and our membership experienced the disappointment of six separate unsuccessful applications to the PBAC before receiving the good news that treatment is soon to be available for all Australians who need it.

Each time a submission was made by the Sponsor of Myozyme - the only TGA approved treatment for Pompe Disease - the APA also submitted a consumer submission of varying complexity. APA members and other stakeholders such as doctors also submitted individual consumer comments and letters to the PBAC at the time of each submission.

It is concerning that 'Flowchart 2 Details of key decisions and stages' does not appear to give due consideration to the submissions made by consumer groups. When the APA made submissions to the PBAC in relation to Myozyme these reflected the concerns and view of the people who would be most impacted by the decision. However, there does not appear to be a system within the guidelines to ensure that critical issues are not overlooked. The guidelines would also benefit from a system to ensure that none of these critical issues are overlooked and offer the opportunity for the PBAC to discuss or question the patient group on their submission or the issues raised.

The APA is aware of the need to review many drugs of great complexity in a few short days. We appreciate the need to streamline the process and system, however these are critical issues that affect the lives and families of patients and need appropriate consideration.

The APA propose that:

- the PBAC guidelines include a specific requirement that patient associations be notified by the Sponsor of their intention to make a submission to the PBAC (for example, at Section E of Flow Chart 1 of the current Guidelines), and
- the relevant Patient Association be invited to present at the PBAC meeting as a key stakeholder.

We believe it is critical for the PBAC to consider the impact of treatment and the impact of their decision on those who they are ultimately making the decision on behalf of. Patient Associations are able to share deep insight and first hand examples of the effect of treatment. We believe that this is extremely important for the evaluations made throughout the stages outlined in the PBAC guidelines.

The Australian Pompe's Association acknowledges the excellent work done by PBAC patient advocate but the PBAC needs to see and be seen by the patients. We also acknowledge the difficulties of confidentiality in regard to such issues as drug pricing but this could be overcome by separate drug pricing discussions.

The Patient groups also need to be included to see the difficult decisions that the PBAC face at each meeting and by being part of the discussion a much better understanding of the issues will develop. Australia has a complex system of treatment approval and it has taken a significant toll on our members trying to understand and represent themselves within the current system, by being part of the process patient groups and patients will better understand the process.

As outlined above, the other area that we would like to draw to the attention of the committee is the fact that the current guidelines to not address the specific challenges relating to submissions for and review of treatments for ultra-rare conditions. Rare diseases and their treatments raise specific challenges and complexities for data collection and the review of cost effectiveness. We appreciate that the findings of the Life Saving Drugs Programme (LSDP) review have not yet been released and may also address this, but as the current functioning of the LSDP is intrinsically linked.

Conclusion

The work of the PBAC is complex and challenging the admission of patient groups as major stakeholder and the opportunity for patient groups to address the PBAC will enhance and provide an opportunity for the PBAC to consider the impact of their decisions and who they are acting for by making such decisions. The inclusion of Patient groups to the PBAC guidelines will ensure that the work of the PBAC is available to all

About Pompe disease

Pompe disease is an ultra-rare autosomal recessive disorder of glycogen metabolism caused by deficient activity of lysosomal acid maltase Alpha-glucosidase. This enzyme deficiency leads to muscle breakdown, causing disability, which further leads to respiratory failure and in infants, cardiac arrest. Pompe disease is a progressive spectrum disorder, with onset dependent on the amount of enzyme naturally produced. Those diagnosed earlier in life experience faster progression of the disease and a far worse outcome. Australian Pompe Association's membership includes five infantile onset, three juvenile onset and 29 adult onset patients living with Pompe disease in Australia.