

Stakeholder meeting outcome statement

Alglucosidase alfa (Myozyme®) for the treatment of Late-Onset Pompe Disease

A stakeholder meeting was held on 9 October 2014 to discuss the development of eligibility guidelines for subsidised treatment of Late-Onset Pompe Disease with alglucosidase alfa (Myozyme®).

The organisations that were represented included the Australian Pompe's Association, Rare Voices Australia, Genzyme and the Department of Health. Also present at the meeting were clinicians experienced in the treatment of both paediatric and adult patients with Late-Onset Pompe Disease in the areas of genetics, neurology and respiratory medicine.

During the meeting there was constructive discussion about a set of draft eligibility guidelines which had been adapted by the sponsor from the 'ASIEM/ANZAN Guidelines and Indications for Therapy in Late-Onset Pompe Disease with alglucosidase alfa (2012)'.

The discussion focussed on criteria for the treatment of juveniles (aged 24 months to 18 years of age) separately from the criteria for the treatment of adults (aged over 18 years of age) with Late-Onset Pompe Disease, in recognition that Late-Onset Pompe Disease is a heterogeneous condition in which an earlier age of symptom onset and diagnosis generally leads to a greater severity in the course of the disease.

The discussion about initial eligibility covered the diagnostic tests required for the confirmation of Late-Onset Pompe Disease and the level of clinical severity required. Eligibility criteria for the continuation of treatment in juvenile and adult patients were also discussed.

In order to monitor the continuous effectiveness of treatment over time, patient-relevant outcomes such as respiratory function (as evidenced by FVC) and muscular function (as evidenced by the 6 minute walk test) were discussed at length. The importance of determining whether a measured change in 6 minute walk test reflects a true change in muscle testing status or is the result of test variability was discussed. Also discussed was the correlation between 6 minute walk test results and parameters such as quality of life and survival. The Department requested further information on this subject from the expert clinicians.

Stakeholders were informed that work would continue on the development of eligibility guidelines for the subsidised treatment of Late-Onset Pompe Disease with alglucosidase alfa and may involve, as necessary, a further consideration by the Pharmaceutical Benefits Advisory Committee (PBAC).

Attendees were thanked for their participation and their ongoing willingness to participate in further consultation.