From: John Forman
Sent: 1 November, 2016 1:50 PM
To: 'caroline.deluca@pharmac.govt.nz' <caroline.deluca@pharmac.govt.nz>
Subject: Feedback on proposal to list enzyme replacement therapies

Hello Caroline,

This feedback is provided by Lysosomal Diseases New Zealand. We provide information, support and advocacy for families affected by the three diseases intended to be treated: infantile Pompe disease, Hurler disease, and Hunter disease. We are happy for this submission to be shared under the Official Information Act.

We are pleased to see progress with funding for these therapies. Despite our past criticisms of Pharmac's reluctance to consider a schedule listing of these and other rare disease therapies, and the numerous delays there have been in getting to this point, this proposal is another significant moment regarding therapies for Lysosomal diseases. This also reflects the similar comments we made in February/March of this year in relation to the schedule listing of Naglazyme for Maroteaux-Lamy disease (MPS6).

In respect of this proposal, we ask you to consider the following points:

Pompe disease:
1 – we note the information in the proposal about the separate consideration of Myozyme for late onset Pompe disease at the November meeting of PTAC. This begs the question of the status of Juvenile onset Pompe disease in all of this, because it is not mentioned as being under consideration via either of these two pathways. Is this an oversight or an intentional exclusion? Regardless of the answer to that question we assume that any cases of juvenile onset Pompe disease could be considered under the exceptional circumstances (NPPA) scheme.

2 – we consider the special authority criteria and renewal criteria for Myozyme for infantile Pompe disease to be acceptable, given their broad similarity with the Australian guidelines, and our understanding of best practice regarding treatment for this disease.

Hunter disease:
3 – in respect of Elaprase for Hunter disease, we raise a question about the status of a patient with Hunter disease where a transplant cannot be performed, either for lack of a suitable donor match or other valid reason, or where the transplant is not successful. The proposal is silent on such a scenario. In such a case, we assume the patient would be eligible for consideration under the NPPA scheme for treatment with the enzyme.

4 – If this assumption is confirmed, then we find the special authority and renewal criteria for Elaprase for Hunter disease, to be acceptable. We note the different approach taken to treatment criteria compared to the Australian guidelines, but in light of evolving practice in relation to treatment of this disease, we consider the proposed criteria to be acceptable.

Hurler disease:
5 – In respect of Aldurazyme for Hurler disease, we raise the same question about the status of a patient where a transplant cannot be performed, either for lack of a suitable donor match or other valid reason,

or where the transplant is not successful. The proposal is silent on such a scenario. Again, in such a case, we assume the patient would be eligible for consideration under the NPPA scheme for treatment with the enzyme.

6 – we note the significant variation from the Australian guidelines for treatment with Aldurazyme, where the enzyme is used to treat the intermediate form of MPS1, Hurler-scheie only. This proposal is silent on the question of access to the enzyme for MPS1 patients other than those with the Hurler form of the disease.

7 – on the assumption that any Hurler patients fitting the scenario outlined in point 5 above, and any patients with Hurler-scheie who is not transplanted, will retain eligibility to make application for treatment with the enzyme under the NPPA scheme, we find the special authority and renewal criteria to be acceptable.

Thank you for the opportunity to make this submission.
Regards, john

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