30 November 2016 –

Enzyme replacement therapies - 3 new decisions and one more review.

There is some very positive news to report this month, and some disappointment as well. LDNZ is delighted that Pharmac has approved the listing of Myozyme for infantile Pompe disease on the schedule. This is the first approval for this treatment and putting it on the schedule means that any baby diagnosed with this disease, and who fits the treatment guidelines, will be automatically given treatment, avoiding delays and anxious waits for special consideration under the exceptional circumstances scheme.

Also positive is the listing of Aldurazyme for Hurler disease, and Elaprase for Hunter disease, where the babies can be treated with the enzyme replacement therapy before and after an intended bone marrow or stem cell transplant, without any special application being needed. These decisions mean there are now 5 of the Lysosomal diseases which have an approval for treatment with ERT, for at least some affected patients.

The disappointment comes in the form of no decision regarding treatment for Hurler or Hunter patients where a transplant may not be possible, and no decision regarding treatment of older patients (over 2 years of age) with Pompe disease. Instead, Pharmac referred the evidence for Myozyme for late-onset Pompe disease back to PTAC, its expert advisory committee, for further consideration. We are stunned that the acceptability of evidence for this treatment is good enough in 76 other countries, yet Pharmac has decided to reinvent the wheel and do a full review of Myozyme for older patients.

So its three steps forward and two back this month, but overall it is positive news. They resisted for overa decade, but our arguments and advocacy were strong. They built a momentum and we are optimistic that more progress will be made in the near future.