

March 2016 - Pharmac has approved funding of Naglazyme for Maroteaux-Lamy disease

It was 11.22 am on 17 March 2016 when LDNZ got the briefing about this - the first listing in New Zealand of a novel treatment for a lysosomal disease since 1999 when Cerezyme was listed for Gaucher disease. This decision is an important breakthrough for LDNZ and represents a significant shift in approach by Pharmac. In the past 15 years Pharmac has been extremely reluctant to provide access to any of the new treatments for Lysosomal diseases. Their resistance required continued strong advocacy from LDNZ, and our efforts have finally been successful.

This is a very small step in terms of the one disease and a tiny number of patients eligible for treatment, but it's a giant step in terms of a change of attitude, a change of approach, and a change of policy on the part of Pharmac, in making this drug available. They are now showing a willingness to be more considerate of other issues, rather than just a narrow focus on the cost effectiveness of a particular drug and the management of the budget. They're looking more broadly at the ethical considerations which guide decision makers on the practical application of the right to health that we all have, but which had previously not been properly recognised.

LDNZ thinks it is also significant that this decision has come at a time when the budget available for pharmaceutical expenditure has been severely constrained and effectively cut over the past few years by the imposition of additional purchasing responsibilities on Pharmac without enough addition funds being allocated to them. This has forced Pharmac to use all of its savings on other drugs to cover the increased responsibilities without matching funds for the other drug purchases they have had to pick from DHBs and the Health Ministry.

We didn't expect to be praising Pharmac, but they deserve recognition for continuing with this funding decision through the orphan drugs fund when they are under considerable budget pressure. Recent debate about the funding of the Melanoma drug, Keytruda, highlights the fact that many current medicine decisions are driven primarily by the extreme budget squeeze, and we are very pleased that for the first time in a very long time, we have escaped that trap.

The good news with this decision highlights the fact that there's still a lot of work to do because there are several dozen patients with other Lysosomal diseases where treatments are available that are funded pretty much everywhere in the OECD countries, but not yet funded in New Zealand. LDNZ will continue its efforts to ensure this momentum continues.