EMBARGOED until noon Tuesday 7 June 2011

Access to Enzyme Replacement Therapy for Lysosomal Diseases

Background: Lysosomal diseases are caused by an enzyme deficiency that leads to a wide variety of symptoms and health problems, with great differences in age of onset across the 45 different diseases in this group. Many of these diseases are fatal at an early age or lead to serious lifelong complications and a reduced life expectancy because of relentless deterioration in health status**. One of these diseases, Pompe disease, is also a neuromuscular disease supported by the Muscular Dystrophy Association.**

Until the 1990s there were no medicines to treat these diseases. However in 1997 enzyme replacement therapy (ERT) began in New Zealand for Gaucher disease. There are currently 20 Gaucher patients in New Zealand who are on funded ERT treatment.

Since the turn of the century five other enzyme replacement therapies have been developed to treat Lysosomal diseases. **All of these therapies are funded for patients in most developed countries in the world, with very few exceptions.**

Recent developments: These new therapies have been under review by Pharmac since 2004. LDNZ, NZORD and MDA have been working constructively with Ministers, Pharmac, officials and industry over many years to win access to these medicines for NZ patients.

These are the major milestones in our work over that time:

- The development of the medicines strategy and its action plan, published December 2007,
- A 2008 review by PTAC of the new ERTs with a recommendation early in 2009 that the exceptional circumstances (EC) scheme is used as the access point for these medicines,
- The National Party's 2008 election pledge of working to find a solution to problems with access to highly specialised medicines,
- The McCormack Panel's 2009 review on high-cost, highly specialised medicines and its suggestion of using EC to deal with these medicines,
- The two Ministers' (Dunne and Ryall) 2010 announcement of Pharmac's review of the EC scheme as a pathway to access such medicines,
- The 2011 consultation by Pharmac of the EC scheme which has just been completed.

When PTAC recommended in 2009 that these therapies be dealt with through the EC scheme, funded treatment for one young boy was approved within a month. The later reviews recommending use of the EC scheme suggested we were on track to a solution.

The Problem: We have now hit a brick wall.

- No further approvals have been given since the young boy's case in 2009.
- The recent review of Pharmac's exceptional circumstances scheme indicates a tougher stance by Pharmac, rather than improved access.
- One recent application for a 46 yr old woman with Pompe disease was recently declined even though Pharmac noted the treatment would offer "improved muscle function ..... followed by likely stabilisation of the disease" and that there is "no other treatment available for this patient's life threatening disease".

Our groups support Pharmac's role as central purchaser of medicines. Their negotiations on price and controls on medicine use, have meant significant savings for our health system and improved use of medicine. ***However, they fail to address the "fairness" and "community values" prescribed by the NZ Medicine Strategy in their policies and decision making process***. They focus excessively on cost issues and budget management in their decision making, and leave out the moral dimension.

Enzyme replacement therapies for these diseases are not perfect treatments. They do not completely cure the disease but they do make significant improvements to health and quality of life. This is very highly valued by patients for diseases that have little or no other effective interventions.

These therapies are not cheap, but most of the diseases will have very small numbers eligible for treatment, usually fewer than 10, some just one or two. **Despite high individual cost, the total cost to our health system would not be large.**

Our groups have persuaded the company producing most of these enzyme therapies to make an offer to government and Pharmac that would provide significant discounts and cap the total financial risk. We understand that an offer has been put on the table but to date Pharmac have effectively rejected the offer.

The Solution: There is a need to address the significant gap in the policy framework that means medicines for very rare "orphan" diseases are significantly disadvantaged in the funding assessment process, because of the complexity of the diseases, limited disease knowledge, limited clinical trial data, very small numbers making unit costs very high, and so on.

Our medicine system needs to provide a counter-balance to that inherent disadvantage through additional considerations for funding of orphan drugs as provided in Australia and elsewhere.

We call on the government to ensure there is a system in place for equitable access to medicines for orphan diseases. Such a policy direction must come from government to guide the way Pharmac assesses these medicines and would allow a fairer deal for patients with these serious diseases.

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