BACKGROUND

An Enzyme Replacement Therapy (ERT) for Pompe disease developed by the Genzyme Corporation, and several others are currently under development.

For the commercial ERT, approval has been given by many nations and is reimbursed by health providers within those nations.

However, we are aware that some nations have not approved this therapy and obstacles to reimbursement exist even within nations that have approved the therapy.

EXPERIENCE

In 2006 the Enzyme Replacement Therapy (ERT) for Pompe Disease, developed by the Genzyme Corporation, was commercialised and was quickly approved by the FDA and the EMEA. It has since been reimbursed in many European countries, in the USA and other countries worldwide, including those with less developed health systems.

Where therapy has not been approved or reimbursed the IPA has had many requests from patients or their national patient groups to support their demands for access to therapy. The IPA has willingly added its voice to these individual campaigns but with mixed success.

At the time of writing (April 2012) we are aware of many wealthy nations, or provinces within nations, that still refuse to reimburse therapy for all who require it; in particular:

- Australia, New Zealand, Scotland, Wales, Canada

Conversely there are less developed nations that do appreciate the benefit of therapy to patients, those known to the IPA include:

- Algeria, Czech Republic, Greece, Hungary, Malaysia, Poland, Slovenia

IPA POSITION STATEMENTS

Orphan Drug Reimbursement

Where a nation or a union of nations has passed an Orphan Drug Act (e.g. USA and EU) it is logical that drugs approved through that process should be considered favourably for reimbursement by health providers within those nations. It makes no sense for governments to recognise the disadvantages to orphan diseases by promoting and supporting research and drug development, only for the same governments, or their health providers, to refuse to reimburse the costs on economic grounds alone.

With Orphan diseases it is not appropriate to apply the same cost-effectiveness criteria that are used for therapies for more common diseases, the small patient numbers inevitably lead to high drug costs due to the rigor required to prove its efficacy and safely and the proportionately higher production, distribution and training costs.

The IPA also encourages pharmaceutical companies to have clear and transparent policies for pricing of their orphan drugs. It is accepted that the unit price for such drugs will be very high at its commercial launch; there are considerable research, development, production...
and distribution costs to recoup within the first few years. But high drug prices put great strain on health provider’s budgets for rare diseases, and prices should be lowered once the drug is established; this is necessary not only to improve naïve patient access to that specific therapy, but also to allow therapies for other rare diseases to be accommodated within limited budgets.

**Benefit of therapy**

Evidence of the medical benefits of ERT for Pompe disease is continually growing both through published case studies and anecdotal evidence. The majority of patients experience a stabilisation of the disease; without therapy the symptoms would progressively worsen, leading to increased disability and inevitably dependence on wheelchair use and ventilatory support. This leads to a greatly reduced quality of life and shortened life-expectancy. The natural course of the disease impacts on the patient, the family and all levels of social interaction, it leads to greater dependency on the state benefit system and prevents the patient and carers from meeting their aspirations in employment and within their family and other social undertakings.

The IPA agrees with the view of most treating physicians that stabilisation of Pompe disease is a good indicator of a successful therapy; preventing further progression of symptoms has an enormous psychological benefit and allows patients to plan for the future and, in many cases, return to activities previously abandoned due to Pompe disease. The IPA is aware of many cases where patients receiving therapy have returned to their original employment. Some have rediscovered skills they had lost to the disease. It is clearly unfair to deny patients this chance of stabilisation.

It is the IPA view that patients should be recognised as full members of society and should not be denied access to approved therapies because of the rarity of their condition. The IPA considers that the current and future contributions to society of patients and carers should outweigh the cost of therapy over the patient’s lifetime.

**Lifetime cost of therapy**

The IPA believes that when health providers consider the lifetime cost of therapy they should not assume that the costs will remain high in the long-term. It is the IPA’s position that the current high costs of therapy should be considered as a medium-term situation and there will be a number of factors influencing the future cost of therapies that should be taken into account for the longer term:

- New therapies are being developed and it is highly likely that there will be competing ERTs by 2015. These therapies promise to be more effective and may allow the administration of reduced doses for some patients.

- There are currently research initiative showing promise for Gene Therapies and it is expected that commercial therapies could be in place before 2020.

- The number of diagnosed Pompe patients is continually increasing such that there is room for competing therapies and the development and production costs will become
proportionately reduced after market exclusivity agreements have expired (2013 in the USA, 2016 in the EU).

- The IPA recognises that the geographic distribution of patients across a nation will be uneven. Where the health provision is funded locally this can lead to high financial burden for one health provider compared to others. This has led to many cases of patients being denied access to therapy. The IPA advocates the adoption of risk-sharing strategies between health providers to apportion the costs of therapy across many local health providers. Such strategies should incorporate clear guidelines and protocols to ensure that the decision to reimburse therapy is made in favour of the patient.

**ADDITIONAL CONSIDERATIONS**

The IPA encourages health providers to carefully consider their charging strategies for drug delivery and pharmacy services; excessive charges for these services may jeopardise future reimbursement of this and future therapies.