Friday 10 March 2017

To Kelly.Traynor@parliament.govt.nz
Deputy Clerk of Health Select Committee

Please accept this letter following the committee's request for a written submission on the matters raised in my petition. Please also let it be noted that I wish to appear before the committee with my supporting advocates, to discuss these important matters and elaborate on the complex issues which are summarised here.

To the Chair and members of the Health Select Committee.

Re: Petition 2014/98 requesting:
That the House of Representatives note that 747 people have signed a petition requesting that the house ensure Pharmac has sufficient resources to deliver on its rare disease policy, and provide urgent access to the eight treatments it signalled when it set up this policy over two and a half years ago.

My name is Samantha Emily Lenik, I am a wife to Stephen Lenik, a Commander in the Royal New Zealand Navy, a mother to Kaelin and Sienna, and I am self-employed. When I was diagnosed in January 2014 with Pompe my world was turned upside down. My roles as wife, mother and business woman, were all put in jeopardy. I was filled with an over-arching fear that I would not be able to fulfil these role, that my life would be severely impacted by my declining health and ultimately by the shortening of my life. What added to my fear and frustration was hearing that there was an enzyme replacement therapy, (ERT), which slows down the progression of the disease, stabilizing the condition, and ultimately giving me more time with my family, but this proven treatment was not funded here in New Zealand!

Pompe disease, also known as Glycogen Storage Disease Type II or Acid Maltase Deficiency, is a very rare, progressive and fatal disorder which damages muscle and nerve cells throughout the body. It is caused by an accumulation of glycogen in the muscles due to deficiency of the enzyme "acid alpha-glucosidase". The build-up of glycogen causes progressive muscle weakness throughout the body and affects various body tissues, particularly, skeletal muscles, diaphragm, nervous system and the heart.

Pompe disease is relentless; it causes untreated patients to waste away in front of loved ones. As the disease progresses patients need more and more support from walking aids, wheelchairs, breathing machines for respiratory support, and eventually nursing home care, even at a young age. A patient's final years are spent in long term palliative care, attached to a mechanical ventilation machine to help them breathe. Pompe disease currently affects 10 New Zealanders.

A disease is considered rare when it affects few people. Despite the rarity of each rare disease taken individually, together they may affect up to 8% of the population. When immediate family and friends are factored in, rare diseases may impact nearly 25% of the population of any country. Rare diseases affect people all over the world, and are a true global health issue. In developing countries the problems are compounded by poverty, scarcity of medical expertise and other resource limitations. But New Zealand is not a developing country, so what limitations do we have that stops us from funding the lifesaving enzyme replacement therapy? Our main limitation is money, Pharmac is not given enough money by the Government to be able to do its job properly, and this means that Pharmac's approval processes for rare disease treatments makes it impossible to fund these treatments. This is highlighted by a quote from Sarah Fitt, Pharmac Operations Manager, in Pharmacy Today where she responds simply by saying, in effect, "we don't have enough money".

In 2006 Sanofi/Genzyme's enzyme replacement therapy, Myozyme, was given US Food and Drug Administration (FDA) approval. Since then 76 countries have evaluated the study data, weighed up the cost and social aspects, and approved funding for Myozyme for Pompe patients in those countries. What factors for consideration do these countries take into account that we do not? They do not limit the cost of the drug to the patient population, they factor into their cost benefit analysis framework additional human rights benefits for medicines which are life-saving orphan drugs.

Rare disorder studies are always challenging, as obtaining large enough numbers to be statistically robust is the consistent challenge. However this should not deny patients effective treatment options when there is positive meta-analyses showing positive primary outcomes. One such meta-analyses has been presented to the Pharmacology and Therapeutics Advisory Committee, (PTAC), and I won't go into it here, but the data demonstrates that alglucosidase alfa has a beneficial effect in LOPD patients. This is confirmed by improvements in survival and ambulation maintained over time, as well as prevention of deterioration in respiratory function. Yet when evaluated by PTAC these findings were dismissed as being weak. The Committee considered the clinical benefits with regards to ambulation and pulmonary function as modest! How is stabilization in mobility and an increase in FVC, (Forced Vital Capacity – respiratory function), modest?! The former greatly helps in quality of life and the latter helps in longevity of life!

The underlying reason as to why these studies are always rejected is because no matter how good the data, the cost is always going to be against rare disease patients because we are small in number. This is demonstrated by the following comments from PTAC; "The Committee considered that despite the significant price reduction included in the Proposal, the treatment remains extremely expensive and poorly cost effective." It will never be cost effective when you are comparing a drug for a small community, versus a drug with similar clinical data results, but that benefits a larger population group. But does that mean the smaller population groups can be so easily dismissed by Pharmac?

Our petition was designed to raise awareness about the lack of funding of rare disease treatments by Pharmac, and the process that these treatments have to go through to get funded. Since our petition was first launched last year, there have been some significant developments in a number of areas:

1 - Regarding the special fund for rare diseases

- A supporting online petition asking for the same outcomes was signed by 2,695 people.
- The RFP launched by Pharmac in 2014 was closed off in December 2016, with a review initiated.
- We estimate that of more than one hundred patients in need of the eight treatments provisionally indicated in the RFP as likely candidates for consideration under the RFP, fewer than five of those patients now have funded access to therapies that were not funded by any other means prior to the RFP.
- Several of the new schedule listings for these eight treatments, and others approved under the RFP, relate to treatments that were already funded for selected patients under the exceptional circumstances scheme (NPPA), thus representing a transfer between budgets, rather than new medicine access approvals.

2 – Regarding the disease I have

- Four of the ten New Zealand adult Pompe patients, including me, gained access to a clinical trial for a new enzyme replacement therapy (ERT) for Pompe disease late last year, run by Amicus Therapeutics. This involves travelling to Adelaide every two weeks for a period of 24 months for infusion of the drug, which is still an experimental treatment, not a proven therapy.
- In November 2016 Pharmac recognised the value of Myozyme for infantile-onset Pompe disease and listed it on the Pharmaceutical schedule, but discriminated against older patients by doing so.
- An application by Sanofi Genzyme for the listing of Myozyme for adult-onset patients on the pharmaceutical schedule has progressed into Pharmac's system and was considered by Pharmac's advisory committee (PTAC) with a recommendation that funding be declined.
- The PTAC minutes give no indication that factors other than benefit and relative cost have been considered in any detail. This is despite the rare disease policy of Pharmac and its $5 Million fund, and Pharmac's new "factors for consideration" in decision-making, which suggested that the inherent disadvantage of rarity, and broader ethical and social factors, should have influence in decisions.
- Sanofi Genzyme have granted compassionate access to Myozyme from March 2017 to four New Zealand Pompe patients.
- The interim solutions for eight Pompe patients does not solve the significant problems that remain with decision-making about funding treatments for adults with this disease, despite it being funded in 76 countries, and New Zealand being just one of three OECD countries that do not.

3 – Regarding Pharmac's decision-making

- It is apparent that a variety of reviews and policy changes have had very little impact on access to specialised medicines for rare diseases. The legislative brief that Pharmac has, is interpreted by them very narrowly in terms of health outcomes and budget management.
- Pharmac has been very reluctant to give specific consideration to issues which are widely accepted, in many comparable jurisdictions, as important factors in policy and decisions regarding rare diseases. These include the human right to health, equity in access to healthcare, reduction of disparities between population groups, and a sense of social responsibility.
- Please note that these criteria regarding rights and ethics are requirements across all other parts of our health system, yet Pharmac seems free to act without a moral compass. Why?
- We believe it is time to change the legislation governing Pharmac to include these wider considerations that are vital to achieving fairness for those with rare diseases.
- We cannot accept that Parliaments intentions in setting up the valuable and important cost saving aspects of Pharmac, was also to leave certain vulnerable groups to be marginalised, excluded and abandoned to die by narrow pharmacoeconomic analysis in budget management.

Please recommend to the House that the legislation is changed to ensure:

- The disadvantage of rarity is balanced with specific counterbalancing policy across all health services, including medicine access, for rare diseases.
- Decisions regarding funding of treatments for rare diseases should be taken elsewhere than in Pharmac.
- A rare disease policy and action plan is established for New Zealand.
- The policy and action plan should include an adequate ring-fenced budget and a broader set of decision criteria for medicines for rare diseases.

The needs of rare disease populations have to be addressed to ensure universal application of the principles of justice and access to health, and contribute to reduce health disparities between populations. Health care and treatment for rare diseases is a human rights issue and right now New Zealand is woefully behind other OECD countries in addressing the needs of its rare disease communities.

I hope you will take the time to consider the points I have raised, and also give me the opportunity to appear before the committee with my supporting advocates, to discuss these important matters and elaborate on the complex issues which are summarised here.

Yours sincerely,

Samantha Lenik