

Hello everyone,

See below for the message our groups sent to the Prime Minister last week and the reply late Friday afternoon.

Essential messages are:

- The National Party pledged 3 years ago to fix this problem of access to highly specialised medicines.
- Multiple reviews have not solved the problem.
- Pharmac still does not have suitable high level policy directions that enable it to deal with such situations.
- Very rare orphan diseases can't compete with cost-effectiveness comparisons against medicines for common disorders.
- Equity and fairness need to be explicitly considered.
- Problems are highlighted by the case of a handful of adults with Pompe disease. With treatment they can get improvements to health and a halt to the decline of a degenerative and eventually fatal disease. Without it they will die.
- Patients in 45 other countries are treated. Why not in NZ?
- Most developed countries now have orphan drug access policies.
- The solution has to be politically driven. That is how it has been dealt with in most countries.
- It is not acceptable that these decisions are left to officials who have neither the policy framework nor the expertise to deal with them.

We have gone back to the Prime Minister's office to ask again for a meeting and are waiting on a response. His office may have overlooked the point that the legislation prevents the Health Minister from getting involved in funding decisions. We have nowhere else to go. The responsibility must rest with John Key to resolve this issue.

Feel free to use this information as the basis for approaches you make to your own media contacts, while adding your own experience and perspectives of course.

Regards, john

John Forman  
Executive Director, NZORD  
New Zealand Organisation for Rare Disorders

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**From:** J Key (MIN) [<mailto:J.Key@ministers.govt.nz>]  
**Sent:** Friday, 14 October 2011 5:14 p.m.  
**To:** John Forman  
**Subject:** RE: An urgent matter needing you to take charge of and resolve

Dear Mr Forman

On behalf of the Prime Minister, Rt Hon John Key, I acknowledge your email of 11 October 2011 concerning access to highly specialised medicines.

Please be assured your comments have been noted.

As the issue you have raised falls within the portfolio responsibility of the Minister of Health, Hon Tony Ryall, your email has been forwarded to his office for consideration.

Thank you for writing.

Regards

Megan Walls  
Correspondence Assistant

**From:** John Forman [<mailto:exec.director@nzord.org.nz>]  
**Sent:** Tuesday, 11 October 2011 12:47 pm  
**To:** J Key (MIN)  
**Subject:** An urgent matter needing you to take charge of and resolve

Dear Mr Key,

I am writing to you with a request that you personally address an issue which our organisations believe only you can resolve.

In your party's 2008 election manifesto National pledged itself to finding a solution to the problem of access to highly specialised medicines. This was an issue that had dragged on for many years prior to 2008 and we welcomed that promise. Since then we have been working with officials, Ministers and other stakeholders to try and get a solution to the problem of access to highly specialised medicines for New Zealand patients.

Despite numerous workshops and reviews during your first term of government, the problem is not yet solved. The most recent review by Pharmac of its Exceptional Circumstances scheme did not fulfil the expressed request of your Ministers, Tony Ryall and Peter Dunne, that the review should deal with it.

Now matters have come to a head. Four patients with life-threatening Pompe disease have been denied access to the treatment (Myozyme) which is proven to improve health status and halt the decline of this serious degenerative disease that robs them of muscle strength and respiratory function.

Pharmac either can't or won't solve the problem. They have prevaricated over this issue for years now, including in the past year where the evidence of benefit has increasingly firmed up. The Minister of Health is prevented by legislation from getting involved in specific funding decisions.

The responsibility to solve this problem must rest with you. These patients are facing a slow death that is now preventable. They must not be abandoned by our government and its health system.

We call upon you to personally take responsibility for demonstrating that your party and your government believe that patients with life-threatening diseases should be given a fair go, and get access to the treatment that is now available.

Patients in 45 other countries are getting treatment. Why not New Zealand patients? Relying solely on the relative costs per quality-adjusted life-year while excluding other considerations should not be the determinant of whether they are treated or not. Nowhere else in New Zealand's public health system are interventions – even those associated with very high costs – subject to this level of scrutiny before patients are allowed to benefit from them. New Zealanders expect a fair go for all, and accept that extraordinary measures will be taken in extraordinary situations.

In all other countries where highly specialised medicines are funded, it appears that political decisions have been taken at the highest level to ensure those with the most serious and urgent need are treated, despite problems relating to cost-effectiveness. The same level of intervention and direction is clearly needed here.

Our request is that you intervene to break this log-jamb, ensure that these patients with Pompe disease are not abandoned and that they do get treatment promptly. We ask that you do this as the first step in actually delivering an access programme for highly-specialised medicines to treat rare "orphan" diseases.

We and the patients we represent anxiously wait your response, and while we appreciate your busy schedule we are very keen to meet with you to discuss this urgent matter.

Yours sincerely,

On behalf of:

The Muscular Dystrophy Association of NZ,  
Lysosomal Diseases New Zealand,  
The NZ Organisation for Rare Disorders.