

From: John Forman
Sent: Monday, 7 November 2011 12:08 p.m.
To: 'j.key@ministers.govt.nz'
Subject: Request for you or a representative of the National party to meet our delegation at Parliament on Wednesday 16 November

Dear John Key,

You have received previous messages from us about the lack of funding of a specialised medicine for Pompe disease, which is the leading current example of the need for an orphan drugs access policy to give fair access to treatment for those with rare diseases.

The Labour government's medicines strategy could have/should have dealt with this but was not implemented. The National party pledged in their 2008 manifesto to get action to sort out access to highly specialised medicines, but it has all come to nothing. The McCormack panel's report and Pharmac's review of exceptional circumstances have not dealt with the problem, with all recent applications for treatment of Pompe disease declined by Pharmac. They do not have a policy framework that can provide equitable access to specialised drugs such as Myozyme for Pompe disease.

We are continuing our campaign with a march from Civic Square to Parliament on Wednesday 16 November, arriving about 1pm at the Seddon statue in Parliament grounds. The four adult patients with Pompe disease will be in wheelchairs, accompanied by a number of supporters.

We are inviting all political parties to be at Parliament to meet our group and our supporters and all will be invited to make a brief announcement (about 5 minutes) of their party's policy in relation to treatment of diseases like Pompe disease that need highly specialised treatments. We hope that the National party will be prepared to reaffirm its earlier pledge to solve this issue. We want you to follow that through by delivering a robust orphan drugs access programme that will ensure treatment for our Pompe patients and have enough teeth to fix this problem into the future for other rare diseases.

We are very happy to discuss this further if there is any more information you require, and we look forward to getting confirmation of your availability, or a representative of the National Party who can speak on your behalf. We have confirmed attendance, or messages to be delivered to our delegation on their behalf, by four political parties so far. Three others have yet to respond to our invitation.

A copy of our earlier request to you is copied below for your information. You referred us to Tony Ryall. However as Health Minister he is excluded from getting involved in such decisions, as you will no doubt recall from the Herceptin case. We consider this a political party manifesto issue and we seek from you the same commitment we are asking of all political parties. We look forward to your response.

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

John Forman
Executive Director, NZORD

From: John Forman
Sent: Tuesday, 11 October 2011 12:47 p.m.
To: 'j.key@ministers.govt.nz'
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 fulfill 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.