

Press release – for immediate release. Monday 7 November 2011.

Rare disease patients to march on Parliament - We want a fair go

Patients with life-threatening Pompe disease will march on Parliament next week to call for a commitment from all political parties to treatment of their disease, and to put in place an orphan drugs access programme to ensure a fair go is extended to all other patients with rare diseases when new treatments are developed in the future.

Five patients with the adult-onset form of Pompe disease have had their applications to Pharmac declined and an appeal rejected. One patient has gained access to a clinical trial in the United States for a second-generation treatment for Pompe disease. The remaining four could have the course of their degenerative and fatal disease improved then stabilised by treatment with the enzyme replacement therapy Myozyme, but funding of the treatment has been denied.

Treatment costs are expected to average about \$300,000 to \$500,000 per patient per year, but with such small numbers the total cost is not a major imposition on our health system, and far less than amounts committed to many other things inside and outside of our health system.

The patients and their supporters will assemble in Civic Square in Wellington at noon on Wednesday 16 November and march to Parliament grounds where they will be addressed by political parties, some of which have already agreed to state their policy publicly. Tariana Turia will speak for the Maori party, Peter Dunne for United Future and Steve Chadwick for Labour. Policy messages will also be delivered from the Green party and NZ First. Responses to the groups' invitation to address their rally are still awaited from ACT, Mana and National parties.

The patients are being supported by the Muscular Dystrophy Association, Lysosomal Diseases NZ, and the NZ Organisation for Rare Disorders. NZORD Executive Director John Forman say the groups have been working through the system talking with Pharmac, government officials, Ministers and politicians for many years to work out a system for access to specialised medicines, but all avenues have proved fruitless.

"Past governments have proposed solutions. The Labour government's medicines strategy could have solved the problem but this part of the strategy was never implemented. The National party's 2008 manifesto pledged a solution for access to highly specialised medicines, but the resulting reviews during their first term led to no change to Pharmac's approach.

"At a political forum earlier this year all the major political parties acknowledged the need for a solution to this problem, with some noting the weakness of the policy framework which means Pharmac continue to assess these highly specialised medicines against medicines for common disease," Forman says. "That means the applications are often doomed right from the start, as Pharmac puts undue emphasis on cost-effectiveness comparisons without taking into account fairness and equity provisions in the medicine strategy and health legislation."

The groups want a political solution to be pledged by all political parties prior to the election so there can be some certainty about policy on access to medicines for rare diseases in the future. Copies of messages to the Prime Minister are copied below as background to this release.

For more information contact John Forman 027 240 3377

On behalf of:

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