

Press release 28 February 2017 (Rare Disease Day).

Pharma company gives up on Pharmac - Gives drug free to NZ patients.

Sanofi Genzyme developed Myozyme, a complex biological treatment for Pompe disease, a Lysosomal disease, over ten years ago. Since that time funded treatment of affected patients has spread to 76 countries. Over that decade, NZ's drug purchasing agency Pharmac has consistently refused to fund it, leaving the 10 patients here abandoned and facing relentless decline in their health and quality of life.

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After substantial renewed efforts to negotiate with Pharmac, and with a recent recommendation from Pharmac's PTAC committee, released early February, that funding should be declined, the company has decided to provide the treatment free to 4 NZ patients.

Chair of Lysosomal Disease New Zealand, John Forman, has thanked Sanofi Genzyme for its generous contribution to the health and wellbeing of these four patients. "It is very nice to see a Pharma company that is prepared to make such a significant contribution without any reimbursement. These companies often get bad press, so this generous act should be noted and the company congratulated and thanked".

Pompe disease was the subject of a petition and campaign by LDNZ late last year. Subsequently 4 patients were accepted onto a clinical trial of a new Pompe treatment in Adelaide, and one patient died in late December, leaving 4 in urgent need of treatment.

"Sanofi Genzyme had been working on a new application to Pharmac for over a year," says Forman. "We had great hopes that a new Pharmac policy relating to drugs for rare diseases, and a new set of factors for consideration in their decision making, might lead to some real change in Pharmac's response to this application. We looked for signs that the inherent disadvantage of rarity in terms of data, evidence and costs, would lead to a different response from Pharmac. We expected Pharmac to consider equity and the reduction of disparities, which are principles that underpin our health system, and we wanted to see active consideration of community values of fairness and a helping hand for those most vulnerable. But the PTAC minutes show scant consideration of anything but evidence and costs."

LDNZ is frustrated that once again, treatments for very rare diseases are given a direct comparison in cost-effectiveness with treatments for common diseases, leading to almost automatic failure for the Myozyme application. Forman says it is particularly frustrating given PTAC's acknowledgement of a significant price reduction offered by Sanofi Genzyme.

The 4 patients' treatment is expected to begin within weeks. They are no longer abandoned to die by our health system, in contravention of the right to health.

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