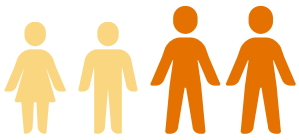
Briefing

Rare Disorders and Medicine Access Aotearoa 2024

An estimated 300,000 New Zealanders live with at least one of over 7,000 known rare disorders. A disorder is considered rare when it affects fewer than or equal to 1 in 2,000 persons in New Zealand. Often complex, debilitating and life threatening, these conditions require a broad range of services.



Half of those affected are children

7,000 +

Known rare disorders

72% of rare disorders are genetic in origin

International studies show that the health care costs of patients with rare disorders can be three to five times higher than for patients without a rare disorder, and the economic burden of productivity-loss and non-medical costs is felt by both affected households and the state.

While less than 10% of rare disorders have an approved treatment available, treatments that are available can make a life-changing - and saving - difference to those who need it.

Rare medicines in New Zealand

New Zealand's investment in rare disorder medicines is extremely poor. We rank 32 out of 32 OECD countries for access to modern medicines.

More than half of the novel drugs approved by the FDA in 2023 were for rare disorders.

< 1%

Less than 1% of Pharmac's pharmaceutical budget in 19/20 was spent on rare disorder medicines.

Between 2011 - 2020 only 11% of the modern rare disorder medicines launched in the OECD over the decade were publicly funded in NZ.

Currently, OECD countries invest on average 1.4% of GDP on medicines. New Zealand invests only 0.4% of GDP.

New Zealand urgently needs to ameliorate it's track record on medicine access, particularly for people living with a rare disorder, to improve health equity and reduce avoidable demand and costs on the health system.

$1.49B

Pharmac's current pharmaceutical budget (equivalent to 0.4% of GDP).

$490M

The amount Pharmac needs per annum to clear the Options for Investment waitlist of medicines.

$4B

The amount of additional new funding needed to meet the OECD average investment in medicines.

New Zealand's first Rare Disorders Strategy

In 2022, the Minister of Health instructed Manatū Hauora to develop New Zealand's first Rare Disorders Strategy. The intention of the strategy is that it will lead to better, more timely services and more equitable support and outcomes for people and whānau with rare disorders.

Rare Disorders NZ, as the only umbrella organisation supporting all New Zealanders who live with a rare disorder, has been involved both as a member of the RDS reference group and as a co-designing partner. We have been advised by the Minister that the final Strategy will be launched in July 2024.

Delivering on the Strategy

For the Rare Disorders Strategy to deliver on it's intentions, access to rare disorder medicines needs to be improved. The current one-size-fits-all model under Pharmac's pharmaceutical schedule does not work for low volume, high-cost medicines, and there are too many inconsistencies under the only other avenue – the exceptional circumstances framework.

Following consultation with domestic and international experts, Rare Disorders NZ recommends that a **single, barrier-free pathway to rare disorder medicines** be established by Pharmac. An assessment pathway for rare medicines with criteria separate to common conditions (as is implemented in Europe, Australia and Japan) will ensure equitable access to medicines for those with rare disorders.

Implement a separate assessment pathway for rare disorder medicines.

$490M

Insist the Government urgently prioritise increasing Pharmac's medicines budget per annum to clear the Options for Investment waitlist.

Implement a strategy to reach the OECD average within the next 5 years.