Re: Proposal to change Pharmac’s policy principles for funding rare disorder medicines

To: Pharmac’s Board (or its delegate), consult@pharmac.govt.nz
Date of Submission: 21 August 2025
Submitted by: Rare Disorders NZ
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Declaration of interest

Rare Disorders NZ works with clinicians, researchers, allied health professionals, academia, government and industry to achieve better outcomes for people with a rare disorder in New Zealand and their whānau. We are funded by grants, donations, fundraising events, Pharma roundtable and a small Te Whatu Ora contract. This submission is in response to the proposal to change Pharmac’s policy principles for funding rare disorder medicines.

Rare Disorders NZ

Rare Disorders New Zealand is the respected voice of rare disorders in Aotearoa. We are the national peak body organisation, supporting the 300 000 New Zealanders with rare disorders and the people who care for them. We help those affected by rare disorders navigate the healthcare system, find information and resources, and connect with support groups specific to their condition.

We proudly advocate for public health policy and a future healthcare system that works for those with rare disorders – using a strong and unified voice to collaborate with Government, clinicians, researchers, and industry experts, to promote diagnosis, treatment, services, and research.

Our vision is for New Zealand to become a country where people living with a rare disorder are fully recognised and supported with equitable access to health and social care.

A rare disorder is a medical condition with a specific pattern of clinical signs, symptoms and findings that affects fewer than or equal to 1 in 2,000 people in Aotearoa New Zealand. Rare disorders include, but are not limited to, rare conditions among genetic disorders, cancers, infectious disorders, poisonings, immune-related disorders, idiopathic disorders and various other rare undetermined conditions. An ultra-rare disorder is a medical condition with a specific pattern of clinical signs, symptoms and findings that affects fewer than or equal to 1 in 50,000 people in Aotearoa New Zealand.

Submission

Rare Disorders NZ supports changing Pharmac’s definition of a rare disorder from 1 in 50,000 to 1 in 2,000. This will bring Pharmac’s definition in line with the Rare Disorders Strategy published by the Ministry of Health in 2024, as well as international definitions.

The proposed change brings all rare disorder medicine applications under the process already in place for what we define as ultra-rare disorder medicines, giving Pharmac a mechanism to consider a funding application before an application is lodged with Medsafe if other criteria are met. We hope this proposed change will encourage more applications for rare disorder medicines as it removes a key barrier that prevents applications currently being made.

For consistency and clarity, Rare Disorders NZ recommends adopting the definition of a rare disorder in the Rare Disorder Strategy which is “*a medical condition with a specific pattern of clinical signs, symptoms and findings that affects fewer than or equal to 1 in 2,000 people in Aotearoa New Zealand*”. This is preferred over the currently proposed definition of *“a clinically defined disorder that affects an identifiable and measurable patient population of less than 1:2,000”.’*

Rare Disorders NZ is concerned that the terms "identifiable" and "measurable" in the proposed definition may introduce an impractical level of specificity or set unrealistic expectations for evidence, given the current limitations in rare disorder data in New Zealand. This could result in very few applications meeting the criteria. The Ministry of Health’s Context and Evidence document[i], which accompanied the Rare Disorders Strategy, states: “We lack specific information on how many people are living with rare disorders and which disorders are most prevalent in Aotearoa New Zealand. Very little information on rare disorders is easy to retrieve from health or statistical data.” Rare Disorders NZ continues to advocate for health, disability, education and other government entities to capture relevant data on rare disorders.

We note that Pharmac is working through options for how to operationalise the proposed definition change, which may include holding more Rare Disorders Advisory Committee meetings or involving other expert committees to support this work. Rare Disorders NZ looks forward to being part of these discussions.

It is important to recognise that an increase in rare disorder medicine applications will require not only additional clinical advice capacity but also enhanced resourcing and more tailored approaches in all phases of Pharmac’s application and funding processes.

We are pleased that Pharmac has acknowledged in this consultation that “it is harder for a rare disorders medicine to get funded.” While the proposed definition change is a positive step that may help more rare disorder medicines enter the assessment process, it does little to address the existing bottleneck which has many rare disorder medicines sitting on the Options for Investment list, awaiting funding. Likewise, it does not address that the research and economic analysis ‘assessment’ phase is not designed for low volume, high-cost medicines for rare disorders.

Looking ahead, we hope to see the development of a dedicated assessment pathway for rare disorder medicines. This would help ensure that innovative treatments with demonstrated clinical benefit can be assessed and funded in a way that is consistent with international best practice.

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[i] Ministry of Health. 2024. Te Rautaki o Aotearoa e Pā ana ki ngā Mate Mokorea: Te horopaki me ngā taunakitanga - Aotearoa New Zealand Rare Disorders Strategy: Context and evidence. Wellington: Ministry of Health.