Re: Proposal to decline some medicine funding applications on the Options for Investment list

To: Pharmac applicationfeedback@pharmac.govt.nz
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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 decline some medicine funding applications on the Options for Investment list

Rare Disorders NZ

Rare Disorders New Zealand (RDNZ) 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 living with rare disorders to navigate the healthcare system, find information and resources, and connect with support groups specific to their condition.

Our vision is for New Zealand/Aotearoa to become a country where people and whānau living with a rare disorder experience best possible health and wellbeing. We seek to achieve this by enabling and empowering people with rare disorders to best benefit from services and therapies available in New Zealand, and by championing their collective voice, advocating for provision of world leading evidence based health, disability, education and other services.

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

RDNZ strongly opposes Pharmac's proposal to decline some medicine funding applications on the Options for Investment (OFI) list. The current lack of transparency around the ranking of medicines on the OFI list is unacceptable and must be addressed. However, RDNZ strongly disagrees that removing the bottom 10 to 20 percent of the lowest ranked medicines from the OFI list is the right way to achieve the stated goal of creating greater transparency and clarity to how medicine funding applications are managed on the OFI.

People living with a rare disorder deserve transparency and honesty about the likelihood of medicines that could save or significantly improve their lives being publicly funded. New Zealand's current position at the bottom of the OECD for access to modern medicines forces many to make impossible decisions, including whether to become "medical refugees" and move overseas to access treatment, or wait hoping that their medicine will be funded soon. These decisions would be a lot more informed if people and their clinicians knew where on the OFI list their medicine sat.

RDNZ proposes increased transparency is achieved through identifying the top 20% and bottom 20% of the OFI list or separating the list into tiers in some other way. If removing the bottom 20% of applications does not cause issues with commercial sensitivity and Pharmac's negotiation position, then we struggle to see how identifying this same group without removing them from the OFI list is any different.

RDNZ advocates for equitable access to modern rare disorder medicines through a dedicated assessment pathway. People with a rare disorder must have access to medicines with proven clinical benefit for their condition. Removing funding applications from the OFI list simply because they are ranked at the bottom due to being low-volume, high-cost treatments moves New Zealand further away from achieving equity for rare disorder patients.

The underlying issue is an inadequate medicines budget, not the length of the OFI list. Pharmac itself describes the OFI list as *all applications that we would fund if the budget allowed it*. Medicines only progress to this list after assessment by Pharmac's specialist advisory committees, whose expert clinical advice confirms that these treatments should be accessible to New Zealanders. Reducing the size of the list may create the illusion of progress but does not improve medicine access for New Zealanders. The OFI list should be used strategically as an evidence-based tool to demonstrate the scale of unmet need and need for increased investment in medicines.

RDNZ sees this proposal as out of sequence with Pharmac's reset programme. Broader and deeper reform is urgently needed, incorporating early and meaningful co-design

with consumers and patients. New Zealand should not have to maintain a waiting list for essential medicines. We urge Pharmac to prioritise meaningful reform of the health technology assessment process, clear the existing application backlog, and actively advocate for a significantly higher medicines budget to meet the needs of all New Zealanders, not just remove medicines from a long waiting list to hide the depth of unmet need.