Re: Review of the Exceptional Circumstances Framework Consultation

To: ecfreview@pharmac.govt.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 regard to the **Review of the Exceptional Circumstances Framework.**

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

Thank you for the opportunity to provide feedback on the Review of the Exceptional Circumstances Framework. Rare Disorders NZ sees five areas of change that are urgently needed in this framework.

1. **Introduction of a Specific Assessment Pathway for rare disorder medicines.** We recommend equitable access to modern rare disorder medicines is established through a specific rare disorder assessment pathway. We see this as an essential outcome of this review.

2. **Future proof the Framework**
   We recommend this review includes a future focus, with a subsequent review date and horizon scanning built in.

3. **Make the system more efficient for low volume medicines**
   We recommend changes are made to reduce administrative load for both clinicians and Pharmac, while increasing consistency and certainty for patients.

4. **Increase transparency and formalise discretion**
   We recommend Pharmac becomes more transparent about decisions on applications made through the Framework. The discretion currently applied within the Framework also needs to be formalised.

5. **Increase guidance and support for clinicians making applications**
   We recommend that Pharmac develop a comprehensive clinician guide, including worked examples of NPPA applications. Additional practical support pathways for clinicians also need to be developed.

Introduction of a Specific Assessment Pathway for rare disorder medicines

The Exceptional Circumstances Framework Review consultation document states that "*the Framework recognises that different funding pathways serve different purposes. The Schedule process supports access for population groups, while other pathways exist to respond to exceptional situations. Together, these processes are designed to work alongside each other so that people can achieve the best possible health outcomes*".

Most people living with a treatable rare disorder who are seeking access to life-saving or life changing medicines in New Zealand are not achieving the best possible health outcomes or receiving international standard of care treatments.

This gap in medicine access for people with rare disorders in New Zealand has been repetitively highlighted but the inequity remains.

- The 2022 Pharmac Review stated that "Even at the lower end, our estimates confirm there is significant unmet need [for people with a treatable rare disorder]".
- The 2025 Pharmac Consumer Engagement Workshop Report identified that there is an unaddressed equity issue in assessments for those with rare disorders, and that under the current HTA process, some treatments will never be funded. An action point in the review was to develop alternative pathways in line with the Rare Disorders Strategy.
- Rare Disorders NZ's 2025 Voice of Rare Disorders survey found that 39% of people surveyed believed they were missing out on some medicines because they were not funded in New Zealand, and 30% of those surveyed were self-funding their rare disorder medicines.
- Medicine New Zealand's 2025 AtoM 5 report identified 26 modern rare disease medicines that are publicly funded in Australia but not in New Zealand as of 30 June 2025.

The current Exceptional Circumstances Framework is not a pathway available to most people with a rare disorder trying to access lifesaving and life-changing low-volume, high-cost medicines. Even those with ultra-rare disorders are told they are not exceptional *enough* to meet the criteria.

The only current alternative, the Schedule process is not working for rare disorder medicine funding applications. Equity for people living with a rare disorder is not adequately considered in the assessment phase and high-cost low-volume treatments don't stand a chance.

This raises a fundamental ethical issue. People with rare disorders should not be disadvantaged simply because their condition is uncommon or the medicine they need is high-cost. Access to treatment should depend on need and clinical benefit. For many people with a rare disorder, the medicine they are waiting for is not a better or more convenient option than what they currently have. They are waiting for their only option to live without significant disability or ill health, or to live at all.

Rare Disorders NZ has long advocated for equitable access to modern rare disorder medicines through a specific rare disorder assessment pathway. It is essential this pathway prioritises equity and the right to the best possible health for people living with a rare disorder.

We ask that as part of this review Pharmac consider and analyse international funding approaches for rare disorder medicines and apply this to the New Zealand context. This work should inform the design of a specific pathway, with implementation progressed as part of this review or as an immediate next step, to ensure Pharmac and the wider health system can respond to the rapidly evolving pipeline of high-cost, low-volume medicines for rare disorders, including one-off curative therapies.

Future proof the Framework

This review needs to get things right for the future. It should include a commitment to a subsequent review date and ongoing horizon scanning, given the rapidly evolving medicines landscape and the growth of personalised and precision medicine.

An outstanding action of the 2022 Pharmac review that was not adequately addressed by the Rare Disorders Strategy was the need to “consider the challenge of funding medicines for rare disorders, taking into account the increasing scale of the problem and the impact that this will have on health services more generally”. This recommended action is wider than the Exceptional Circumstances Review, but the importance of its consideration in this work cannot be understated.

There is very little hope within the rare disorder community for the future of medicine access in New Zealand. The 2025 Voice of Rare Disorders survey found that, among those who had thought about it, almost all (96%) were either worried or very worried that promising future treatments and medicines (e.g. gene therapy/CRISPR) will not be funded by New Zealand’s public health system.

As highlighted in our first point, this review provides an opportunity for Pharmac to future-proof a pathway for access to modern rare disorder medicines, including high-cost one-off curative therapies that do not currently have a pathway or funding mechanism that is fit for purpose. We hope this opportunity is not wasted.

Make the system more efficient for low volume medicines

This review presents the opportunity to make practical changes to the Exceptional Circumstances Framework processes to make the system more efficient for low-volume low-cost or cost-neutral medicines and supplements, particularly where the medicine is already recognised as clinically necessary and does not require the same level of analysis as a high-cost and/or high-volume funding application. This is especially important for rare disorders, where medicines may be used by very small patient groups, supply can be tenuous, and stable ongoing treatment is essential to patients avoiding serious harm.

Rare Disorders NZ recommends that Pharmac create a streamlined pathway for low-volume medicines and supplements, especially cost-neutral products and like-for-like substitutions. Where a supplier or sponsor can demonstrate that an alternative is cost-neutral and clinically equivalent to an already funded product, there should be an expedited process that does not require the same level of assessment. This would allow Pharmac to focus its clinical and analytical resources where they are most needed, while improving access, continuity of care and choice for patients with rare disorders.

The Exceptional Circumstances Framework should have a process to allow greater flexibility for low-volume medicines, particularly where there is foreseeable supply issues or stock constraints. In these situations, listings should not be limited to a single brand or formulation where clinically appropriate alternatives exist. Instead, listings should be based on the generic medicine wherever possible and allow for multiple formulations, so that continuity of treatment is not compromised by procurement or availability issues.

We also see a need for a clearer process to move medicines that are repeatedly accessed through NPPA onto the Pharmaceutical Schedule without requiring a full assessment process. Where a medicine is already being accessed through NPPA for a small number of patients, there should be a simple streamlined pathway to transition that medicine onto the schedule for the relevant group. This would reduce administrative burden for clinicians and Pharmac, as well as improving certainty for patients and whānau.

Increase transparency and formalise discretion

Rare Disorders NZ recommends that transparency is increased around how decisions are made within the Exceptional Circumstances Framework, particularly in relation to NPPA applications. We would like to see clearer explanations of why applications are approved, declined, or do not meet principles.

At present, there is limited visibility on how it is determined whether an application meets the principles. There is also limited visibility on how the factors for consideration are applied to NPPA decisions compared with those used for medicines assessed through the Schedule pathway. It difficult to understand why some applications succeed while others do not, and whether consistent and equitable approaches are being applied across pathways.

We also ask that discretion is formalised and that the relationship between pathways is clarified. This is particularly important where a Schedule application is in progress and a NPPA application is then made for the same indication. Rare Disorders NZ has received mixed responses from Pharmac on whether a NPPA application can be approved in these circumstances, or only if the named patient is considered "exceptional" compared to the wider group covered by the Schedule application. This lack of clarity creates uncertainty for clinicians and patients.

We recommend that NPPA applications can proceed even when a Schedule application is being progressed. Without this, there is a clear disincentive for clinicians and suppliers to submit a Schedule application for a medicine that could otherwise be approved through NPPA. Patients are also affected. Those who might have received treatment through NPPA can be left without access while waiting for the lengthy Schedule process to conclude.

Rare Disorders NZ would like to see more clarity around what constitutes "exceptional" circumstances. While it is understood that there is no fixed numerical threshold, the absence of clearer guidance on how exceptionality is determined creates uncertainty for clinicians about whether to apply and presumably inconsistency in decision-making by Pharmac. Greater transparency and formalisation of what "exceptional" circumstances means would support more equitable and predictable outcomes, as well as increase clarity for clinicians considering applications.

Increase guidance and support for clinicians making applications

Rare Disorders NZ recommends increased guidance and practical support for clinicians and pharmacists engaging with the Exceptional Circumstances Framework.

Currently there are not clear pathways to support clinicians and pharmacists to obtain pricing, quotes, and secure supply of medicines when preparing NPPA applications. Greater support is needed to ensure clinicians can efficiently source medicines, understand supply options, and navigate procurement requirements. There needs to be a pathway for support managing procurement issues and supply constraints when they arise.

Rare Disorders NZ has long been asking for Pharmac to develop a comprehensive clinician guide for NPPA applications. We would like to see this guide developed as a requirement that comes out of this review. The guide needs to include worked examples of NPPA applications including clear explanations of how applications did or did not meet the principles and why applications were approved or declined. Variability in experience and knowledge across clinicians contributes to inequity in access, as some patients benefit from more informed or experienced applicants than others. A comprehensive guide will go some way to addressing this inequity while removing some of the administrative burden on clinicians.

Conclusion

This review is an opportunity to ensure the Exceptional Circumstances Framework works for those it is intended to support, including people living with rare and ultra-rare disorders.

People with rare disorders should not be disadvantaged because their condition is uncommon or the medicines they need are high-cost. For many people living with a rare disorder access to medicine through Pharmac's pathways is their only option to live without significant disability or ill-health, or to live at all.

Rare Disorders NZ asks for a more transparent, efficient, equitable, and future-proofed Exceptional Circumstances Framework that includes a specific assessment pathway for rare disorder medicines. We encourage Pharmac to use this review to make meaningful and enduring change, not tick a box.