Re: Healthy Futures (Pae Ora) Amendment Bill

To: Committee Secretariat, Health Committee
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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 Healthy Futures (Pae Ora) Amendment Bill.

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.

Introduction

Rare Disorders NZ supports, in principle, the intent of the Healthy Futures (Pae Ora) Amendment Bill to improve the effectiveness of health service delivery for all New Zealanders. We have identified areas in the existing Pae Ora (Healthy Futures) Act and the Bill where there is additional potential to make changes to fully achieve this intent. These areas are outlined below.

Make the Rare Disorders Strategy a legislated health strategy

The Rare Disorders Strategy was published after the Pae Ora (Healthy Futures) Act was enacted. There is now a clear opportunity to incorporate it into the legislation as a health strategy, as was done with the Mental Health and Wellbeing Strategy, which was added in 2024.

The Ministry of Health released the Rare Disorder Strategy[i] In 2024, during the current Government's term. The Rare Disorder Strategy made clear commitments to integrating rare disorders into health system planning. The omission of rare disorders from the current amendment Bill suggests that this commitment has not been upheld in its drafting. The Strategy states:

> *"In the future, decision-makers will consider rare disorders as they decide priorities for evolving and changing the system….People and their whānau living with rare disorders will become one of the population groups that decision-makers routinely think about when designing system improvements."*

Clauses 21–24 of the amendment Bill introduce mandatory targets into the Government Policy Statement (GPS) and require health strategies to align with them. The proposed GPS target areas are broad and risk not addressing the unique and complex needs of people with rare disorders unless this population is explicitly considered and planned for.

Without a Rare Disorders Strategy that is current and gives effect to the targets set in the GPS, people living with rare disorders will continue to fall through the cracks. Ultimately, without action, people living with rare disorders will make up the tail end of the statistics that fail to meet national health targets.

Legislating the Rare Disorders Strategy as a health strategy is the only way to genuinely demonstrate commitment to the estimated 300,000 New Zealanders living with a rare disorder. It would ensure they are consistently considered in future system changes, investment decisions, and performance monitoring, as is required by the Strategy itself.

Rare Disorders NZ recommends the Rare Disorders Strategy be incorporated into legislation as a health strategy.

Require health strategies to include action plans and accountability measures

Rare Disorders NZ has observed that many strategies, while well-intentioned, often don't drive meaningful change and become symbolic rather than actionable.

Strategies accompanied by action plans that include mechanisms for monitoring, evaluation, and public accountability will go a lot further in improving the effectiveness of health service delivery for all New Zealanders than strategies that exist only as high-level commitments or aspirational goals.

Rare Disorders NZ recommends amending the Act to require all health strategies are accompanied by action plans that outline how objectives will be implemented and measured.

Ensure equitable access to effective and appropriate health services

In the foreword to the 2024 Rare Disorders Strategy, Dr Diana Sarfati (then Director General of Health), acknowledged that "people and their whānau living with rare disorders often face barriers in getting timely, effective and equitable health care".

The proposed removal of the Health Sector Principles from the Act removes the focus on the health sector being equitable and ensuring that population groups have access to services in proportion to their health needs.

While Rare Disorders NZ supports retaining the Health Sector Principles, we believe that, at minimum, the Bill must explicitly commit to providing equitable access to effective and appropriate services, not just "quality services."

Clause 5 of the Bill amends section 3 to insert a new purpose *to ensure that patients get timely access to quality health services.*

This does not go far enough. For people with rare disorders, access to a quality service that is not appropriate or effective for their disorder may be ineffective or even harmful.

Rare Disorders NZ recommends the new purpose in section 3 be changed to: *to ensure that patients have timely, equitable access to effective, appropriate and quality health services.*

Retain mandatory requirements for expertise

Public health measures are particularly important for protecting people living with rare disorders. They are a population that are often medically vulnerable and require complex, coordinated, and specialised care. The health system must be equipped with robust, evidence-based, expert-led oversight to support their needs.

Rare Disorders NZ is concerned about the removal of specific requirements for collective knowledge, expertise and experience to be met when appointing board members and the expert advisory committee on public health. Amendments in clauses 12, 16, and 33 significantly broaden ministerial discretion over appointments, without safeguards to ensure balanced and qualified membership.

Eliminating the requirement for expertise in key areas such as population health, health equity, Te Tiriti o Waitangi, health protection, health promotion and epidemiology creates a real risk of gaps in knowledge and leadership. This change could dilute trust, reduce transparency, and politicise appointments.

Rare Disorders NZ is also concerned these proposed changes, along with the removal of the Health Sector Principles, do not support addressing structural inequities and ensuring culturally safe, informed care for all, particularly for Māori and for people living with rare disorders.

The latest Voice of Rare Disorders Survey shows a need for continued focus on cultural capacity within the health system. It found that while the majority of people felt culturally safe when visiting health services, 19% of Māori living with a rare disorder considered visits to a nurse, doctor, health service, hospital service or genetic testing service to be culturally unsafe.[ii]

Rare Disorders NZ recommends maintaining the current requirements for the board and expert advisory committee on public health appointments.

Amend Pharmac related legislation

Pharmac's statutory objectives

Rare Disorders NZ recommends that additional amendments be made to the Bill to change the objectives of Pharmac. These changes are necessary to align Pharmac's purpose and processes with the recommendations of the 2022 Pharmac Review, the Minister's Letter of Expectations 2024/25 and 2025/26, the Valuing Life Summit 2024 White Paper, and the 2025 Independent Consumer Engagement Report.

These changes are essential to support the cultural reset Pharmac is currently undertaking and to shift its focus away from a narrow emphasis on cost containment. A purely cost-based approach risks distorting decision-making and often leads to sub-optimal outcomes for patients, particularly in relation to rare disorder medicines. For people living with rare disorders, emerging therapies may be high cost but offer significant long-term savings through reduced pressure on the wider health and social systems and/or dramatically improve quality of life for both patients and carers.

To address these issues, Rare Disorders NZ recommends that Section 68 of the Act be amended as follows:

- Amending objective (a) to read "to secure for eligible people in need of pharmaceuticals, the best health outcomes that are reasonably achievable from pharmaceutical treatment" (thereby deleting "and from within the amount of funding provided")
- Including a new objective (b): "to take into account to take into account broader societal, economic, and non-health outcomes, including fiscal impacts to government, when making medicines and medical devices purchasing decision"
- Retaining the current objective (b) as objective (c)

Rare Disorders NZ recommends amendments to Pharmac's statutory objectives to ensure its decision making better reflects broader health, social, and economic outcomes and is aligned with recent reviews.

Consumer and patient consultation

Section 70 of the Act requires Pharmac to consult in implementing objectives and performing functions, and in performing its functions, Pharmac must, **when it considers it appropriate to do so**...consult on matters that relate to the management of pharmaceutical expenditure with any sections of the public, groups, or individuals that, **in the view of Pharmac**, may be affected by decisions on those matters;

Rare Disorders NZ believes that rather than requiring Pharmac to consult the net effect of Section 70 is to permit Pharmac not to consult when it should.

The 2025 Pharmac Consumer Engagement Workshop Report by Dame Kerry Prendergast found that "Because there is no existing framework for engagement, the way Pharmac currently engages with consumer/patient representatives is regarded as uneven, unequal and inconsistent. Pharmac's organisational culture is also a problem, seen as slow to respond, lacking respect for consumer/patient representatives, and highly defensive when called to account". This means "there is a gulf between Pharmac management and the consumer/patient representative community".

Many people living with rare disorders have been left feeling marginalised, antagonised and alienated by Pharmac over the past several years. Rare Disorders NZ recommend that Section 70 be deleted in favour of including patient consultation as one of Pharmac's functions in section 69, allowing less discretion by Pharmac and supporting sustained delivery of workstream two of Pharmac's reset program focused on enhancing consumer engagement and trust.

Section 71 (b) obliges the Pharmac Board to appoint "a consumer advisory committee to provide input from a consumer or patient point of view". Successive Boards have responded to this requirement largely by appointing members to the Consumer Advisory Committee (CAC) who represent consumers in the broadest of lay terms as representatives of potentially vulnerable population groups, rather than representatives of actual patients who are most directly and adversely impacted by Pharmac's decisions. The CAC has seldom included representatives with experience of rare disorders, or representatives of many other groups who are most directly and adversely impacted by Pharmac's decisions. Rare Disorders NZ has not seen the CAC engage with or represent the rare disorder community.

Rare Disorders NZ believes that it is imperative that directly impacted patients are fully represented and recommends that this section be amended to "…a Consumer and Patient Advisory Committee to provide input from a consumer **and** (ie replace the current word "**or**") patient point of view"

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Rare Disorders NZ recommends deletion of Section 70 and amendments to Section 71 (b) to strengthen Pharmac's obligations to consult with patient groups when it should.

Pharmac's functions

Rare Disorders NZ also recommends that the functions of Pharmac in section 69 of the Act are reviewed to ensure that they reflect the intention of the current Pharmac reset program which aims to "lay the foundation for a more transparent, outward focused, and collaborative organisation". In particular we recommend that Pharmac's functions include an expectation that Pharmac will consult when it should do so. As previously mentioned, the medicines landscape is evolving and it is imperative that the legislation is future proofed so that it does not act as a barrier to progress, particularly for those living with rare disorders.

Summary

Rare Disorders NZ recommends the Bill is amended to:

- Legislate the Rare Disorders Strategy as a health strategy under the Healthy Futures (Pae Ora) Act;
- Require health strategies to include action plans and accountability measures;
- Retain commitments to equitable access to health services in the original Act by retaining the Health Sector Principles or amending clause 5 of the Bill so the new purpose in section 3 is changed to: ensure that patients have timely, equitable access to effective, appropriate and quality health services;

- Preserve existing expertise requirements for appointments to the board and expert advisory committee on public health.
- Update Pharmac's legislation, including its statutory objectives, to ensure its decision making better reflects broader health, social, and economic outcomes, and patients' voices, and is aligned with recent reviews and strategic direction.

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

[ii] HealthiNZ. 2024. Impact for whānau māori of living with a rare disorder in Aotearoa New Zealand. https://raredisorders.org.nz/media/pages/file/7d/5295-rdnz-whanau-maori-white-paper-layout-web.pdf