“best possible health and wellbeing for people and whānau living with rare disorders in New Zealand/Aotearoa”

Submission to:	Finance and Expenditure Committee (FEC)
Subject:	Budget Policy Statement 2025
From:	Rare Disorders New Zealand (RDNZ)[1]
Date:	3rd February 2025

Recommendations

Rare Disorders New Zealand (RDNZ) recommends that BPS 2025:

1. Allows for a 2025 Budget which meets the following RDNZ expectations:
   a. Te Whatu Ora will be resourced to urgently commence implementation of the Rare Disorders Strategy by no later than 1 July 2025.
   b. Resources will be allocated to enable the establishment of a national rare disorders centre of expertise
   c. Pharmac will be resourced to purchase medicines for significantly more people with rare disorders
2. Includes a set of authentic wellbeing objectives as required by the 2020 amendment to the Public Finance Act
3. The policy areas to be focussed on in Budget 2025 be amended as follows:
   a. Lifting economic growth through measures to address New Zealand’s long-term ***wellbeing and*** productivity challenges.
   b. Keeping tight control of government spending while funding ***health and other…*** Government policy commitments and cost pressures that cannot be met from reprioritisation.
   c. Implementing a social investment approach to drive better results from the Government’s investment in ***medicines and other health and*** social services and thereby improve life outcomes for people with high needs.

Introduction

At RDNZ our mission is to enable and empower people with rare disorders to best benefit from services and therapies available in New Zealand, and to champion their collective voice, advocating for provision of world leading evidence-based health, disability, education and other services. We do this to make it possible for people and whānau living with rare disorders to experience their best possible health and wellbeing.

About 300,000 New Zealanders have one or more of the over 7,000 known rare disorders which are thought to be prevalent in New Zealand. Approximately 50% are children, 78% of

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[1] https://www.raredisorders.org.nz/

respondents to a 2023 RDNZ survey[2] said they experience rare disorder-related difficulty with seeing, hearing and/or moving, and 72% of disorders are genetic in origin. People with rare disorders are high users of the country's health and disability services, yet they frequently report dissatisfaction with the interactions and outcomes that they experience.

Following RDNZ's advocacy over more than two decades the Pharmac Review Report[3] recommended development of a Rare Disorders Strategy (RDS). After being reviewed by then Minister of Health Hon Dr Shane Reti the RDS was published by Manatū Hauora in July 2024[4]. The RDS provides for its implementation over a ten-year time period, commencing from its publication date.

Frustratingly, it seems unlikely that little if any implementation progress will have been made during the RDS's first year post-publication. This is because Te Whatu Ora has indefinitely refused to engage in the Strategy's implementation while they continue to go through the current "reset". Furthermore, the post-budget 2024 provision to Pharmac to enable the Government's promised cancer drugs to be funded resulted in disproportionately very few rare disorders medicines making the cut.

RDNZ supports a collective of over 150 disorder-specific support groups, representing over 30,000 individuals living with rare disorders. They are telling us that after waiting for more than two decades for the RDS they are angry that its implementation has been delayed by at least a year, if not more, primarily because of Te Whatu Ora's apparent indifference. (Any Finance and Expenditure Committee members who are sceptical as to the validity of these assertions are invited to view this short video clip[5] of Te Whatu Ora's Executive Leadership Team (ELT)'s fumbling through their inadequate responses to Health Committee questions during 2024's 'Scrutiny Week').

In response to the foregoing two paragraphs RDNZ has launched a petition, which we expect to deliver to Parliament in April 2025 or thereabouts, calling for Te Whatu Ora to develop an RDS implementation action plan, commencing no later than 1st July 2025.

While RDNZ was extremely disappointed in then Minister of Health Hon Dr Shane Reti's lack of leadership in declining to require Te Whatu Ora to engage with implementing the RDS and his reportedly washing his hands of any further engagement and involvement[6], RDNZ recognises that accountability for Te Whatu Ora ELT decision making ultimately lies with its Chief Executive whose term of office concludes in June 2025. RDNZ will be calling for her successor to reverse the incumbent's decision-making and get on with the RDS implementation. We hope that this will be reinforced by incoming Minister of Health Hon Simeon Brown.

Meanwhile, Associate Minister of Health (Pharmac), Hon David Seymour, has, in his letter of expectation to Pharmac's Chair Paula Bennett asked Pharmac to "work with the Ministry of Health as it develops the Rare Disorders Strategy"[7]. With the development of the RDS now complete, Pharmac and RDNZ have been engaging with each other in its implementation. The continued gross underfunding of rare disorders medicines notwithstanding RDNZ has

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[2] Impact of living with a rare disorder in Aotearoa New Zealand. RDNZ. https://raredisorders.org.nz/about-rare-disorders/2024-white-paper/

[3] Pharmac Review Final Report 2022. https://www.health.govt.nz/system/files/documents/publications/pharmac-review-final-report.pdf

[4] https://raredisorders.org.nz/about-rare-disorders/rare-disorders-strategy/

[5] Health NZ execs grilled by MPs on Strategy implementation. 6 December 2024. https://raredisorders.org.nz/learn/health-nz-execs-grilled-by-mps-on-strategy-impleme/

[6] TVNZ news clip. 14 December 2024. https://www.youtube.com/watch?v=O_5EV1UpYoY&feature=youtu.be

[7] Minister's Letter of Expectations 2024/25 to Pharmac Chair. https://pharmac.govt.nz/assets/Uploads/Letter-of-Expectations-2024-25.pdf

appreciated ongoing opportunities to engage on complex issues with both the Pharmac Board Chair and members of its executive and other staff in ways that we have not always experienced hitherto.

Budget Policy Statement 2025: RDNZ overview

There are some aspects of the 2025 Budget Policy Statement that are consistent with and have the potential to support RDNZ’s 2025 Budget expectations, and which should therefore be retained in modified form, including:

- The Government’s overarching goals to: build a stronger, more productive economy that lifts real incomes and increases opportunities for New Zealanders; deliver more efficient, effective and responsive public services to all who need and use them – in particular, to … improve health outcomes and educational achievement; and….restore discipline to public spending.

- The policy areas for focus in Budget 2025 including: lifting economic growth through measures to address New Zealand’s long-term productivity challenges; implementing a social investment approach to drive better results from the Government’s investment in social services and thereby improve life outcomes for people with high needs; and…funding…high priority Government policy commitments that cannot be met from reprioritisation.

RDNZ supports the retention of modified versions of this content because:

- Investing in early and accurate diagnosis for people with rare disorders, and early delivery of evidenced based care pathways and therapies can have a significant impact on their ability to live as well as possible for as long as possible and thus become net contributors to the building of a stronger more productive economy.

- More efficient, effective and responsive public services, together with discipline in public spending, will create room for the needs of people living with rare disorders to have their needs supported, resulting in their experiencing a range of improved life outcomes, including health and educational outcomes.

- A social investment approach to drive better results from the Government’s investment in services, and thereby improve life outcomes for people with high needs, is directly relevant to people with rare disorders and their need at times for expensive therapies which can sometimes be difficult to justify in the absence of an investment approach which looks at the longer-term benefits to society as a whole

- Funding high-priority Government policy commitments that cannot be met from reprioritisation is also directly relevant to rare disorders because of the policy commitment enshrined in the Government’s Rare Disorders Strategy – which should be considered a high priority for Budget 2025 given Te Whatu Ora’s track record of dragging the chain on its implementation – and the lack of any likelihood that the commitment will be met from the reprioritisation represented by Te Whatu Ora’s current reset.

Despite these positive elements RDNZ is nevertheless not convinced that the BPS content is strong enough to ensure that the resulting budget will meet the expectations of people living with rare disorders, hence this submission.

RDNZ Expectations for Budget 2025

RDNZ’s submission is that the 2025 BPS settings and content should be able to provide for a 2025 Budget which meets the following RDNZ expectations:

1. Te Whatu Ora will be resourced to urgently commence implementation of the Rare Disorders Strategy by no later than 1 July 2025.

2. Resources will be allocated to enable the establishment of a national rare disorders centre of expertise

3. Pharmac will be resourced to purchase medicines for significantly more people with rare disorders

Te Whatu Ora will be resourced to urgently commence implementation of the Rare Disorders Strategy by no later than 1 July 2025.

In July 2023 RDNZ was assured[8] by then Health Minister Hon Dr Ayesha Verrall that there would be sufficient funding in the then envisaged four-year funding envelope to allow for the implementation of the RDS to get underway. In theory, and as we’ve already pointed out to HNZ Commissioner Dr Lester Levy[9], implementation of the RDS is consistent with his aim of redirecting expenditure to front line clinical services, which means if his reset is successful, it should be possible to implement the RDS within the existing resource envelope. However, we remain sceptical because:

- Dr Levy is to be supported with a one billion dollar “letter of comfort”[10], indicating that he’s not confident that there’s sufficient fat in the system to enable front line services to be delivered within the allocated funding

- Te Whatu Ora’s recently released three-year workforce plan[11] fails to recognise that any workforce planning is required to support people with rare disorders, let alone implementation of the RDS (an omission which could have been avoided had there been early engagement with the RDS). Workforce development is required across all of the seven RDS relevant domains identified for action by RDNZ[12], not least in the areas of genetic diagnoses[13] and development and implementation of clinical care pathways[14].

- Te Whatu Ora is proposing as part of its reset to halve the size of its perinatal screening services team at a time when the service should be preparing itself for embracing new diagnostic technologies and expanding its effectiveness and diagnostic reach. RDNZ has

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[8] 11th July 2023. Meeting between Dr Verrall, RDNZ Chair James McGoram and CE Chris Higgins

[9] Letter to Dr Levy. 11th September 2024. https://raredisorders.org.nz/media/pages/file/7e/letter-to-lester-levy-regarding-implementation-of.pdf

[10] 3rd December 2024. Health New Zealand | Te Whatu Ora Statement of Performance Expectations 2024/25 p. 41. https://www.tewhatuora.govt.nz/publications/statement-of-performance-expectations-2024-25

[11] Te Whatu Ora. 10th December 2024. Health Workforce Plan 2024. https://www.tewhatuora.govt.nz/publications/health-workforce-plan-2024

[12] 7 priority areas for rare: Strategic priorities to improve health and wellbeing for people living with a rare disorder https://raredisorders.org.nz/about-rare-disorders/7-priorities-for-rare/

[13] The advent of whole genome sequencing technology will not only be a game changer for people living with rare disorders, but will have significant workforce development issues for existing screening services, genetic counselling services and pathology services

[14] Te Whatu Ora has a team of two staff to manage its national clinical care pathways initiative, which is inadequate for the task of expanding the service beyond the one rare disorder covered by the programme.

formally expressed its opposition to this proposal[15]

While we don't necessarily expect the Government's 2025 Vote: Health budget appropriations to make specific provision for implementing the Rare Disorders Strategy (although it would be immensely helpful if it did) it is clear to us that Te Whatu Ora's current resource envelope is insufficient to support implementation of the RDS, and that additional resources will need to be allocated.

Resources will be allocated to enable the establishment of a national rare disorders centre of expertise

For the past eighteen months RDNZ has advocated for the creation of a national virtual rare and undiagnosed disorders centre of expertise[16], inspired by similar programmes in Australia, Europe and the UK. RDNZ's framework is designed to support a range of improved life outcomes including social, educational, vocational, health, financial and general quality of life. Although implementation of the RDS would be a significant contributor to the effectiveness of the centre of expertise, RDNZ sees it as a cross-Ministry initiative to be resourced externally to Vote: Health.

Ultimately the centre of expertise will contribute to a more efficient health sector as a result of: marshalling and focusing already existing sources of expertise; earlier accurate diagnoses; earlier access to effective support services; and more efficient use of resources as diagnostic and support odysseys are shortened resulting in fewer demands on over-stretched systems. In the short term however establishment and bridging support will be required through the 2025 and 2026 budgets.

To support Budget 2025 to resource the Rare Disorders Strategy and establish a national rare disorders centre of expertise, RDNZ recommends that the Budget Policy Statement be amended by:

1. Including a set of authentic wellbeing objectives as required by the 2020 amendment to the Public Finance Act, rather than merely repeating the Government's overarching goals for its term of office. This amendment is crucial for people living with rare disorders because:

   a) It is our fundamental belief that it is a basic duty of the New Zealand Government to support its citizens to live well for as long as possible, and that this right should be extended to all New Zealanders with rare disorders. This basic duty should be a key driver of how it allocates resources and distributes wealth through successive annual Budgets.

   b) New Zealanders with rare disorders are currently afforded neither the best possible opportunities nor the same rights as other citizens to be supported to live well for as long as possible. RDNZ's White Papers[17] [18] reporting on the experiences of New Zealanders living with rare disorders, together with a wealth of patient stories, demonstrate that this is because:

      - New Zealand's current newborn screening programme appears to give insufficient

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[15] Submission on the National Public Health Service Consultation Proposal re perinatal screening services. 22 January 2025. https://raredisorders.org.nz/media/news/file/2025/01/27/20250116-national-public-health-service-submission.pdf

[16] https://raredisorders.org.nz/media/pages/file/de/rsca-centre-of-expertise-131123.pdf

[17] RDNZ. Impact of living with a rare disorder in Aotearoa New Zealand in 2024. https://www.raredisorders.org.nz/assets/Documents/5295_RDNZ_White-Paper-Layout_WEB.pdf

[18] Impact for Whānau Māori of Living With a Rare Disorder in Aotearoa New Zealand 2024. https://www.raredisorders.org.nz/assets/Documents/5295_RDNZ_Whanau-Maori-White-Paper-Layout_WEB.pdf

consideration to the contribution that early newborn genetic diagnoses can make to subsequent preventative family planning decisions

- Newborn screening arrangements are becoming increasingly anachronistic, falling behind international best practice, and lacking integration with other emerging screening technology, including whole genome sequencing.
- For some conditions there has been misalignment between availability of screening tests and availability of treatment resulting in delays in treatment which in turn militates against its effectiveness.
- Pathways to support best practice clinical support for people with a rare disorder diagnosis are at rudimentary at best.
- Inadequate funding is resulting in lifesaving, life-extending and life-enhancing medicines being denied to those who would benefit.
- We are advised anecdotally that demand for modern genetic testing technology and expertise is outstripping supply and availability, resulting in delayed diagnoses and access to effective treatment.
- Clinicians, including both general practitioners and specialists are often not sufficiently equipped or resourced to accurately diagnose rare disorders.
- Clinical responses to rare disorders are often based on inaccurate diagnoses which means that patients' health outcomes either fail to improve or become worse because they receive inappropriate or harmful treatment.
- There is a dearth of coordination services to support people to navigate the complexities of the health system, which are especially challenging for people living with rare disorders.
- Disability support is experienced as discriminatory, insufficient, difficult to access and not enabling people with rare disorders to live good lives.
- New Zealand underinvests in health research[19], including rare disorders research, which could identify new and better interventions, treatments and cures.

c) We are concerned that the absence of clear wellbeing signals in the BPS will result in service compromises which will be deleterious to the quality and length of life of New Zealanders living with rare disorders.

2. Amending the policy areas to be focussed on in Budget 2025 to:
    a. Lifting economic growth through measures to address New Zealand's long-term productivity *and wellbeing* challenges.
    b. Keeping tight control of government spending while funding *health and other* ~~a limited number of high priority~~ Government policy commitments and cost pressures that cannot be met from reprioritisation.

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[19] NZHR. April 2024. FEC Submission re BPS 2024. https://nz4healthresearch.org.nz/wp-content/uploads/2024/04/NZHR-Budget-Policy-Statement-submission-220424.pdf

Pharmac will be resourced to deliver medicines to significantly more people with rare disorders

Pharmac is under-resourced, and its current budget does not allow it to purchase the life-improving medicines that New Zealanders need. New Zealand continues to languish at the bottom of the OECD access to medicines league table[20]. The post Budget 2024 allocation to enable the Government’s pre-election cancer medicines promises to be honoured resulted in disproportionately few rare disorders medicines being funded compared to demand, as evidenced by some 50 support groups for people with specific rare disorders for which medicines are available globally but unavailable in New Zealand.

To address this RDNZ expects Budget 2025 to commit to annual increases to Pharmac’s budget to move from the current expenditure of 0.4% of GDP to the OECD average of 1.4% of GDP over the next four years. This funding envelope would be directed in the first instance to resourcing a life-improving rare disorders access to medicines scheme. Achieving the current OECD average within four years will require cumulative allocations of one billion dollars per year[21], and would be justified on the basis that this would constitute a good social investment, with longer term economic benefits resulting from recipients collectively being net contributors rather than so called net burdens to the New Zealand economy and society.

Although more analysis needs to be done RDNZ believes that investing in the future wellbeing of people with rare disorders (and others) has the potential to result in significant reductions in the economic burden both to the country and its citizens,[22] and to gains in national productivity and prosperity. RDNZ submits that a BPS which values an investment approach to informing its expenditure decisions will be an important enabler of realising these gains.

The 2025 Budget Policy Statement stops short of addressing these issues and RDNZ therefore recommends that the policy areas to be focussed on in Budget 2025 be amended to:

1. Implementing a social investment approach to drive better results from the Government’s investment in ***medicines and other health and*** social services and thereby improve life outcomes for people with high needs.

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[20] https://www.medicinesnz.co.nz/our-industry/comparable-countries
[21] MNZ. Graeme Jarvis email communication. 27 May 2024.
[22] BERL. March 2023. Rare Disorders Insights Report. Pathways towards better health outcomes. https://raredisorders.org.nz/media/pages/file/fb/rare-disorders-insights-report-final.pdf