

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

Submission to: Health Committee
Subject: [Gene Technology Bill \(GTB\) 2025](#)
From: Rare Disorders New Zealand (RDNZ)¹
Date: 17th February 2025

Recommendations

Rare Disorders New Zealand (RDNZ) supports the intent of the Gene Technology Bill but recommends that the legislation provides for:

1. The unique needs of people with rare disorders
2. Appropriately balanced safety measures to prevent any potential harm to individuals with rare disorders (who may be more vulnerable to unintended consequences of gene technology).
3. Timeliness of decision making to prevent avoidable mortality and re-invention of the wheel
4. Mechanisms to guarantee that individuals with rare disorders, regardless of their ethnicity or socio-economic status, can benefit from gene technology advancements.
5. Compassionate access mechanisms
6. Meaningful consultation with the rare disorders community.

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 respondents to a 2023 RDNZ survey² 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.

Rare Disorders Strategy

Following RDNZ’s advocacy over more than two decades the Pharmac Review Report³

¹ <https://www.raredisorders.org.nz/>

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>

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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 2024⁴. The RDS provides for its implementation over a ten-year time period, commencing from its publication date. RDNZ holds that its contents are a statement of government policy which government agencies are obliged to implement, and should not be permitted to opt out of.

The RDS’s Priority 1 - Kia tika ngā whakaritenga, kia pai ngā mahi tautiaki - Gearing the system for quality care - references the introduction of new capabilities as follows:

“Horizon scanning will inform early work to prepare for possible introduction of developing technologies and capabilities that can potentially benefit people with rare disorders. A workstream will clarify risks and benefits and will progress the conditions for safe adoption of technologies and capabilities, allowing for ongoing yet flexible early preparation. Other work to occur well in advance of adopting new technologies and digital capabilities will be to develop ways to assure their safety and quality and to communicate about them. People and their whānau living with rare disorders will provide significant input to this work.”

Gene Technology Bill

In public opinion polls undertaken in 2020 and 2022 about 50% of respondents agreed that genetically modifying organisms is a safe and effective way of making new medicines, about 30% disagreed and about 24% didn’t know⁵. Anticipating therefore that the Bill may have its detractors RDNZ wishes to express its support both in general, and as being consistent with the above requirements of the RDS. However we also submit that further consideration of the Bill should take into account the specific requirements of New Zealanders living with rare disorders, as follows:

Specific Provisions for Rare Disorders: The bill does not explicitly address the unique needs of individuals with rare disorders. Rare Disorders New Zealand argues that the bill should include specific provisions to ensure that gene technology advancements are accessible and beneficial to those with rare genetic conditions.

Risk Management and Safety: While the bill aims to provide risk-proportionate regulation, Rare Disorders New Zealand nevertheless emphasises the importance of appropriately balanced safety measures. We advocate for robust risk assessment processes to prevent any potential harm to individuals with rare disorders, who may be more vulnerable to unintended consequences of gene technology. Such processes however should not be drawn out, nor impeded where evidence thresholds are harder to achieve for rare disorders compared to more common conditions

Timeliness of Decision Making. One of the potential consequences of decisions that are unnecessarily too long is that people with rare and other disorders could deteriorate or die while waiting for a therapy to be approved, including for clinical trials. The GTB is ambiguous about whether the interface between the Gene Regulator, MedSafe and any additional requirements of the proposed Therapeutic Products legislation will create more work. Medsafe processes could be hastened by relying on the authority of international regulators, such as the FDA. In short RDNZ argues that there should be no unnecessary re-invention of any New Zealand or international wheels. RDNZ further recommends that the legislation itself (rather than separate regulations) provides for statutory time frames for the gene regulator, once again promoting timely decision making.

⁴ <https://rareorders.org.nz/about-rare-disorders/rare-disorders-strategy/>

⁵ New Zealand Speaks! 2022 Kantar NZHR Opinion Poll. New Zealanders for Health research 2022.

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Equitable Access: There is concern that the bill may not adequately ensure equitable access to gene technology treatments for all New Zealanders. Rare Disorders New Zealand calls for mechanisms to be put in place to guarantee that individuals with rare disorders, regardless of their ethnicity or socio-economic status, can benefit from these advancements. One way that this could be achieved is through fair and proportionate cost recovery regimes which support rather than militate against equity of access by vulnerable population groups - including those living with rare disorders.

Compassionate access: There is a likely need for some form of compassionate access exemption mechanism. There may well be instances with gene editing technologies etc where there is low/negligible risk to the environment or the wider public but there may be some risk to the potential patient who would like to receive the treatment (it may be deemed experimental treatment in a clinical setting). The mechanism would be used in medical situations where a patient has a very unique clinical situation/diagnosis, and it is unlikely that obtaining a licence for the treatment would be feasible. It would be somewhat akin to the Section 29 exemption that exists presently in the Medicines Act that allows doctors to prescribe unregistered medicines on a named-patient basis.

Consultation and Representation: Rare Disorders New Zealand stresses the need for meaningful consultation with the rare disorders community. We believe that the voices of those directly affected by rare genetic conditions should be included in the decision-making processes related to gene technology regulation.

A handwritten signature in black ink, appearing to read "Chris Higgins".

Chris Higgins
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