

Organisation: Rare Disorders New Zealand (RDNZ)

Submission to: Pae Ora Legislation Committee ([Pae Ora Legislation - New Zealand Parliament (www.parliament.nz)](https://www.parliament.nz/en/pb/sc/scl/pae-ora-legislation/tab/mp))

Subject: [Pae Ora (Healthy Futures) Bill](https://legislation.govt.nz/bill/government/2021/0085/latest/LMS575405.html)

**Kerry’s story**

**Example:** Kerry started to have some varied symptoms which were concerning, and she visited her GP to ask for help, the GP was unable to offer any diagnosis. Kerry continued to have increased symptoms and returned to the GP 3 times, after which time the GP assumed it was due to anxiety although she was adamant it was not. With support from her partner Kerry encouraged the GP to complete further investigations and think outside of the ‘common’ box; Kerry eventually was given a diagnostic test which confirmed a rare disorder.

Once diagnosed the journey had only just begun and there were 1. barriers for treatment, access to treatment 2. coordinating all the different specialists in absence of a central person coordinating things as would occur with diabetes, cancer or a common condition, 3. No clinical pathway or standard of care being followed here in NZ even though international standards exist a/ and were the next hurdle, etc.

The Pae Ora (Healthy Futures) legislation as currently drafted does little to nothing improve Kerry’s situation or health outcomes, or to ensure that s/he and hundreds/xxx hundred like him/her and their whanau and families will experience the healthy future promised by the legislation.

In order to ensure that Kerry and others experience significant and positive outcomes from the Pae Ora (Healthy Futures) legislation RDNZ submits that the Bill be amended as set out and recommended in the following table:

| **#** | **clause/section** | **issues** | **RDNZ recommends that:** |
| --- | --- | --- | --- |
|  | [General Policy Statement](https://legislation.govt.nz/bill/government/2021/0085/latest/d1586289e2.html#LMS575404) (GPS) | Successive reviews of the publicly-funded health system in New Zealand, including the 2020 Health and Disability System Review, the subsequent 2021 White Paper and indeed the Pae Ora (Healthy Futures) Bill itself have repeatedly overlooked the consistently poor outcomes experienced by those diagnosed and living with rare disorders. RDNZ has survey evidence which reflects international reports to show wide-ranging impact on persons with rare disorders in absence of specific policy and holds that the second paragraph of the GPS, which states that “*one of the root causes of… inequity and variation was the structure of [a] health system…that had become fragmented and complex, leading to unclear roles, duplication, misalignment, and a lack of a common whole-system ethos”,* applies particularly to those living with rare disorders | The opening sentence of the General Policy Statement be amended to: *Successive reviews of, and submissions addressing, the publicly-funded health system in New Zealand…have found consistently poor outcomes for some groups, in particular Māori, Pacific peoples, people living with rare disorders, and people with disabilities, and significant unwarranted variation in service availability, access, and quality between population groups and areas of New Zealand.* |
|  | [Interpretation](https://legislation.govt.nz/bill/government/2021/0085/latest/LMS575481.html) | RDNZ believes that the legislation should make explicit reference to rare disorders, which should be defined as per international norms best exemplified by European Union Orphan Drug Regulation 141/2000, which defines a disease or disorder as rare when it affects less than 1 in 2000. This is a population or community of scale with over 300,000 New Zealanders impacted and their whānau.  Furthermore the Bill makes frequent references to the “term population groups” without providing a corresponding interpretation of what this means. | The Interpretation section of the Bill be amended to include:  *“rare disorder means a disease or disorder which affects less than 1 in 2000 people in the New Zealand population”*  *“population groups means Māori, Pacific, people with a disability, and people with a rare disorder”* |
|  | [Health system principles](https://legislation.govt.nz/bill/government/2021/0085/latest/LMS575484.html) | Clauses 7(1) (b) and (c) of the health system principles require engagement with…other population groups…to develop and deliver services and programmes that reflect their needs and aspirations…and provision of opportunities for Māori to exercise decision-making authority…having regard to…the interests of other health consumers.  Clause 7 (4) states that these two health system principles do not apply to Pharmac and the performance of its functions.  Pharmac’s inadequate engagement with people with rare disorders contributes to poor health outcomes by Creating undue barriers to equitable access for small populations and innovative modern medicines. Without treatment persons with rare disorders impact hospital and ICU beds, require family carers and create a high societal economic burden  RDNZ maintains that Pharmac should be bound by clauses 7(1) (b) and (c) in the same way as all other components of the health system. Inclusion cannot exclude a community of scale and still remain equitable. | The health system principles section of the Bill be amended by deleting clause 7 (4) in its entirety. |
|  | [Key roles and health documents](https://legislation.govt.nz/bill/government/2021/0085/latest/LMS575557.html) | RDNZ maintains that people with rare disorders, including [condition], have long been neglected by the health system, as illustrated by Kerry’s story above. We believe that people with rare disorders have unique issues and challenges which should be recognised through a specific health strategy[[1]](#footnote-1). | Sections 10 (1)(a) 29 (1)(b) “key roles and health documents” and “overview of important health documents” be amended by adding  *(v) Rare Disorders Health Strategy*  The Bill be amended by including a new Section 41 as follows:  ***Rare Disorders Health Strategy***  *(1) The Minister must prepare and determine a Rare Disorders Health Strategy.*  *(2) The purpose of the Rare Disorders Health Strategy is to provide a framework to guide the health system in improving health outcomes for people with rare disorders.*  *(3) The Rare Disorders Health Strategy must—*  *(a) contain an assessment of the current state of health outcomes for people with rare disorders and the performance of the health system in relation to people with rare disorders; and*  *(b) contain an assessment of the medium and long-term trends that will affect the health of people with rare disorders and health system performance; and*  *(c) set out priorities for services and health system improvements relating to the health of people with rare disorders, including workforce development.*  *(4)* ***Subsection (3)****does not limit what may be included in the Rare Disorders Health Strategy.* |
|  | [Functions of Health New Zealand](https://legislation.govt.nz/bill/government/2021/0085/latest/LMS575493.html) | Clause 14 (3) states that “in performing any of its functions in relation to the supply of pharmaceuticals, Health New Zealand must not act inconsistently with the pharmaceutical schedule”.  The problem with this clause is that it will result in people such as Kerry (above) being denied life-saving and life improving therapies which will enable them to contribute to the economy and society generally through valued social and occupational roles, simply because Pharmac claims it does not have the resources or mandate to fund such treatments.  RDNZ maintains that there has to be a provision to override Pharmac in exceptional circumstances where a good efficacy and funding case can be made. Health New Zealand should not have to automatically have its mandate to achieve best health outcomes for all held hostage by Pharmac’s heavily proscribed decision making processes. | Clause 14 (3) be amended to:  *“in performing any of its functions in relation to the supply of pharmaceuticals, Health New Zealand may in exceptional circumstances and with the consent of the Minister of Health, act inconsistently with the pharmaceutical schedule where this is warranted by societal costs and benefits”* |
|  | [PHARMAC](https://legislation.govt.nz/bill/government/2021/0085/latest/LMS575612.html) | As above, the problem with Pharmac’s current objectives is that people such as Kerry are being denied life-saving and life improving therapies which will give them a healthy future and enable them to contribute to the economy and society generally through valued social and occupational roles, simply because Pharmac claims it does not have the resources or mandate to fund such treatments.  RDNZ maintains that there should be an exceptional circumstances provision which would allow Pharmac to spend beyond its funding envelope where the costs of investing in a treatment can be justified by the overall benefits to society. RDNZ further believes that to minimise the need for the proposed “exceptional circumstances” provision there should be a requirement for Pharmac to be sufficiently funded so that New Zealanders receive pharmaceutical treatments in a way that is consistent with what is available to citizens of comparable advanced economies. | Section 61(a) be amended to the objectives of Pharmac are to *“secure for eligible people in need of pharmaceuticals, the best health outcomes that are reasonably achievable from pharmaceutical treatment, either from within the amount of funding provided, or in exceptional circumstances and with the consent of the Minister of Health, as otherwise warranted by societal costs and benefits.”*  Section 62 be amended to include a new subclause 62(3): *“Pharmac shall be allocated sufficient funds to enable it to carry out its objectives in a way that is consistent with comparable international norms”* |
|  | [Regional Arrangements](https://legislation.govt.nz/bill/government/2021/0085/latest/LMS575629.html) | RDNZ notes that the Health and Disability Review report contemplated 6-8 DHBs, the subsequent health reforms White Paper proposed four Health New Zealand subregions, and the current Bill leaves the actual number to be determined by the Minister. Irrespective of the details Kerry and people like him/her are not served well when services are organised regionally. People with rare disorders deserve to receive evidence-based services which are consistently available, delivered with consistently high quality in accordance with internationally agreed standards of care for their particular condition, as part of a nationally managed service stream. This is the opposite of the current post-code lottery arrangements which would risk being perpetuated under any future regional arrangements. We believe that the same arguments could be made for other conditions (such as mental health and cancer services for example), and that rather than have a patchwork quilt where some services are provided regionally and some nationally, it makes best sense for them all to be provided along national service lines. People with rare disorders could come under the umbrella of a dedicated national rare disorders and precision medicine service (RDNZ’s preference), or be a discreet sub-service of a broader national service. | Section 97(1)(a) be deleted and replaced by  *National service arrangements*  *The Governor-General may, by Order in Council, on the recommendation of the Minister, make regulations…*  *(a) specifying national service arrangements—*  *(i) through which Health New Zealand and the Māori Health Authority must provide and arrange services; and*  *(ii) which must be maintained by the Health New Zealand and the Māori Health Authority* |
|  | Other |  |  |
|  | Other |  |  |

1. The final RDNZ version of the submission will amplify this point, referencing and drawing from RDNZ’s previous and ongoing submissions/efforts to introduce a rare disease strategy [↑](#footnote-ref-1)