

Pae ora: responsiveness to rare disorders in our future health system

Webinars co-hosted by the Ministry of Health | Manatū Hauora and Rare Disorders New Zealand

Q&As from Webinars

I have a question concerning the current rare-disorder health expenditure in NZ and how it compare to Australia's health expenditure on rare disorders, e.g.

Estimates of rare disorder health expenditure are not available for New Zealand or Australia (though hospital costs of rare disorders have been estimated in Western Australia¹).

Economic analyses of rare disorders and associated costs have started appearing in the international economic and health literature^{2 3 4 5}.

Overall health expenditure in Australia (estimated at \$US5,900 per capita in 2020) is considerably higher than that in New Zealand (estimated at \$US4,200 per capita in 2020). This difference is largely accounted for by the difference in the countries' gross domestic product per capita.⁶ A reasonable starting assumption is that rare disorders expenditure will, similarly, be higher in Australia than in New Zealand.

Country health expenditure is one important factor in health outcome differences between countries. Expenditure does not fully account for outcome differences for country populations⁷ or for people with rare disorders⁸.

Will the Ministry release information concerning the funding of life-saving generic drugs (as opposed to patented ones)?

Pharmac is the Government's funder of pharmaceuticals in New Zealand and is the best source of information on funded generic and patented medicines. Many products that we think of as life-saving (such as those used in a health emergency like adrenaline for anaphylaxis or naloxone for opioid overdose) are generic.

In deciding which medicines it will fund, Pharmac examines the health impacts of medicines (including impacts of preventing an early death which we can think of as 'life-saving') and the costs relative to those health impacts. All medicines must be approved by Medsafe as safe, effective and produced to quality standards.

In general, generic medicines tend to be less expensive than original branded medicines. However, this is not always the case. For rare disorders, and for technically challenging production techniques, there may not be incentives for competing manufacturers to produce generic medicines, given the costs involved in set-up, quality assurance and regulatory approval by Medsafe.

¹ [The collective impact of rare diseases in Western Australia: an estimate using a population-based cohort](#)

² [Socio-economic costs of rare diseases and the risk of financial hardship: a cross-sectional study](#)

³ [The national economic burden of rare disease in the United States in 2019](#)

⁴ [The IDEaS initiative: pilot study to assess the impact of rare diseases on patients and healthcare systems](#)

⁵ [Can you hear us now? The impact of health-care utilization by rare disease patients in the United States](#)

⁶ [Global Health Expenditure Database](#)

⁷ [Here's how countries compare on healthcare expenditure and life expectancy](#)

⁸ [Do EU5 Countries with Favourable Healthcare Expenditure and Reimbursement Indicators Have Better Patient-Reported Access to Treatments for Rare Diseases?](#)

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Pharmac regularly reviews the medicines it funds. Generic medicines may become available at lower cost, and/or may drive down the cost of branded comparators. Where Pharmac can buy medicines at lower cost, this frees up funding for other, new or additional medicines. Even where there are no generic medicines for certain rare disorders, generic and other lower-cost medicines contribute through freeing up funds for other medicines including those for rare disorders.

Pharmac has published reports on its funding for rare disorders medicines on its website^{9 10}.

Have these questions been distributed yet through a survey administered via Rare Disorders NZ, e.g.? If not, are there any plans to carry out survey research? Feedback would perhaps be more representative and hopefully guide better targeted strategy development.

Rare Disorders NZ (RDNZ) undertakes periodic surveys to understand the issues important to members and publishes the results together with insights and recommendations^{11 12}. RDNZ plans to repeat their survey before the end of 2023, and will provide details via their newsletter¹³ and website¹⁴.

Recommendations that followed the 2021 survey were:

- Recommendation 1: Continue to widen engagement with communities of people living with a rare disorder to provide a collective voice, in particular with Māori, Pacific and ethnic minorities.
- Recommendation 2: Increase collaboration amongst different stakeholders to implement person centred co-ordinated models of care for better quality life for people with rare disorders, including those that are yet to be diagnosed.
- Recommendation 3: Promote alternative assessment pathways to provide better access to modern, life changing medicines for people with rare disorders.
- Recommendation 4: Continue to advocate for development of a New Zealand Rare Disorders Health Strategy and Action Plan aligned with other current healthcare implementation plans.

RDNZ uses the survey results and insights to make submissions across a range of policy portfolios and consultations, including for some of the Pae Ora strategies.

The webinar questions are consistent with those asked in other Pae Ora strategy input opportunities and with RDNZ submissions. It is helpful to Manatū Hauora to have current insights from webinar participants to add to the input already available through the RDNZ surveys and submissions.

⁹ [2019-Report-Funding-Medicines-for-Rare-Disorders](#)

¹⁰ [Funding data for rare disorders medicines](#)

¹¹ [2019 Voice of Rare Disorders Survey](#)

¹² [2021 Voice of Rare Disorders Survey](#)

¹³ [Sign up to receive the RDNZ newsletter](#)

¹⁴ <https://www.raredisorders.org.nz/>

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Where will the rare disorders strategy underway sit with these other strategies?

The Pae Ora strategies are being developed to be published in July. A rare disorders strategy is being developed on a longer timeframe; it will be informed by and further build on the Pae Ora strategies.

Having input from the rare disorders community to the broader strategies is important so that the overall direction of system improvements supports better health outcomes for people with rare disorders. These strategies will provide a foundation and a rare disorders strategy will promote improvements specifically focussed on better health and wellbeing for people with rare disorders.

Given this is an exercise in data collection for medium to long term Pae ora goals. What is being discussed to provide relief to people with Rare Disorders, now?

Across the health system many people are providing care, support and advice to people with rare disorders. While not perfect, a lot of good care is provided and many people involved in healthcare are looking to make improvements week by week. Improvements are also a focus for Te Whatu Ora | Health New Zealand, for many practitioner or service provider groups and for cross-sector work like that to improve child wellbeing.

Questions about providing relief to people with rare disorders, now, need to be addressed to those responsible for funding or providing health care. Where your main health provider is not able to answer, Te Whatu Ora will in most cases be able to answer. The Nationwide Health & Disability Advocacy Service¹⁵ may be able to help.

Manatū Hauora | the Ministry of Health, in developing the Pae Ora strategies, is looking at the direction of improvement needed for New Zealanders' health in the coming 5-10 years and further ahead. The aim will be to move the system as far as possible towards gaining the best health outcomes for all NZers including for those with rare disorders and undiagnosed disorders and other particular and diverse needs.

Developing a rare disorders strategy provides the focus for a closer look at what barriers in the system can be lowered or overcome, and what opportunities there are now that may not have existed in the past. The aim is to move towards better lives for people with rare disorders.

How will the Pae Ora strategy fit within what is needed at a regional level? Will support be available to alleviate the travel and access of support? Will Pae Ora work in with the Ministry of Education & look to future needs of children with RD's?

Your input is highlighting the importance of access and support for people from all regions and diverse communities, along with the need for work across sectors such as with the education system. This input will inform the Pae Ora strategies.

¹⁵ [Nationwide Health and Disability Advocacy Service](#)

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It is worth noting that this input is in keeping with what Te Whatu Ora | Health New Zealand has been set up to do. Its functions, set out in the Pae Ora (Healthy Futures) Act include, among other things, to:

- provide or arrange for the provision of services at a national, regional, and local level
- develop and implement locality plans
- improve service delivery and outcomes for all people at all levels within the publicly funded health sector
- collaborate with other agencies, organisations, and individuals to improve health and wellbeing outcomes and to address the wider determinants of health outcomes
- work with the Māori Health Authority when performing any functions.

Te Whatu Ora is working towards more equitable access to services for all people and places, but change will take time.