



An initiative to mobilize patient advocates—including from indigenous (Māori) communities—to provide input on the country's first rare disorder strategy.

### Context

New Zealand has a robust community of patient advocacy groups supporting people living with a rare disorder, however, the country does not have a formal rare disorder strategy. The New Zealand government has not prioritized the needs of those with rare disorders—including timely diagnosis—or ensured a coordinated approach to supporting people with a rare disorder, raising concerns about adverse health outcomes and restricted access to lifesaving treatments that have been approved for similar patients in neighboring Australia.

As part of a major review of the country's drug purchasing agency, a fortuitous recommendation emerged in 2022 to develop a Rare Disorder Strategy—a longstanding goal of the rare disorder community. The National government tapped the Ministry of Health (MoH) to develop the new strategy.

Seizing an unprecedented opportunity for advocacy, Rare Disorders NZ (New Zealand)—an umbrella organization—led the charge in mobilizing a diverse group of patient groups to provide input on the strategy from the patient perspective, including from the marginalized Indigenous (Māori) community.



# Informing New Zealand's first rare disorder strategy\* as of April 2024

## Background

Rare Disorders NZ is a well-established organization that publishes a bi-annual Voice of Rare Disorders Survey, highlighting challenges the rare disease community faces.<sup>5</sup> The 2023 survey results showed that access to diagnosis was a major concern: over half of respondents reported that they waited more than one year to get a diagnosis and, for almost one in five respondents, the journey took more than 10 years.

This situation did not improve between 2019 and 2021. There were multiple contributors to these delays, including lack of rare disease data in electronic health record systems—which can lead to challenges accessing rare disease services—insufficient communication between healthcare professionals and specialists, and a limited emphasis on rare disorder research.

Over the past few years, Rare Disorders NZ has been able to significantly increase the number of people participating in this survey, from 300 to 1100—including 140 Māori—excellent representation from this population.

With the announcement of a forthcoming Rare Disorder Strategy, Rare Disorders NZ set out to work closely with the MoH and built a strong relationship to ensure that the strategy reflected the community's concerns. The organization facilitated the government team's access to key information about rare disorders to inform the strategy, highlighting, for example, the extensive wait times for diagnosis.

Rare Disorders NZ also collaborated with the government to gather input from patients and clinical experts to inform the strategy. They organized online focus groups with questions that prompted responses on devices that were shared in real-time to spark conversation. The government also interviewed 20 families of Māori descent, which was valuable in designing a strategy that must address the needs of the most underserved population in the country.

When the draft strategy was shared in late 2023, patient advocates were surprised that there were several sections yet to be completed even though they had provided specific recommendations. In response, Rare Disorders NZ made a formal submission outlining the community's demands for what the strategy should include, describing how to complete the missing sections, and raising additional areas that the strategy should address.

Rare Disorders NZ helped provide a unified voice on priority issues for the government to consider and include in the strategy. Together with other patient advocacy groups, they stated their demands more openly than before and made their expectations clear—especially for an accurate and rapid diagnosis. The group's advocacy was noticed with admiration within and beyond the rare disorder community.

Rare Disorders NZ published a white paper on Rare Disease Day 2024 that included the results from the 2023 Voice of Rare Disorders Survey as well as the organization's four

advocacy priorities for the forthcoming national strategy:<sup>5</sup>

- 1. Develop a Rare and Undiagnosed Disorders Centre of Expertise** to provide early and accurate diagnosis, evidence-based best practices for both diagnosed and undiagnosed patients, and family support through coordination experts who help navigate the health system.
- 2. Ensure patients can access essential medications** for rare diseases.
- 3. Ensure data on rare disorders is incorporated in the electronic health system** to expedite diagnosis.
- 4. Implement the Rare Disorders Strategy** in partnership with Rare Disorders NZ to ensure it is accepted by the rare disorder community and implemented.

The same clinical experts whom the government had consulted when conducting initial interviews publicly supported these demands, enhancing their credibility and, hopefully, encouraging the government to meet them. The white paper also highlighted how the country's current systems suffered from institutional racism and must do better when engaging with Māori communities to avoid a tokenistic way of addressing health concerns.

As of April 2024, a revised strategy has not been released publicly, but the rare disease advocacy community is hopeful that the government will incorporate its demands into the final strategy.

## Learnings

Rare Disorders NZ's perseverance and successful mobilization of patient advocates—including those from underserved communities—highlight the value of uniting behind a shared vision to advocate effectively with government on behalf of all people living with a rare disorder.

- **Be bold and push to get your voice heard:** Rare Disorders NZ realized that it was not being invited to co-create the country's rare disorder strategy (as had happened in Australia) and, therefore, should not assume that the government would reflect the patient perspective. The organization was effective in coordinating multiple opportunities to mobilize rare disease advocates who provided their input on the forthcoming strategy.
- **Elevate community voices:** Rare Disorders NZ was contacted by the government to ensure that the rare disorder strategy it was developing truly served the community. First-hand data from people living with a rare disorder was the best source to elevate their experiences and needs. For example, patients reported that they were being denied the basic right to access disability services because they had not received a definitive diagnosis—a compelling argument to accelerate diagnosis.
- **Start with a focus on the most underserved:** Rare Disorders NZ felt strongly that it should underscore the needs of the indigenous population, which are often neglected, and place these front and center to advance equity. Ensuring that the government understood the challenges of rare disorders among the Māori was an important initial step toward making progress for all New Zealanders with a rare disorder.



- **Get the clinical community on board:** Given that physicians are highly influential with the government, it was important to join forces with the clinical community. Rare Disorders NZ briefed healthcare professionals on patients' demands for what should be included in the strategy. The fact that the clinical community ultimately endorsed Rare Disorders NZ's submissions to the government was a win.
- **Recognize the value of advocacy – even if it may not lead to success:** According to Rare Disorders NZ, even if the Rare Disorder strategy does not reflect everything patients are seeking, mobilizing and uniting the advocacy community—patients and clinicians—has been critical in raising awareness of rare disorders.

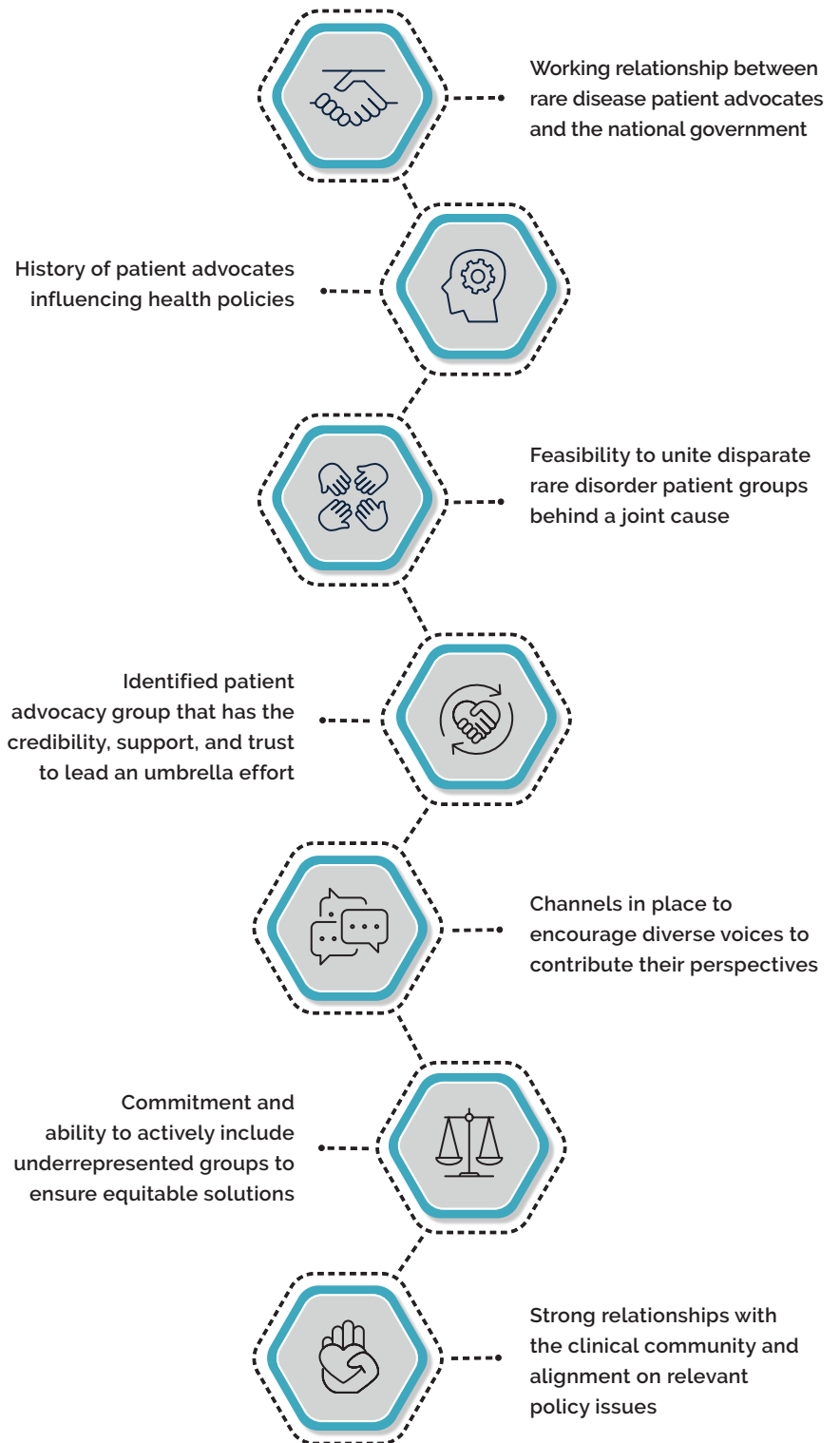


## Goals & next steps

New Zealand's Rare Disorder Strategy is expected to be released in 2024. The general sentiment within the rare disorder community is that while the strategy may not be perfect, it is an important milestone to leverage and build upon to improve the lives of people living with a rare disorder.



## Success factors & considerations when developing a similar model



# Update as of July 2024

## New Zealand's first Rare Disorders Strategy

After 20 plus years of advocacy from Rare Disorders New Zealand and a diverse set of patient groups, New Zealand's Ministry of Health finally delivered its first Rare Disorders Strategy in July 2024.

The Strategy provides guidance to health entities on how to better support people living with a rare disorder and reflects input from the rare disorder community on many important points, including the need to collect information on rare disorders in national data sets. One of the most significant achievements is the requirement that decision makers must routinely consider people living with a rare disorder when designing health system improvements.

The Strategy did not include every recommendation from patient groups, and Rare Disorders New Zealand will continue to advocate for a separate assessment pathway for rare medicines and acknowledgment that rare disorders affect every aspect of an individual's life, not just health.



Te Kāwanatanga o Aotearoa  
New Zealand Government



## Te Rautaki o Aotearoa e Pā ana ki ngā Mate Mokorea – Aotearoa New Zealand Rare Disorders Strategy