

HEALTH SELECT COMMITTEE SUBMISSION

Petition of Sue Haldane for Rare Disorders NZ: Develop a National Rare Disorder Framework

PETITION REQUEST

That the House of Representatives urge the Government to acknowledge the universal challenges faced by people living with a rare disease, and the unfairness within the current system, by committing to the development of a New Zealand National Rare Disorder Framework.

PETITION REASON

1 IN 17 NEW ZEALANDERS HAVE A RARE DISORDER



- Evidence shows that collectively, rare disorders are not rare, with a potential 10,000 types; however, most people (80%) will fall into only 150 different types of rare disorder.¹
- Those living with a rare disorder in New Zealand face inequitable access to diagnosis, treatment and care due to lack of awareness of the collective barriers.²
- There is no comprehensive data or measures of this population currently in New Zealand, therefore no corresponding visibility or action.
- International evidence highlights that rare disorders disproportionately affect children and women as their main carers. With policy for those with rare conditions there is the opportunity to support the goal of eliminating child poverty and embed a living standards framework.

RECOMMENDATIONS

1. ***Acknowledgement of rare disorders as a vulnerable population (based on international expectations and evidence) and commitment to develop a National Rare Disorder Framework***
2. ***Specific data collection on people living with rare disorders***
3. ***Increase in funding and sustainability for the peak body, Rare Disorders NZ***

NATIONAL RARE DISORDER FRAMEWORK

A cohesive National Rare Disorder Framework would address the holistic and broad areas impacting people with rare disorders and offer a comprehensive, future-focused plan to improve health and wellbeing for the 300,000 people impacted. Bringing together associated officials to ensure that the newly established Health NZ and Maori Health Authority can provide preventative and effective health

¹ *Estimating cumulative point prevalence of rare diseases: analysis of the Orphanet database* (<https://www.nature.com/articles/s41431-019-0508-0>)

² NZ Voice of Rare Disorders White Paper (https://raredisorders.org.nz/assets/Uploads/VOICE-OF-RARE-DISORDERS_WhitePaperV5.pdf)



and social supports for the most vulnerable who often fall through the gaps. This would bring New Zealand into alignment with global expectations and international frameworks, such as the UK Rare Diseases Framework.³

Following engagement with our national collective of more than 100 rare disorder support groups, Rare Disorders NZ identified **seven key elements** of a rare disorder framework: **Diagnosis; National rare disorder registry; Planned pathways for critical care; Access to appropriate disability and social support; Rare disorder medicines; Research; and Workforce development.**

SIGNIFICANT BENEFITS

A rare disorder framework for New Zealand will:

- Help ensure that people living with a rare disorder are included and have improved healthcare/wellbeing through responsive access to diagnosis, medicines and services. This is universal healthcare in action.
- Mean that the wellbeing of people with rare disorders is acknowledged and factored into government policy - including health, education, employment, and welfare/social services. This would incorporate preventative health economy policies.
- Support the Government with meeting its goal of eliminating child poverty and embed a living standards framework for some of New Zealand's most vulnerable people.
- Provide vital support for carers of people with rare disorders - disproportionately women, who may also be suffering from rare disorders themselves (e.g. genetic conditions).
- Ensure this vulnerable group receives the vital mental health support at diagnosis and along the pertinent stages of their health journey.
- Help New Zealand meet its international obligations (UN Declaration of Human Rights, CEDAW, UNCROC, Disability Convention, SDGs).
- Ensure New Zealand aligns with international standards to reduce inequity so people living with rare disorders are supported like in other OECD countries, such as the UK, Australia and the USA.

RARE DISORDER DEFINITION

Unlike the US or EU, New Zealand has no official definition of what constitutes a rare disorder, however ,they are considered to be life-threatening or chronically debilitating disorders, which are statistically rare – and have an estimated prevalence of less than 1 in 2,000, and have a high level of complexity and uncertainty.

Examples of rare disorders include some well-known conditions such as cystic fibrosis, Batten's disease, Ehlers-Danlos syndrome, muscular dystrophy, and also rare cancers - plus other conditions with no formal diagnosis.

³ <https://www.gov.uk/government/publications/uk-rare-diseases-framework>



BIOGRAPHIES

Sue Haldane is a mum and advocate working for more equitable and holistic healthcare for people living with a rare disorder. Sue's daughter, Lizzie, has a genetic rare disorder, 22q Deletion Syndrome, which leads to heart abnormalities, weakened immune system and developmental delays.

Rare Disorders NZ was founded more than 20 years ago and is the only organisation representing all New Zealanders living with a rare disorder and their whanau. We are the connector hub and collective voice of rare health and work to ensure equitable treatment for the rare disorder community. Our collective includes more than 100 rare disorder support groups and partners including the Royal NZ College of GPs, and international alliances.

CONCLUSION

Anyone, anytime, could find themselves or a loved one having to cope with the symptoms of a rare disorder and the irony is this situation is not rare. While the disorders themselves may be diverse, the collective population is large, with the same discriminatory barriers being faced by the wider rare disorder community.

The reality of living with a rare condition can mean waiting years for a diagnosis, juggling specialist appointments and no clear pathway to care or social supports including mental health and education support. This community needs recognition, awareness and inclusion for positive health and social outcomes.

International evidence shows that rare disorders need specific pathways and policies, and taking action makes economic, social and moral sense. New Zealanders are shocked to learn that New Zealand lags behind most OECD countries in recognition and support for people living with rare disorders. Most other countries including Brazil, China, the Philippines, Sweden, the UK and Australia have taken action to ensure equitable diagnosis, treatment and care.

Rare Disorders NZ wants to work with the Government to integrate and align rare disorders within the reformed health system, the living standards framework and wellbeing budget, as well as co-create a National Rare Disorder Framework to improve health and wellbeing for all including the most marginalised.

We would appreciate the opportunity to appear before the Committee to give an oral submission to amplify the voice of the rare disorder community, and showcase ways we can work together to progress a cohesive and equitable national framework for people living with a rare disorder and their whanau.

Lisa Foster
Chief Executive
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Sue Haldane
Mum and advocate

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REFERENCE REPORTS AND DOCUMENTS

- 1) Australian National Strategic Action Plan for Rare Diseases
(<https://www.health.gov.au/resources/publications/national-strategic-action-plan-for-rare-diseases>)
- 2) UK Rare Diseases Framework
<https://www.gov.uk/government/publications/uk-rare-diseases-framework>
- 3) EU Rare 2030: Foresight in Rare Disease Policy
http://download2.eurordis.org/rare2030/Rare2030_recommendations.pdf
- 4) APEC Rare Disease Action Plan
(<https://www.apec.org/rarediseases/News/APEC-Rare-Disease-Action-Plan-Sets-Precedent-to-Integrate-Rare-Disease-Care-by-2025>)
- 5) UN Member States include rare diseases in political declaration on universal health coverage
<https://www.rarediseasesinternational.org/un-member-states-include-rare-diseases-in-political-declaration-on-universal-health-coverage>
- 6) NZ Voice of Rare Disorders White Paper
https://raredisorders.org.nz/assets/Uploads/VOICE-OF-RARE-DISORDERS_WhitePaperV5.pdf
- 7) Fair for Rare NZ campaign
https://raredisorders.org.nz/assets/4277_Rare-Disorders-Fair-for-Rare-booklet-3.0-kw.pdf