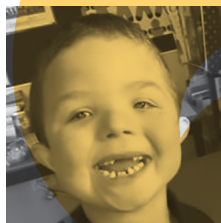




Fair

FOR RARE

CALL FOR NATIONAL STRATEGY FOR
RARE DISORDERS IN NEW ZEALAND



“No country can claim to have achieved universal healthcare if it has not adequately and equitably met the needs of those with rare diseases.”

HELEN CLARK, UNITED NATIONS

Statement from UN Development Programme Administrator Helen Clark to the International Conference on Rare Diseases & Orphan Drugs, Cape Town, 20 October 2016

Rare is Everywhere

People you know, people in your community, are living with a rare disorder. Collectively¹, rare disorders affect a huge slice of New Zealand society, with 1 in 17 people (around 300,000 New Zealanders) affected. The ripple effect on whānau equates to one third of our population.² Most of the over 6,000 known rare diseases disproportionately affect children, adolescents, and young adults.

Rare Disorders NZ, and the support groups we represent, are demanding that people living with rare disorders be formally recognised within our legislation as a population group. This would allow the establishment of mechanisms to measure health impact, and lead to specific health strategies to address the issues and challenges faced by this group. Systemic barriers and discrimination affect all individuals with rare conditions, and without a box to tick, many fall between the gaps, unable to access services and support.

Sometimes the public perception is that curing rare disorders is too difficult and costly for our country to achieve; however, the cost of denying access

to life-improving effective treatments and care may cost society more.

Those with rare disorders are not demanding miracles, just the chance to experience a fair go and equitable access to treatments and care that offer a better quality of life. These are goals our country can achieve, and has a duty to strive for.

Why should a person have a more difficult pathway to support just because of the complexity or rarity of their disorder type? Yet, this is the reality in Aotearoa.

There is an opportunity to change this picture; to improve health outcomes and remove systemic barriers. A rare disorder health strategy could align and interconnect with those already in place to make it a simple and complementary process of leaving no-one behind.

Let's work together to restore faith in our health system, by respecting and including persons with genetic, rare and undiagnosed conditions.



Lisa Foster
Chief Executive

¹ RDNZ follows the European Union policy which defines a disease or disorder as rare when it affects less than 1 in 2,000.
² We estimate that as many as 1.5 million people in NZ either have – or live with someone with – a rare disorder (around 30% of the population).

OVER
6,000
distinct rare diseases

Each one affects
fewer than **1 IN**
2,000
PEOPLE

Affects
6%
of the
population

72% 
ARE GENETIC

ABOUT
50%
of the people affected
by rare disorders
are children

300,000
New Zealanders live with
a rare disorder

IMPACT OF LIVING WITH A RARE DISORDER IN NEW ZEALAND

1 IN 3
people with a rare disorder spent an average of 13 days as an in-patient in hospital in the past year

54%
felt the costs associated with managing their rare disorder were hard to manage

1 IN 17
were in ICU for an average of 7 days

70%
had decrease in income and limited employment options

32%
required more than 2 hours per day for illness-related daily tasks

ALMOST **50%**
felt that organising care was difficult to manage

1 IN 5
took 10 years to get a diagnosis

Our Vision

People living with a rare disorder will have improved healthcare and wellbeing through access to diagnosis, medicines and services

DIAGNOSIS

Early and accurate diagnosis of rare diseases

PLANNED PATHWAYS FOR CLINICAL CARE

Coordinated and integrated pathways for cohesive healthcare

ACCESS TO DISABILITY AND SOCIAL SUPPORTS

Implement simple mechanisms to ensure appropriate access to disability and social supports

RARE DISORDER MEDICINES

Equitable access to modern rare disorder medicines through a specific assessment pathway

RESEARCH

Coordinated and funded programme of research for rare disorders

NATIONAL RARE DISEASE REGISTRY

Capture relevant data on rare disorders in New Zealand

WORKFORCE DEVELOPMENT

Planned training on rare disorders for health professionals and support staff

“ I feel I am just as sick as any cancer patient but there is no financial support available and even worse, no social support or recognition of my invisible illness. All my issues are considered ‘invisible illnesses’ and that’s exactly how I feel – invisible to our health system.”

“ Because my son’s rare disorder was not well known he did not qualify for funding even with his limitations and behaviour issues. However, now with his additional diagnosis of autism, doors are finally being opened.”

“ The supports provided should **NOT** be based on whether it is a well-known disorder.”

“ We had to leave New Zealand to gain life-saving treatment. I would say the health system completely failed us, despite the best efforts of some people working in it.”



Our Call For Action

Rare Disorders NZ, and the support groups we represent, are calling for action by our leaders to recognise the combined needs of the rare disorder community as a distinct population, and commit to a National Health Strategy for Rare Disorders.

A practical, inclusive approach can transform lives. RDNZ, and the groups we represent, can offer the blueprint and expertise to start this co-design process. Our vision is for a healthier, preventative and future-focused health system that reduces the time to diagnosis, that commits to training and education of healthcare professionals and provides equitable access to services and support.

Aotearoa New Zealand needs a health system that is Fair for Rare. Now is the time for change. The end of 2021 saw the UN formally recognise the challenges facing people living with rare conditions, and 2022 will see New Zealand map out a complete restructure of our health system.

We have the evidence and data from our 2021 Voice of Rare Disorders Survey to show the real impact of living with a rare disorder in New Zealand and to highlight the gaps and barriers in the system. With this information, a targeted approach can be applied to ensure these inadequacies are overcome.

Treatment, care, and support for rare disorders is inadequate in New Zealand and must be improved

Let's work together to co-produce and design a stronger healthcare system that includes the most vulnerable, by committing to adopt strategies to address the challenges facing those with rare disorders and improve access to quality treatments, care and support.

Indifference is not an option.

ABOUT RARE DISORDERS NZ

At Rare Disorders NZ we work to amplify the collective voice of rare disorders in New Zealand.

Rare Disorders NZ works with more than 125 different rare disorder support groups, representing more than 20,000 people living with rare disorders. We are the only

national organisation supporting all New Zealanders who live with a rare condition, and the people who care for them.

Rare Disorders NZ is part of the global rare disease community, including Rare Disease International and the Asia Pacific Alliance of Rare Disease Organisations.

New Zealand's International Commitments

- UN Resolution on Persons Living with a Rare Disease was adopted by all 193 UN Member States on 16 December 2021 and is the first UN document to recognise the specific challenges of Persons Living with a Rare Disease and their families
- UN 2030 Sustainable Development Goals and New Zealand's 2019 People's Report highlights the vulnerability and unmet needs of 300,000 New Zealanders living with rare diseases
- NZ Public Health and Disability Act 2000 and Statements in the Health and Disability Commission Code of Rights detail expectations to provide comprehensive services for all New Zealanders
- APEC Rare Disease Action Plan provides objectives and pillars to facilitate Asia Pacific alignment and partnerships, with development of policies and regulations for rare disease populations

Please help us continue to support people living with rare disorders in Aotearoa New Zealand.

givealittle.co.nz/org/rare-disorders-nz



RARE
DISEASES
INTERNATIONAL



Asia Pacific
Alliance of Rare
Disease Organisations



raredisorders.org.nz

PATIENT GROUPS

22q11 Foundation
Australia and New Zealand

The New Zealand
Acromegaly Society

The Arthrogyposis Group
New Zealand

ALD Foundation Trust

Alpha-1 Association
New Zealand

New Zealand Amyloidosis
Patient Association

The Angelman Network

Arachnoiditis New Zealand

Arachnoiditis Collaborative
Research Network &
Support

Arnold Chiari Malformation
Support Group

Brain Tumour Support NZ

Charcot Marie Tooth NZ

Complex Regional Pain
Syndrome (Kiwi CRPS)

Congenital Cytomegalovirus
Support Group

Cornelia de Lange Syndrome
Support Group

Cri du Chat Support Group
of New Zealand

Cushings Syndrome
Support Group

Cyclic Vomiting Syndrome
New Zealand

Cystinosis Support Group

Cystic Fibrosis NZ

Debra New Zealand

New Zealand Dystonia
Patient Network Inc.

New Zealand Eating
Disorders Career Support

Ehlers-Danlos Syndromes
New Zealand

Fabry Australia

Fabry Support Group NZ

Fanconi Anaemia Support
Australasia

Foetal Anti-Convulsant
Syndrome New Zealand

FOXG1 New Zealand
Support Group

Fragile X New Zealand

Friedreich Ataxia Research
Association

Guillain Barré Syndrome
Support Group NZ Trust

HAE Australia

Haemophilia Foundation of
New Zealand

Hereditary haemorrhagic
telangiectasia Support
Group

Hirschsprung's Support
Group

Homocystinuria (HCU)
Network Australia

Huntington's Disease
Association of New Zealand

HypoPARA New Zealand

IIH Brains Trust

Intracranial Hypertension
New Zealand

Immune Deficiencies

Foundation of New Zealand

Immune Thrombocytopenia
Purpura (ITP) NZ

The New Zealand LAM
Charitable Trust

Leukaemia & Blood Cancer
New Zealand

Leukodystrophy Resource
Research

LI-Fraumeni Syndrome
Association

Little People of New Zealand
(LPNZ)

Lysosomal Diseases
New Zealand

Mal De Embarquement
Syndrome Support

Mast Cell NZ

Motor Neurone Disease
New Zealand

Defeat Multiple System
Atrophy Down Under AU NZ

Muscular Dystrophy
New Zealand

Myasthenia Gravis
Support Group

The New Zealand
Neuromuscular Disease
Registry

Oculo Dento Digital
Dysplasia Support Group

Orthostatic Tremor
Support Group

Osteogenesis Imperfect
Support Group

Parenteral Nutrition
Down Under

Perthes Support Group

Perry Syndrome
Support Group

Pitt Hopkins Syndrome

Potio NZ Inc

New Zealand Pompe
Network

Porphyria Support Group
(NZ)

Prader-Willi Syndrome
Association NZ

Restless Legs Syndrome
Support Group

Sarcoidosis New Zealand

Sarcoma/Bone Cancer
Awareness

Scleroderma Support group

Spinal Muscular Atrophy
New Zealand

NZ Stills (AOSD/JIA)
Support Group

Tarlov Cyst New Zealand

Team Telomere

Trigeminal Neuralgia
Support New Zealand

Tuberous Sclerosis Complex
New Zealand

Unique - Understanding
Rare Chromosome and Gene
Disorders

Von Hippel-Lindau Alliance

New Zealand Williams
Syndrome Association

New Zealand XXY
support group

