# **Rare Disorders NZ: 2020 Election Manifesto**

**Issue 1 – Call for National Rare Disorder Framework**

Rare disorders: Policy makers often fail to realise that while infrequent or ‘rare’ disorders (diseases that affect 1 in 2,000 people or less) have relatively few patients individually, collectively have a substantial population with 300,000 New Zealanders affected. They are chronic, progressive, degenerative, and disabling. People living with rare diseases face many common challenges, such as delayed or inaccurate diagnosis, difficulty accessing care and lack of knowledge or access to expertise. For the individual sufferer this is a disaster, and for an economy it represents a significant direct and indirect cost. Therefore, most developed countries make this group a public health and social funding priority as this makes economic, social, and moral sense. New Zealand also has current international commitments to include people with rare disorders within Universal Health Coverage and aligned expectations and actions are expressed within the current APEC Rare Disease Action Plan pillars. Therefore, now is the time to afford this group higher priority within New Zealand.

**Solution** – Rare Disorders NZ and the support groups we represent are calling for acknowledgement and awareness of the common challenges faced by people living with a rare disease, along with a commitment to address these challenges through the development of a **New Zealand National Rare Disorder Framework**. Fairness means having the vision and leadership to develop public policy and actions to improve access to information, diagnosis, care pathways, access to disability, social supports, and medicines as well as to promote research in this field.

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| **Policy Question** |  |
| Will your party commit to a National Rare Disorder Framework? |

**Issue 1 – Diagnosis**

**Solution** – **Early and accurate diagnosis of rare disorders to avoid the diagnostic odyssey and prevent unnecessary interventions.**

Having a diagnosis often means having choices and the avoidance of lost opportunities.

* Connection to Global Commission to end the Diagnostic Odyssey for Children with a Rare Disease <https://www.globalrarediseasecommission.com/>
* Expanding best practice in ante-natal and newborn screening to ensure early intervention
* Genomic access for more complex and undefined cases

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| **Policy Question** |  |
| Will your party commit to an early diagnosis for all with a rare disorder? |

**Issue 2 – PLANNED PATHWAYS FOR CLINICAL CARE**

**Solution** – **Recognition of and removal of structural barriers that prevent cohesive healthcare.**

Due to high uncertainty for many with rare disorders the importance of being given a coordinated “holistic” approach to care is essential. This would enable inter-disciplinary services and effective collaboration between all health professionals, community service providers, Ministry of Social Development and Ministry of Health.

* Navigator for rare and complex cases
* Access to the right treatment
* Access to appropriate specialists with effective cooperation across disciplines
* Access to centres of excellence for specific “grouped” disorders (metabolic, skeletal, neurological, etc) which link to International sites (WHO Collaborative Global Network for Rare Diseases (WHO CGN4RD)
* Care across the DHB boundaries removing postcode lottery
* Transition to adult services - clear plans and processes

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| **Policy Question** |  |
| Will your party commit to a planned pathway for clinical care? |

**Issue 3 – ACCESS TO DISABILITY AND SOCIAL SUPPORTS**

**Solution –** **Implement simple mechanisms to ensure appropriate access to disability and social supports.**Access based on clinical need assessmentnot by diagnosis. Establishment of a simplified process to enable access to disability support services that includes the less common, more complex conditions so that no one is left behind because they do not tick a box.

* Implement specific mechanisms and service design that ensure **integrated care provision** and connection across ministries and organisations
* Assessment processes which include people with rare disorders – holistic assessment framework (factors for consideration)
* Inclusion of income support in the coordinated care model (exclusion often occurs with undiagnosed or poorly understood conditions)
* Adequate respite care and carer support payments

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| **Policy Question** |  |
| Will your party commit to people with rare disorders having access to disability and social support when required? |

**Issue 4 – RARE DISORDERS MEDICINES/DRUG ACCESS PROGRAMME**

**Solution** – **Equitable access with an appropriate assessment pathway and criteria for rare disorders which takes into consideration their small patient populations, progressive disease, and limited data.**

* A dedicated, adequate and ringfenced budget
* Assessment criteria separate to common conditions (as is implemented in Europe, Australia and Japan)
* Separate management processes
* Equity as the central principle to ensure balanced assessment and that cost per person is not a constant barrier

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| **Policy Question** |  |
| Will your party commit to a review of PHARMAC to ensure equitable access to medicines for rare disorders? |

**Issue 5 – RESEARCH**

**Solution** – **A funded national coordinated programme of research on rare diseases that includes active participation by patients, carers and patient advocacy groups. Improved government commitment and associated funding for rare disease research.**

* Includes research into diagnosis, prevention, treatment, service delivery, disability support
* Provision of opportunities for collaboration and connection between researchers

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| **Policy Question** |  |
| Will your party commit to a funded national coordinated programme of research on rare disorders? |

**Issue 6 – NATIONAL RARE DISEASE DATABASE**

**Solution** – **Improved data to ensure accurate awareness of the demographics of rare disorders and to allow responsive access to each other, to research participation and to clinical trials that may be available. Medical research on rare diseases is fast becoming an important source of advances in medical technology and genomics, which could hold the key to future treatments for all diseases.**

* Use of clear coding for rare disorders
* Alignment with Orphanet descriptions of 6172 clinically unique RDs excluding groups of disorders and disorders subtypes

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| **Policy Question** |  |
| Will your party commit to a national rare disorder database? |

**Issue 7 – WORKFORCE DEVELOPMENT**

**Solution** – **Clear planning for sustainable training to ensure workforce are ready and available – including genetic counsellors, biostatisticians, trained specialists, allied health professionals and lab workers.**

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| **Policy Question** |  |
| Will your party commit to workforce development? |