

Lysosomal Diseases New Zealand – Strategic plan for 2015 to 2017

Mission:

Lysosomal Diseases NZ is a charitable trust dedicated to improve contact between families affected by lysosomal diseases within New Zealand, and supporting research into the causes and treatment of lysosomal diseases and improvements in the clinical care of affected people.

Past activities and achievements:

LDNZ was formed in 1999 and incorporated as a charitable trust in April 2000. Its first action was to bring together various informal support groups that existed for different diseases under the lysosomal umbrella, and ensure that there was a “home” for those in New Zealand with any lysosomal disease, including those conditions where no other family contacts or networks existed.

Since 2000 we have:

- Built family contacts from a base of just a few dozen to more than 120 families today.
- Provided advice and support to families regarding healthcare access, disability support issues, disease information, family contacts, and medicine access.
- Promoted improvements to diagnosis and clinical care including boosting genetic services, metabolic services, care pathways, newborn screening, and palliative care.
- Promoted equity of access to medicines, especially orphan drugs, and supported access to clinical trials for new therapies.
- Organised family meetings and conferences with medical and scientific participation.
- Supported research into lysosomal diseases.
- Built collaboration with patient groups, medical teams and researchers internationally.

Governance:

LDNZ is governed by a trust board and employs Jenny Noble as a part-time Administrator/Field Officer to carry out the major tasks to achieve its mission.

5 strategic priorities:

In December 2014 the board of LDNZ reviewed its activities and confirmed these priorities for the future:

- 1 – Direct support and information for families.
- 2 – Promoting improvements to health and disability support systems.
- 3 – Support and promotion of research into lysosomal diseases.
- 4 – Organise and participate in conferences.
- 5 – National and international lobbying, advocacy and networking to assist our goals.

Action plan:

The 5 strategic priorities give rise to the following action plan for LDNZ.

1 – Direct support and information for families.

- Maintain an up-to-date website with information, contacts and links.
- Provide responses to enquiries by email or phone to all those seeking information.
- Ensure at least annual contact with all families to maintain connections and identify any assistance that may be needed.
- Provide regular newsletters and updates, including via social media.
- Provide direct support and information regarding orphan drug access, hospital admissions, palliative care, disability support provision, etc.

2 – Promoting improvements to health and disability support systems.

- Make submissions to Ministry, DHB and other relevant officials to identify problems and suggest solutions for better care.
- Maintain a focus on improvements to diagnosis and clinical care through strengthening genetic, metabolic and related services outreach and responsiveness.
- Advocate for timely adoption and adequate funding of innovations in technologies such as non-invasive prenatal testing, newborn screening, pre-implantation genetic diagnosis, and whole genome and whole exome sequencing, to ensure best practice in prevention, diagnosis and care.
- Advocate for an improved and fair carer payments system.
- Promote choice and flexibility in respite care and carer support systems.
- Seek a rare disease policy and action plan, including an orphan drugs access policy, to incorporate all of these health and disability issues.

3 – Support and promotion of research into lysosomal diseases.

- Advocate and fundraise for existing animal models of lysosomal diseases in New Zealand.
- Work with researchers to find new animal models for these diseases.
- Explore biobanking of samples from lysosomal disease families for research.
- Explore the development of a patient registry for our diseases.

4 – Organise and participate in conferences.

- Investigate the feasibility of an Asia Pacific regional conference, or an international conference on lysosomal diseases, to be held in NZ in 2016 or 2017, in conjunction with QMB, ICORD, APARDO and other possible collaborators.
- Identify key conferences at which LDNZ should be represented to maintain our links and influence.

5 – National and international lobbying, advocacy and networking to assist our goals.

- Maintain our connections with lysosomal and rare disease networks worldwide to learn and to contribute our experience to their discussions.