mnd
New Zealand

THE MOTOR NEURONE DISEASE ASSOCIATION OF NEW ZEALAND

RESEARCH STRATEGY
2019-2022

CURE • CARE • COMMUNICATE • COLLABORATE

INTRODUCTION

MOTOR NEURONE DISEASE (MND) refers to a group of progressive neurodegenerative disorders affecting the motor neurones, the most common of which is amyotrophic lateral sclerosis (ALS).

Based on data from international studies, it is estimated that **there are over 300 people living with motor neurone disease in New Zealand** at any one time, and more than 100 people will die of the disease each year(1). Median survival time from onset to death is 3–4 years for ALS(2). The exact prevalence of MND in New Zealand is not known, however recently published MND mortality data would suggest that the prevalence may be higher than in other countries (3).

The Motor Neurone Disease Association of New Zealand (MND New Zealand) is a small not-for-profit organisation whose main purpose is to help support people with MND and their carers. There is no cure for MND, but until there is, the MND New Zealand vision is: “Together we provide the best care and support for those living with MND.” The aim of the MND New Zealand Research Strategy is to develop a globally connected, comprehensive, national MND research programme that ensures all people with MND living in New Zealand receive the best evidence-based care and support available, whilst remaining part of the international effort to identify the causes of, and ultimately a cure for, MND.

TOGETHER WE PROVIDE THE BEST CARE AND SUPPORT FOR THOSE LIVING WITH MND

NZ MND RESEARCH STRATEGY

WHY

BEST CARE AND SUPPORT UNTIL WE FIND A CURE

HOW

FUND, PROMOTE, ENCOURAGE AND TRANSLATE THE BEST RESEARCH

WHAT

GLOBALLY CONNECTED, COMPREHENSIVE MND RESEARCH PROGRAMME

OUR VALUES

MND New Zealand has established the following organisational values as being integral to the success of our vision.

PEOPLE FIRST

People with MND, families/whānau, staff, and our wider MND community are at the core of what we do and how we work.

SUPPORTIVE

We work alongside people with MND, as well as with their families/whānau, to support them in what they want to achieve.

PARTNERSHIP

We work together with our stakeholders as one team.

PROFESSIONAL

We maintain the highest professional standards with all of those we work with.

HONESTY

We are upfront, honest, and show integrity in all that we do.

BACKGROUND

MND RESEARCH IN NEW ZEALAND

Motor neurone disease research in New Zealand is still in its relative infancy. There are large gaps in the literature regarding the epidemiology, aetiology, diagnosis, and clinical management of MND in New Zealand. Following the global success of the Ice Bucket Challenge in 2014, motor neurone disease saw an increase in research activity internationally. Concomitantly, New Zealand was fortunate to have the return from the UK of Dr Emma Scotter, who established a team dedicated to motor neurone disease research at the Centre for Brain Research at the University of Auckland. Through interactions with the Hugh Green Human BioBank and the New Zealand Neurological Foundation Douglas Human Brain Bank, Dr Scotter's lab uses primary human brain cells and human brain tissue to study motor neurone disease, focusing on TDP-43 proteinopathy and the role of non-neuronal cells(3). Since her return, Dr Scotter and her team have also published articles revealing the higher death rates from MND than other countries using mortality data for New Zealand, and the presence of C9ORF72 and UBQLN2 mutations as causes of amyotrophic lateral sclerosis in New Zealand(3,5).

Until recently, MND New Zealand had minimal involvement in the MND research field. Following the increasing interest in motor neurone disease, it seemed timely to focus on the research and awareness objectives of the organisation. A fundraising walk held in Christchurch in 2014 was attended by over 650 people and it was proposed that a similar event be held nationwide in six main centres in 2015. The goal of the Walk 2 D'Feet MND campaign would be to improve awareness of the disease and raise money for research, with half of the funds raised going towards establishing the MND New Zealand Research Fund. Over the next three years MND New Zealand saw an exponential growth in the annual event, with 18 cities and 6000 people taking part in 2018. To date, close to $800k has been raised from the walks, with approximately $400k going to the research fund.

Following consultation with other MND organisations and research groups, it was agreed that an essential first step to developing an MND research programme in New Zealand was to establish a registry. Working together with Associate Professor Richard Roxburgh, Kerry Walker, and Miriam Rodrigues, who already had experience with building a registry, the New Zealand MND Registry was established in May 2017 as a direct result of the MND New Zealand Research Fund. The aim of the registry is to facilitate "participation in national and international clinical trials and research; and assist in the planning of research''(6). The research fund also supported the development of The New Zealand Motor Neurone Disease Research Network in 2017 by Dr Scotter and Jayne McLean. The network provides a centralised website informing others of MND research in New Zealand, allowing for interaction between MND biomedical and clinical researchers, allied health professionals, and other researchers, and encouraging new MND research(7). It recently initiated the Rare Genetic Diseases of the Brain Symposium for Researchers of MND and other rare genetic diseases of the brain in Queenstown. This was supported by MND New Zealand and provided a platform for the discussion of the latest advances in research. Importantly, key connections were made with Australian counterparts, who presented the most recent findings from SALSA-SGC (the Sporadic ALS Australian Systems Genomics Consortium) and discussed the potential for future collaboration.

The New Zealand MND Research Network now lists the profiles of all researchers and currently known and completed research taking place in New Zealand, including:

- The Centre for Public Health Research at Massey University, which has completed a New Zealand population-based control study to investigate associations between occupational and environmental exposures and MND.

- The Department of Communication Disorders at the University of Canterbury, which is assessing the use of alternative and augmentative forms of communication in people with motor neurone disease, and a separate study assessing the impact of swallowing skill training protocol in patients with ALS.

- The Swallowing Research Laboratory at the University of Auckland, which is investigating the specific effects of motor neurone disease on swallowing in comparison to other diseases and to the healthy population.

- The Respiratory Physiology Laboratory team at Christchurch Hospital, who are focusing on the optimisation of respiratory function testing in MND patients, aiming for prediction of disease progression.

- The Thought Wired team are currently researching and developing a brain-computer interface for people with severe physical disabilities, such as motor neurone disease and cerebral palsy. The main aim is to create a completely physical-free communication system that utilises the electrical signals of the brain to control applications on a computer.

Both the New Zealand MND Research Network and the New Zealand MND Registry have played a crucial role in establishing a platform for MND research in New Zealand. MND New Zealand now commits $37k to the registry and $15k to the network annually.

To maintain the highest governance standards, and in keeping with an overall review of the organisation's strategic objectives, a research strategy was required to ensure remaining funds prioritised research activity that would be of the greatest benefit to our clients.

> ‘To date, close to **$800k** has been **raised** from the walks, with approximately **$400k going to the research fund.**’

WORKING WITH OUR PARTNERS

DEVELOPMENT OF OUR STRATEGY

The development of the strategy began with the establishment of the Research Working Group in February 2018. In June, a research development session was held with working group council and staff members, Dr Emma Scotter (Scotter Lab., Centre for Brain Research) and with contributions from the New Zealand MND Registry and New Zealand MND Research Network.

The MND New Zealand National Council's Research Working Group recognised and supported:

- People living with MND in New Zealand being part of the decision-making and solution.
- Collaboration with the research, medical, and allied health sectors to ensure quality outcomes.
- A comprehensive research programme ranging from discovery, to testing potential treatments, clinical research, and social care.
- Partnerships across the global and national network of organisations supporting research into MND.

The group proposed a collaborative model developing a strategy with input from people with MND and their families as well as from the wider sector, including researchers, clinicians, clinical nurse specialists, genetic counsellors, and other allied health representatives. Both internal and external consultation would be used to identify views on and practical challenges to MND research. To reflect our diverse community, feedback from different regions and from a range of different ethnicities would be sought.

To ensure that people living with MND in New Zealand and their whānau were included in the development of the research strategy, questions about research were included in the national community survey commissioned recently by MND New Zealand and sent to people with MND, families and carers, and health care professionals throughout the country. This included questions about how important research was, whether MND New Zealand should fundraise to support MND research, and which areas of research they thought were the most important. The survey also included questions that would inform future strategy, such as current access to health services and up-to-date information. Participants overwhelmingly agreed that MND New Zealand should fundraise to support MND research (91%). Research was rated the third most important "purpose" by all respondents, and the most important purpose by people with MND. At 91%, the mean of all groups showed that most respondents considered "developing clinical pathways" to be most important research area. At 86%, 85%, and 85%, the next most supported research areas were "improving quality of life", "finding a cure" and "connecting New Zealand to global MND research".

Feedback on the draft strategy was then sought from external key stakeholders including Dr Emma Scotter, Associate Professor Richard Roxburgh, Kerry Walker, New Zealand MND Registry Curator, and the New Zealand MND Research Network. MND New Zealand also sought input from the Neurological Foundation of New Zealand as part of the Neurological Alliance of New Zealand. MND New Zealand is also part of the International Alliance of ALS/MND Associations, and will use information from the forthcoming symposium to further inform our strategy.

CURRENT SITUATIONAL ANALYSIS

Following a review of current MND research in New Zealand and consultation with our key stakeholders, MND New Zealand established the following issues:

1

There are large knowledge gaps regarding the epidemiology, aetiology, diagnosis, and clinical management of MND in New Zealand.

2

There is a severely restricted workforce capacity, with a lack of researchers and supervisors available.

3

New Zealand has a relatively small population of people with MND and is geographically isolated, limiting access to the rapidly changing global research environment.

4

Inequities in access to healthcare exist geographically and culturally, and there is a lack of translation of international research into best practice, evidence-based care of people with MND.

5

There is a need for cultural awareness and input from Māori/Pasifika health groups in the development and implementation of the research strategy.

6

Biomedical and clinical researchers fail to engage in MND research.

7

Current researchers are working in isolation, unaware of others working in the same field, and there are limited opportunities for researchers to interact with one another.

8

Limited funding for research is available and inefficiencies exist in current research, with existing data and/or samples not being fully utilised.

9

Limited resources are available from within MND New Zealand, which is reliant on public donations for research.

10

There is a lack of strategic oversight, with no clear set of focus areas to ensure remaining funds prioritise research activity of the greatest benefit to people with MND.

11

There are limited opportunities for those impacted by the condition to influence research priorities.

12

Public awareness about the need for New Zealand-based research and funding and what research is currently taking place is lacking.

OUR GOAL

A GLOBALLY CONNECTED, COMPREHENSIVE, NATIONAL MND RESEARCH PROGRAMME

To address these issues, four strategic objectives have been established that will form the basis for the development of the MND New Zealand Research programme moving forward. MND New Zealand will support only the best research that provides the greatest opportunity to meet the following key objectives: Cure, Care, Communicate, Collaborate.

1. CURE

Advance our understanding of the causes of MND, particularly within the New Zealand environment, and connect our clients and their family/whānau to future trials and treatments.

2. CARE

Enable all people with MND, and their families/whānau, to lead the best quality of life possible through access to optimal clinical and social care using evidence-based practice.

3. COMMUNICATE

Share the latest advances in research and clinical management with all of our MND community in a timely and comprehensible manner, to enable informed decision-making and encourage research activity and exchange of knowledge.

4. COLLABORATE

Promote the best conditions for growth and development of New Zealand's MND research by fostering national and international partnerships and exploring new opportunities for research funding.

1. CURE

Without fully understanding the cause of a disease, development of treatment will be limited. It is essential that we advance our understanding of the causes of MND, particularly within the New Zealand environment, to enable further treatments, ultimately leading to a cure. This will include genetic research, disease-modeling, identification of biomarkers, drug development, and other treatment initiatives, as well as known environmental factors that are linked with MND.

A key priority is to improve our knowledge of the epidemiology of MND in New Zealand, including identifying any ethnic differences, which will be essential in connecting our unique population to future trials and treatments. This includes genetic screening to better understand the New Zealand MND cohort and identify those who will potentially benefit from future gene therapies. We will need to ensure that the infrastructure necessary to connect New Zealanders to effective treatments when they become available overseas is identified and developed. By understanding our patient population, and having a comprehensive registry complete with genetic profiles, we will be better positioned to promote New Zealand as a potential trial site for future MND clinical trials.

Within the next three years we will aim to have a comprehensive "opt out" New Zealand MND Registry with over 90% of people with MND listed, and with genetic screening offered at the time of diagnosis, ensuring that our population is "trial and treatment ready". We will support New Zealand's growing research community nationally and work with clinical leaders and researchers to build national and international alliances to connect New Zealanders to global research initiatives.

KEY PRIORITIES

- Work with the New Zealand MND Registry to establish the registry as "opt-out"
- Partner with clinical leaders and researchers to connect New Zealanders to global research initiatives
- Improve our knowledge of the epidemiology of MND in New Zealand, including gene typing

2. CARE

In order for people with MND and their family/whānau to lead the best quality of life possible, they must have access to the best clinical healthcare available, which uses evidence-based practice. This includes timely, equitable access to multidisciplinary specialist care and standardised national clinical care pathways for all people with MND. This is not possible without a thorough understanding of current health care service delivery in each district throughout New Zealand from diagnosis through to end-of-life care, including scope and timeliness. We need to identify barriers to providing best practice care and any disparities in the provision of current services.

We will support research that improves our knowledge of the current clinical care of people with MND, in addition to clinical research that aims to minimise disease impact from initial diagnosis through to end-of-life care. This includes, but is not limited to, research in all health care disciplines that fills knowledge gaps in respiratory and nutritional support, symptom control and palliative care, use of assistive technology and mobility aides, and psychological and social support. The knowledge gained will in turn be used to continually improve clinical management of MND.

Within the next three years we will have a complete understanding of how health care is delivered in each region and within different groups by working with GPs, neurologists, and other specialists and allied health providers, and we will support the development of best practice guidelines and the implementation of standardised clinical pathways for MND care in all DHBs. In addition, we will support and collaborate with research that aims to reduce physical, social, and cultural barriers to health care, allowing equitable access to multidisciplinary specialist care nationwide.

KEY PRIORITIES

- Nationwide review of health care delivery in each region from initial diagnosis to end of life care
- Development of best practice guidelines for the clinical management of MND in New Zealand, and the implementation of standardised clinical pathways for MND care in all DHBs
- Identify barriers to timely, equitable access to multidisciplinary specialist care and collaborate with health care research that aims to remove them
- Strengthen our relationship with clinicians and allied health professionals to encourage and promote clinical research

3. COMMUNICATE

With the amount of MND research continuing to grow internationally, MND New Zealand has a responsibility to disseminate this information to our clients in a timely and easily comprehensible manner. Our recent community survey of people with MND and carers confirmed the findings in the literature that this group has a very high need for information, and gets a lot of that information from MND New Zealand, particularly via the support team, the website, and publications. It is therefore vital that we work with researchers and clinicians to become a trusted source of accurate, up-to-date information and provide regular research news, particularly that which is relevant to New Zealand. We must ensure that we maintain a two-way channel of communication with our community, updating them on our progress, listening to their research views and involving them in decision making.

Over half of the respondents from the community survey don't follow the MND New Zealand Facebook page, so it is important that we continue to improve the ways by which we share the latest advances in research. These include face-to-face contact, print, and digital communications, and would also include facilitating information sessions and online forums between researchers and clients as well as regular research updates for our support team.

The survey also identified specialists as an important source of knowledge. We will look for ways to enhance our relationship with health care providers to ensure they have access to up-to-date information, to assist clinical team decision-making and ensure evidence-based practice. This may involve hosting forums for interactive discussion around the latest research. Exchange of knowledge within the research community is also essential to increase research activity and collaboration. Working with the New Zealand MND Research Network, we will identify ways to increase interactive discussion about MND research, both online and face to face.

KEY PRIORITIES

- Development of a research–specific communication programme for all members of the MND community including staff and health care providers, with publications, online content, and face-to-face discussion opportunities
- Maintain a two-way channel of communication with our community with regular updates on current projects supported by MND New Zealand and opportunities to influence research priorities
- Establish an expert panel to provide advice and ensure accurate and comprehensible information is disseminated
- Identify ways to facilitate knowledge exchange between researchers and increase research activity

4. COLLABORATE

In order to promote the best conditions for the growth and development of New Zealand's MND research nationally, it is vital that we form strategic partnerships with government agencies, healthcare research funders, and neurological groups, as well as academic institutions and professional bodies.

We need to develop the MND research workforce by creating opportunities for academics and tertiary students, and supporting the New Zealand MND Research Network to promote MND to the research community. We will explore new opportunities for research funding and work with global and national research initiatives that leverage funding from other sources and promote collaboration and cross-disciplinary research.

As a patient-representative organisation, MND New Zealand is an advocate for our clients at both a local and national level. It is essential that we identify social barriers to participation in research trials, including financial, legal, and ethical barriers, and work with government to influence relevant policy and legislation.

KEY PRIORITIES

- Strengthen relationships with associated neurological groups and other MND organisations to ensure strategic alignment and efficient use of resources
- Support the New Zealand MND Research Network to promote MND to the research community, and partner with academic institutions and professional groups to create opportunities for academics and tertiary students
- Ensure that MND New Zealand is represented at a national level to influence relevant research policy and funding

> ‘**A key priority is to improve our knowledge of the epidemiology of MND** in New Zealand, including identifying any ethnic differences, which will be essential in connecting our unique New Zealand population to future trials and treatments.’

OUR STRATEGY IN ACTION

The administration of MND New Zealand research grants will be through MND New Zealand National Office. Requests for funding will be considered via a competitive peer review process involving consultation with a panel of experts by the General Manager and MND Community and Research Advisor. Recommendations will then be forwarded to the MND New Zealand National Council for final approval.

Details of MND New Zealand’s research strategy will be available on MND New Zealand’s website, along with a summary of all projects and proposals that receive MND New Zealand research funding support. An annual update will be published to summarise research projects and milestones and their alignment to MND New Zealand’s research strategy.

TARGETED INVESTMENT IN PRIORITY AREAS

MND NEW ZEALAND RESEARCH FUNDING PROGRAMME

1

Building workforce capacity and collaboration: financial support for NZ MND Research Network, fellowships, PhD scholarship top-up grants, interactive forums, and travel grants.

2

Advance knowledge in the cause and treatment of MND in New Zealand: project grants for innovative New Zealand biomedical and clinical research, health services research, and ongoing support of MND Patient Registry.

3

Strategic partnership and initiatives: aligning with other neurological groups where common aims exist to maximise impact; promoting collaboration and leveraging funds from other sources to meet goals.

SUMMARY

MND research has grown exponentially in the last decade. Never before has there been such a desire to understand the causes of and find new therapies for this complex condition. It is imperative that people living with MND in New Zealand are included in these international efforts. In the last three years, MND New Zealand has helped to lay the foundations for a small but rapidly growing field of MND research in New Zealand. This has only been made possible through the tremendous community support for MND New Zealand, and the hard work of a small but dedicated group of researchers. However, there is still much work to be done if we are to bring the global successes of the future to our national stage.

Without knowing the demographics of people with MND in New Zealand and the health services they currently receive, we cannot translate international research findings into health care policy and clinical pathways. A nationwide study that identifies gaps in the clinical management of MND is urgently required to ensure that our community receives the best care and support possible.

Currently we lack the research personnel required to bring clinical trials to New Zealand. MND has been of little interest to neurologists and medical students in the past, as the condition has limited treatment options. We need to communicate the rapidly changing and exciting environment to clinicians, allied health professionals, and those who are training in order to build an MND workforce to deliver the therapies that will "care" today and "cure" tomorrow. We need to invest in our current researchers and use our limited funds wisely through a research grant scheme. Though comparatively small, this seed funding may attract bigger investment and at the same time help to promote MND as a potential career option to future researchers.

To date, all research supported by MND New Zealand has been as a direct result of public donations. As a small organisation with limited funds, it will be essential going forward to consider how to ensure the ongoing sustainability of the research fund and seek funding from additional sources. We must continue to engage our supporters, listen to their views on research, and feed back to them about the progress we are making. At times there will be a need to prioritise some projects over others, particularly given the rapidly changing research environment internationally. The community survey has already provided useful information about preferred directions for research, and as other opportunities arise it will be important to maintain a two-way channel of communication with our community. At the same time, we need to understand the barriers our researchers face and assist them to reduce these by building the workforce capacity, sourcing funds, and influencing governmental policy. Being geographically isolated, we face challenges with access to clinical trials, and we need to consider cost-effective ways to maintain connections to international research, as well as any associated legal and ethical implications.

By targeting our funds to the areas of greatest need with the greatest opportunity for strategic progress, and fostering the best conditions for growth and development of New Zealand's MND research nationally, we have the opportunity to lead the development of a national research programme that will ensure that people with MND receive the latest evidence-based care and access to new international treatments as they develop.

> ‘ We need to **communicate** the **rapidly changing** and exciting **environment** to clinicians, allied health **professionals** and those who are training in order to build an MND workforce to deliver the therapies that will **"care" today** and **"cure" tomorrow**. ’

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