



MND Research Network Newsletter

Summer 2021

Welcome to our summer newsletter with the latest updates from the MND Research Network. The last few months have been tough for Aotearoa with COVID-19 making its reappearance on our shores. Lockdowns across the country have had an impact on research and meant some studies have come to a halt or are delayed while the labs remain closed and COVID-19 restrictions are in place.

For those of you working in our hospitals and health care services it has also been a challenging time working on the front line under different conditions, and reprioritising workload. It is fair to say many of us are suffering from Zoom fatigue!



At the start of August, we were excited to host Dr Emma Scotter and Dr Luke McAlary for our second teleconference. Thank you to those who attended from various backgrounds all with a shared interest in MND, especially our presenters who are doing fantastic work in the biomedical field of MND. We received positive feedback from attendees who found the presentations informative and interesting. For those of you unable to attend, a [synopsis](#) of each speaker's presentation is available on the website. A recording of this teleconference is also available to watch in your own time, [click here to view](#). Our final teleconference for 2021 is on **Thursday 2nd December**. Keep reading for more information about our presenters and topics for this meeting.

It is an exciting time for MND research in New Zealand with upcoming research studies planned. There are also some wonderful opportunities available for someone who is interested in pursuing a funded masters or PhD qualification. You can read all about these in this newsletter.

If you know anyone interested in MND research, please encourage them to connect with us via [Twitter](#), [Facebook](#), or the [MND Research Network website](#). We hope 2022 will allow opportunities for us to network in person again as we adapt to living with COVID-19 in our community.

Kia kaha
Dympna Mulroy
NZ MND Research Network Manager

2nd December New Zealand Teleconference



The next teleconference is scheduled for Thursday 2nd December from 10am to 12pm. Please share information about this teleconference within your networks and to others who have an interest in MND research. [Click here](#) to access the Zoom details, and calendar link.

We are delighted to announce the following presenters for December, including Dr Natalie Leigh who recently completed her PhD, Dr Mandy Wilkinson and Justin Jordan, and Dr Alan Stanley, who will each discuss their upcoming New Zealand MND research projects.



Dr Natalie Leigh; Doctorate of Clinical Psychology, Lancaster University, United Kingdom

Dr Leigh recently completed her PhD which aimed to learn whether there is a relationship between levels of stigma and levels of psychological distress experienced by individuals with motor neurone disease. The study also aimed to determine the strength of this relationship compared to other important factors such as symptom severity and social support. Many eligible New Zealanders participated in this study through the New Zealand MND Registry. Dr Leigh will present the results from her study which will be of relevance to the New Zealand MND community.



Dr Mandy Wilkinson; Centre for Postgraduate Nursing Studies, University of Otago & Justin Jordan; Medical Student, University of Otago

Dr Wilkinson and a group of researchers from the Canterbury Respiratory Research Group, Christchurch Hospital, and University of Otago Christchurch campus are studying the role and impact of having an MND clinical nurse specialist. This is a combined quantitative and qualitative study. Dr Wilkinson will discuss the qualitative part of the study which involves interviewing patients to explore views around the role of the MND Clinical Nurse Specialist (CNS) in Canterbury.



Justin will discuss the quantitative part of the study which will analyse local data around the prevalence and incidence of MND in Canterbury, including survival rates, times to treatment, hospital admission, and timing of the interventions before and after the implementation of an MND CNS and MND multidisciplinary clinic.



Dr Alan Stanley; Neurologist, Hawkes Bay DHB

Dr Alan Stanley is chairperson of the MND Clinical Working Group and is involved in several MND research-related projects. Dr Stanley and Dr Claire Reilly (MND New Zealand Community and Research Advisor) are investigating the costs associated with caring for people with MND in New Zealand. Dr Stanley will discuss this study which is due to start in the coming months. Dr Stanley will also present the national guidelines for the assessment and management of MND that have been developed by the MND Clinical Working Group.

[Research Opportunities](#)

There are three exciting MND related research opportunities available in New Zealand, two masters and one PhD. They are fully funded and start the first semester 2022. Applications are open now and you can read about each of them below. Please forward this information to anyone who is interested in completing further study in the field of MND.

[Prevalence of MND in New Zealand – Masters Student Scholarship](#)

An exciting research opportunity has become available for those interested in completing a master's degree and contributing towards the New Zealand MND research portfolio.

MND New Zealand is funding a study on the "*Prevalence of MND in New Zealand*". This is a nationwide, point prevalence study of MND in New Zealand using capture-recapture methodology. It is anticipated that this project will take place over 12 months. They are offering a \$10,000 stipend (tax free), plus domestic tuition fees for 1 year. The start date is January – February 2022. This is an exciting opportunity to kick start a research career and make a significant contribution to the rapidly evolving field of MND research. More information is available on The University of Auckland [Find a Thesis](#) or [click here](#) for details.

[Why are two Xs better than one? Modifying X inactivation for the treatment of X-linked neurological disorders – Master's and PhD Scholarship](#)

Brain disorders caused by faulty genes on the X chromosome occur earlier, more severely, or exclusively in males. In females on average only half of cells switch off the faulty X chromosome while half switch off the functioning X chromosome. This study proposes to improve this mechanism, to treat X chromosome diseases.

Two fully funded positions are available, one PhD and one masters. The successful candidates must be eligible for enrolment in the specified course at the University of Auckland. Successful candidates will have completed a qualifying degree in Biological Science, Biomedical Science, Pharmacology, Physiology, or Medicine. Engineering and Computer Sciences applicants with a Neuroscience interest are also welcomed.

Both students will be based at the newly refurbished MND Scotter Lab at the School of Biological Sciences Thomas Building, University of Auckland. The PhD position includes a stipend of \$35,000 per annum for three years, and institutional fees paid. The masters position includes a stipend of \$22,000 for one year, and institutional fees paid. [Click here](#) for further information about the research study and application process.

[Research News – National and International](#)

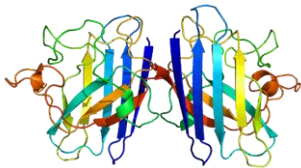
News releases and research updates are posted on our website - [research news page](#) and social media pages – [Twitter](#) and [Facebook](#). Follow us to keep updated regularly on what is happening in MND research.

[The New Zealand Genetic Frontotemporal Dementia Study](#)

Approximately 15% of people who have motor neuron disease (MND) are also diagnosed with a specific type of dementia called frontotemporal dementia (FTD). Overlap between MND and FTD occurs at clinical, genetic, and pathological levels. The NZ Genetic Frontotemporal Dementia Study (FTDGeNZ) is a multidisciplinary, inter-institutional initiative in search of pre-clinical diagnostic markers of dementia in a large New Zealand family. Dr Brigid Ryan is leading this study at the Centre for Brain Research at the University of Auckland. The aim of FTDGeNZ is to identify accurate markers of disease onset, risk, and progression that are relevant to both familial and sporadic FTD. The focus is on non-invasive, cost-effective diagnostic markers, in the hope that they will ultimately be used widely as a screening tool. Understanding the natural course of FTD may also elucidate the pathological processes underlying related dementias. This is the only study of its kind in New Zealand and one of the largest pre-clinical studies of a family cohort with a single mutation internationally. Dr Ryan and some participants recently spoke about this study on Our Changing World podcast. [Click here](#) to listen to the Podcast. You can also read a [news article on Radio New Zealand](#).

Tofersen (BIIB067) Biogen; Symptomatic Adults with SOD1-ALS

In the previous newsletter, we reported that Biogen's phase 3 VALOR study to evaluate the efficacy, safety, and tolerability of tofersen 100 mg in adults with SOD1 MND was due to be completed in July 2021. Results from this trial were announced at the American Neurological Association Conference in October. You can read the full article release [here](#) or listen to the presentation by registering on the [Biogen website](#).



The results indicated that tofersen did not meet the primary endpoint of change from baseline to week 28 in the Revised Amyotrophic Lateral Sclerosis Functional Rating Scale (ALSFRS-R).

Despite this, trends favouring tofersen were seen across multiple secondary and exploratory measures of biologic activity and clinical function. In addition, a pre-specified integration of data from VALOR and its ongoing open-label extension study (OLE) reinforced these findings and showed that early tofersen initiation led to less decline across multiple measures of motor function, respiratory function, muscle strength, and quality of life in people with SOD1-ALS. Most adverse events in both VALOR and OLE were mild to moderate in severity, including procedural pain, headache, pain in extremity, falls, and back pain.

SOD1-ALS is a rare, genetic form of ALS that accounts for approximately two per cent of the estimated 168,000 people who have the disease globally. Currently, there are no genetically targeted treatment options for ALS. In light of the findings from these studies Biogen is actively engaging with regulators, the medical community, patient advocacy groups and other key stakeholders around the world to determine potential next steps for the use of tofersen.

Tofersen (BIIB067) Biogen; Presymptomatic Adults with SOD1 mutation.

In the last newsletter we also shared Biogen's announcement on trialling tofersen for presymptomatic adults with SOD1 mutation, known as the [ATLAS trial](#). Several clinical sites have been announced including one in New South Wales. Unfortunately, they have decided not to bring it to New Zealand at present. Individuals must have a protocol-defined rapidly progressive SOD1 mutation and be clinically presymptomatic for ALS. The development of drugs for presymptomatic adults of MND highlights the importance of current studies investigating the genetic causes and associations of MND and early identification of individuals with a genetic form of MND.

Amylx - AMX0035 for MND

AMX0035 is an oral drug combining two compounds called sodium phenylbutyrate (PB) and tauroursodeoxycholic acid (TUDCA). These protect nerve cells from damage by targeting the endoplasmic reticulum and mitochondrial-dependent neuronal degeneration pathways in ALS patients.

AMX0035 was tested in a phase 2/3 clinical trial called [CENTAUR \(NCT03127514\)](#), which consisted of 137 participants recruited across 25 sites in the United States through the Northeast ALS (NEALS) Consortium. This trial achieved its primary endpoint of slowing decline in function as measured by the ALS Functional Rating Scale-Revised (ALSFRS-R) relative to placebo. AMX0035 is the first investigational therapy to demonstrate statistically significant benefit on this prespecified primary outcome in people with ALS since edaravone. It should be noted that this trial was developed for individuals with more rapidly progressive disease, making interpretation difficult for the wider population of people living with ALS/MND.

In April, the FDA requested data from a large Phase 3 clinical trial called [PHOENIX \(NCT05021536\)](#) before considering AMX0035 for approval. In September, Amylyx Pharmaceuticals announced their plans to submit a new drug application to the FDA for approval of AMX0035 for MND. Similar applications, supported by CENTAUR results alone, are currently being reviewed by Health Canada and set for filing with the European Medicines Agency by the end of the year. Amylyx is also actively discussing with health authorities elsewhere the best path for approval in their respective countries.

In the meantime, Amylyx are going ahead with plans for the Phase 3 PHOENIX trial. Estimated enrolment is 600 participants with ALS whose symptoms began in the past two years — this criterial will be more reflective of the wider population of people living with MND. Recruitment for this study has not yet commenced and estimated completion time is early 2024. The longer and larger phase 3 PHOENIX trial will add rigour to the confirmed findings as we continue to learn more about the effect of AMX0035 in MND.

Resources

Alzforum



Alzforum is a web-based scientific community dedicated to understanding Alzheimer's disease and other related neurodegenerative disorders. The website reports on the latest scientific findings, creates and maintains public databases of research data as well as producing online discussion forums. There is a range of publications on MND/ALS which can be easily found via the search box. [Click here for more information.](#)

Global Health Data Exchange

The University of Washington's Institute for Health Metrics and Evaluation has created the world's most comprehensive catalogue of surveys, censuses, vital statistics, and other health-related data. It's the place to start your health data search. They have a useful [GBD comparison interactive tool available](#). Data made available for download by IHME can be used, shared, modified, or built upon via the Open Data Commons Attribution License. To find out more or to access this information [click here](#).



New Zealand Association of Clinical Research



New Zealand Association of Clinical Research (NZACRes) is a professional association for clinical researchers in New Zealand. Membership is open to all those who have an interest in clinical research. Their goal is to promote and support clinical research within New Zealand through the provision of networking and educational opportunities, industry resources, and collaborations to address industry issues and create solutions. NZACRes also provides the clinical research community with a unified voice and a means for industry consultation. Membership is currently **FREE until March 2022!** Further information is available [here](#).

Conferences

We are excited to announce an **Australia and New Zealand MND Symposium**, scheduled to take place in Australia March 2022, date to be confirmed. With the absence of a face-to-face conference in 2021 it is hoped travel restrictions will have lifted for those who wish to travel to this event. We are hoping to



arrange a meeting in New Zealand for those unable to travel abroad where attendees can discuss the symposium and network with other New Zealand MND researchers. We will be in contact with additional information as it becomes available.

For a full list of upcoming conferences, webinars, and recordings of previous webinars please go to the [events section](#) on our website. Below is a list of some upcoming conferences next month that are more accessible as travel restrictions remain ongoing.

[Allied Professionals Forum \(APF\) | 3 – 4 December 2021 | Online](#)

The Allied Professionals Forum (APF) is an educational and training forum for allied health professionals who specialise in ALS/MND. This international forum offers healthcare professionals from around the world an opportunity to share ideas on good practice in the daily management of people with ALS/MND. The International Alliance of ALS/MND Associations will host a virtual Allied Professionals Forum spread over two days to accommodate time zones. Information about this forum is available [here](#).

[32nd International Symposium on ALS/MND | 7 – 10 December 2021 | Online](#)

Each year, the Symposium attracts over 1,000 delegates, representing the energy and dynamism of the global MND research community. It is the largest medical and scientific conference specific to MND/ALS and is the premier event in the MND research calendar for discussion on the latest advances in research and clinical management. Following the success of last year's first virtual Symposium and the ongoing challenges caused by the global pandemic, the MND Association's 32nd International Symposium on ALS/MND in December 2021 will once again be held online. [Click here for more Information.](#)



I hope you enjoy reading this newsletter. If you would like to see anything in our next newsletter, please [contact us](#) by the end of February.

It is hard to believe the end of the year is upon us. We will be closed from Friday 24th December until Wednesday 12th January 2022.

As this is the last newsletter for 2021, I want to thank all our supporters who have participated in the Network over the year. Those who attended and presented at our teleconferences, supported us with the “Cuppa tea for MND” event, participated in our survey, those who joined the Network this year, and all New Zealanders who are passionate about MND research. 2022 is looking like an exciting year for MND research as more opportunities and knowledge arise.

*Wishing you and your loved ones a
Merry Christmas and a Safe and Happy New Year*

We look forward to connecting with you again in 2022!

THANK YOU!