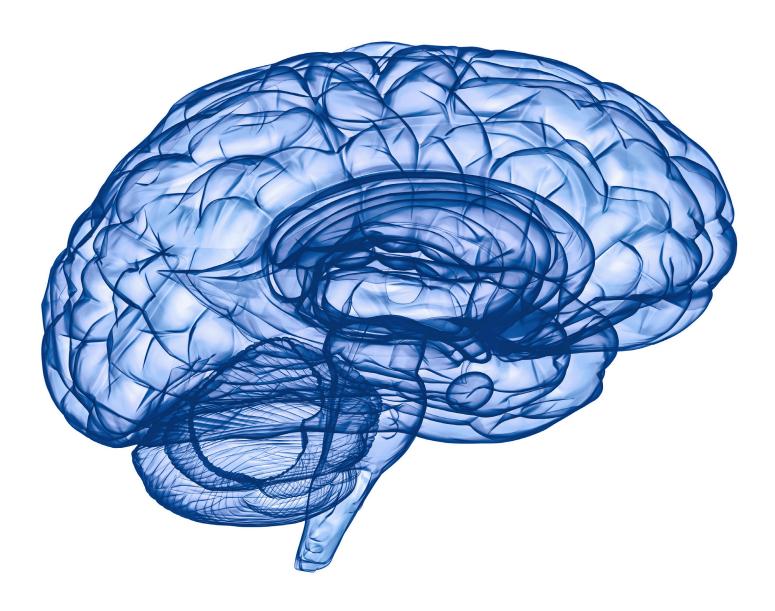
AUS & NZ MND Research Symposium



Symposium Schedule

April 28th-30th, 2022





DAY 1: April 28th, 2022

9:30 Registration & Morning tea

Session 1

MND Care and Research; now and into the future

Chairs: Rebecca Sheean & Gethin Thomas

| 10:30 | Welcome to Country |
|-------|--|
| 10:35 | Welcome on behalf of the MNDRA and FightMND; Fiona McIntosh |
| 10:40 | Address by Local Delegate |
| 10:50 | A Personal Perspective (PLEX); Mel Syron |
| 11:00 | MND Collective overview; Rebecca Sheean |
| 11:15 | An innovative approach to patient management, clinical trials, |
| | and evidence-based policy development, is required to provide |
| | coordinated, personalised care and find an effective treatment |
| | for Motor Neurone Disease (MND); Matthew Kiernan |
| 11:30 | An Overview of New Zealand Motor Neurone Disease Research |
| | Strategy; Claire Reilly |
| 11:45 | MNDSA Clinical Pathway and Referral Network; Tracey Watters |
| 12:00 | SALSA; Anjali Henders |
| 12:15 | Panel discussion |
| | |
| 12:30 | Lunch - Sponsored by <i>Illumina</i> |



Advancing MND research to understand the causes of MND

Chairs: Bradley Turner & Sarah Kerwin

| 1:20 | Probing the integrity of interneuronal inhibitory circuits in |
|------|---|
| | ALS with TMS-EEG; Mehdi Van den Bos |
| 1:32 | Altered SOD1 maturation and post-translational modification in |
| | post-mortem familial and sporadic ALS spinal cord; Benjamin Trist |
| 1:44 | Arginine-rich C9orf72 ALS proteins stall ribosomes in a manner |
| | distinct from canonical ribosome-associated quality control |
| | substrates; Danny Hatters |
| 1:56 | Validation of the GGNBP2 gene as an ALS risk factor using |
| | Drosophila: links to synaptic development and autophagy; |
| | Sarah K. Kerwin |
| 2:08 | Characterising myelin structure and composition using a |
| | combination of human post-mortem tissue and patient |
| | iPSC-derived spinal cord organoids; Samantha Barton |
| 2:20 | Ablation of free fatty acid receptor 2 (FFAR2) signalling |
| | accelerates early disease progression in the SOD1G93A |
| | mouse model of MND; John Lee |
| 2:32 | Q&A All speakers |
| | |
| 3:00 | Afternoon tea |
| | |



Rapid Fire Talks: Preclinical studies in MND

Chairs: Sarah Rea & John Lee

| 3:30 | A copper chaperone-mimetic polytherapy for SOD1-associated amyotrophic lateral sclerosis; Victoria Shephard |
|------|---|
| 3:36 | Polytherapy for the treatment of familial SOD1 ALS; Jeremy S. Lum |
| 3:42 | Effects of antiretroviral therapy on motor behaviour, TDP-43 |
| | proteinopathy and immune response in a motor neuron |
| | disease mouse model; Megan Dubowsky |
| 3:48 | A high-throughput flow cytometry drug screen to discover new |
| | treatments for motor neurone disease; Nicholas Geraghty |
| 3:54 | ALS monocyte-derived microglia-like cells reveal cytoplasmic |
| | TDP-43 accumulation, DNA damage, and cell-specific |
| | impairment of phagocytosis associated with disease |
| | progression; Anthony White |
| 4:00 | Glucose dyshomeostasis in the SOD1G93A mouse model |
| | of MND; Tanya McDonald |
| 4:06 | Investigating the role of calpain cleavage as an early pathogenic |
| | mechanism in mouse models of Machado Joseph disease and |
| | motor neuron disease; Katherine Robinson |
| 4:12 | Developing scalable and rapid human iPSC-derived neuron |
| | models to investigate TDP-43 pathology; Matisse Jacobs |
| 4:18 | Validating Bruton's Tyrosine Kinase (BTK) as a druggable |
| | therapeutic target for Motor Neuron Disease; Sara Jose |
| 4:24 | Developing new viral-mediated mouse models of TDP-43 |
| | Pathology; Heledd Brown-Wright |
| 4:30 | Sarm1 knockout modifies biomarkers of neurodegeneration |
| | and spinal cord circuitry but not disease progression in the |
| | mSOD1G93A mouse model of ALS; Sharn Perry |
| 4:36 | MND muscle fails to respond to motor neuron pro-synaptic |
| | signals to form postsynaptic specializations; Peter Noakes |
| 5:00 | Poster Presentations and Welcome drinks |
| | Even numbered posters 5 pm - 5:45pm |
| | Odd numbered posters 5:45 pm - 6:30pm |
| 7:00 | Day 1 concludes |





Day 2: April 29th, 2022

9:00 Registration

Session 4

Recent discoveries on the role of TDP-43 in the pathogenesis of MND

Chairs: Emma Scotter & Adam Walker

| 09:30 | Non-biased identification of new modulators of MND-associated |
|-------|--|
| | TDP-43 aggregation; Rebecca San Gil |
| 09:42 | miR-23a suppression accelerates functional decline in the |
| | rNLS8 mouse model of TDP-43 proteinopathy; Aaron Russell |
| 09:54 | pTDP-43 pathology-associated increase in microglial CD68 |
| | expression in human Amyotrophic Lateral Sclerosis; |
| | Molly Swanson |
| 10:06 | In vivo characterisation of human TDP-43 reveals a critical role |
| | for posttranslational modifications for condensate formation |
| | and aggregation; Marco Morsch |
| 10:18 | Linking TDP-43 pathology, motor cortex hyperexcitability and |
| | motor neuron demise in ALS; Catherine Blizzard |
| 10:30 | Q&A All speakers |
| | |
| 10:50 | Morning Tea |
| | |



Fostering treatment development and clinical trials/biomarkers in MND

Chairs: Cathy Blizzard & Allan McRae

| 11:20 | Lipid profiles in blood of mutant SOD1 mice have diagnostic, |
|-------|---|
| | progressive and pharmacodynamic biomarker properties; |
| | Sophia Luikinga |
| 11:32 | Comparative RNA-seq reveals upregulated tissue generation |
| | in skeletal muscle of amyotrophic lateral sclerosis cases to |
| | controls; Anna Freydenzon |
| 11:44 | The 1H-MRS Metabolite Signature of Cortical Hyperexcitability |
| | in ALS; Sicong Tu |
| 11:56 | Systemic central nervous system delivery of therapeutic |
| | antisense oligonucleotides with blood-brain barrier |
| | crossing peptides; Fazel Shabanpoor |
| 12:08 | A multimodal, nerve-muscle combination therapy strategy |
| | for MND; Peter Crouch |
| 12:20 | Q&A All speakers |
| | |
| 12:40 | Lunch |



Enhancing clinical research and improving quality of life/clinical trials in MND

Chairs: Susan Mathers & Frederik Steyn

| 1:30 | Grief, depression and anxiety in bereaved caregivers of |
|------|---|
| | people with Motor Neurone Disease: A population-based |
| | national study; Samar Aoun |
| 1:42 | Online Carer's Questionnaire (OCQ) Behaviour Screen; |
| | Amelia Ceslis |
| 1:54 | Polysomnographic titration of non-invasive ventilation in motor |
| | neurone disease (3TLA): Study protocol for a randomised |
| | controlled trial; Abbey Sawyer |
| 2:06 | Neck Weakness in MND/ALS: An Investigation of Prevalence |
| | and Physiotherapy Management - A Retrospective Descriptive |
| | Study; Trinh Sia |
| 2:18 | NIV@Home: A pilot randomised controlled trial comparing |
| | home-based versus day admission models of non-invasive |
| | ventilation (NIV) implementation; David Berlowitz |
| 2:30 | Lung volume recruitment therapy in people with motor neurone |
| | disease and slowly-progressive neuromuscular disease: |
| | a randomised controlled trial; Nicole Sheers |
| 2:42 | Q&A All speakers |
| | |
| 3:00 | Afternoon tea |
| | |



Rapid Fire Talks: Patient-focussed studies in MND

Chairs: Colin Mahoney & Kelly Williams

| 3:30 | Towards quantitative measurements of iron in the spine and brain in MND using Magnetic Resonance Imaging; Thomas Shaw |
|------|--|
| 3:36 | Comparison of hip- and wrist-worn actigraphy for assessing functional decline and disease progression in patients with MND; Cory Holdom |
| 3:42 | Uncovering Predictive Urinary Biomarkers for Motor Neuron Disease; Mary-Louise Rogers |
| 3:48 | Low Hypothalamic Volume with Low BMI is Associated with Shorter Survival in Patients with ALS; Jeryn Chang |
| 3:54 | Hypermetabolism is not associated with increased levels of cytokines in patients with ALS; Stephanie Howe |
| 4:00 | Familial ALS-associated SFPQ variants promote the formation of SFPQ cytoplasmic aggregates that reduce surface AMPA receptor expression in primary neurons; Victor Anggono |
| 4:06 | Functional characterisation of the amyotrophic lateral sclerosis risk locus GPX3/TNIP1; Fleur Garton |
| 4:12 | Rare short tandem repeat expansions present in Australian sporadic ALS and FTD cases; Lyndal Henden |
| 4:18 | Genetics of Motor Neuron Disease in New Zealand; Emma Scotter |
| 4:24 | Leveraging cryptic relatedness for gene discovery in Australian and New Zealand ALS and FTD cohorts; Kelly L. Williams |
| 4:30 | Assessing the quality of evidence in studies aiming to improve the psychological well-being (PWB) of MND carers: Results of a systematic review of carer interventions; Paul Cafarella |
| 4:36 | Opportunities to enhance participation in psychological interventions for people with motor neurone disease and their family caregivers: Important considerations for service implementation; Sarah Velissaris |
| 4:42 | Q&A All speakers |
| 5:00 | Presentation and Poster Awards |
| 5:10 | Closing Remarks; David Ali |
| 5:20 | Day 2 Concludes |
| 7:00 | Conference Dinner; MND and ME |

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Day 3: April 30th, 2022

9:00 Arrival and Morning tea

10:30 Welcome on behalf of FightMND

and MNDRA; David Ali

10:35 Lived Experience Insights – what do we

want from research?; Sean Dorney

Session 1Causes and biology

10:45

The MiNDAUS Registry-manage, share, contribute;
 Catherine Hansen

- 2. MND Triggers The environment versus genes; lan Blair
- 3. We hear a lot about TDP43 what is it, what does it do and how can we target it for treatments?; Fiona Bright
- 4. Panel Discussion

12:15 Lunch

Session 2 Treatment and Care

13:15

- Current trials in Australia and their respective stages.
 What does the MND trial pipeline look like?; Colin Mahoney
- 2. Genetic counselling; Vrunda Sane
- 3. Prism an app to help organise your MND logistics; Jane Milne
- Compassionate Communities: Supporting those caring, dying and grieving; Samar Aoun
- 5. Panel Discussion
- 15:15 Closing remarks; Fiona McIntosh
- 15:30 Meeting Close

