InTouch

KIA NOHO TATA Inclusive | Inspiring | Informative Winter 2017 Issue 95

Winter rituals

How to keep well and sleep tight

Home sweet home

Tenancy rights explained

Tried and tested

Understanding clinical trials

On the agenda

Updates from our AGMs

Muscular Dystrophy New Zealand

InTouch

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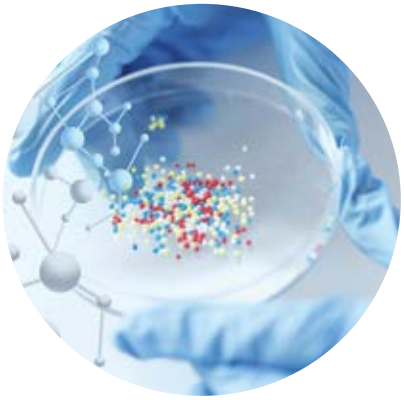

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Muscular Dystrophy New Zealand

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We would also like to acknowledge our corporate sponsors:

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Also thanks to the ANZ Staff Foundation, the Rehabilitation Welfare Trust, the ARA Lodge No 348 IC Charitable Trust, the Clyde Graham Trust, NZ Post Community Post, Auckland Council, Richdale Charitable Trust and the Independent Living Service for their continuing support.

Catching up with Heather

Tēnā koutou katoa, greetings to you all

It is with some sadness that I am writing this column, as it will be my last as the Chairperson of the MDANZ.

My relationship with the association is a long-standing one, going back almost 30 years. I am very proud to be associated with an organisation that has demonstrated such resilience and strength, and delivered such high quality services to our members over many years.

I would like to take this opportunity to thank the departing National Council members for their time and commitment over many years. It can be a tough gig at times, trying to fit these responsibilities into busy lives, but you have all done just that. Thank you!

Welcome to the new National Council Members and congratulations Ken Green, on becoming the Chairperson and Trevor Jenkin on becoming the Vice Chairperson. You will undoubtedly continue with the passion and commitment in your respective roles that I have seen you display as council members. That augurs well for the Muscular Dystrophy Association of New Zealand.

Indeed, I feel I am leaving MDANZ in very good hands. The Chief Executive, the operations team, and the fieldworker service are now well-established in their roles, and services for members are more responsive than they have ever been.

As with any developing entity, MDANZ has further growth and strategic development to do. That is how it always is in the ever-changing world of human services. However, the foundation is solid and well-prepared for what lies ahead.

I wish you all well for the future.

Ngā manaakitanga

Heather Browning
MDA Chairperson

It might be you ...

or a family member, a neighbour or a friend. It could be a wee baby, or a retiree, it could happen at any stage in life.

Muscle weakness and wasting conditions can strike anyone of any age, of any ethnicity. These disabling conditions are called neuromuscular conditions with most but not all being genetic in origin.

We provide services to people with neuromuscular conditions – services that are unique and help them to live their life to its fullest.

You can help by:

- **Telling family members affected by a neuromuscular condition about us.**
- **Supporting our fundraising efforts.**

Muscular Dystrophy Association Patron, Judy Bailey.

In touch with Ronelle

He aha te mea nui o te ao? He tangata, he tangata, he tangata!
What is the most important thing in the world? It is the people, it is the people, it is the people!

I reflected on this Māori proverb, or whakataukī, as I travelled around meeting members and supporters at the annual general meetings (AGMs) held across the country. This whakataukī sums up a foundational principle for MDANZ, as the future of our organisation depends on member engagement and good governance relies on the capability, goodwill and enthusiasm of our committee members. Without people change doesn't happen, and we will be unable to keep moving forward. So it is with this in mind that I extend a sincere thank you to all who have stepped into governance roles to serve for a term.

At this time, we farewell Heather Browning and thank her immensely for the expertise, time and dedicated support she has provided to MDANZ and the operational team during her term as Chairperson. We now welcome Ken Green to the role and look forward to continuing to develop and grow as a member-driven organisation. With Ken's appointment, we have achieved a new milestone of having two individuals with lived experience of a neuromuscular condition in the senior leadership positions of Chairperson and CE. I think this brings another strength to our organisation. To find out more about the outcome of each AGM, see the round up on page 8.

As winter approaches, we have health needs to think about in our community. We had an amazing response to our recent member survey about the pneumonia vaccine. The results are discussed in the MDA News pages and will inform our strategy for improving funded access to the vaccine. The team have also compiled some information about the importance of sleep for this issue. Most of us know that getting a better sleep is important for our energy levels, mood, and overall health. However, in my experience, this is easier said than done when in pain and trying to manage with floppy limbs.

I'm a huge fan of hot water bottles, electric blankets, anti-flamme and multiple pillows to prop up the arms and legs that won't stay in place. Interestingly the team don't recommend wine in their story on page 12, but I know of plenty who do! Whatever you do to get a good night's sleep I hope you keep dreaming, Dream big, and remember when you wake up, you get coffee.

Ngā mihi mahana,
Warm regards

Ronelle Baker
Chief Executive

Support Us

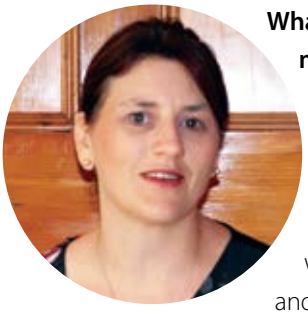
Any donation, big or small makes a difference. Donations of $5 or more are tax deductible.

Call: 0900 426 93 to make an automatic $15 donation.

Online: Donate any amount securely online. **www.mda.org.nz**

A cup of tea and a catch up with … Elizabeth McCallum

Each issue we introduce a MDANZ team member:



How long have you worked for the Muscular Dystrophy Association and what do you do?

My role is Office Manager in our Wellington Branch and I started at the beginning of this year.

What qualifies as a great day at work for you?

Achieving at least one item on my list of things to do.

If resources and funds weren't an issue, what would you like to see our members enjoying?

Freedom! It's such a precious gift that until we lose it, or have it taken away from us, we often don't even realise how much it means.

What's the perfect morning tea for an office shout?

Ah, the perfect morning tea means being a lady enjoying High Tea with precious friends and family (not always possible as we all live in different cities!), whilst the perfect office shout is baking a cake and sharing it with Dympna, plus anyone else who visits us. (Let me know when you're visiting and I'll see what I can do).

What are you passionate about?

Giving those who have so much ability to do so many things, the opportunity to do so. Everyone has a role and part to play, it's up to us to help them explore their qualities and assist them to achieve what they want to.

Hidden treasure

A few months ago, the National Office team set a day aside to do a tidy up. While we expected to find some interesting things, we didn't expect to uncover gems.

At times, it felt like we were going through the annals of MDA history. One ring binder we pulled out was dusty with rusty hinges. Opening it revealed the very first Annual Report and correspondence organising raffles and other events. It seemed like a snapshot in history as we turned the pages that had been created by typewriters, and found addresses that simply had road names with no numbers. Times clearly have changed. This was obvious when we found a photo of an MDA Ball held in the 1970s. My gaze immediately went to the old -fashioned power chair while everyone else in the office laughed at the orange and brown colour scheme and the amazing hairstyles.

It's empowering to go through the archives of our organisation's history because it highlights the passion of many who have been involved in changing the world we live in. There have been many positive changes over the years, from scientific breakthroughs to improved community access. One old article highlighted the ingenuity of a family who created a hoist to go in their van. This is now commonplace. However, what this truly highlights, is the impact that people have on one another and how through working together, we can all make a difference. As an organisation created by members, it is important to remember that each one of us is helping make a better future.

Natalie Brunzel

Old scrapbooks, photos and newsletters are a valuable part of MDANZ history.

Thanks Nic!

Riding to raise money for MDANZ

Nic with Olympic rower turned cyclist Hamish Bond.

Getting up at dawn is challenge enough for most teenagers, but when Nic Brockelbank set his alarm for before dawn on March 23rd, getting up early was the least of his worries.

Nic started training months ago for the early morning event at Cambridge's Avantidrome. He wanted to see how many laps he could cycle in an hour, and asked people to support him by donating money to the Muscular Dystrophy Association.

The event was a huge success. Nic exceeded his goal and cycled 113 laps in the hour. He raised $5660.10 which will go towards helping other young people with muscular dystrophy take part in the Duke of Edinburgh Hillary Award.

There were some high-profile supporters on the day, including Olympic rower turned cyclist Hamish Bond and World team sprint champion Sam Webster. Breakfast TV covered the entire event live.

MDA news

Funding the pneumonia vaccine

Thank you so much to everyone who responded to our email survey about the pneumonia vaccine. It was the biggest response to a survey ever, and shows how important the issue is.

There are two pneumococcal vaccinations available (Prevenar 13 + Pneumovax 23). Both are funded for specific set of conditions that puts an individual at a higher risk of contracting pneumonia, currently this does not include neuromuscular conditions, despite expert opinion recommending both. At the moment, members of MDANZ are encouraged to receive the vaccinations, and we will reimburse them for the costs of approximately $70 for Pneumovax 23.

We wanted to survey our members to find out more about how many of you have had pneumonia, how it affected you, and what you think about the vaccine. Here are some of the findings;

- 31% of the people who replied had been sick with pneumonia
- Of that group, 13% have been hospitalised, with an average stay of between 7 and 13 days and between two and four weeks off work or school
- 72% of respondents had never been informed of the vaccines
- 80% said they would receive the vaccines if they were free at their GP

Survey results will be used in our submission to Pharmac to include those with neuromuscular conditions under the criteria for free vaccines.

Save the date:

Friedreich Ataxia Family Information Day

Saturday 15th July 2017, 10.30am at MDANZ National Office, 419 Church Street East, Penrose, Auckland

Snacks and lunch will be provided for all guests. Speakers include: Professor Martin Delatycki, Murdoch Children's Research Institute; Dr Louise Corben, Murdoch Children's Research Institute Collaborative Clinical Research Network in Friedreich Ataxia (CCRN); Dianne Boon FARA NZ, and Ronelle Baker Chief Executive MDANZ.

All adults and children with Friedreich ataxia are invited to participate in the Friedreich ataxia natural history study. The aim of the study is to assess which assessment scales are the best to test how people are affected with Friedreich ataxia. This is very important in considering drug trials and how to assess the benefit or otherwise of any drug being tested. This study, part of the Collaborative Clinical Research Network in Friedreich Ataxia (CCRN) will help us all move forward to our first treatments for Friedreich ataxia. Let us know if you are interested in participating in this study. We will provide you with more information and confirm your private appointment time of approximately 40 mins with Martin and Louise closer to the event.

RSVP 18th June 2017 by email info@mda.org.nz or phone 0800 800 337.

Please include how many people will be attending with you and whether you wish to be part of the measurement studies.
We have some funding available to help with travel costs.

Update from the NZ Neuromuscular Disease Registry

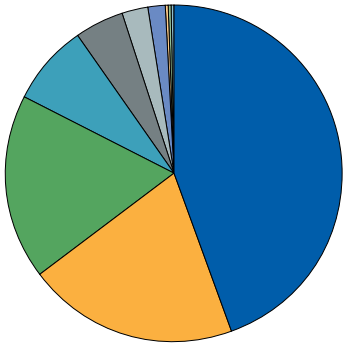
The NZ Neuromuscular Disease Registry is a nation-wide registry for people living in New Zealand with any disorder supported by the Muscular Dystrophy Association of New Zealand.

Its primary aim is to enable people with neuromuscular disease to participate in research and clinical trials. A developing aim of the Registry is to obtain genetic diagnosis for patients who only have a clinical diagnosis.

MDANZ is the primary sponsor of the Registry and it also actively promotes and supports the work of the Registry by informing members about it. This has generated 165 self-referrals to date. MDANZ's Fieldwork Service has also provided an informed consent process to over 420 people. Other sources of referral to the Registry are the Principal Investigator, Dr Richard Roxburgh, who runs neurogenetic clinics in Auckland, other neurologists from throughout the country; Genetic Health Service NZ, paediatric neurologists and prevalence studies, like MD Prev and Impact CMT. GPs, the National Metabolic Service and General Paediatricians have also referred a few people.

Over 1000 people have enrolled since the registry's launch in August 2011 and of these 8 have been involved in clinical trials, 134 in other disease specific research and over 750 have contributed anonymised data to cohort studies. As a result, the Registry has facilitated almost all other neuromuscular research currently taking place in New Zealand in recent years. If you are interested in enrolling please talk to an MDANZ Fieldworker or email the Registry Curator registry@mda.org.nz

Sources of referral to the NZ NMD Registry



- MDANZ Fieldworkers 44.5%
- Principal Investigator 20.3%
- Registry Curator (includes self-referrals) 17.8%
- Neurologists 7.9%
- Genetic Health Service NZ 4.6%
- Paediatric Neurologists 2.6%
- Prevalence studies (AUT) 1.8%
- Paediatricians 0.3%
- GPs 0.2%
- Metabolic Service 0.1%

NZNMD REGISTRY

Introducing …

Gemma Poke, our most recent trustee tells us about herself.

Gemma Poke

I am a clinical geneticist based in Wellington, and see patients across the lower North Island and upper South island. I have worked in New Zealand since 2013. I completed my genetics training in Edinburgh, and prior to that did genetics and adult medical training in Brisbane. I have a longstanding interest in neurology and neuromuscular conditions, dating back to my time as a medical registrar. For the past two years, I have been on the steering committee for the MD-Prev study, and specifically, have been looking at the utility of genetic testing for the diagnosis of genetic muscle disorders. I am in the early stages of supervising a genetic counselling masters student, who will be studying family communication in myotonic dystrophy families. I have a strong interest in the "mainstreaming" of genetics, whereby genetic tests are ordered and interpreted by non-genetics specialists. This requires a close working relationship between neurology and genetics.

AGM news

A great night in Hamilton

The AGM was an inspiring combination of important business and interesting speakers.

The Annual General Meeting (AGM) for the national association was held a little later this year, to allow time for the organisation to transition to a new reporting framework, which required consolidation of financial information across national office, all branches and the research trust.

Members, supporters and staff joined the AGM which was held at the Distinction Hotel in Hamilton on the evening of Friday 29th April. The Chairperson's report was presented by Sophie Tauwehe Tamati on behalf of outgoing Chairperson Heather Browning and Chief Executive Ronelle Baker facilitated the evening. The outcome of the National Council elections were announced and we welcomed Ken Green and Trevor Jenkin to the respective positions of Chairperson and Vice Chairperson. Kerry Stephenson and Jan Daly were re-elected for a further two year term on council and Tristram Ingham was elected as a new Councillor at Large for a two year term.

We were delighted to have a range of informative and inspiring guests, including MDANZ Ambassador Nic Brockelbank who spoke about his interests and what motivates him to keep setting and achieving personal goals. Associate Professor Alice Theadom from Auckland University of Technology shared results from the MD-Prev study, providing new insights into the needs of our community. It was also great to hear that a number of additional sub-studies are now taking place because of the overall success of the MD-Prev study.

Top: Past and present National Council members, and their partners, enjoy dinner.
Above: Speakers Alice Theadom and Nic Brockelbank.

Regular *In Touch* contributor Dr Richard Roxburgh shared his excitement about a breakthrough treatment for SMA and special guest Dr Larry Stern gave closing remarks at the conclusion of the event, acknowledging the work of MDANZ and the high proportion of resources being dedicated to member services, which he felt was unprecedented in similar organisations across the world. Acknowledgements were made to all contributors and outgoing Councillors, and attendees were invited to join in the Freedom waiata (song) at the close of the evening.

Because Ken Green's movement to the role of Chairperson created a casual vacancy on council, Brent Walker was appointed to this casual vacancy by resolution of the council at their face to face meeting on Saturday 29th April, in accordance with clause 11.1 of the MDANZ constitution. Brent will serve a one year term under this appointment and is eligible to seek re-election at the 2018 AGM.

AGM news

From the branches

Northern

The Northern Branch AGM was held at the National Office in Penrose, Auckland, on Saturday 8th April. Pro-bono Accountant Raema Inglis presented a financial summary for 2016 and the positive year-end position was noted as a highlight.

Current serving Chairperson Trevor Jenkin was re-elected into this role, and Joy Jenkin, Andrea Clive, Michael Schneider, Lew Pulman and Rebecca Poad were all re-elected onto the committee. Acknowledgements were given to outgoing committee member Raymond Mok, and the Branch welcomed Corinne King as a new committee member.

Guest speaker Dr. Gina O'Grady gave a presentation on treatments and research in childhood onset neuromuscular conditions.

Wellington

The Wellington Branch AGM was held at the Walter Nash Stadium in Lower Hutt on Sunday 26th March, with National Council Chairperson Heather Browning in attendance. Heather addressed the AGM and spoke about the importance of local governance in a strong member-centric organisation.

Acknowledgements were given to outgoing Chairperson Peter Tegg, Treasurer Dianne McKellar, and long serving committee member Barbara McAdam.

Current serving committee member Annelize Steyn was unopposed in her nomination for Chairperson. Annelize will also take up the role of Wellington Branch Representative on the National Council. Re-elected Branch committee members include John and Lyn Hawkins, Liz Mills and Andrew Holt. New member Jon-Paul Bignold was welcomed to the committee, and volunteer Urmit Patel to the role of Treasurer.

Canterbury

The Canterbury Branch AGM was held at the Papanui Library in Christchurch on Saturday 18th March and attended by members from near and far. The meeting began with a show of respectful recognition for Sue Robinson, a life member and close friend to the Branch.

Longstanding treasurer Phil Austin officially stepped down from the committee, as did former secretary Paul Freeman. Andrew Munro was re-elected as Branch Chairperson and Warren Hall re-appointed as Vice Chairperson and Branch representative on National Council. Long-serving Committee members Vivienne Palmer, Earl and Yvonne Mason were also reappointed. New committee members welcomed at the AGM were Rebecca McClean as Treasurer, Bonny Stephens as Secretary, Paula Holden and Emma Shewan, Delwyn Christie and Kerry Smith. It was noted that having three committee members from the Nelson/Blenheim region was beneficial for broader representation at a branch governance level.

West Coast member Anna Osborne and Chief Executive Ronelle Baker were invited guests who addressed the group. Acknowledgement and flowers were given to Branch Office Manager Eris Le Compte, who is retiring following 11 years of service.

Southern

The Southern Regions Branch AGM was held on Saturday 4th March at the new office premises in Caversham, Dunedin.

After more than two decades serving on governance, Raewyn Hodgson stood down as Chairperson and nominations were called from the floor. Robbie Verhoef was duly elected to the role, with Rebecca Croxen reappointed as Treasurer, and Fi Murch joining the committee as Secretary. Leslie Facoory, Andrea McMillan and Barb Kitto were reappointed as committee members and Shaun Baker was welcomed as a new committee member.

Sincere thanks was offered to Raewyn as the outgoing Chairperson and other committee members Mary Burn, Allan Hodgson and Olivia Samson for their contribution and support in 2016.

The branch reflected on the recent establishment of a fieldworker service and Jo Smith gave an introduction to her role. Chief Executive Ronelle Baker spoke about MDANZ's plans for the coming year and Programme & Service Advisor Miriam Rodrigues gave an update on research and breakthrough treatments for neuromuscular conditions.

BRANCH news

Catching up with news from around the country

The cold fingers were worth it! Everyone had a great day ice skating.

Northern

One of the big events so far this year was a trip to an Auckland ice skating rink. We had members from all over the region – from Kerikeri up north and as far south as Te Awamutu. It was a lot of fun with plenty of skidding, skating, cold fingers and hot chips.

This event is always popular and we're looking forward to having it again in a few months when the weather starts getting warmer again. We're very grateful to Paradice Skating rink for how well they look after us.

support@mdn.org.nz

Southern

It's hard to believe it's six months since the fieldworker service began in Otago and Southland. It's been a busy few months with plenty going on. The office is now fully functional with plenty of space to hold a social get-together. Watch this space for events coming up later this year!

We have had an impressive 16 new referrals in the past six months and fieldworker Jo Smith has been able to provide and outreach service to many of our new and existing members. She has been actively promoting our services by building relationships with key personnel in local hospitals, medical centres and other healthcare practices. She has also had the chance to promote MDANZ on a local radio station.

Networking with other allied healthcare professionals has been key in educating the community about muscular dystrophy and the services that MDANZ provides.

In collaboration with National Office, the Seasons for Growth programme (designed to help adults and children to understand and manage change, loss and grief) will be offered as a pilot to Southern Branch members. We are planning to deliver a three-hour adult seminar in Invercargill and North Otago, with a group in Dunedin. Please contact Jo as soon as possible to register your interest.

Also on the cards is a mens social event planned for the next few months. Let Jo know if you are interested and she will include you in the planning process.

An established coffee group for parents with children with disabilities meets in Dunedin twice a month. This fun and friendly group is run by Parent to Parent. Please contact Jo for further details.

We can also support other members interested in meeting up regularly for social connections. Please make contact and we can work together to make this happen.

September is our awareness month, with the appeal day on Friday 29th. We are looking for volunteers who could spare some time, or donate items for raffles. Your time and generosity is greatly appreciated.

Thanks to everybody who has supported the branch and our fieldworker service in recent months.

joanne@mda.org.nz

Wellington

Wellington members have enjoyed many events recently throughout the region. Penny, our fieldworker based in the Hawkes Bay, enjoyed taking part in an event hosted by Sailability Hawkes Bay. Members certainly tested Penny's skills out on the water. We were a group of 30, so there were lots of boats out that day. Big thanks to Katy and the crew again from Sailability Hawkes Bay for their effort and for pranking the fieldworker with a soaking while sailing by! If you haven't yet come along, we would love to see you next time. Check out the Hawkes Bay 'Sailability Hawkes Bay Sailors' page on Facebook for loads of photos.

office.mdawgtn@xtra.co.nz

Fieldworker Penny Piper and her son James joined members for a great day out on the water.

Canterbury

Our camp was held during the first week in March at Hanmer Springs. It was very successful, even though the numbers attending were down. The highlight was a jet boat ride on the river (pictured) Our next camp will be the children's camp, in October.

All children living in the South Island aged between six and 18 years are invited. This will be from 2nd to 5th October at Mt Hutt Retreat, Methven. Awesome activities are available including archery, frisbee golf, jet boating, camp fire, and hot pool visits to name a few. So if you are interested, contact your field worker or phone the Christchurch office – 03 377 8010 to let us know.

Planning is also underway for a mid-winter barbeque, so pencil Sunday 25 June into your calendars. More details will come later.

At the end of April we said goodbye to Office Manager Eris who has retired. Eris has given 11 years of faithful service to the branch. Thank you Eris!

mdacanty@xtra.co.nz

Jet boating was a camp highlight.

Good night, sleep tight

How to get the rest your body needs

An expert guide to strategies for sleep, and members share their tips.

Sleep is one of life's essentials. In fact, we spend nearly one-third of our lives sleeping, but getting enough sleep can be a major issue for people with neuromuscular conditions. Here's our guide to getting more, better quality sleep.

The importance of sleep

Sleep is important as it affects our health and ability to function and avoid illness by supporting our immune system. Most people know that sleep allows our bodies to rest and conserve energy, but it also decreases blood pressure, heart rate, breathing and body temperature, and helps to regulate appetite and weight, and control blood glucose levels. During sleep our brains remain active - laying down memory, restoring daytime mental function and carrying out tasks that lead to physical growth.

Lack of good sleep can lead to excessive daytime sleepiness, tiredness and lethargy, morning headache, poor memory, anxiety and depression.

Sleep and neuromuscular conditions

Neuromuscular conditions are often associated with difficulty in obtaining a good night's rest and sleep, with respiratory problems often being the culprit. These tend to manifest clinically when the associated condition progresses over time. Early detection and treatment of breathing difficulties are very important to improve sleep and other health outcomes. Many types of breathing assessments can help evaluate your respiratory status, the three most common are a peak flowmeter, pulmonary function testing and pulse oximetry. Ideally, these assessments should be performed at symptom onset, or diagnosis, with regular monitoring by a health care professional.

Breathing and coughing techniques can help maintain healthy lung function. These can be done several times a day by taking five to 10 deep breaths with a short rest in between, to strengthen the lungs and help them expand fully. Self-assisted cough techniques, chest physiotherapy and airway clearance device such as an insufflator-exsufflator, high frequency chest wall oscillation, a flutter and the acapella device can help with cough and airway secretion clearance. Suctioning can also be performed to clear airway secretions.

Ventilation will be recommended if breathing is becoming problematic and is either non-invasive or invasive. Both types require a medical device at some level to assist your breathing by allowing your respiratory muscles to rest. You may start using a noninvasive ventilation device, then progress to a bi-level, positive airway pressure device and eventually an invasive ventilator.

Non-invasive ventilation provides pressure during inspiration and expiration through an external mask, nasal prongs or a sipper tube.

Bi-level devices are positive airway pressure devices that create air pressure and airflow coordinated with your breathing. It delivers inspiratory positive airway pressure (IPAP) when you breathe in, pushing air into the lungs. This is followed by a significantly lower expiratory positive airway pressure (EPAP) that allows you to exhale.

Invasive ventilation requires a tracheostomy tube or ET tube that is inserted directly into your airway to deliver air to your lungs and can be used to partially or fully help your lungs function with condition progression.

It is useful to develop a bedtime ritual or routine so that your body knows you are getting ready to go to sleep.

There is a variety of different **assistive equipment** that may help with getting to bed and getting a good night's sleep. Mechanical beds may help with height adjustment and positioning. Transfer boards and mechanical lifts make it easier and safer for family and caregivers to move you if needed, particularly at night into bed. It is important to ensure you have the right mattresses, pillows and cushioning to allow you maximum comfort for a good night's sleep. It is important to discuss this with your occupational therapist. Your equipment should be regularly reviewed as your needs will change over time with your progressing condition. If you've had equipment for a long time, is also important to check for wear and tear and ensure your equipment is still in good condition for use. Medical alarms to call for help may also be useful if you have limited mobility.

A **mobility program**, which includes various physical and occupational routines, as well as proper positioning,

Managing fatigue is also important so you can get the most out of each day and get to bed in a better state.

energy conservation, breathing exercise and quality of sleep can help lessen some of your symptoms as well. Therapies such as range-of-motion exercises and stretches help prevent freezing of the joints of your knees, hips, feet, elbows, wrists and fingers. This can in turn make you more comfortable at night.

Managing your day is an important factor in getting a good night's sleep. Getting exposure to natural light helps the body to maintain a sleep/wake cycle and recognise when it is day and night. Managing fatigue is also important so you can get the most out of each day and get to bed in a better state. If you can, try to set aside time to rest between performing daily living activities. These activities (e.g., bathing, dressing and eating) should be spaced apart to allow this to happen. When performing them, sit down whenever possible to reduce unnecessary steps. It is a good idea to ask someone to help if the activity causes you to become short of breath. In addition, the time of day you perform various activities should be taken into consideration, because you may have more energy in the morning than later in the day. If you do have daytime naps, limit these to 30minutes max. It is important to slow down in the evening and **avoid labour intensive activities** within three hours of bedtime.

It is useful to develop a **bedtime ritual** or routine so that your body knows you are getting ready to go to sleep. This can include an hour of quiet time before bed, doing things such as reading or listening to music. It is also important to try and keep your sleep regular, with the same bedtime and the same getting up time. Aim for eight hours of sleep each night.

Try to **avoid stimulants** such as caffeine, nicotine and alcohol before bedtime. Watching television, using laptops or mobile phones can also be stimulating and would be good to avoid at least one hour before bed. This is also not a good time to have upsetting conversations as these can stimulate strong emotions that make it hard to sleep.

Nutrition can also have an impact on your sleep and you may want to discuss with your doctor or adietitian if needed. Generally, heavy meals should be avoided within two hours of bedtime. Spicy food, citrus or fizzy/carbonated drinks can cause indigestion and disrupt sleep if eaten close to bedtime.

Quality of sleep can be improved by using **medical equipment** correctly. **Positioning** can also be helpful. By elevating the head of the bed, you may experience less shortness of breath. The head of your bed can be raised by using extra pillows under your head, neck and chest, or placing pillows or blankets under the mattress, or between the mattress and box spring.

Make sure your sleeping environment is pleasant, and think about blackout curtains, adjustment of the room light, temperature and noise levels, which can enhance overall quality of sleep.

For more information please refer to the Healthy Sleep Hygiene factsheet from Respiratory team, Auckland DHB.

Stacy Spence

Stacy, has Duchenne muscular dystrophy and is on a ventilator 24/7. He shares his secrets for a good night's sleep.

The comfort I get in bed is important as I have recently experienced pressure sores from my wheelchair seating. The comfort in my bed, and the lack of pressure, aids the healing of my skin and wounds.

I have trialed different mattresses including air mattresses with little success. They are just not suitable for me. My current sleep system, which I find most suitable for me consists of an electric adjustable bed, Softform Premier mattress with Roho mattress (4 section) over this.

The bed required some modification to the control unit as I do not have the strength in my fingers to use a standard one. This allows me to adjust my back angle throughout the night as needed and with the Roho mattress I find this works very well for me, allowing a comfortable night's sleep.

Natalie Brunzel

Navigating the system when you need a new bed can be daunting, Natalie shares her experience.

We all crave a good night's sleep. Considering the countless bedding options and advancements in technology available, you'd think this would be easy to achieve. However, too much choice can be overwhelming. How do you navigate the system when you need a new bed?

Firstly, you receive a referral to your Community Occupational Therapist (OT), this can come from your doctor, or in my case, via another OT I was working with. Good things take time and in Auckland the waiting lists are long. It took six months before I saw the Community OT. When she arrived, she was brilliant. We discussed all the changes I might need in my home. When we discussed the bedding, the system became a little more complicated. She said we needed an appointment with the Positional Management Clinic.

I had never had an appointment with a positional OT before. I just devised my own system of what felt comfortable, using ordinary pillows of varying sizes which I had acquired over the years. I was slightly nervous about what they would say about this method. However, they could only improve it, well at least that's what I thought. After a lengthy discussion and a thorough examination of my abilities (and limitations), the OT drew up a plan. The next step was to seek funding. My current bed had an air mattress but I had long decided that I didn't want an air mattress anymore. I find them noisy and cold to lie on. I discovered just how cold an air mattress is compared to non-air when I received my first trial mattress. I don't think I will ever go back.

Due to my use of a Vendlet, a system that helps to turn me at night, the bed frame it attaches to needs to be strong enough to hold the weight of the Vendlet. The mattress needs to be under a certain height to ensure the turning function isn't compromised. This is a delicate balance to achieve, especially when you require a soft bed.

During this process, it was established my Vendlet was outdated. Luckily, I had seen a newer version of the Vendlet at the *Show Your Abilities* expo. I noticed the new one had motors attached to each arm of the Vendlet, this is a new feature now available.

After a lengthy discussion with the Vendlet supplier, they said this type of turning system would not work for someone that transfers into bed. So, I would not be able to up-grade as this would mean that I wouldn't be able to transfer safely in and out of bed. However, the Sales Representative assured me a basic system is coming on the market soon. The motors would not be on each arm of the apparatus, making it friendlier for people who transfer in and out of bed.

I'm still going through this process, but have learned you need to ask questions and keep asking questions. Trust your instincts. None of the positional pillows that were ordered for me were suitable, and we have decided to keep using my system. Do what works. Remember, you're the one who has to sleep in your bed each night!

The life-changing power of research

A signficant way to support and advance the treatment of neuromuscular conditions

A guide to understanding what's involved in taking part in clinical research and knowing what to expect.

About clinical research

Clinical research is medical research that is carried out on humans. Individuals volunteer to participate in studies that aim to uncover better ways to treat, prevent, diagnose and understand human disease.

Clinical research includes both clinical trials that test new treatments and natural history studies, which provide valuable information about how diseases progress.

If you are considering taking part in a clinical trial, the doctor in charge of the trial will give you a lot of information about the treatment being tested, the possible results and the possible side-effects. It is always worth finding out as much as you can before you agree to take part.

The most comprehensive online listing of trials is at www.clinicaltrials.gov where you can search for trials for a particular condition. There is an ethical requirement for trials to be listed on the clinicaltrials.gov website or an equivalent type of website.

BEWARE

If you are considering participating in a clinical trial that is not listed on the clinical trials website be suspicious that it may not be an ethically approved clinical trial. Ask the researchers why their trial is not listed and talk to your doctor about it.

You will never have to pay to participate in an approved clinical trial. If a company is suggesting that you will have to pay to participate you should reconsider participation.

What is a clinical trial?

A clinical trial is a rigorously controlled test designed to examine the safety and/or effectiveness of drugs, devices, treatments, or preventive measures in humans. Clinical trials follow a strict protocol to ensure that the testing is completed as quickly and safely as possible and accurately answers the questions being asked. Although the start of a clinical trial is cause for optimism, it must be remembered that only 20 percent of all studies are successful and therefore they aren't a guarantee for a treatment.

What do I do if I want to participate in a clinical trial?

Join the NZ NMD Registry. You can do this by talking to an MDANZ Fieldworker or by contacting the NZ NMD registry curator via email: registry@mda.org.nz

You can also directly contact the centre or the company involved in the clinical study. They will get in touch with your local doctor whose involvement is essential.

What are the different phases of a clinical trial?

Phase I is usually quite small and almost always designed purely to assess the safety of the new treatment and how well it's tolerated. Phase I studies are usually done using healthy volunteers.

Phase II can last up to two years. It tests the effectiveness of a treatment on a larger number of patients. Participants are sometimes divided into groups and the benefit of the drug is compared to a placebo, which could be described as an 'empty' drug and is sometimes talked about as a 'sugar pill' or a 'sham procedure'. Usually the patients don't know whether they have been given the real drug or the placebo. The trial is then known as a 'blinded study'. Phase II trials are sometimes divided into phase IIa and phase IIb.

... people taking part in clinical trials are followed up using even more stringent assessments than usual, even after the trial has finished.

Phase IIa is specifically designed to determine the best dose of the drug.

Phase IIb is specifically designed to study how well the drug works at the dose determined in the phase IIa study.

Phase III involves a larger number of patients and follows the same process as Phase II. This step can take two to three years. The aim is to gain a more thorough understanding of the effectiveness and benefit of the drug and to test whether it's as good as, or better, than the current standard treatment.

Phase IV evaluates the long term risks and benefits of the drug once it's available on the market, also known as the post-marketing phase.

Why participate in a clinical trial?

People have different reasons. Some want to have a more active role in their own health care or would like to benefit from new research developments before they become more widely available. They should keep in mind that although the start of a clinical trial is a very promising sign, it isn't a guarantee for a treatment. Another advantage is that people taking part in clinical trials are followed up using even more stringent assessments than usual, even after the trial has finished. This close attention could result in better management of the condition.

Clinical trials inevitably carry a risk and so it is very important that an informed decision is made. Understanding the details of the clinical trial process and the impact it has on participants and their families is essential before a final commitment is made.

What are the risks and disadvantages of taking part in a trial?

Many people are understandably eager to get involved in clinical trials and it's very important that they understand what is involved. They should discuss the study in detail with the trial nurse or doctor as well as their own doctor and family before giving their consent to take part.

The main disadvantage is that studies often involve multiple and frequent visits to hospital. This is obviously not always easy or practical. Procedures could be painful, for example injections and biopsies and, of course, there is always the risk of an adverse reaction to the treatment. Participants in a trial also have to keep in mind that the treatment they receive might not provide any direct benefit for them – there is a chance they might be given a very low dose of the drug, or even a placebo.

Who can participate in a clinical trial?

All clinical trials have guidelines about who can take part. The factors that allow someone to participate in a clinical trial are called 'inclusion criteria' and those that disallow someone from participating are called 'exclusion criteria'. These criteria are based on such factors as age, gender, the type and stage of a disease, previous treatment history, and other medical conditions. Before joining a clinical trial, a participant must qualify for the study.

Participants in a trial also have to keep in mind that the treatment they receive might not provide any direct benefit for them.

Inclusion and exclusion criteria are not used to reject people personally, instead they:

- Keep the participants safe, for example another underlying condition could make participation in the trial dangerous.
- Help ensure that the researchers are able to produce reliable results and therefore get the treatment to market as quickly as possible so that the wider population can benefit.
- Increase the reliability of the results by ensuring that everyone taking part has similar symptoms at the beginning of the trial. Otherwise it is difficult for the

researchers to interpret the results because they won't know if the reason one patient responded to treatment and another didn't is due to the drug or differences in their condition to begin with. This is especially helpful in the early phases of a clinical trial when there are only few participants.

In most circumstances, people who wish to participate in a clinical trial will find it easier if they live relatively near the team of people who are conducting the research, because they need to be monitored frequently. The clinical trial organisers will usually reimburse reasonable travel costs.

What do I need to know before I enrol in a clinical trial?

You should know as much as possible about the clinical trial and feel comfortable asking the members of the healthcare team and the research team questions about it.

The following questions might be helpful to ask:

- What is the purpose of the study?
- Who is going to be in the study?
- Why do researchers believe the experimental treatment being tested may be effective?
- Has it been tested before?
- What kinds of tests and experimental treatments are involved?
- How do the possible risks, side effects, and benefits in the study compare with my current treatment?
- How might this trial affect my daily life?
- How long will the trial last?
- Will hospitalisation be required?
- Who will pay for the experimental treatment?
- Will I be reimbursed for other expenses?
- What type of long-term follow up care is part of this study?
- How will I know that the experimental treatment is working?
- Will results of the trials be provided to me?
- Who will be in charge of my care?

Miriam Rodrigues

Auckland Powerchair Football Club Incorporated (APFC)

Powerchair football is a competitive team sport for people with physical disabilities who use powerchairs for their mobility. The rules are similar to outdoor soccer with a few modifications. The game is played in a gymnasium on a regulation basketball court. Two teams of four players use their powerchairs to attack, defend and spin-kick a 13 inch football in an attempt to score goals. Every powerchair has a guard to hit the ball.

Want to join our practice and give it a go?

If you're keen to be part of a competitive and fun team sport, use a powerchair, are aged 8 or over, and can communicate with other players and have sufficient control of your wheelchair, this is a great way to make new friends.

Next Training Practices: Guards are supplied

Sunday 14 and 28 May
Sunday 11 and 25 June
Sunday 16 and 30 July
from 1pm-3pm

Auckland Spinal Rehab Unit Gym
30 Bairds Rd, Otara

Contact: Jan Fels on 027 428 0272
or felsp@slingshot.co.nz
Jenny on aucklandpfc@gmail.com
Find us on Facebook @AuckPFC
APFC Website https://auckpfc.wordpress.com

Follistatin gene therapy improves muscle function of people with IBM

A naturally-occurring protein used in trial

Promising results from a phase 1 trial for sporadic inclusion body myositis (sIBM) have been published in the scientific journal, *Molecular Therapy*. The trial tested the safety of a follistatin gene therapy, developed by Milo Biotechnology in USA.

Follistatin is a naturally-occurring protein that blocks myostatin. Myostatin is a protein that limits muscle growth and stops our muscles from becoming too big. Blocking myostatin allows the muscles to grow and become stronger, which could potentially be beneficial for people with muscle-wasting conditions such as IBM.

The follistatin gene contains the instructions to make follistatin protein. Injecting a copy of the follistatin gene via a virus vector into the muscle could increase the amount of follistatin protein and help to build muscle.

The results from the trial showed that the follistatin gene therapy was safe and well-tolerated. It also improved the participants' muscle function.

Biopsies from the trial participants' muscles also showed that the follistatin gene therapy reduced fibrosis (scarring) and improved muscle regeneration.

In October 2016, follistatin gene therapy received Orphan Drug Designation from the FDA. This is also now being trialled for Becker and Duchenne muscular dystrophies, as well as IBM. R

Molecular patch therapy for DM1

As with most neuromuscular conditions, myotonic dystrophy type 1 (DM1) is caused by genetic defects and treatments remain symptomatic. Researchers are therefore continuing to strive towards the development of treatments that directly target the genetic cause of the condition. This may have the potential to reverse, or at least slow down, the deterioration of tissues affected by the condition, including the movement muscles, heart, diaphragm and brain.

DM1 is caused by an expansion of DNA within the DMPK gene. This leads to the production of mutant DMPK RNA, which gets trapped in the nucleus and has toxic effects to the cell.

Professor Matthew Wood at Oxford University is developing a molecular patch that could be a potential treatment for people with DM1 that binds to the mutant DMPK RNA and blocks its toxic effects. The molecular patches will be linked to short fragments of protein called peptides that ease delivery into cells of muscles and other parts of the body. Mouse models will be used first to establish safety, efficacy and dosing.

This research will also help to further enhance molecular patch technology, which will be beneficial for the neuromuscular field in the long-term. R

ACE inhibitors help Duchenne and Becker muscular dystrophy

Managing heart muscle weakness

A phase 3 trial, recently published in the journal, *JAMA Cardiology*, has found that treatment with ACE inhibitors before heart weakness is detected is likely to be beneficial for people with Duchenne and Becker muscular dystrophy.

ACE inhibitors are drugs that widen the blood vessels, making it easier for the heart to pump blood around the body. People with Duchenne and Becker muscular dystrophies eventually develop weakened hearts (cardiomyopathy) and so are often prescribed ACE inhibitors.

There are currently no clear recommendations about when people with Duchenne or Becker muscular dystrophy should start ACE inhibitor therapy. This study aimed to find out

whether it might be beneficial to begin ACE inhibitor therapy before the onset of heart weakness.

The researchers used Magnetic Resonance Imaging (MRI) to assess the heart health of 42 teenagers with Duchenne or Becker muscular dystrophy. This was carried out at the start of the study and then again two years later. At the start of the study, participants did not have detectable heart weakness but did have some fibrosis (scarring) in their hearts.

The progression of heart fibrosis was much slower in participants who received ACE inhibitor therapy compared to those who did not. Fibrosis stiffens the heart and increases the risk of heart failure, so slowing its progression is extremely beneficial.

Although this study involved small numbers of participants, its findings are important for determining how cardiomyopathy is best managed in Duchenne and Becker muscular dystrophies. It suggests that ideally people with these conditions should begin ACE inhibitor therapy when fibrosis is first identified and before heart weakness occurs.

FDA to review Soliris for MG

Alexion Pharmaceuticals recently announced that the FDA will review its investigational drug eculizumab (brand name Soliris) for the treatment of refractory generalised myasthenia gravis (MG) which occurs in a subset of MG patients. An FDA decision on the drug is expected in October. The company also has submitted a marketing application to the European Medicines Agency (EMA) to gain approval for the drug in Europe.

Both the US. and EU marketing applications are supported by comprehensive data from the phase 3 REGAIN study, which showed clinically meaningful improvements such as an improvement in the Quantitative Myasthenia Gravis (QMG) score, a physician-administered test that assesses MG severity.

Soliris works by targeting part of the immune system called the complement system, which is responsible for helping antibodies clear damaged cells and potentially toxic microbes that could cause infections. In MG, antibodies whose job it is to target these toxic pathogens instead inappropriately recruit the complement system and target the space across which nerve fibers transmit signals to muscle fibers, called the neuromuscular junction (NMJ). Soliris is thought to work in MG by inhibiting the complement pathway to prevent destruction of the NMJ, and is a promising treatment for the condition which has rarely responded to other medication available.

Research into treatments for SMA

SMA is our *Condition in Review* this issue (see page 24).
Here's a guide to the latest research and drug approval for the condition.

In December 2016 the U.S. Food and Drug Administration (FDA) approved nusinersen (Spinraza ™) as the first drug approved to treat children and adults with spinal muscular atrophy (SMA). The drug is administered by intrathecal injection into the fluid surrounding the spinal cord. It is designed to increase production of the full-length SMN protein, which is critical for the maintenance of motor neurons.

Similarly in Europe, the European Medicines Agency (EMA) recommended Spinraza for approval following assessment of two pivotal controlled studies, ENDEAR (infantile-onset SMA) and CHERISH (later-onset SMA), which both demonstrated the clinically meaningful efficacy and favorable safety profile of SPINRAZA. A decision from the European Commision to make this available is expected in the next few months.

Avexis-AAV9 vector gene therapy is another new therapy delivered intrathecally. This consists of vectors that introduce SMN1 gene, and needs to be performed in early phases of the condition before motor neuron dies. This drug has had significant positive results in mice models with a single injection. Phase 1 trials in humans are ongoing in 15 infants less than 6 months of age. Early data from the trial has indicated positive results with the oldest being 21 months of age, 2 of whom can walk independently and none are on permanent ventilation.

Motor scores also continue to improve.

Currently cellular and molecular studies are being conducted to seek understanding of the mechanisms that trigger motor neurons to degenerate.

Scientists are developing a broad range of model systems in animals and cells to investigate disease processes and expedite the testing of potential therapies. Animal models of SMA are critical tools in discovering and developing new therapies. Scientists have developed new zebrafish, mouse, and pig models, including models of less severe SMA types 2 and 3, which may greatly facilitate the identification of new therapeutic targets and candidate therapies.

Researchers are also looking for biomarkers for SMA.

Recent trials involving pharmaceuticals in SMA included celecoxib, riluzole, pyridostigmine and 4 Aminopyridine, olesoxime, Sodium Phenylbutyrate, a combination of valproic acid/valproate and carnitine/levocarnitine, growth hormone, hydroxyurea, and in men a combination of levoprolide and testosterone.

Monitoring SMA Drugs

A SMA Registry workshop held in Amsterdam in May has produced an updated core dataset for the TREAT NMD Global SMA Registry. This will enable the global Registry to be utilised for the ongoing monitoring of people with SMA who are taking medications newly approved by the regulators, such as Nusinursen (also known as Spinraza in the USA), to treat their SMA. Miriam Rodrigues, Curator of the NZ NMD Registry, was one of a number of attendees at the workshop, which was organised and funded by Biogen and TREAT NMD.

IMPROVING QUALITY OF LIFE


Bariatric Lux/Ultra Low


Vendlet V5S


Diagonal Toilet Lift


Ropox All-in-One Hoist

Bariatric Lux - The Bariatric Lux 4 section electric bed offers both functional design and excellent quality. The beech wooden paneled bed ends have been developed to offer the user an attractive domestic look . The extra sturdy frame allows a safe load of up to 318 kg. Operation via the hand control allows the occupant to select various positions as required. The bed offers two various heights ranging from 42 cm and 81.5 cm (LOW VERSION:25.9 cm-62.5 cm). The mattress platform also offers trendelenburg positions. Two further actuators beneath the platform allow full profiling capability of the backrest and knee break features.

Vendlet V5S/Speed Adjust - The New VENDLET V5S is an automatic patient turning system for moving and handling the bedridden client with limited resources. When the bars are raised the VENDLET V5S works as a side rail. The side rail function is tested and approved by TÜV. The speed adjustment makes it possible to reduce the speed of the bars to 75 percent or 50 percent of the normal speed. Each system is supplied with 2x slide sheets and 2x Turning sheets as standard.

Diagonal Toilet Lift - Toiletlift Diagonal is based on the natural movement of standing up, offering optimum support, with both feet firmly planted on the floor, during the 'stand up' phase. An important feature of this Toiletlift is the ability to adapt to the users specific height and weight. With no bar in front of the Toiletlift, the user is able to move their feet back to facilitate 'evacuation'. Ideal for users who have multiple sclerosis, Motor Neurone disease, post Polio, Hemaplegia, Strokes and other disabling conditions. The weight rating of the AEROLET Diagonal is 150 KG with a bariatric version rated at 250 KG also available. The toilet lift can also be fitted with a Bidet.

Ropox All-in-One Hoist - Our "All-in-One" active hoist can be changed from a standard hoist to a standing hoist without the use of any tools. As a Combined patient- and stand-up hoist the product provides excellent support and safety for the user and it improves the working area conditions for the helper when lifting the patient in different situations.

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Spinal Muscular Atrophy (SMA)

Understanding the recessive genetic disorder

Spinal muscular atrophy (SMA) is a recessive genetic disorder. It is caused by a loss of anterior horn cells (spinal motor neurons). Messages from the nerve cells in the brain (called upper motor neurons) are transmitted to nerve cells in the brain stem and spinal cord (called lower motor neurons) and from them to specific muscles. Upper motor neurons instruct the lower motor neurons to produce movements in the arms, legs, chest, face, throat and tongue. This loss of spinal motor neurons means that there is a lack of nerve signal from the spinal motor neurons to the muscle, which causes the muscle to waste away (atrophy) leading to weakness. The muscles affected are used for activities such as crawling, walking, sitting up, and controlling head movement. In some cases of SMA, the muscles used for breathing and swallowing are affected.

Approximately 1 in 6,000 to 1 in 10,000 babies are born with SMA.

Causes of SMA

SMA Types I-IV is a result of a lack of a protein called survival motor neuron protein (SMN). The reduced amounts of this protein result in the spinal motor neurons not being maintained properly and they die. This in turn leads to atrophy of the muscle because the muscle is no longer being innervated. SMA is an autosomal recessive condition meaning that an affected person has inherited a faulty SMN gene from each of his or her parents.

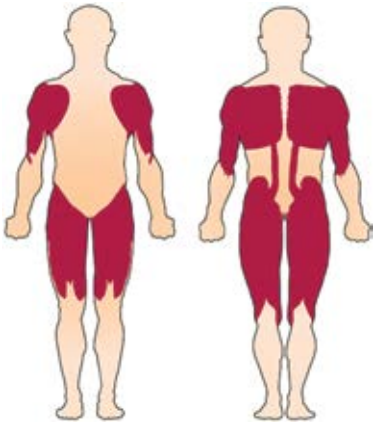


Image credit: mda.org

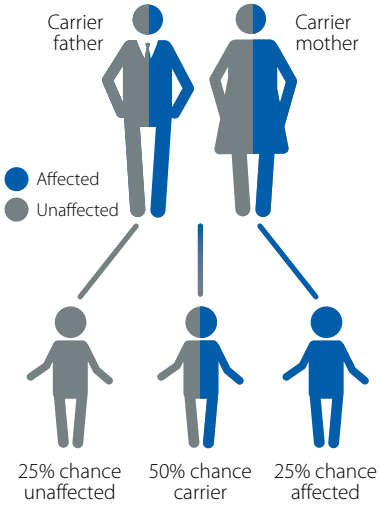
Approximately 1 in 6,000 to 1 in 10,000 babies are born with SMA.

Levels of SMN protein is primarily controlled by the *SMN1* gene and influenced by the *SMN2* gene. Alterations in *SMN1* causes SMA and the number of copies of *SMN2* influence the severity of the condition with type 1 being the most severe through to type IV being the least severe. The *SMN2* gene makes some SMN protein but most of what it makes is non-functional and is discarded by the cell. The small amount of functional SMN that *SMN2* produces influences the severity of SMA, with the greater the number of *SMN2* copies a person has the less severely affected they will be. The extra amounts of survival motor neuron protein produced by the 3 or more extra copies of the less efficient *SMN2* gene has a protective effect and the severity of the condition is reduced.

Other forms of SMA include; X-linked spinal bulbar muscular atrophy (Kennedy's disease), dominant spinal muscular atrophy with affected lower extremity (SMA-LED) are all caused by defects in different genes.

Inheritance Pattern

Spinal muscular atrophy is inherited in an autosomal recessive pattern, which means both copies of the *SMN1* gene have mutations that affect the production of SMN protein from the gene. The parents of an individual with an autosomal recessive condition usually each carry one copy of the mutated gene, hence they are called carriers. Carriers typically do not show signs and symptoms of the condition as they each still have one functional gene, which is sufficient to avoid symptoms. Two carrier parents



When two carrier parents have a child, there are three possible outcomes. With each pregnancy, there is a 25% chance that the child will be affected.

have a 1 in 4 chance (25%) in each pregnancy of having a child affected with the condition. In rare cases (approximately 2%) people with the condition have a new defect in one of their genes. In this situation only one parent is a carrier and the other defect occurred randomly within the individual. This is called a *de novo* mutation (new and not inherited).

Diagnosis of SMA

The diagnosis of spinal muscular atrophy (SMA) is established in individuals who have;

- A history of motor difficulties
- Evidence of motor unit disease on physical examination
- Mutation detected in the *SMN1* gene (this can be carried out via a blood test or saliva sample) which indicates whether there is a deletion or other mutation of the *SMN1* gene. This test identifies at least 95 percent of SMA Types I, II, and III.
- Electromyography (EMG) records the electrical activity from the brain and/or spinal cord to a peripheral nerve in the arms or legs that controls muscles during contraction and at rest and nerve conduction velocity studies (NCS), which measure electrical energy by assessing the nerve's ability to send a signal.

Individuals with SMA type II often have average intellectual skills during the formative years and above average by adolescence.

Features of SMA

Type I spinal muscular atrophy (also called Werdnig-Hoffman disease) is a severe form of SMA that is evident at birth or soon after birth and results in a rapid decline and death within two years. Affected infants may initially meet developmental milestones but then quickly decline and fail to meet motor milestones although they remain bright and have expressive faces. They have poor muscle tone (hypotonia) and most are unable to support their head or sit unassisted due to the severe muscle weakness. Babies with SMA type 1 have respiratory difficulties and usually require ventilation. They often have swallowing problems that may lead to feeding difficulties, choking or gagging. Other features can include small local involuntary contractions of the tongue, mild contractures often at the knees and an absence of tendon reflexes.

Type II spinal muscular atrophy is characterised by muscle weakness that develops in children between ages 6 and 12 months. Children with type II can sit without support, although they may need help getting to a seated position but they never achieve unaided walk. Fasiculations or trembling of the tongue and finger trembling is almost invariably present and approximately 70% of individuals have an absence of tendon reflexes. Individuals with SMA type II often have average intellectual skills during the formative years and above average by adolescence.

Type III spinal muscular atrophy (also called Kugelberg-Welander disease or juvenile type) has milder features that typically develop between early childhood, after ten months of age, and adolescence. Individuals with type III spinal muscular atrophy achieve walking but may lose this ability over time. Type III is characterised by proximal limb weakness with the legs more severely affected than the arms.

Type IV spinal muscular atrophy or adult onset spinal muscular atrophy is a relatively mild condition with affected individuals usually experience mild to moderate muscle weakness, tremor, twitching, or mild breathing problems with the onset

of these signs and symptoms not occurring until adulthood. Typically, only muscles close to the center of the body (proximal muscles), such as the upper arms and legs, are affected in type IV spinal muscular atrophy

Management of SMA

Treatment for SMA involves multi-disciplinary management of the symptoms and prevention of complications.

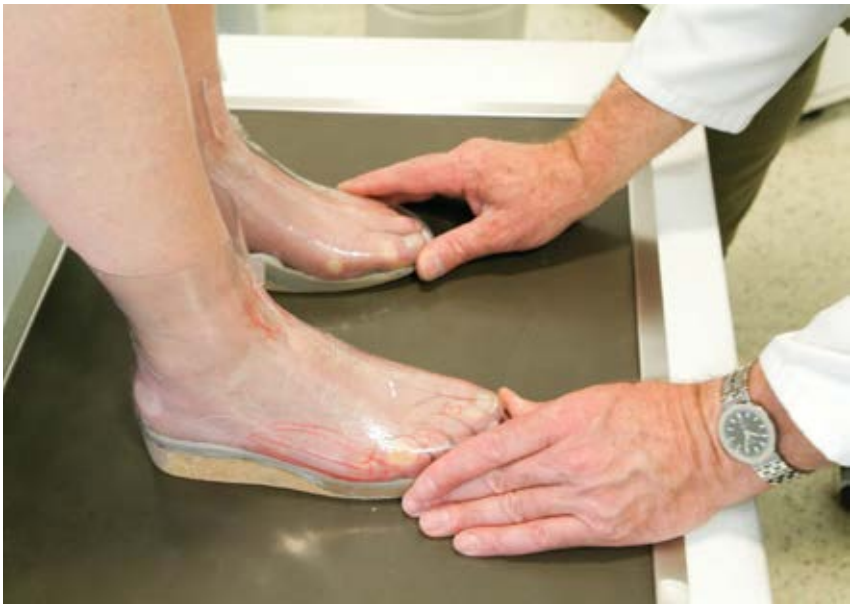
Poor weight gain, sleep difficulties, pneumonia, scoliosis, and joint contractures are common complications and management revolves around preventing or treatment of these.

Physiotherapy, occupational therapy, and rehabilitation may help to improve posture, prevent joint immobility, and slow muscle weakness and atrophy. Stretching and strengthening exercises may help reduce spasticity, increase range of motion, and keeps circulation flowing. Applying heat may relieve muscle pain.

Assistive devices such as supports or braces, orthotics and wheelchairs may helpful in maintaining mobility.

Good nutrition and a balanced diet are essential to maintaining weight. Chewing and/or swallowing difficulties can be addressed with input from speech language therapy and dietetics. When nutrition is a concern in SMA, placement of an naso-gastric feeding tube or gastrostomy (PEG) may be appropriate.

It is vital that respiratory problems are well-managed. As respiratory function deteriorates, ventilation, via tracheotomy or non-invasive intermittent positive-pressure breathing device, is effective.



Assistive devices such as supports or braces, orthotics and wheelchairs may be helpful in maintaining mobility.

Weakened muscles may lead to curvature (scoliosis) of the spine. Surgery for scoliosis in individuals with SMA II and SMA III can be carried out safely if the forced vital capacity is greater than 30%-40% and can help maintain adequate respiratory function.

Muscle relaxants such as baclofen, tizanidine, and the benzodiazepines may reduce spasticity. Botulinum toxin (Botox therapy) may be used to treat jaw spasms or drooling. Excessive saliva can be treated with amitriptyline, glycopyolate, and atropine or by botulinum injections into the salivary glands.

Regular evaluation by a multi-disciplinary team every six months, or more frequently for children who are weak, to assess nutritional state, respiratory function, and orthopaedic status (spine, hips, and joint range of motion) is recommended.

Genetic counselling is available to families who have a diagnosis of SMA. This service provides information, helps families understand inheritance patterns and what this means in their family, as well as enabling people to make informed family-planning decisions. ®

Living and hoping

Brent Walker shares his journey with SMA, the power of mainstream schooling, and the exciting potential of treatment breakthroughs.

I was born with SMA type 2, but back in 1994 it took a while to get a diagnosis. In fact, many specialists told my parents I was simply being lazy. It wasn't until two years later, in March 1996, that genetic testing confirmed SMA.

My parents found this a shock, but also a relief. It meant finally getting to the bottom of what was wrong with their son. They had many questions as to what the future might hold. But having such a complex disease in the mid 90's, meant there was no sign of a cure or treatment on the horizon.

Living with SMA brings countless challenges. However, having said that, I don't usually think of them as "challenges", but rather things I have to work through in order to have a better quality of life.

Some of the most significant obstacles my family and I faced were during my school years, making sure I was included. Attending school camps every year required a huge amount of effort from my parents, as well as from me. It wasn't simply a case of making it to camp, but it was about being included in as many of the daily activities as possible, while keeping up with my daily cares in a new and often not particularly accessible environment.

Being mainstreamed right through my school years was one of the biggest and best experiences I could have had. It made me feel like any other student. And hopfully, it didn't just benefit me, because I think it helped change the perceptions many people have.

By the end of sixth form, I was pretty much over school. As soon as I achieved NCEA level two, I was gone, heading straight into part-time work at my parents' growing business. Over the next three years, I developed many skills and got a taste of the real world outside of school.

This led to me deciding to move out of home in Te Awamutu and to Hamilton. Things happened very quickly. My initial plan was to spend a few years living in group care houses. But when the perfect two-bedroom unit became available, I jumped at the opportunity. Skipping the transitional stepping stones, and moving straight to my own place, filled me with confidence, and I knew this big step was the right one.

My most recent big accomplishment was travelling to the USA last year for a holiday and the chance to visit a close friend, who also has SMA. I went with the assistance of my 19-year-old brother and one of my amazing support workers. The checklist of things you have to think about for someone like me travelling abroad is brain-melting. For most of my life, that job was left up to Mum. But by the age of 22, I needed to take on the responsibility myself. It also meant I could get up to things Mum wouldn't approve of!

Brent is keenly watching treatment developments for SMA.

I am now more focused on my health. This new focus came at the end of last year, with news that a new drug, Spinraza was approved by the FDA in the USA. For the first time in my life, it meant there was some hope.

I didn't use to pay much attention to treatments in development. I preferred to live in the here and now. But I have spent hours researching this drug and think it is looking positive.

I recently caught up with my former paediatrician to see what he thought. His opinion was positive, but he cautioned me that the initial price of this particular treatment could be the biggest barrier. For now, it's a waiting game, hoping for the New Zealand government and its agencies to approve this drug in the near future.

GUEST
speakers

Restoring balance in an online world helps teens struggling with negative thoughts.

Helping teens struggling with depression

RAYMOND MOK

Sparx is a free online tool developed by the University of Auckland to help teenagers who are struggling with depression and stress.

Disclosure: I hadn't heard about Sparx until I was asked to write a review on it, and I am not a teenager.

I find Sparx more like a short course, than a game, in the sense that it is obviously intended to be a tool for teenagers to learn skills that will help them feel better mentally. Sparx seems less entertaining than most of our modern commercial video games. It has a low difficulty level and only basic 3D graphics by today's standards. It has many other game characters you can interact with, but it is a single player game. The upside is that the player can easily learn some ways of thinking and doing things to cope with life, without spending too much time on a game. Anyone can play Sparx on a computer – you don't have to be depressed to play it. The things taught in Sparx are not new ideas to me. However, it is useful in reminding me to keep active and get help. Sparx also teaches you how to help others feel better. .

What does Sparx involve? The objective in the game is to restore the balance of a "game world" that has been plagued with negative thoughts symbolised by "Gnats". To progress in the game, the player has to get rid of Gnats, solve simple puzzles, obtain positive "Sparks" and collect "Power Gems". Sparx offers a form of Cognitive Behavioural Therapy (CBT), which shows you ways of thinking and acting to improve how you feel. CBT can come in the form of counselling, self-help books, or a computer program. It is one of the main recommended treatments for young people and adults with depression. Through a guide (a game character), Sparx teaches you how to relax, keep active, relate to others, solve problems, keep life balanced and get help.

Just before playing the first level and twice more throughout the course, you will be asked nine multi-choice questions regarding your mood. For example: Over the last two weeks have you been feeling down, depressed, irritable, or hopeless? A) Not at all. B) Several days. C) More than half the days. D) Nearly every day. Then Sparx will tell you whether you might be stressed or down or you might have some signs of depression. However, Sparx emphasises that it does not give diagnosis and that only a health professional can diagnose depression. If you don't find Sparx helpful, they recommend speaking to a counsellor, doctor, youth worker or calling Youthline or Lifeline 0508 4 SPARX (0508 477 279). Call 111 if you or someone else might be unsafe right now. The only thing I find inadequate is the options to answer the questionnaire. There's no option in-between "Not at all" and "Several days".

I probably wouldn't find Sparx useful if I was very depressed. In that case, speaking to a real person who can understand is more useful. However, Sparx does encourage its users to seek help as needed.

Sparx is a useful tool, that looks like a game, for some people with depression and stress to feel better. It may not work for everyone, but there are many other options in terms of getting help.

Raymond was born in Hong Kong, grew up in Auckland and lives in Hamilton. He lives with Duchenne muscular dystrophy. His hobbies include blogging, songwriting and Toastmasters. He has a degree in Computer Science and a Postgraduate Diploma in Business Administration.

On board for a successful trip to Wellington.

My challenge to you

OLIVIA SHIVAS

I've heard lots of stories about travelling with a disability. Unfortunately many of them are about bad experiences – but not all of them.

A few weeks ago, I went to Wellington for the day to speak at a journalism conference. I have travelled by myself before, but I'm always a bit nervous because regardless of whether you have a disability, things don't always go to plan. However, everything did go to plan! The airport staff were amazing, the flight crew was helpful, and taxi drivers knew how to fold my wheelchair without me having to explain. While it's beneficial to bring up negative experiences and discuss solutions when things don't go to plan, I think it's also important to bring attention to positive things.

Talking about travel, I'll never forget my longest trip away when my family and I lived in Malaysia for six months. My dad had resigned his job of 20 years, we had rented out our family home and my brother and I signed up to correspondence school lessons. We volunteered at Bethany Home, a school for kids with disabilities set in the countryside. My mum is from Malaysia so it was also a great opportunity to spend time with her family. Every day I would do my school work in the morning, then in the afternoon I would help out at the school, doing anything from feeding another child their lunch, to teaching a class a new game, or cleaning up the equipment room.

As a wheelchair user, it would be easy for me to think I can't help others in need.

One thing I learnt on this trip was that we all have skills we can use to help other people, whatever our abilities are. As a wheelchair user, it would be easy for me to think I can't help others in need. But I can actually do a lot. I discovered I could share the rules for a fun game even if it was something physical I couldn't play myself, and I could empathise and understand what other young people were going through, and share advice.

The purpose of volunteering at this school certainly wasn't so I could feel better about myself. But it opened my eyes to what it's like to live with a disability on the other side of the world, and to be grateful for what I do have. It was a time when I focused on the needs of others, and was able to forget the problems in my own life.

Making friends at Bethany Home.

My challenge to you for the next couple of weeks is to do something kind for someone else, whether it be playing board games with residents at a retirement village, or baking cookies for teachers at a local school. We can all do something to make another person smile.

Olivia is the Rangatahi representative on National Council. She lives with central core disease and has a passion for seeing young people reach their full potential. Olivia has a Bachelor of Communication Studies and works at Attitude Pictures, a TV production company that promotes the stories of people with disabilities.

Ask the **PANEL**

Your tenancy rights explained

DR HUHANA HICKEY

Q: What are my rights as a tenant if I have a disability? Also, if my condition deteriorates, can I expect my landlord to pay for modifications such as a ramp to the front door or allow them to be fitted if the funding comes from elsewhere?"

As a person with disabilities, it is notoriously difficult to get any modified housing in the private sector or in Housing New Zealand. There are some disability agencies who run social housing for their communities, such as IHC and CCS Disability Action, but it pays to know your rights, no matter who your landlord is.

Here are some basic rights:

A tenancy is a contract between a landlord and a tenant. It outlines particular conditions of a tenancy and both the landlord and the tenant must sign the agreement, names of who is involved, a list of any chattels, and the date the tenancy begins and ends (if it is fixed term). The contact address for the landlord must be documented on the agreement.

There are four types of tenancies:

1. Fixed-term tenancy – this is where there is a specific timeframe around being in a property. If you wish to leave the tenancy at a different date, you must apply to the tribunal to have that done.
2. Periodic tenancy – only ends when the landlord or the tenant ends the tenancy.
3. Service tenancy – this is an employer/employee arrangement of housing via employment. Services are covered under the Residential Tenancies Act.
4. Boarding house tenancy – covers boarding rooms in a house with shared facilities for more than 28 days.

When paying rent, the landlord can increase the rent but it has to be six months or more between each rise, and can only happen after the landlord has given 60 days' notice.

A bond is usually paid and is up to four weeks rent. It must be lodged with the Ministry of Business, Innovation and Employment within 23 days of the bond being paid. If there is any damage to the property, the landlord can keep the bond if it's deemed to cost that amount, or more, to repair any damage.

Landlords cannot discriminate on the grounds of sex, ethnicity, religion, marital status, age or even if you are unemployed. If it's an accessible house, they cannot deny you because of disability.

The landlord is required to advise you of any difference to the contract, and of any changes. If you feel you are facing discrimination, the best way is to lodge an application for a hearing at the cost of $20.44, so you can get an impartial ruling.

Any modifications needed?

This is difficult because if you don't own the home, the landlord must give permission for any changes to be made to the dwelling.

If the landlord won't pay for the modifications, the funding will likely come from the Ministry of Health or ACC (depending on criteria). If you apply to the Ministry of Health and they fund the modification, this will come out of your allocation for funding, and in most cases you can only get it once, unless you can make a case for further funding in the future, if your needs change and you have to move house.

To discuss modifications or any of these issues, you need to talk to either your Ministry of Health provider or your ACC provider.

Dr Huhana Hickey MNZM has a background in human rights and disability law, she is currently a post doctoral research fellow at AUT where she is studying the health and disability needs of whānau hauā. She remains committed to ensuring all persons with disabilities and their whānau know of and have access to their rights.

Curing the incurable

DR. RICHARD ROXBURGH

What we're learning about genetic conditions.

Neurogenetic conditions are the most curable incurable diseases. A real life example of this is the story of spinal muscular atrophy, which began unfolding over 100 years ago. In the 1890's two European neurologists, Guido Werdnig followed by Johann Hoffman, described spinal muscular atrophy in babies.

More than 50 years later, in 1956, Swedish neurologists, Lisa Welander and Erik Kugelberg described spinal muscular atrophy in older patients. They understood that this was a similar sort of disorder that Werdnig and Hoffman had described decades earlier because by then neurophysiological tests had been developed. These new kinds of tests showed that this was a nerve problem causing muscle weakness rather than the disease being primarily in the muscle (like in the muscular dystrophies – which can look very similar clinically).

Evidence continued to grow during the 1960's with doctors describing families with members who experienced severe early onset SMA and members with mild later onset disease. It was becoming apparent that both the severe condition occurring in babies and the mild later onset disease were caused by the same thing.

Thirty years later the gene for SMA called SMN1 (short for survival of motor neuron 1) , was discovered by linkage of autosomal recessive SMA that led to a particular place on chromosome number 5. Over the following years the genetics were further elucidated.

Individuals affected with SMA have pieces missing from each of their two copies of survival motor neuron gene but some are very severely affected while others are not so. This is because just upstream of the SMN1 gene is another gene called SMN2 which is identical to SMN1 except at one position in the gene which means that most of the protein made from this gene is incomplete. However about 10 percent of the protein is the full working version of the SMN1 protein – just like what would have been produced by the SMN1 gene had it been working. It is possible to have several copies of the SMN2 gene and it has been shown that each of them can produce a little full length SMN1 protein and that, on average the more copies a person has the less severe is their disease.

Knowing the gene for SMA meant that the human gene could be used in animal models to learn more about how it worked and to screen for potential treatments. For example, the human gene could be inserted into mice and then the mice tested to see if they could be cured. If the drugs pass tests in mice then they may be able to be further developed in humans.

Nusinursen is one such drug. Nusinursen is a treatment for SMA now approved by the USA's FDA for all types of SMA. Nusinursen is like a Bandaid plastered over the defect in the SMN2 gene so that it produces more of the full length SMN1 protein.

Nusinursen needs to be administered directly into the fluid that surrounds the spinal cord and it needs to be regularly administered – currently the recommended dosing is about four injections a year.

Another gene- based therapy currently being trialled, the Avexis Trial, involves a one-off injection into the spinal fluid of a harmless virus carrying a functional SMN1 gene that is thought to integrate into the patient's own genetic code and start producing SMN1 protein.

The success of both these treatments illustrate why genetic diseases are the most curable incurable diseases. Firstly we know exactly what the first step is that has gone wrong in the disease and has caused all the problems. Secondly we can make incredibly accurate models in various animal models which we can use to understand how that first genetic misstep leads to the clinical condition, and so that when medications are successful in these models they are likely to be effective in humans. Thirdly patients can take part in drug trials and be sure that they genuinely do have the condition, and not some other lookalike condition.

Dr. Richard Roxburgh FRACP PhD is a Consultant Neurologist at Auckland Hospital.

Muscular Dystrophy New Zealand

About us

MDANZ is a trusted source of specialist information and provides a range of free services and practical support for individuals, families and whānau with lived experience of rare neuromuscular conditions.

The Muscular Dystrophy Association of New Zealand Inc., commonly known as MDANZ, began in the late 1950. Since then MDANZ has broadened its scope to support many other neuromuscular conditions. We are proud to have Judy Bailey and Dame Susan Devoy as our longstanding patrons.

Our unique governance structure ensures leadership of the organisation by individuals and family members with lived experience of a neuromuscular condition. We have four regional branches that are supported by the National Office based in Auckland.

We want New Zealanders with lived experience of neuromuscular conditions to experience freedom of choice in a responsive society.

To achieve this mission, we provide;

- Free information and advice, through our website, an 0800 info line and in paper booklet form
- A nationwide fieldworker service for personalised support
- Free loan of resources, such library books, recreational beach chairs and cough assist machines
- Funded support for counselling
- Discretionary funding for life enhancing resources not covered by government
- A high quality quarterly magazine to inform and inspire our membership and broader communities of support
- Funding for neuromuscular research and a mechanism to help New Zealanders to access clinical trials and new treatments
- Education workshops for members, health professionals, schools and others
- Advocacy and lobbying at a community or national level
- A platform for support groups and peer to peer networking

MDANZ is a registered charity and relies almost entirely on donations from the public, trusts and other businesses/ organisations to continue its work in the community.

Our Team

Ronelle Baker
Chief Executive

Miriam Rodrigues
Programme and Service Advisor

Brian Hadley
Accountant and Business Manager

Chris Light
Member Resource Assistant

Miriam Hanna
Information and Resource Coordinator

Amanda Lam
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Council Representatives

If you want issues brought to National Council meetings, talk to your branch representative. They have the responsibility to raise your issues at National Council meetings and to make sure you are heard. Your branch representatives and their contact details are as follows:

Northern Branch

Trevor Jenkin. Ph: 021 267 4380
Email: trevor.jenkin@gmail.com

Wellington Branch

Annelize Steyn. Ph: 021 480 108
Email: kilmarnock.annelize@gmail.com

Southern Branch

Robbie Verhoef. Ph: 021 044 9437
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Canterbury Branch

Warren Hall. Ph: 03 329 4390
Email: warrenjh@xtra.co.nz

Conditions covered by MDANZ

Muscular Dystrophies:

Becker Muscular Dystrophy

Congenital Muscular Dystrophies and Congenital Myopathies

Distal Muscular Dystrophy

Duchenne Muscular Dystrophy

Emery-Dreifuss Muscular Dystrophy

Facioscapulohumeral Muscular Dystrophy

Limb-Girdle Muscular Dystrophy

Manifesting carrier of Muscular Dystrophy

Myotonic Dystrophy

Oculopharyngeal Muscular Dystrophy

Diseases of the Motor Neurons:

Spinal Bulbar Muscular Atrophy (Kennedy's Disease and X-Linked SBMA)

Spinal Muscular Atrophy - all types including Type 1 Infantile Progressive Spinal Muscular Atrophy (also known as Werdnig Hoffman Disease)

Type 2 Intermediate Spinal Muscular Atrophy

Type 3 Juvenile Spinal Muscular Atrophy (Kugelberg Welander Disease)

Type 4 Adult Spinal Muscular Atrophy

Hereditary Spastic Paraplegias (HSP)

- all types:

Also called Familial Spastic Paraparesis

Leucodystrophies

- all types.

Metabolic Diseases of muscle *- all types including:*

Acid Maltase Deficiency (also known as Pompe's Disease)

Debrancher Enzyme Deficiency (also known as Cori's or Forbes' Disease)

Mitochondrial Myopathy (including MELAS, MERRF, NARP and MIDD)

Phosphofructokinase Deficiency (also known as Tarui's Disease)

Phosphorylase Deficiency (also known as McArdle's Disease)

Diseases of the Peripheral Nerve:

Charcot-Marie-Tooth Disease (CMT) (Hereditary Motor and Sensory Neuropathy) - all types

Dejerine-Sottas Disease (CMT Type 3)

Hereditary Sensory Neuropathy

Inflammatory Myopathies:

Dermatomyositis

Inclusion Body Myositis

Polymyositis

Diseases of the Neuromuscular Junction:

Congenital Myasthenic Syndrome

Lambert-Eaton Syndrome

Myasthenia Gravis

Myopathies *- all types:*

Andersen-Tawil syndrome

Central Core Disease

GNE Myopathy

Hyperthyroid Myopathy

Hypothyroid Myopathy

Myofibrillar myopathy

Myotonia Congenita (Two forms: Thomsen's and Becker's Disease)

Myotubular Myopathy

Nemaline Myopathy

Paramyotonia Congenita

Periodic Paralysis

Inherited Ataxias:

CANVAS

Friedreich Ataxia (FA)

Spinocerebellar Ataxia (SCA)

Neurocutaneous Syndromes *- conditions affecting the brain and the skin:*

Central Cavernous Hemangioma

Neurofibromatosis Type 1

Neurofibromatosis Type 2

Schwannamatosis

Tuberous Sclerosis

Von Hippel Lindau Syndrome

Should you have a query regarding a condition not listed please contact us on 0800 800 337 or email info@mda.org.nz

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Answers: TA versus other powerchairs

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