InTouch

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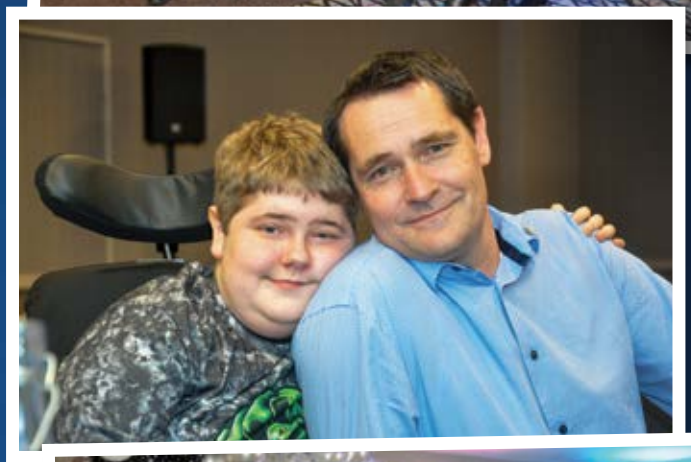

60 YEAR MDA 1959-2019 ANNIVERSARY

Our Jubilee and Conference

A crusader for the cause

Queen's Birthday Honour for a tireless advocate

Celebrating each other's strengths

With friendship, laughter and trust

Brothers in arms

Knowing your limits, but never giving up

Muscular Dystrophy New Zealand

InTouch

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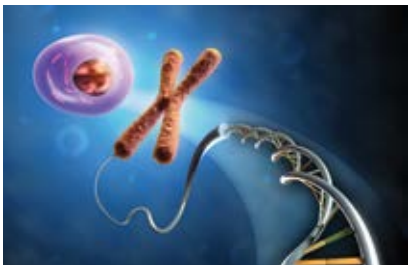

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Also thanks to Allied Medical, Biogen and Sanofi Genzyme, the ARA Lodge No 348 IC Charitable Trust, the Clyde Graham Trust, NZ Post Community Post, Auckland Council, Richdale Charitable Trust and the Independent Living Service for their continuing support.

Korero with Trevor

Let's get out there and educate our wider community this September.

The 2019 Neuromuscular Education & Advocacy Seminar which took place on August 3 was, in my mind, MDANZ's best event yet. It was great to see so many of our members, whānau and health workers attend.

The exciting programme and line up of speakers aimed to improve the health and wellbeing and advocacy skills of individuals, families and whānau living with neuromuscular conditions, through research, education and collaborative practice and I'm sure everyone went away with more skills and knowledge.

Over that weekend, we also celebrated MDANZ's 60th Anniversary with a fantastic Jubilee event. It was wonderful to celebrate and reminisce on how far the association has come in 60 years.

I am excited to be leading the organisation into the next phase of growth and pledge to use my business experience and knowledge to help it become even stronger and more sustainable for the next 60 years ahead.

Spring is a busy time of year for MDANZ as we head into the annual appeal month in September. In recognition of global awareness days for several of the conditions that MDANZ covers, this September we will be holding a month-long Freedom campaign with a key focus on the week of 6th – 13th September.

Last year, we were really impressed by how our members and supporters got involved in our conversation about Freedom. We want to keep the conversation going for another year and ask, "What does Freedom mean to you?"

Let's join our voices and raise awareness about our needs and aspirations. Join us to raise awareness and vital funds for the thousands of Kiwis affected by neuromuscular conditions.

There are many ways you can get involved, whether it be through social media, volunteering to fundraise for your local branch, displaying a collection box in your workplace or holding a mufti day at school.

Let's get out there and educate our wider community this September.

Ngā mihi mahana,

Trevor Jenkin
National Executive Chairperson

The seminar: Trevor Jenkin and council member Tristram Ingham.

SMA funding decision seen as devastating

Families consider moving overseas for treatment.

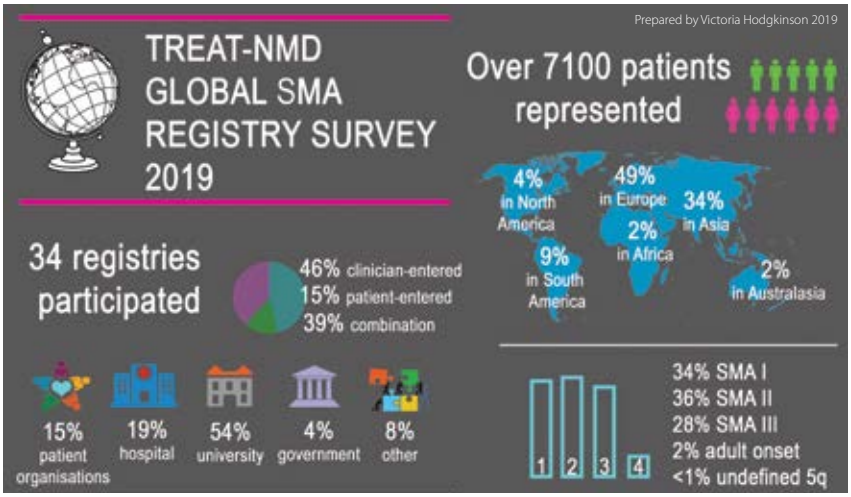
Pharmac and funding for medicines for rare disorders has been making news headlines a lot.

MDANZ members with spinal muscular atrophy (SMA) are working hard to impress upon the decision-makers the importance of access to treatment. A survey sent out by MDANZ in June to families with SMA, to inform a presentation to the Health Select Committee, told us that 100 percent of people completing the questionnaire were aware of nusinersen (also known as Spinraza) as a treatment for SMA.

Upon learning that the treatment was effective in slowing or halting the progression of SMA 95 percent felt positive or hopeful.

Ninety six percent felt hopeful when they learned that Spinraza was being considered by Pharmac for funding. But when they learned that the decision to fund had been deferred 91 percent felt negative or despondent.

One quarter have considered or sought professional support such as counselling to cope with the situation and one fifth thought that a family member had sought counselling to cope with the situation.



Two thirds of the adults with SMA and all of the parents of children with SMA have investigated moving overseas to access treatment and the majority (86 percent) do not have confidence in the current Pharmac model. When asked to describe in their own words the impact on them of Pharmac's deferral of a decision on Spinraza, the term 'devastating' was most frequently used. Some families said that the situation was causing them to leave the country and one family said they will sit out two years in Australia to meet the residency requirements in order to access treatment for their child.

The treatment clearly works, is life-saving and restores physical abilities to people with SMA. New Zealand needs to get on board with the rest of the world.

Explore, discover and live

If you are looking for any information, advice, support or equipment relating to disability in New Zealand then the national disability information website https://firstport.co.nz is a great place to start. The website, which replaced the Weka website, includes extensive lists of disability support services for each region which Firstport says can provide you with the specialist knowledge, advice and resources you need for your individual circumstances.

It holds information about a whole range of relevant topics so you can "access all you need to know in one place. From transport to education, funding assistance to housing modifications, Firstport can help you find what you need".

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

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Thank you. We greatly appreciate your support.

Bradley's enthusiasm, humour and kind-hearted thirst for life recognised

The discretionary fund has been renamed.

The MDANZ Members' Discretionary Fund has been renamed The Bradley Jenkin Memorial Fund in recognition of the years of unpaid service and commitment made by Trevor and Joy Jenkin, and in keeping with the generous spirit of giving demonstrated by their late son Bradley, who sadly passed away from complications of Duchenne muscular dystrophy aged 17.

Dr Tristram R. Ingham, the vice-chairperson of MDANZ's National Council, said the decision was made by a resolution in council and that all other award criteria will remain the same, with the fund awarded quarterly.

Tristram said it was a great pleasure for the National Council "to be able to make this gesture for Trevor and Joy, not only as an enduring legacy for their hard work and sacrifice, but because Bradley's enthusiasm, humour, and kind-hearted thirst for life embodied the MDANZ vision of 'freedom beyond limits'".

Bradley Jenkin.

The last funding announcement on May 22, coincided with what would have been Bradley's 21st birthday and, in future, the second round of the four funding rounds each year would occur around this date.

The council also appointed Joy Jenkin (councillor-at-large) to the award panel with full voting rights. N

New DMD group for members

Your association is establishing a Member Reference Group for Duchenne muscular dystrophy made up of individuals and parents of children with lived experience of DMD.

The aim of the new group is to bring a consumer perspective to the prioritisation and implementation of the DMD-related activities undertaken by MDANZ.

The formation of this group also reflects our core MDANZ values of Empowering/ Whakamanatanga, Proactive/Kōkiritanga and Connected/Tūhonotanga.

If you're interested in learning more please email: info@mda.org.nz with 'DMD Member Reference Group" in the subject line. N

Duke of Edinburgh: An adventurous journey

Leadership lessons, sailing, virtual reality gaming, a wild scavenger hunt and adapted yoga and meditation were just some of the adventures our Dukies undertook.

At the end of 2018, our five Dukies were nearing completion of achieving their bronze in the Duke of Edinburgh's Hillary Award and an adventurous journey was the final step they needed to undertake.

The November 30 to December 2 adventure presented each of them with a variety of activities and workshops to challenge their thinking and teamwork and take them out of their comfort zones.

Day one saw Jack Lovett- Hurst and Grace Chapman take flights to Auckland for the adventurous journey, while Dylan Schneider and Ella Mills made their way to the Waipuna Hotel & Conference Centre to start the journey officially. This was the first time the Dukies could meet and get to know one another.

Ronelle Baker facilitated the first workshop, which included discussions and brainstorming around what leadership means, before the Dukies picked Ella as their leader for the scavenger hunt.

Next up was an adapted yoga, stretching and meditation session with the first day ending early to allow each dukie to get some rest before a busy few days ahead.

Day two saw four training topics covered – food and nutrition, first aid and personal safety, map reading and route finding to get the group ready to travel to One Tree Hill where the scavenger hunt took place.

Larking around at the Maritime Museum.

Ella was given the first clue and she worked with each Dukie to quickly solve it and head to the next area. Each clue included a specific challenge to solve in order to lead them to where the next clue was hidden.

It was a wet and windy day and the Dukies supported each other and moved together as a team. Towards the end of the scavenger hunt, they picked up the pace to cross the finish line just in time for a delicious lunch hosted by Joy and Trevor Jenkin.

As a reward for finishing the scavenger hunt our Dukies then headed to a virtual reality gaming experience in Mt Roskill to have some fun. This was again designed to take them out of their comfort zones and try something completely different. Those playing had to wear goggles and use a remote to control the virtual reality world.

At first the Dukies were somewhat hesitant and unsure, but by the end they had spent nearly an hour chatting, laughing and enjoying the new activity.

Day 3: The last activity on the adventurous journey was a sailing experience on the *Ted Ashby* with the Maritime Museum which saw the Dukies sail across Auckland's Waitemata Harbour. Grace helped the crew to get ready for sail on the way out and on the way back in. Once back on land, the Dukies toured the museum.

MDANZ would like to thank Ronelle, Marty Price and Amelia Noyes for preparing and planning the adventurous journey. Thank you to Joy and Trevor for their support throughout the weekend and also to our amazing Dukies and their parents.

A cup of tea and a catch up with Natalie Foote

Each issue we introduce a MDANZ team member.

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

I started in November 2018 as the board secretary and I am now working as the marketing and communications manager and executive assistant to the national executive chairperson.

What qualifies as a great day at work for you?

A great day at work means ticking all the boxes off my to-do list, working with the marketing and fundraising

team to implement our goals and supporting Trevor so he can fulfill his busy and demanding role.

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

Supportive, equal and inclusive opportunities enabling our members to fulfil their passions.

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

A freshly baked homemade cake to share.

What are you passionate about?

Getting my work/life balance right by spending quality time with my family and friends, reading, baking and a weekly run to clear the head.

Have licence, will drive

Getting a full driver licence is no mean feat.

Melanie Wickenden (nee Eggers) has just passed her full Driver Licence and it has been quite a journey, she explains.

As a first step when Melanie, who featured in the Winter 2019 issue of *In Touch*, went for her learner's Driver Licence she downloaded an app on her phone and iPad and practiced every minute she had to spare. And when she went for the theory test she achieved 100 percent. Melanie tells *In Touch* she then had her learner's licence for two years and rode a moped during that period so she could learn the road rules and get more confident. Her birth father Simon took her for her first drive in his

car on Easter weekend and they used the empty carpark of a local store.

"I also got driving lessons, a local driving instructor was great at helping me as I couldn't retain information very well due to short term memory loss after having my brain surgery. My husband Dave helped me learn as well.

"I kept reading my road code and made sure I knew the rules, but Dave had a heart attack on Christmas Day and was sent to Wellington Hospital and when we came back he couldn't drive, so that meant I had to."

Melanie, who has glycogen storage disease, went for her restricted test but failed and two weeks later tried again and passed. "I then got my car (Doris) a Mazda Demio and drove everywhere. Two months later I passed my full licence first time."

She says while she had been quite content thinking she would never drive, "I wake up every morning with a smile knowing I have my full licence and my own car. I really enjoying driving. The inside of Doris is all pink. Why? Because I can."

Freedom
Muscular Dystrophy Association NZ

We are nearly there: Our Freedom Campaign 2019

Our month-long Freedom Campaign in September will celebrate the triumphs and challenges our members face as we work to raise both awareness of neuromuscular conditions and funds for local services.

The vision of 'Freedom beyond limits' is at the heart of everything we do at MDANZ to help our members have the freedom to make their own choices and decide for themselves how to best live.

This is why our Annual Freedom Appeal is so important.

We kick it off on September 6 for the start of an intensive week of activities focused on our national fundraising with big pushes on social media, on the crowd funding platform Givealittle and through the branch events outlined on the next page.

We will be celebrating our strong and courageous members by sharing their stories on social media and at the same time raising awareness of neuromuscular conditions.

Ensuring that people throughout New Zealand are aware of the conditions that our members live with is important so that we can have open conversations about how our members' lives can be enhanced through advocacy, changes in legislation and better care and knowledge.

Fundraising is our other focus as it ensures the sustainability of our organisation so our members can continue to access services such as our fieldworkers, free counselling, funding opportunities, this *In Touch* publication along with great social events.

This dual purpose allows us to provide further opportunities for our members.

We'd love you to come along to the fundraising events and support your community so please make sure the dates on the next page are in your calendar.

And watch out for us on social media leading up to the events as a 'like' or 'share' can go a long way.

And we can't thank enough the many volunteers, both in governance and on the ground, that give their time and effort, year after year, to continue this meaningful work.

If you want to get involved, in any capacity, please contact your local branch or fieldworker to express your interest. Alternatively, you're welcome to register your interest through our website www.mda.org.nz.

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RESPIRATORY TECHNOLOGIES

Freedom diary dates

Put these dates in your diary for our upcoming Freedom Appeal. It's going to be the best one yet.

6th Sept and 7th September - Airport collections between 8am and 5pm.

7th Sept – DMD Awareness Day

7th Sept – Southern Community BBQ at Dunedin Bunnings

Come along and grab a snack while supporting muscular dystrophy at the same time.

16th Sept – Southern Quiz Night in conjunction with Parent to Parent at Northstar Oamaru

How good is your general knowledge? Enjoy a laugh and a drink in support of two amazing organisations.

22nd Sept – Northern High Tea in Central Auckland

Calling all ladies to a Sunday afternoon gathering with small sandwiches, scones and tea, of course.

24th Sept – Southern Quiz Night at the Kensington in Dunedin

Another test of your general knowledge. Maybe you'll be better than the first time or just coming back for more.

29th Sept – Limb Girdle Awareness Day N

The Queen's Birthday Honour is not the first award Allyson Lock has received. In February this year she was awarded the Rare Disease Day Patient Advocacy and Support Award, which she received from Governor-General Dame Patsy Reddy at a special ceremony at Government House.

A crusader for the cause

Celebrating a tireless advocate for people with Pompe disease

Becoming a Member of the New Zealand Order of Merit in the Queen's Birthday Honours for her services to people with rare health disorders hasn't stopped Allyson Lock in her crusade.

The listing in the Queen's Birthday Honours 2019 under the Member of the New Zealand Order of Merit read: "Mrs Allyson Sarah Lock, of Masterton. For services to people with rare health disorders." And no one could have been more surprised than Allyson.

She tells *In Touch* she never expected to get such an honour.

"Other people get that sort of thing and I don't feel it is deserved. It's crazy that I can get that honour advocating for a drug they won't give me."

She adds she is grateful for the honour but will be more so when Myozyme for Late Onset Pompe Disease (LOPD) gets funding.

Allyson has been advocating for Pharmac to fund Myozyme since 2010 when she found she had LOPD and, after joining MDANZ, she went on to establish the Pompe Network New Zealand, which offers "support, information, friendship and hope" to people affected by Pompe.

There are currently only 12 people diagnosed with Pompe in New Zealand which the network's website describes as "a rare, inherited and often fatal disorder that disables the heart and muscles".

Allyson currently has a petition before Parliament calling for Pharmac to fund Myozyme and urging the Minister of Health to provide additional funding to Pharmac to enable the drug to be subsidised.

And while she has petitioned Pharmac for nine years, she has no idea why it keeps getting knocked back.

"They keep moving the goal posts. I just keep knocking on the door but they make all sorts of excuses. They say there is no evidence that it works, but 75 other countries fund it."

She says she is tired of fighting but has to hope it will happen.

Allyson even went as far, at one point, to ask representatives from Pharmac to afternoon tea at her home "to see me with my family and see how it affects our family".

"I said to them thank you for coming, this is not an ambush, but please explain it to me and my husband, in front of my sons."

As Allyson sees it, it is not just Pharmac but also wider Government funding that is the issue.

After her diagnosis she luckily qualified for a clinical trial for a new ERT (enzyme replacement therapy) in 2011 in Florida and after six months there, she continued to travel to Brisbane for the treatment until a site was set up in New Zealand.

She says this trial medicine kept her "alive and functioning and stopped me going downhill any further" for five years until the trial was cancelled. She was without any treatment for nine months and was back to being bed ridden and "could either have a shower or make dinner, but not do both. One or the other was all I could manage".

But in early 2017, Sanofi Genzyme offered people with Pompe in New Zealand access to its International Compassionate Access Programme for the drug Myozyme and Allyson has improved since.

Today, while she has a cleaner, she manages her home on the rural outskirts of Masterton, with her husband Steve and two teenage sons, two noisy Alexandrian parrots and sundry other animals. Her eldest son is living in Auckland.

She now uses a mobility scooter as her balance is bad and it is a safety issue. "But I have lot more energy these days and love to zip-zap around and get things done."

Allyson, her husband Steve and sons Ben and Josh. Her eldest son, James, lives in Auckland.

While she has known she has Pompe for nine years it was a long road to get the initial diagnosis.

This included numerous trips to doctors and specialists over the years and she says when she was 26 some in the medical profession "started to think I was nuts. They thought I was making things up … they thought I was faking it.

Life is good on the outskirts of Masterton.

She was 45 when she was diagnosed and she says it was actually a relief to have a diagnosis after two decades of seeking help.

"You do start to doubt yourself. And you get a real aversion to going to the doctor and I still have that, despite having a great GP now."

She was 45 when she was diagnosed and she says it was actually a relief to have a diagnosis after two decades of seeking help.

"You have to go with your gut when you know something is wrong. Keep pushing and try not to let [doctors] make you feel like you are a pest."

Both parents have to be carriers of Pompe and each child has a 25 percent chance of being affected. Her twin brother has it too, but not as severely as Allyson. Her sons are not affected.

Although she had the symptoms in her 20s, it didn't

dawn on her that she might have a rare disease. And this is borne out by other people's experiences including a Southland woman who, after seeing a television item on Allyson, realised she had the same symptoms.

And despite having access to Myozyme now Allyson is still pushing for Pharmac to fund the medication. She doesn't want anyone who has Pompe to "feel that their country doesn't think they are important".

"If I was hurt, if I was a drunk driver and killed someone, they would still save my life but I was hurt through nobody's fault and they say we are not helping you."

Her other work has also included helping set up the Gaucher Association of Australia & New Zealand.

"Because I had set up one group, patient advocates reached out and asked me to help set up their association. So many people have helped me, I like to pay it forward. That is really important," she says.

Oliver Groom and his support person Nicole Grimme out for lunch in Kerikeri with Nicole's dad, Stuart.

Celebrating each other's strengths

Friendship, laughter and trust

Friendship, laughter and deep mutual trust and respect lie at the heart of what makes the team of Oliver Groom and his support person, Nicole Grimme so successful.

There is a lot of laughter and fun coming down the phone line as *In Touch* talked to Kerikeri-based Oliver Groom and his main support person Nicole Grimme.

And the close friendship forged over the past 10 years is obvious as they talk about how their shared sense of humour helped them click straight away.

But there is also real respect as Nicole talks about Oli's inner strength after a recent major medical event and his strong musical talent. (See box story.)

"Our focus is on not what he can't do. Instead we work around things and work on ways to make something happen. We don't let things stop us," she says.

And they have gone somewhat out of Oli's comfort zone in recent years, including a cruise around the Pacific and a South Island motorhome trip, with Oli saying Nicole is good at encouraging him a bit further. "In a good way; most of the time".

They also point to out that the mutual trust they've built up means Oli has undertaken adventures he might not have done so with other people because they know each other so well.

While it is harder to get out and about just now, Oli points to a cruise around the Pacific Islands to Vanuatu as a recent highlight.

He says there were good facilities for wheelchairs on the ship, although there were a couple of places they couldn't disembark because of the access. One memorable moment was "sitting at the back of the ship in the middle of the ocean - it was something I had never imagined was achievable".

The pair have also been to MDANZ camps as well as around the South Island in a mobility campervan.

Oli says this was an awesome trip as they went down to his brother's wedding in Queenstown for a couple of weeks.

"It's such a great way to travel, there was plenty of room and a hoist shower and rails and a hook-up sling across the ceiling to the bed."

Oli, who has Duchenne muscular dystrophy, now finds it harder to get out and about after complications with a PEG tube in his stomach.

Nicole says he had massive internal bleeding and blood transfusions, but surgery wasn't possible because Oli couldn't go under an anaesthetic.

Since then he has not been able to be in his wheelchair for very long periods as his breathing gets worse when he is upright. For his last hospital visit they had to pre-arrange an ambulance to Auckland.

At present, Oli needs 24/7 help with the majority being provided by Nicole, backed up by other support people, and his mother, Clare, helps out when she can. The house Oli lives in is owned by his grandfather and Oli also shares it with three flatmates.

"Our focus is on not what he can't do. Instead we work around things and work on ways to make something happen."

Oli says having flatmates helps pay the bills and his sister also lived there until quite recently. The house is also home to Oli's two cats, one is a 'guard' cat called Kat .

They joke too about some of the less than ideal support people 27-year-old Oli has encountered over the years.

There was one who was drunk when they arrived for a shift and passed out on the couch. So, Oli had to call Nicole in. He says it was lucky he was on the computer and could message Nicole.

There was another who waved herbs around as she thought this might "cure" Oli.

"She didn't last long," he says.

And while they do joke about the mishaps, saying they could write a book about it, they add that the support services from MDANZ and CCS Disability Action have been fantastic.

The pair first met when Oli was in high school as Nicole, who emigrated from the United Kingdom about 16 years ago, was a teacher aide at the school at the time.

Oli had spinal surgery in 2007, and in their 10 years together Nicole has also become Oli's advocate around issues like funding and other support services. But, most importantly, his friend.

A passion for music

Due to health reasons it is harder for Oliver Groom and his support person Nicole Grimme to get out a lot now, but Oli keeps very busy with his great passion, music. He released his first album in 2014 called Lucid Frequency under the name State of Flux (See https://soundcloud.com/state-of-f)

Nicole says Oli is a very talented musician and composer but that he doesn't recognise this talent himself. Oli will only admit to *In Touch* that he's is something of a perfectionist around his "electronic chill out" music which he composes on his computer.

Oli also works on his music with friends playing the guitar and Nicole's daughter sang on one track. The Northern MDANZ office has also helped him publicise his music to members.

Current musical favourites are Fat Freddy's Drop and Salmonella Dub and the last local concert he attended was at Western Springs in Auckland to hear Guns N' Roses.

They laugh again when they recall one concert they attended where the accessible space was a hard wooden stand knee-high off the ground which, needless to say, no one in a wheelchair could get on to. They sent a letter of complaint after that experience.

Have you used your Discount Card yet? If not, you're missing out!

How does your Muscular Dystrophy Member Discount Card work?

Simply show the supplier account number on the card to the cashier OR enter the promo code listed on the card when ordering online.

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Marian, Discount Card Holder

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Matt, Discount Card Holder

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60 YEAR
MDA 1959-2019
ANNIVERSARY
Our Jubilee






A mighty significant milestone

Celebrating 60 inspiring years.

MDANZ patron Judy Bailey beautifully summed up the spirit of the 60th Jubilee celebration in her written address to the attendees when she said: "All power to you all and may MDANZ grow and flourish for another sixty years and beyond."

Judy said in her address (which was read by Trevor Jenkin) the event was a mighty significant milestone in the life of a wonderful association.

"It has been my honour to have been a patron of MDANZ for more years than I can remember, though not quite 60!"

She said she came on board around the time Dame Susan Devoy was raising the association's profile with her amazing walk through the length of the country. "That walk was a game changer for MDANZ and really brought neuromuscular conditions to the national consciousness.

"I have met so many extraordinary people at various events over the years, the remarkable Hibbend family, Nic Brookelbank with his fab cookbook and great soccer skills, Liam Gleeson, who's now about to sit Level 1 NCEA and is already making a name for himself with his designs for hot wheel cars.

"There are too many people to mention individually but each and every one of them is achieving and inspiring others on a daily basis."

She also said that now we can celebrate another group of extraordinary young people.

"A bronze Duke of Edinburgh Award is no small achievement. It's a delight to see you Dukies continuing to push the boundaries and leading the way for others. Warm congratulations to Marty Price for the energy and dedication he brings to the programme."

Jack Lovett-Hurst, Ella Mills, Dylan Schneider, Olivia Shivas and Grace Chapman (who wasn't able to attend) were all awarded their Bronze certificates and badges on the night, while Denise Ganley and Katie Noble were awarded life membership and presented with their certificates. Other Life Members unable to attend were John and Lyn Hawkins, Rae Mahoney and Raema Inglis.

The glorious balloon display donated by Janella's Party Hire ensured the event looked as festive as everyone felt.

Left: Life members with Trevor Jenkin: Katie Noble and Denise Ganley. *Right:* Our Dukies, Ella Mills, Jack Lovett-Hurst, Dylan Schneider, Olivia Shivas. Grace Chapman was unable to attend.

All welcome

The start of a great day.

A grand total of 107 members, whānau and health professionals turned out at Waipuna Hotel and Conference Centre in Auckland for the Freedom Beyond Limits Neuromuscular Education and Advocacy Seminar, followed by the 60th Jubilee celebrations.

National executive chairperson, Trevor Jenkin, kicked off the August 3 seminar welcoming the delegates and making special mention of those who had travelled some distance to attend, including guest speaker Rob Besecker and long-time MDANZ supporters Professor Larry Stern and Jacqueline Stern.

He also thanked the very generous sponsors for making the event possible: Biogen (gold), Roche (silver), Breathing and Medical Respiratory Technologies, New Zealand Powerchair Football and Allied Medical, along with supporting sponsors the Neurological Foundation, the Centre for Brain Research at the University of Auckland and the Auckland Harley Davidson group and event organiser, Icon Events.

The mihi whakatau was delivered by Ron Baker and MDANZ member Dion Ponga offered a reply on behalf of the association.

Climbing your own mountain

Making the choice to live the best life.

Chicago based author, international speaker and health worker, Rob Besecker, told the seminar he does everything he can to live the best life he can to pursue his dreams.

His book *Everest Strong: Reaching New Heights with Chronic Illness* is an inspirational memoir published in 2017 and Rob outlined the long and painful journey that has led him to help others live their best lives.

He spends much of his time advocating for hospice care patients and their families and he says on his website his passion for healthcare comes from his own experience living with chronic heart ailments and muscular dystrophy which gives him "a relentless drive to squeeze the most value out of each day".

He told the seminar about the physical and mental challenges that led him on a journey of incredible adventure and self-discovery. He went from months spent in hospital beds, questioning his chances of survival, to leaping toward his biggest challenge: a hiking expedition to the base camp of Mt. Everest.

An athlete and successful scholar, at 24 years old he began having heart problems which continued to escalate to the point where he underwent heart surgery. He continued to have all kinds of health problems but realised he "didn't need a fairytale ending," but just needed to remember every time he got knocked down to get back up again.

Rob Besecker.

In 2011 he endured further major health issues and despite some questioning his survival, in 2012 he began to make a full recovery. When he has been asked how he recovered he says: "What other choice did I have? You can give up or get up and keep going and my choice was to do that."

He trained hard for the trip to Base Camp including extensive altitude training and was successful in completing the 65 kilometre journey to Base Camp in 11 days.

Rob told the seminar his message was that we all go through obstacles and challenges throughout life and they are different for everyone "so when you have the opportunity to help someone [do so] and when you have struggles yourself reach out to people for help".

See www.robbesecker.com

Chronic Sorrow: Moving from grieving to gratitude

What happens when you are grieving over something invisible, how do you let people know you are experiencing a grief process?

Chronic sorrow is the name for a hidden suffering people have to manage which can be caused by the ongoing grief felt when a loved one is diagnosed with a chronic health issue or disability, according to Dr Kirsty Ross a senior lecturer in clinical psychology and director of the Psychology Clinic at Massey's Manawatu campus.

She told a breakout session at the Neuromuscular Seminar that this chronic sorrow can occur when an event happens that leaves a loved one changed forever. It isn't clinical depression; you function perfectly well but there is an underlying sadness there which is not always picked up by other people, so it can be very isolating.

Some of the emotions you might feel include anxiety, low mood, trauma response from procedures and feelings of isolation.

She noted too that this chronic sorrow is a normal parental reaction to having a child with a chronic illness or disability. You are grieving but repeated triggers elevate this grief.

Kirsty sees this as a normal emotional reaction to loss; loss of social connections as people may back away; loss of confidence in parenting skills and even a loss of privacy if in home care is needed.

She said too that the emotional response is recurrent and it intensifies during the initial presentation of the illness, during developmental transitions, increasing care demands and during periods of new or worsened symptoms.

Kirsty Ross.

But what happens when you are grieving over something invisible, how do you let people know you are experiencing a grief process?

The impacts of chronic sorrow can include searching for reasons for what has happened leading to guilt or blame; fatigue – both physical and emotional along with levels of hyper-arousal and hyper-vigilance and a high level of emotionality.

There has been a lot of research on the negative outcomes of the impact of a long-term health condition but people are now starting to look at resilience and overcoming these impacts. But before moving to positivity "you have to sit in the dark and make the darkness beautiful".

Kirsty says to manage chronic sorrow you might think about reading about the condition to prepare for future changes; working in a collaborative way with health professionals; community education; stress relieving activities such as mindfulness or meditation and eating, sleeping and exercising well.

Social interaction particularly with people in the same situation can help as can self-awareness and acceptance of emotions and communicating these to others.

She said the quality of social support is very important; people try to fix things but you can say to them; "I just need you to listen."

She says it is important to "sit in the dark" and acknowledge your feelings and then focus on the positive. Resilience skills can be learnt. Resilience is getting up in the morning and doing stuff, weathering the storm.

She also pointed to positive coping looking to values and beliefs; focusing on your strengths and having self-compassion, maintaining hope and optimism and gratitude.

Accessing new medicines

From molecule to medicine is a long and risky process.

Graeme Jarvis.

Better partnership and collaboration, more transparency and more timely decision making along with better funding for Pharmac are needed to improve access for New Zealanders to new medicines, according to Graeme Jarvis, the CEO of Medicines New Zealand.

He described the very long process involved in getting new medicines approved noting that from molecule to medicine is a risky and long process.

It can take 12 to 15 years to go from discovery through preclinical to clinical trials to regulatory review and approval and scale up to manufacturing and the chances of being successful with a new concept are very remote, he said. Only one in 10,000 molecules makes it through the process to become a new medicine.

In addition, the cost of developing a new medicine is very high and has risen from around $300m in the 1970s to about $4.1 billion today because of the increased complexity of medicine, higher cost of trials and higher medicine failure rates during development.

He said the easy-to-treat conditions were dealt with in the 1960s and 70s, so the focus moved to more difficult conditions meaning more complex medicines development in the recent decades.

Currently, there are some 7,000 modern medicines globally under development of which around 1,300 are for neurology and 1,100 for immunology.

In New Zealand getting medicines to patients involves several steps. Firstly, the Ministry of Health (Medsafe) determines if the medicine is safe and effective. Then Pharmac's Pharmacology and Therapeutics Advisory Committee (PTAC) reviews funding applications for new medicines.

PTAC makes recommendations on which medicines should be funded. And then Pharmac determines if it will actually publicly fund those medicines.

The timeframe for Pharmac's decision-making before patient access to publicly-funded medicines averages 3.6 years. But for other medicines there is a waiting time between the PTAC recommendation and Pharmac's funding decision and some medicines are still waiting for funding decisions even after almost 10 years including some rare diseases medicines.

New Zealand also had a significantly lower percentage of modern medicines publicly funded compared with other countries, sitting at 17 percent compared with 96 and 94 percent in Germany and the United Kingdom. We also spend far less on pharmaceutical expenditure as a percentage of the total health expenditure than other countries.

He says it is "a myth" that New Zealand can't afford new medicines and one study shows that the medicines budget shrank in real terms by 0.3 percent between 2007 and 2018.

Graeme says New Zealand has a lot of catching up to do and his very clear message is that the pharmaceutical budget has not kept up with other types of health investment as our population and disease burden has grown.

It was a busy day for sponsors and seminar attendees.

Exciting new treatments for different conditions

How some of the treatments are purported to work is rooted in how the condition itself is caused.

Long-time contributor to *In Touch* consultant neurologist Associate Professor Richard Roxburgh, shared with the seminar some of the most exciting new treatments for neuromuscular conditions and how they work. In many cases how the treatment is purported to work is rooted in how the condition itself is caused. This can make for some complex information!

Richard explained what causes spinal muscular atrophy (SMA), Duchenne muscular dystrophy (DMD), facioscapulohumeral muscular dystrophy (FSHD), myotonic dystrophy, Pompe disease, and Friedreich's ataxia. Treatments for these conditions are either in the latter stages of the clinical trial process or, as in the cases of nusinersen (Spinraza) and Zolgensma for SMA and enzyme replacement therapy for Pompe disease, have reached the market.

The underlying cause of SMA is a genetic alteration that results in insufficient amounts of an important protein, Survival Motor Neuron (SMN) being produced. Nusinersen works by converting a similar gene that doesn't produce very much SMN protein into one that produces normal amounts of it.

Nusinersen has to be given regularly though and by injection into the spinal column. It is also very expensive. Another treatment called Risdiplam works via a similar molecular mechanism but is an oral medicine instead making the logistics of taking it much easier. An application to approve this drug is being made right now.

One of the first true gene therapies to be approved by the US FDA is Zolgensma for SMA. This genetic therapy is delivered to the body's cells by a non-harmful virus and is able to produce the Survival Motor Neuron protein. It is one of the world's most expensive drugs.

Duchenne muscular dystrophy is caused by any number of different genetic alterations in the dystrophin gene. Some treatments are specific to particular genetic alterations, including Ataluren, which is approved in the US and Europe and also Eteplersen, which is approved in the USA only.

Other treatments being trialled in DMD are improvements over already established treatments, such as Vamorolone as a replacement for steroid therapies like prednisone and deflazacort.

Richard described the complex genetic mechanism that causes FSHD and explained how one drug that's about to enter phase 2/3 trials, Losmapimod, appears to work at the genetic level by suppressing the expression of the gene (DUX4) that, when over-expressed, causes FSHD.

Treatments aimed at increasing muscle mass are being trialled in FSHD and appear to be helpful.

Richard is running a clinical trial for people with Pompe disease where the enzyme that's missing in people with Pompe disease is replaced and is also chaperoned by another drug that helps get the enzyme into the cells where it's needed.

To keep up with the clinical trials in your area check out clinicaltrials.gov and enter the name of the condition that you're interested in into the keyword search.

By Miriam Rodrigues

Associate Professor Richard Roxburgh.

Matthew (left) and Andrew Willetts (right).

Brothers in arms

It's about knowing your limits, but never giving up

Two Oamaru brothers, who share a close bond, are working hard to share their skills and learning with MDANZ members, both in their Southern Region and through the National Council. They have learnt that it's about knowing your limits but never giving up.

Oamaru based Andrew and Matthew Willetts share a closer bond than many brothers. As they both have intermediate form of muscular dystrophy, somewhere between Duchenne and Becker muscular dystrophies, each of them has someone who just "gets it", Andrew tells *In Touch*.

He says it helps having a brother with the same condition because you know what to expect and you have got someone who understands completely.

Both Andrew, who is 28, and Matthew, who is 25, live independently, Andrew in a flat on his own, which he says is freezing the day *In Touch* calls, while Matthew lives with his husband Hamish, not too far away.

Both brothers are very active members of MDANZ and have been members of the organisation for most of their lives. Andrew is now chair of the Southern Region and elected onto National Council and Matthew is the Southern rep on the council.

Andrew says he feels honoured to be on the National Council with "a whole bunch of strong people, who are so positive".

One of the things Andrew is proud of in the Southern Region is everyone's willingness to help each other out.

"It is a really important thing to find people who know how this works, and we do rely on each other. I am lucky because I've got my brother but other people don't have that and it's important to find someone who understands."

The region has regular functions and get togethers where MDANZ members from all over the very wide-spread area can get together for fun and networking.

Andrew recently travelled to the small town of Gore for a coffee group and even in such a small area 15 people turned out for the event.

The region has between 80 and 100 members and a good number of them work with the local fieldworker, Jo Smith.

Andrew is passionate about the work Jo does in the region which he sees as hugely beneficial, noting that the southern branch lost services some years ago and had no fieldworker for quite a long period.

He credits Raewyn Hodgson, who was Southern Region chair and on the National Council for many years, with ensuring the branch grew and thrived. "Without her there would be no Southern Branch, because of a lack of support, but getting a fieldworker is huge with members as far afield as Queenstown, Invercargill and Bluff."

Andrew has been a teacher aide at a local primary school for the last two and a half years and loves it. He started an education degree in Christchurch but fatigue associated with MD meant he changed to a Certificate in Learning Support.

"I was in Christchurch for four and a half years, but when the fatigue got really bad I decided to move home."

He has now renewed his studies and is finishing a Bachelor of Arts in Educational Psychology. He is doing this remotely through Massey University, which he thoroughly enjoys especially the interaction with the other students via video conferences and intensive learning weeks.

Once completed he would like to look at working with the Ministry of Education to help students with different needs.

Matthew is not working at the moment but has a Certificate in Interior Design and would ideally like to set up a business in that area.

Andrew manages well living on his own but says financially it can be a bit tough as he is only working part-time and, even with some government help, it's still hard to manage big bills.

The cost of living is an area he sees as an issue for a lot of members as many have to rely on government benefits.

Their parents both live in Oamaru and they see them regularly and Andrew says the support is there if they need it.

He says when they were younger, their parents encouraged them towards difficult decisions and pushed them to do their best.

Asked about the challenges he and Matthew have seen MDANZ members facing, Andrew points to the lack of understanding of some medical professionals who don't know enough about MD.

The cost of living is an area Andrew sees as an issue for a lot of members, as many have to rely on government benefits.

"If you go to the doctor you spend half your time explaining your condition and some are not familiar at all."

He says it is hard to get the message through that you know yourself and your condition really well, that you monitor your health closely and that you know when you need antibiotics because you can't afford to get ill.

And for Andrew working in a class of young children, who often have flu and colds, this means very careful monitoring of his health indeed. As he sees it little bugs can do big damage if you have MD.

Another area of concern in smaller towns is getting an appointment with a GP, as you can wait three to four days before someone is free.

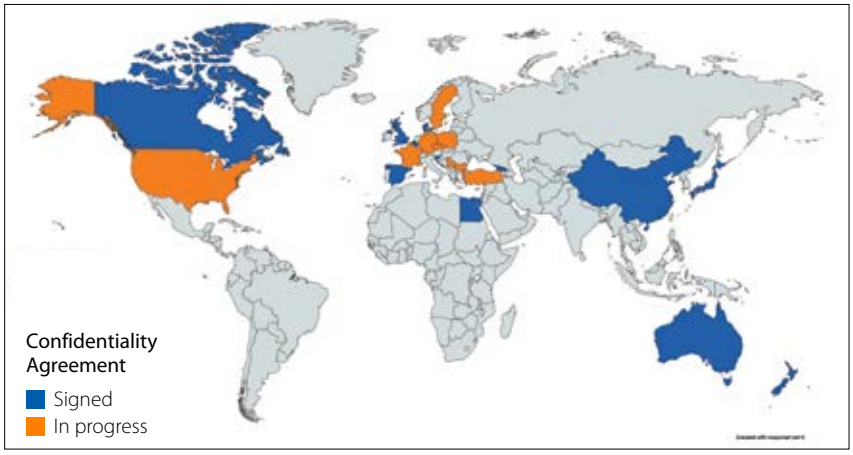
One of the things he tries to pass onto people is the importance of looking after yourself, making sure you know the signs and to tell the doctor, " I know me, I know my health" and don't be afraid to ask others for advice.

"Specialists do help, but at the end of the day it is about needing to know what your limits are and not giving up. Push it, but don't push it [too hard]."

Andrew also tries to eat healthily and to keep up his stretching and exercises, but there is the time factor at play between work, study and MDANZ responsibilities. He jokes that he might get a bit lazy sometimes or forget. But sometimes, he says, "life should get in the way".

A new potential treatment for myotonic dystrophy

New treatments need to go through a stringent testing process.



Treat NMD coordinates a global network of registries for people with myotonic dystrophy.

Every two years the International Myotonic Dystrophy Consortium meets to further advances in the field of care and cures for myotonic dystrophy. In 2019 this meeting took place in Gothenburg, Sweden.

Myotonic dystrophy is one of the more common neuromuscular conditions with seven to eight people of every 100,000 diagnosed with the condition in New Zealand and probably many more affected.

The meeting focused on getting trial and treatment ready. A considerable number of companies are competing to be the first to have a treatment for myotonic dystrophy but before regulators approve new treatments they need to go through a stringent process of being tested in people.

Firstly in healthy volunteers then in people with the condition to show that the treatment actually works.

For a clinical trial to be able to demonstrate whether a treatment works it's important that the people participating in the trial are receiving a similar standard of care – otherwise it's impossible to know whether the measured effect of the treatment is due to the different care or is it due to the treatment?

Care guidelines are also useful for health practitioners who may not have experience of myotonic dystrophy and are seeking answers to questions about how to best care for their patients with it. To those ends the US-based Myotonic Dystrophy Foundation has funded the development of consensus-based guidelines to care for myotonic dystrophy type 1, myotonic dystrophy type 2 and also congenital myotonic dystrophy.

You can find links to these on MDANZ's website or contact the MDANZ office for a copy.

Another aspect of being ready for clinical trials is knowing where people with myotonic dystrophy live and having some basic information about them readily available. Treat NMD coordinates a global network of registries for people with myotonic dystrophy. The map [left] illustrates countries from around the world that have an agreement in place or are in the process of joining the collaboration with Treat NMD.

New Zealand's Associate Professor Richard Roxburgh is the group lead. If you think you might be interested in being part of research please contact the NZ NMD Registry registry@mda.org.nz or an MDANZ fieldworker. Ⓡ

Rituximab for severe myasthenia gravis

Rituximab (sounds like 'rye-TUX-ih-mab') is also known as Mabthera and has been used in the treatment of rheumatoid arthritis and certain forms of cancers. Rituximab belongs to a group of medicines known as biologic medicines. These are medicines that target specific chemicals in your body involved in the inflammatory response that can occur in myasthenia gravis. From August 1 people with myasthenia gravis that hasn't responded well to other treatments, will be able to be prescribed Rituximab on special authority by their neurologist. Ⓡ

X-linked conditions

If there's a known family history of an X-linked condition such as myotubular myopathy, Duchenne or Becker muscular dystrophy, Kennedy's disease or CMT1X, genetic counselling is recommended for women in the family to assess their risk of being a carrier.

Kennedy's disease, myotubular myopathy, Duchenne muscular dystrophy, CMT1X, Becker muscular dystrophy: What do these different neuromuscular conditions have in common?

They're all caused by alterations in genes we carry on our X chromosomes.

Females have two copies of the X chromosome and males just one copy so when there's a mistake in a gene that's on the X chromosome females have a got a second 'good' copy that provides back up but males don't and so will be affected by the X-linked condition.

This means that there are some special characteristics of families that carry a recessive X-linked condition – you'll never see an affected father pass the condition to his son and all the daughters of an affected male will be carriers for the condition.

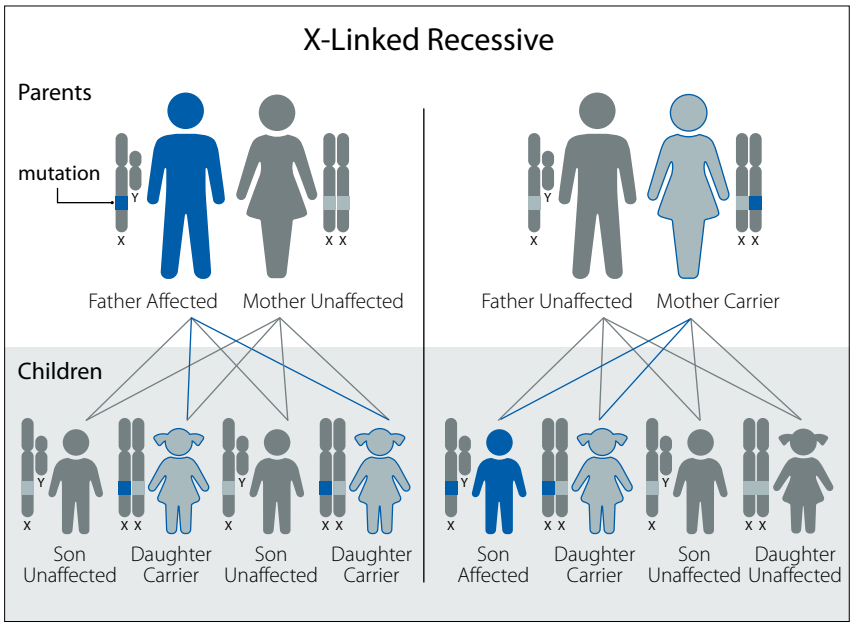
That's because fathers pass the Y chromosome to their sons and the X chromosome to their daughters and in the case of a man with an X-linked condition that X chromosome carries the genetic mistake that is the cause of his condition.

Often in a recessive X linked condition there is no family history of the condition. This can be for two reasons, firstly a mistake on a gene that's on the X chromosome can be passed hidden through generations because females carry another X chromosome which has the healthy working gene on it that masks the effect of the gene with the mistake on it.

You'll never see an affected father pass the condition to his son and all the daughters of an affected male will be carriers for the condition.

Every time a woman has a child she passes one copy of her two X chromosomes to that child – it could be the copy with the working gene or it could be the copy with the mistake on it.

If the child is a girl and she receives the copy with the mistake on it then she is a carrier for the condition (just like her mother). If the child is a boy and he receives the copy with the



Mode of inheritance.

mistake on it then he'll be affected by the condition because he lacks a second X chromosome to make up for the altered copy.

Secondly, during the process of DNA replication mistakes can be introduced. These are termed 'de novo mutations' meaning that they have occurred newly for the first time in an early stage of foetal development.

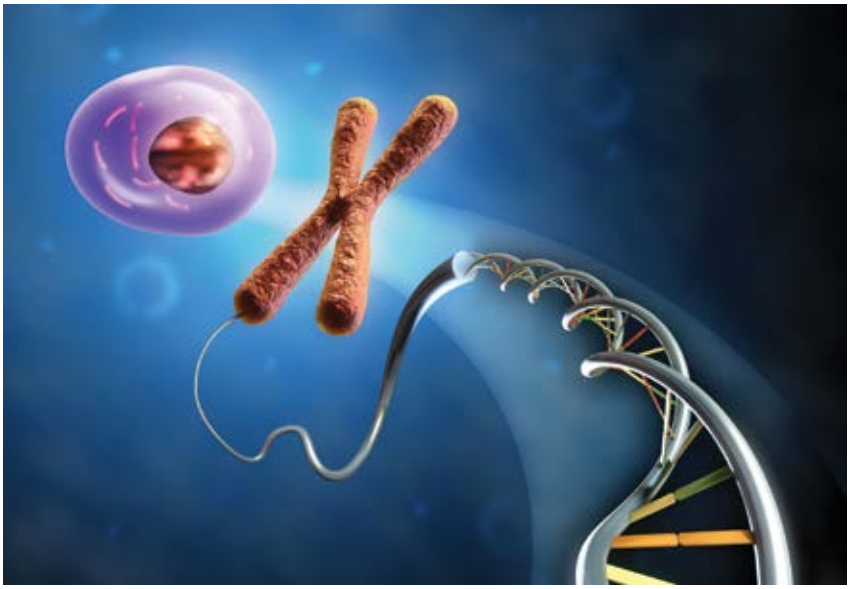
If this happens in the X chromosome of a male foetus then that male will be affected.

Some areas of the genome are more prone to becoming altered than others, for example the dystrophin gene is a particularly large gene and about one third of boys with Duchenne muscular dystrophy have the condition, not because they inherited an X chromosome with a mistake in the dystrophin gene from their mother but because the mistake in the dystrophin gene happened for the first time very early in their development.

Although recessive X-linked conditions usually occur in males these conditions do not happen exclusively in males and sometimes carrier females are affected, although usually it is considerably more mild.

One reason that some women are affected by a condition that is caused by a mistake in a gene on one of their X chromosomes is due to a skewing of the inactivation of the X chromosome that carries the working copy of the gene.

In females one of the two X chromosomes in each cell becomes genetically inactive early in development and remains untranscribed throughout life. This is termed X-chromosome inactivation. The result is that the effective amounts or dosages of products of X-linked genes are equal in males and females.



Often in a recessive X linked condition there is no family history of the condition.

X-inactivation occurs randomly throughout the cells in the body but sometimes the randomisation is not so random and is skewed towards the inactivation of the X chromosome that has the working copy meaning the copy containing the mistake is active in more tissues of the body.

Without X inactivation males and females would have different amounts of the proteins produced by the genes on the X chromosome.

The existence of X inactivation was first suggested by Mary Lyon in 1961. For a time this suggestion was known as the 'Lyon hypothesis,' and the inactive X chromosome was said to be 'lyonized', however these terms aren't usually used these days.

On rare occasions a female might have two altered copies of a gene present on both of her X chromosomes, in which case she will show the same signs and symptoms as an affected boy.

If there's a known family history of an X-linked condition such as myotubular myopathy, Duchenne or Becker muscular dystrophy, Kennedy's disease or CMT1X genetic counselling is recommended for women in the family to assess their risk of being a carrier.

Genetic counselling can be accessed by contacting Genetic Health Services New Zealand. Visit this website for more information: www.geneticheathservice.org.nz

Being present and living right here, in the moment

Like many youngsters Ethan and Ollie King love their matchbox cars, gaming and riding a quad bike. And no one could be stronger advocates for their two boys with Duchenne muscular dystropy than mum and dad Corinne and Mike King. Corinne says to trust yourself, you do know what's best for your child.

Fun in a wheelbarrow bath in the paddock.

When did you find out about the DMD diagnosis and how did this process take place?

Well, I had always worried that Ethan (who is now 8 years old) was not developing the same as his peers, he met his milestones but they were always delayed.

By the time he was two and a half, I had my concerns, particularly because he couldn't run or climb. I queried this with Plunket and took him to a chiropractor, but everyone assured me that all kids develop at a different pace.

Then Ethan's younger brother, Ollie, arrived and had a very acute breathing condition, so all our focus went into him. He had airway surgery at four weeks old and required very intensive care (this condition is not related to MD).

My mum looked after Ethan for the many extended stays we had in Starship with Ollie and she noticed that he didn't want to walk very far and the muscles in his calf would get very tight.

So I took Ethan to the GP and he noted the Gower's manoeuvre to get off the floor [gets up by first getting on to hands and knees and then, from a kneeling position, pushing with hands against knees and thighs until upright] and asked if he had trouble with stairs.

It was actually that moment I knew this was something much more than slow development and the GP referred us to a paediatrician and he asked similar questions.

He then rang me later that day and wanted my husband and I too come see the neurologist ASAP, I couldn't wait and begged him to tell me what they thought was wrong.

I'll never forget the words 'Muscular Dystrophy' as surprisingly I'd had a neighbour with DMD when I was young, so I knew quite a lot about it.

They took more bloods and we waited for them to confirm it was Duchenne, although my Googling made me pretty sure it was. The official diagnosis of Duchenne muscular dystrophy came via email, which was tough.

Then everyone tried to stop me getting Ollie tested. I think they thought it would be too much for me, as it was a 50/50 chance he would also have the defective gene and sure enough he also carried it and would also have DMD.

It was heart breaking and very surreal for a long time, to be honest.

How has the relationship with your school been and what modifications have been made to support your children?

The relationship with school has been good, with both boys at school now. I think I spend more time talking to the SENCO [special education needs co-ordinator] than I do my husband lol!

Ollie (5 years old) has started just this term, he has a medical stroller

that he uses to go out to the field and the teacher or a teacher aide pushes him around the track.

It has been quite a fight to get ORS [Ongoing Resourcing Scheme] funding for him, but we got it in the end with limited teacher aide time.

We have applied for a power wheelchair for him to give him more independence at school. But he can join in with most activities.

The school has been very receptive to trying new things with Ethan. When he started to get tired at school, he used his balance bike to help get around at morning tea and lunch.

Then he got a small mobility scooter and ramps needed to be added to clear the door etc. Someone in the class is assigned to getting Ethan's scooter in and out every day and he uses his wheelchair in class and sometimes out of class when he is tired or sore.

We are also waiting for a power wheelchair for Ethan, as he now requires more seating support as the condition is progressing.

What activities do your children enjoy and how do you encourage that around the house?

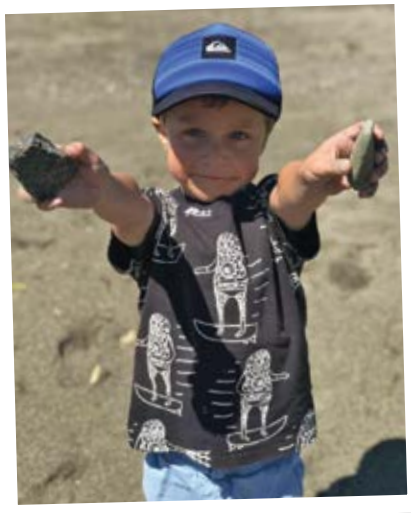
Like a lot of kids, the boys love ipad time and gaming. We have an Xbox room set up just for this purpose to encourage the boys to sit down when they have friends over.

Ethan loves riding his quad bike, which he can still do if he gets inside straight afterwards for a shower and we limit the amount of time he is on it.

He has a friend who brings his motorbike over to ride with Ethan.

A recent stay at Starship for Ethan.

I try to remember it's not just for my boys, it's for those boys yet to be diagnosed coming through the system behind us.



Ollie poses for the camera.

Both boys also love playing matchbox cars, so we have a huge car mat set up with car ramps and way too many matchbox cars and they love playing with these together.

From a parents' perspective, what are your thoughts on having lived experience of this condition?

It's tough, as other children are gaining independence and their parents can worry about them less, the polar opposite is happening in our world.

That can be very isolating. We went from Ethan playing Rippa Rugby and having a drink in the clubrooms with other parents to not being part of that at all, as Ethan now can't play. That was only in two years.

Other parents say you are lucky that you don't have to get up early on Saturday morning and get down to the rugby field, but they don't get it. It's just like many things with kids with disabilities, we are excluded.

And that is the tricky thing, no one that lives a life with 'typical' children can ever really get it. It's like having a baby, you can't understand it till it's your reality.

So at times it can be hard to relate to others, having children with severe medical conditions can be all consuming and it's hard to see the wood for the trees.

Do you have any tips when dealing with the health system and other health professionals in New Zealand?

- Do your own research. We are so lucky to be live in this

technological age where you have access to what is happening at the global level. Know what the standard of care is in the U.S and Europe for your condition and demand this for your children in New Zealand.

- Remember that your condition is rare, never assume doctors have all the knowledge they need to know about your child or your condition. You are an expert in your condition. Trust your instincts, if you think something is wrong, you are probably right!
- It takes a long time to understand how the health system in New Zealand works, I'm still not sure I know sometimes. It can be very frustrating constantly advocating for your child, but I try to remember it's not just for my boys, it's for those boys yet to be diagnosed coming through the system behind us.
- Know your child's genetic mutation so that you know exactly what medical trials are out there to help your child, if they become eligible. Health professionals know their specialist area very well but they don't see the big picture often. It's not really their job to do so, I guess, so it's important to remember that.
- There is a place for both medical and natural and I think both complement each other well. Our boys take steroids, but I also swear by homeopathic sprays, supplements and natural based muscle rubs. You know what's best for your child, the doctors play an important role in this but they aren't the only ones that can help with such a multi-faceted complex condition such as DMD.

You are an expert in your condition. Trust your instincts, if you think something is wrong, you are probably right.

Top: Waihi Beach with dad, Mike: Ethan on his quadbike and Ollie in the buggy. *Above:* A family walk in Whangarei with mum, Corinne.

Is there anything you would like to add that would be helpful for parents or people living with the same condition?

Never be afraid to speak up for your child or your child's condition, if you don't no one will.

Living with the knowledge that your child, or children in our case, has a terminal illness is tough, talk to people about some of your thoughts because otherwise it messes with your head.

If you can't talk to a friend, maybe a counsellor would be a good ear. It's hard to not worry as a parent with a child with significant medical needs, you worry they might fall; you worry they might get sick because of being immune suppressed due to steroids; you worry that muscle pain is something more. You just worry you've missed something important and whether you are doing enough as their parent.

And you worry a lot about the future, what does it look like, will you cope as a parent? Will your child cope living with this and all that's to come.

But it's important to put all that aside and try to be present, try to live right here in the moment because no one really knows what's to come in the future.

And never give up hope. The medical advances that have been made in DMD in the last two years, in particular, have been nothing short of amazing. Trust yourself, you do know what's best for your child.

Seeing opportunities, not obstacles

Aucklander Brian Dalton hasn't let his diagnosis of Kennedy disease stop him from enjoying his retirement, including an extended tour around Europe.

Like most men with SBMA spinal and bulbar muscular atrophy (Kennedy disease) I was originally diagnosed with motor neurone disease (ALS) in 1998. My condition worried my neurologist because I had muscular as well as sensory damage which doesn't present in ALS.

I was invited to attend a neurological conference in Auckland in 2000 where an American voice in the audience asked me questions which were very different to any I had answered before.

I was then sent for a DNA test and finally diagnosed with Kennedy disease. In the early 2000s the internet was not as informative as it is now, and to find any information was virtually impossible. In the 20 or so years since the diagnosis I have come across only one doctor who had any knowledge of the disease. This includes neurologists. Now with the internet we have been able to spread the word about Kennedy's and get in touch with people with it from all over the world.

How does the condition affect you in your everyday life and how have you overcome these challenges?

With my muscles weakening I had to give up my job being an aerial survey photographer. I found a position in customer services in the tool industry, and when this became hard to manage I retired at 69.

I have gone from crutches to a manual wheelchair and now we have purchased a battery driven one. I can still get around on crutches but this can be a bit slow.

I fall often so I have to be careful getting around. Picking something up from the floor can be challenging and getting out of a chair is difficult.

I lost all ability to swallow solids a few years ago and had a PEG inserted for feeding. I miss eating the foods I love as I am an avid cook and have always enjoyed the kitchen and trying new recipes.

There are KDers all over the world, who readily share their concerns, their successes, their ideas and advice.

Our glassware and crockery sets get a good workout, as they are washed or loaded into the dishwasher. We have purchased a set that bounces better for day-to-day use. Our good stuff I have had to promise not to touch! (This is after breaking our wedding glasses in one swoop).

Brian and his wife Tracey in Matera, the city in southern Italy that is mostly cave dwellings. It was an adventure second to none, he says.

Some gadgets don't work and some are invaluable and by trial and error we have found what works for us. We have set our house out to make it as easy as possible to move around. Furniture is placed out of pathways, no loose rugs and walkways kept clear. (This is near impossible with grandchildren, but I would never have it otherwise). Occupational therapists visited and arranged for a ramp to be built to allow wheelchair access into the house from the driveway.

What does your everyday life look like now?

Thankfully I can enjoy a nice shower and take care of my own personal needs unassisted. I attend a neurofit and a hydro session at AUT once

a week. This will hopefully help keep the abilities I have for as long as possible. I am also working on cataloguing my negative and transparency photographic library.

I enjoy reading and I spend a lot of time playing games with, or reading stories to, my granddaughters who live with us.

I can still drive but getting through the shops with a trolley is a bit challenging so now I go shopping with someone else.

I do the laundry and manage to hang it out if there is not too much wind!

Do you have any tips when dealing with the health system and other health professionals in New Zealand?

You must remember that with a rare disease like Kennedy disease the person with it knows more about the symptoms, problems and treatment than most people out there, including health professionals, this is just the way it is.

You will know if a professional has done their homework as soon as the meeting starts. Make your voice heard, be insistent, give your professional as much information as you can.

Brian, in his wheelchair being loaded onto the train in Florence. The service he used in Italy is called Sala Blu and it specialises in disabled access to trains. Brian says you make contact before your trip and check in an hour before your departure. You and your luggage are collected from the waiting room and transported to the train. A lift picks you up, lines you up with the door and in you roll right next to the seating/parking area for wheelchair users.

Make your voice heard, be insistent, give your professional as much information as you can.

Is there anything you would like to add that would be helpful for people living with the same condition?

We have joined the Kennedys Disease Down Under and Kennedys Disease-Raising Awareness Facebook pages. There are KDers all over the world, who readily share their concerns, their successes, their ideas and advice.

I have found this invaluable, knowing that most members are going through the same problems and you can share how you handle them and discuss and gain knowledge that you would never acquire anywhere else.

Everyone on there, including myself, to begin with take the decision of mobility aids as defeat. But over the years I have learnt that when you are ready, and only you will know, to make a decision like using crutches, or investing in a wheelchair, it is a step to 'freedom' in a way.

When I retired Tracey planned, booked and researched a three-and-a-half-month overseas trip to France, Italy and Barcelona in Spain.

In her research she booked 31 different accessible places to stay. All very different, we enjoyed staying in convents, hospitals, trullos, hotels, BnB's, caves and farm stays. All were accessible and all came with an ensuite.

By doing it this way we saved a fortune compared to what tours cost. Our three-and-a-half month holiday cost the same as a three week tour.

You have to see opportunities and not obstacles. When booking to go to Matera in Southern Italy, only two establishments got back to us, the first one saying, 'Don't come to Matera, it is a city of stairs', the second said, 'If you can't walk on the pavement, you walk on the road'. We went to Matera and had a wonderful time.

When booking for Venice everyone said we were mad. We happened to get there just after a marathon so there were ramps on most of the larger bridges. The other ones we tackled slowly. We loved it.

Don't let anything hold you back, with a supportive partner (which Tracey fits the bill by 2000 percent) anything can be achieved. We may have missed out on a few things but what we did see far outweighed the things we missed.

Muscular Dystrophy New Zealand

Help us raise money without spending a cent.

You have the power!

Community Power is a new power company that wants to make a difference to the lives of New Zealanders.

To do so, it shares a portion of its profits with charities such as the Muscular Dystrophy Association of New Zealand (MDANZ).

By switching your power company to Community Power, you will stay on your current rates, or pay less – you won't pay any more for power. And by simply paying your power bill, Community Power will donate a portion of their profits to MDANZ and acknowledge you for this donation. It won't cost you a cent more and you won't have to dip into your own pockets.

This is the most powerful fundraising initiative we've ever launched. We don't need your money, we just need your power!

I've made the switch. Will you make it too?

Trevor Jenkin
National Executive Chairperson

Yes I will! How do I switch?

1. For a free, no obligation quote, email a copy of a recent electricity bill to: **support@communitypower.co.nz**
2. We will let you know how much your donation will be and if there are any savings for you.
3. Then just say "Yes" to start donating at no cost to you, we do the rest.
4. Your electricity supply will not be interrupted, we will take care of everything.
5. If you receive a network company dividend or rebate you will continue to receive this.
6. If you receive a prompt payment discount you will continue to receive this.
7. We will advise you on each monthly invoice of the amount you have donated and of your total donations made since joining us.
8. **Remember, your donations come from our profits, not your pocket. Thank you for helping.**

In partnership with COMMUNITY POWER *Power of Together*
www.communitypower.co.nz/md

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 1950s. 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 core team

Trevor Jenkin
National Executive Chairperson

Miriam Rodrigues
Programme and Service Advisor

Brian Hadley
Accountant and Business Manager

Dympna Mulroy
Member Services Manager

Natalie Foote
Marketing and Communications Manager

See more about our team at www.mda.org.nz

Northern Branch


Fieldworkers: *Darian Smith and Rachel Woodworth*
Office Manager: *Denise Ganley*
Ph: 09 415 5682 or 0800 636 787
Email: support@mdn.org.nz

Central Branch

Fieldworkers
Ph: 0800 886 626
Email: info@mda.org.nz

Canterbury Branch


Fieldworkers: *Paul Graham and Marty Price*
Office Manager: *Vivienne Fitzgerald*
Ph: 03 377 8010 or 0800 463 222
Email: mdacanty@xtra.co.nz

Southern Branch

Fieldworker: *Jo Smith*
Office Manager: *Vivienne Fitzgerald*
Ph: 0800 800 337
Email: joanne@mda.org.nz

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

Michael Schneider. Ph: 021 851 747
Email: spider@spider.co.nz

Central Branch

Bernadette Ingham. Ph: 027 600 3868
Email: members.central@mda.nz

Southern Branch

Matthew Willetts.
Email: willetts.matthew@yahoo.com

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

Spinal disorder - Mobility impairment - Walking impairment - Arthritis - Multiple sclerosis - Cerebral palsy
Neurology - Muscular dystrophy - Amputation - Parkinsonism - Back pain - Stroke - Restricted growth

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Independence for anybody with a disability who has reduced mobility.
More functional than a wheelchair or walker for indoor tasks.
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