



Muscular
Dystrophy WA

2018 Annual Report

MUSCULAR DYSTROPHY WA (MDWA) IS A SMALL ORGANISATION MAKING A **BIG IMPACT**. WE ARE **PASSIONATE ABOUT IMPROVING THE QUALITY OF LIFE** FOR PEOPLE LIVING WITH NEUROMUSCULAR CONDITIONS. WE WORK WITH **HUNDREDS OF FAMILIES**, HELPING THEM GET THE BEST SUPPORT AND SERVICES THEY NEED AND **CONNECTING THEM TO OTHERS WITH SIMILAR CONDITIONS**, ENABLING THEM TO **LEAD FULL LIVES...**



...WE CALL IT



Vale Mr Stan Perron AM CitWA

For more than 40 years he was one of MDWA's most loyal supporters, a generous donor and committed Patron who constantly encouraged us to work harder to support research and provide better services to people with neuromuscular conditions and muscular dystrophy.

After a lifetime of giving back to help others, it's only fitting that we take this opportunity to pay tribute to the late Mr Stan Perron AM, an exceptional businessman who built an empire after a difficult start to life. He was undoubtedly one of Perth's most generous philanthropists and a genuinely humble and passionate man.

Mr Perron was our Patron for the last 32 years. During that time, he maintained a deeply personal interest and stayed abreast of the latest research and MDWA programs making a difference to the community, as well as extending his support philanthropically through The Stan Perron Charitable Foundation.

In addition, Mr Perron leaves his legacy for years to come through the Perron Institute of Translational Science and his continuous investment into research and future treatments for neuromuscular conditions, including muscular dystrophy.

This support was further extended by Perron Group's Toyota WA, who came on board as the major sponsor of the Toyota Muscular Dystrophy Golf Classic more than a decade ago.

Mr Perron was honoured with many awards and was appointed a Member of the Order of Australia for service to business, commerce and to the community, all deservedly so.

However awards were never Mr Perron's motivation. He treated everyone equally, no one was more important than anyone else. His unwavering business ethics were the cornerstone of the Perron Group.

Mr Perron remained an unassuming, fair and private person, demonstrating humility within a business environment as well as through his philanthropic giving.

His passing in November 2018 was sudden and unexpected. This wonderfully generous gentleman leaves a hole in the hearts of many.

His legacy is rich and will live on through his charitable foundation that will continue to touch lives, just as it has done for the last 40 years.



The Board

MDWA is fortunate to boast a dedicated board who ensure the association remains focussed on achieving our mission. The board is elected by the association's members during the annual general meeting.



BRAD GIRDWOOD,
President

Board member since 2014



BRENDAN MURPHY,
Vice President

Board member since 2014



TANVI HARIA,
Treasurer

Board member since 2016



JODIE HATHERLEY,
Member

Board member since 2015



SUSAN TRAHAR,
Member

Board member since 2010



JANE EDWARDS,
Member

Board member since 2014



DR PETER W ROWE,
Member

Board member since 2012



AARON SCHIER,
Member

Board member since 2017



SHANE POWELL,
Member

Board member since 2017

President's Report

I am honoured to once again address you as president of our board. 2018 was another fantastically successful year for our organisation carrying on the great work of our first 50 years.

I would like to thank all of our board members for their support during the year and their ongoing commitment to our community. To be honest, our job is made simpler by the tireless efforts of our CEO, all staff members and volunteers. On behalf of the whole board, I thank you all.

During the year we saw the departure of long-serving board member Geoff Woods. I would like to acknowledge and thank him for more than 15 years' service on the board. He was instrumental in shaping our organisation and the way we operate. I would also like to welcome Tanvi Haria to the position of treasurer, which she has very willingly taken on.

Towards the end of the year we were deeply saddened by the passing of our Patron, Mr Stan Perron. Words do not show justice to his generosity and philanthropic achievements for so many groups and charities, particularly in Western Australia. There is no doubt his rich legacy will live on through these organisations. Our heartfelt condolences go to his family and friends, the wonderful people at Toyota WA and everyone at the Perron Group. He will be sadly missed.

To our honorary life members, volunteers, donors and partners, another round of thanks for your support. The entire board is privileged to serve our community and that will continue in our commitment to research initiatives, social support, advocacy and provision of help wherever possible.

Brad Girdwood, President



Staff

Hayley Lethlean, CEO, is employed to drive the realisation of MDWA's vision. With enthusiasm and dedication, she leads the team of staff to implement the strategic goals and objectives of the organisation in line with the Strategic Plan. She works with all sub committees of the board including the Finance, Audit and Risk Committee, enabling the board to fulfil its governance function.

Piper Marsh, Community Services Manager is responsible for improving the delivery of overall community services from MDWA. She manages the Community Services Department to ensure strategic objectives are delivered through a consistent, quality and compliant program whilst liaising directly with relevant statutory and voluntary organisations.

Mark Hullett, Partnerships & Fundraising Manager, ensures key partnerships are maintained and continue to grow. With an in-depth knowledge of corporate partnerships and community fundraising, Mark is responsible for developing and enhancing existing and new fundraising events & activities, alongside the Brand Awareness & Engagement Manager.

Emma Kenton, Brand Awareness & Engagement Manager, plans, develops and directs marketing and communications efforts to increase the value, performance and market growth of MDWA by focussing on ensuring the right message is delivered for the organisation and its services. She's responsible for putting MDWA on the map, to raise awareness of the condition and to increase revenue streams alongside the Partnerships & Fundraising Manager.

Brianna O'Donnell, Communications Manager, is responsible for communicating key messages to defined target audiences to establish goodwill and understanding between MDWA and its stakeholders and to deliver information pertinent to our community and supporters. This is proactively achieved through managing social media and

chasing broadcast, print and new media opportunities. She also handles the communications journey for participants in fundraising events.

Toby Gummer, Administration & Information Systems Manager, provides a wide range of administration services and support to facilitate the activities of MDWA. He's responsible for implementing and overseeing IT needs, ensuring systems and processes run efficiently and effectively. As well as maintaining our databases and processing banking, he works closely with the entire staff to provide support for events, meetings and other activities.

Sarah Williams, Community Services Officer, engages and works with the muscular dystrophy community to provide individual advocacy and support in response to their needs and wellbeing. Working closely with the Community Services Manager and alongside the Community Programs Officer, she also provides training, education and individual support which builds the skills, knowledge and capacity of the community.

Brooke McGregor, Community Programs Officer, coordinates the delivery of the community services programs, social activities and social wellbeing initiatives. Working alongside the Community Programs Officer and under the direction of the Community Services Manager, she ensures events and activities are relevant and meet the needs of our community. She also provides our volunteers with the support and training needed to ensure programs are delivered successfully.

Cathy Donovan, Bookkeeper, works closely alongside the CEO and Treasurer to oversee all financial aspects of MDWA. From managing our budgets and cash flow, to processing income and expenses and coordinating the annual audit, she ensures the smooth running of the organisation's finances behind the scenes.



CEO's Report

Firstly I'd like to acknowledge how deeply saddened we all were by the passing of our longstanding Patron Mr Stan Perron and we continue to hold his family and loved ones in our thoughts. We are forever grateful for Mr Perrons' philanthropic and charitable heart and his legacy will live on through our relationship with the Perron Group and all at Toyota WA.

Over the past 51 years we've made incredible progress providing ongoing support and investment into research. In 2018 we continued that commitment by granting an extension to Dunhui Li, our PhD student working with Prof Sue Fletcher and Steve Wilton at Murdoch University and The Perron Centre.

Our support of Paediatric Respiratory Physician Dr Adelaide Withers through Telethon Kids Institute and their study of the clinical predictors of respiratory failure in paediatric neuromuscular conditions is ongoing.

We have also contributed to two exciting new projects through Perth Children's Hospital assisting with the dietitian in attendance at neuromuscular clinics; and increased hours for the neuromuscular nurse to enable the safe delivery of the newly-approved TGA drug Spinraza® for spinal muscular atrophy.

While we know that research advances remain essential, there is work to be done in providing daily support for those living with muscular dystrophy and neuromuscular conditions. After listening to our community during the past 12 months, our primary focus was to devote energy and time into increasing our resources to support them. We welcomed Brooke and Sarah to the team in 2018 and their enthusiasm, knowledge and genuine caring nature provided expansion to the support services already offered.

Being a small not for profit we know increased awareness is essential for our growth and to keep increasing our services, so we were delighted that Emma Kenton officially joined the Communications and Fundraising team this year. By expanding within these areas we increased our capacity to raise funds and our footprint in WA.

It was always going to be a tough job following the events and fundraising efforts of our 50th anniversary and while we have posted a deficit this year, this was anticipated and planned, but ensured our community needs could be enhanced. Once again we thank our longstanding corporate partners Toyota WA, Kailis Bros and Myattsfield Vineyards for their commitment and dedication. We also acknowledge the individual fundraisers who went above and beyond to back us and generate ongoing income. Fundraising events like the Toyota WA Golf Classic, Dwellingup100 and Truffles for Muscles all exceeded fundraising expectations and 2018 also saw the successful delivery of the new Busselton Toyota Golf Day in Dunsborough.

Our team extends to our board and our volunteers, and I thank the board for their unwavering guidance and focus on ensuring we keep kicking our strategic goals. Operationally everyone stayed on task, continuously reflecting on our strategy when planning events, education and activities with our community. I thank you all for your ongoing commitment to our cause and for working hard to deliver on our goals.

I acknowledge our Ambassadors Adam Voges, Simon Beaumont, Ross Wallman and Renee Baker who continue to raise our profiles and in turn, money, as they represent us in the media and wider community.

Collaboration remains one of the biggest opportunities for all like-minded organisations and nationally Muscular Dystrophy Foundation alongside the States and Territories it represents secured an NDIS grant to deliver a national digital resource to the Australian neuromuscular community. This initiative was driven by MDWA and we will continue to work diligently with our community to bring this initiative to fruition over the next 18 months.

At a State level, collaboration with Telethon Kids Institute, Rare Voices Australia, Spinal Muscular Atrophy Australia, Save our Sons Duchenne Foundation and those residing at the Centre for Neurological Support continued to deliver opportunities for networking and tangible outcomes for the community.

In closing, 2018 was another successful year for MDWA, but it doesn't stop here. I promise to always put the needs of our diverse and beautiful community first when making business decisions, because they are at the heart of what we are and what we do. I feel privileged that we have the opportunity each year to work harder in providing *Support for Life* to them.

Hayley Lethlean, Chief Executive Officer



Treasurer's Report

On behalf of the Board I am pleased to present the 31 December 2018 Annual Financial Report, which includes the Financial Statements and Independent Audit Report.

The Statement of Comprehensive Income shows a modest surplus prior to research grants for the year of \$67,599 compared to last year's surplus prior to research grants of \$258,502. The reduced surplus was largely due to increased expenses for the community services program and reduced net income from general donations, bequests and value of investments compared to last year.

Although the Perth economy has continued to remain subdued and net fundraising and donations are lower for the year, net grant income has increased to \$320,438 (increase of \$142,383). 2017 is acknowledged as an important milestone, celebrating the 50th anniversary of MDWA resulting in significantly increased revenue raised through donations and fundraising. It is pleasing to note the ongoing generosity of donors and the community has continued with positive support for the Truffle for Muscles Auction, the Toyota Muscular Dystrophy Golf Day and other

initiatives and grants pursued through the excellent efforts of the MDWA team. These are continuing to generate consistent income for the association.

Due to continuing low interest rates on bank deposits and our investments in growth equities and managed funds in the last 12 months, the total market value of investments of \$1,571,272 reflects a reduction of \$98,097 compared to prior year. Whilst the lower value is reflective of general economic and market conditions, our investment strategy continues with a responsible and long-term investment perspective in targeting equity markets. This provides us with better short-term income by way of fully franked dividends and managed fund income distributions with the long-term view to higher capital growth in future years. Our long-term view remains at a minimum three to five years.

Finally, the net assets of the association decreased slightly during the year from \$2,382,347 to \$2,298,232 as at December 2018, after the research grants and scholarships of \$151,714 compared to last year's \$81,882.

I take this opportunity to acknowledge the significant and valuable contribution made by our outgoing Treasurer Geoff Woods who dedicated his service to the Association over the past 15 years. I thank the board, members and community for their continued support.

Appreciation is also extended to our CEO Hayley Lethlean and her dedicated staff and team of volunteers for their fantastic contribution, commitment and professionalism during the year.

Tanvi Haria, Treasurer





Community Services Program

We anticipated 2018 would be a year of returning to normalcy after the hectic year of celebrating our 50th Anniversary in 2017, however 2018 had other ideas. It began with Jessica Crute, our Community Services Officer, leaving in February. This provided an opportunity to look at the structure of the Community Services Department and to really consider the current and future needs of the community and sector. After all of this was taken into consideration we welcomed Sarah Williams into the new role of Support Services Officer and welcomed back Brooke McGregor after an eight year hiatus, into the role of Community Programs Officer, both in April 2018.

With the newly expanded capabilities of the Community Services Department, we believe we are now better placed to provide the support services the community require. We are already seeing the evidence of this with a record number of 41 social programs being conducted in 2018, up 24% on our previous record year of 2017 of 33. We also saw the introduction of a couple of new programs – Coffee Mornings and Family Days.

The Community Services Department is very proud of their 2018 achievements and believe we have laid the foundations in our quest to provide the Support for Life the community not only need, but want.

Social Programs

SCHOOL HOLIDAY PROGRAM (SHP)

We commenced 2018 with a SHP in January, the first time the program has ever been conducted during the Christmas holidays. The children that attended each day participated in activities such as nature flag making, pizzas, tie dying, a scavenger hunt and a cartooning workshop.

The July program was held at Rocky Bay due to the refurbishment at the Niche. We thank Rocky Bay for their generous support on the day.

CAMP

2018 saw a major change for our camp funded by Lotterywest & Telethon. We moved from our long established home at Landsdale Farm School to Ern Halliday Campsite. At this new venue the campers were able to experience a more authentic supported camp environment which offered a multitude of activities including archery, scavenger hunts and campfires. However, by far the highlight was the Urban Indigenous Art session.

The Community Services team would also like to thank the Western Electric Sports Association for their time and support of our school-aged programs.





ADULTS WITH NEUROMUSCULAR CONDITIONS

Adults with neuromuscular conditions attended two events during 2018. At the first event they enjoyed a wine and cheese night during March which was supported by Myattfield Vineyards who showcased some of their best wines.

In August they also enjoyed a beer, pizza and football night.

YOUNG ADULTS SOCIAL GROUP (YASG)

Four YASG events were conducted in 2018. The first was at the fabulous February Fringe Festival event. Next they competed against each other to be named Pub Quiz Champion in June and in August they were among the first to experience movies at the Old Girls School. October saw our social group members get together to celebrate Oktoberfest at The Belgian Beef Café and the year culminated in the YASG Christmas lunch at Coast.



MALE CARERS

Our male carers learnt how to craft beer at The Monk's Brewery School in Fremantle in March and then joined us for The Westwinds Gin Masterclass at the Leederville Hotel in December.

FEMALE CARERS

While the male carers learnt how to craft beer and gin, our female carers enjoyed a wine tasting at Sandalford Wines followed by a relaxing 2 course lunch at Mandoon Estate. December then saw our female carers enjoy the city views during a decadent high tea at C Restaurant.



All of our adult events are very important to MDWA as it allows us to link people with MD, parents, partners and family members to others experiencing similar circumstances. It also provides a great opportunity for some much-needed and well-deserved laughs.

HALE MATES

Following on from a very successful first year of this vital capacity building program, MDWA and Hale School, with the support of Telethon, looked to increase the key aspects of this program during 2018, in particular providing greater opportunities for peer mentoring and social inclusion. This was achieved through:

- > Providing the Hale School peer mentors with greater opportunity to direct the program and, in doing so, develop greater program ownership
- > Delivering more one-on-one and small group activities to encourage greater inclusion and participation. This included well-considered, inclusive activities such as orienteering, cooking, tower building, gardening and science experiments, ensuring all participants had fun and there was maximum participation



HALE MATES CONTINUED

- > Providing greater capacity building/self-development activities to assist with social interaction and community engagement. Activities included gaming opportunities, working as a team with remote control race course, pool party fun and giant game competitions

2018 saw six Hale Mates participants and 12 Hale mentors attend on a regular basis.

The Hale Mates program finished the year with a huge Christmas pool party where mentors and participants swam, had loads of fun and reflected on a wonderful year.

The Community Services Team would like to thank Hale School, the 2018 Hale Mentors, Wendy Keene, Matt Haddon and Ross Wallman for making this program such a success.

COFFEE MORNINGS

We introduced the neuromuscular community to our Coffee Morning program held on the first Tuesday in every month. The mornings provide a chance for people in the community to drop by have a cup of coffee, meet some new people, catch up with old friends or just chat with one of our staff members.

FAMILY DAYS

The second new program introduced in 2018 was our very successful Family Day. This program was developed as a transition program to assist young children with neuromuscular conditions to move into our school-aged services.

We see these days as a way for young children and families to meet other MDWA families in a relaxed and familiar community setting. This means the kids will have the best opportunity to meet some of the other participants attending our programs while their families are present. Parents also have an opportunity to get to know our staff and other members of the neuromuscular community.

The first Family Day was held at Perth Zoo in April followed by lunch time at AQWA in July.

ANNUAL COMMUNITY DAY

As always, 2018 ended with our Annual Community Day. Even a little bit of rain could not dampen our spirits as 100 members of our community enjoyed a BBQ and games in Kings Park. A big thank you to Lotterywest for again supporting this day. Thanks also to Beyond Bank for supplying the volunteers to cook and serve our BBQ and to Princi Butchers for supplying the meat.

This day is always a great opportunity for the neuromuscular community to connect, relax and reflect on the year that has been.





Education Program

Two significant education events occurred last year:

- > SMA Wellness Program – We supported SMA Australia with their February Wellness Program where 26 people heard about the latest research information including updates on Spinraza
- > An Evening with MD Reserachers – inconjunction with the Telethon Kids Institute, 50 members heard presentations from Dr Andrew Wilson and Dr Adelaide Withers on Respiratory Health & Lunch Function in MD and New Standards of Care in MD

Professional Development

The Community Services Team also participated in a number of professional development opportunities to ensure we are able to always provide accurate and up to date information. This included:

- > Attending the ACTT Now Save Our Sons Duchenne Foundation Conference
- > Participating in a range of NDIS education sessions, workshops and forums
- > Undertaking training as a Resilience Doughnut Facilitator
- > Training in family and domestic violence
- > Participating in SECCA's sexual health training

Empower Fund

The first Empower Grants were awarded in 2018, with a total of 14 people receiving support across two rounds. Sports wheelchairs, travel expenses, home and car modifications were just some of the items to be funded, worth more than \$31,000 – and we only expect this demand to grow next year.

Grants pay for items that cannot ordinarily be funded or supplied via other agencies like the NDIS, and where a strong need can be demonstrated.

Below are just a few stories of how the Empower Grants changed our community's lives last year:

With his Empower Grant, Ross was able to modify his vehicle so that he can load and unload himself into his van, meaning he no longer has to rely on taxis to get from A to B and his wife doesn't have to do all the heavy lifting anymore. Getting around on his own has brought back the independence he's missed for so long and understandably this has had a huge impact on the way he feels in his mind as well.

Jakob's dream was to travel to Melbourne and attend a two day celebration of hardcore music. It sounds simple, but is no easy exercise with the added expense of travelling with a support worker and sorting out accessible transport. Thanks to the Empower Fund, this dream to rock out at Lairfest became a reality.



We were able to help Rob and Kylie get back on the road with a new starter motor for their car. The cost was a big outlay in one hit, so Rob and Kylie applied for a hardship grant and we were able to grant funds quickly. For Rob, having accessible transport to get to and from work is essential and their vehicle is his only way of getting out with his electric chair which allows him to be a lot more independent than using his manual chair.

Logan received funds for the materials to have serial casting of his feet, a process that stretches out his tendons to stop his feet from turning or becoming contracted so they sit flat on his footplates and he can wear shoes comfortably. Such a simple thing for him to want to be able to do, but not something that was funded elsewhere.

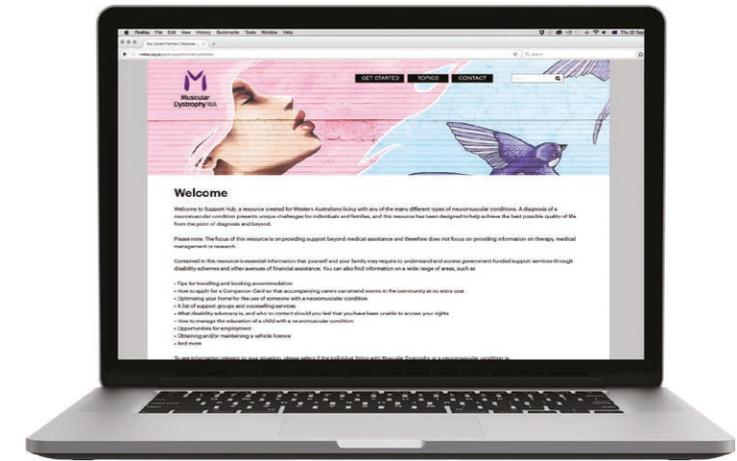
Support Hub

After a lot of development work through 2016-2017 the Telethon funded Support for Life website was launched. This website aims to harness the collective knowledge of the neuromuscular community and house it in one accessible location.

People can access information on funding and financial schemes, support organisations, equipment and travel/transport.

The name has since been changed to Support Hub as we adopted Support for Life as our organisation's motto.

The Support Hub can be viewed at www.mdwa.org.au/support-hub.



400%
increase in contact with our community - nearly 2,500 individual contacts each year

41
social programs delivered in 2018 - up 24% from 2017

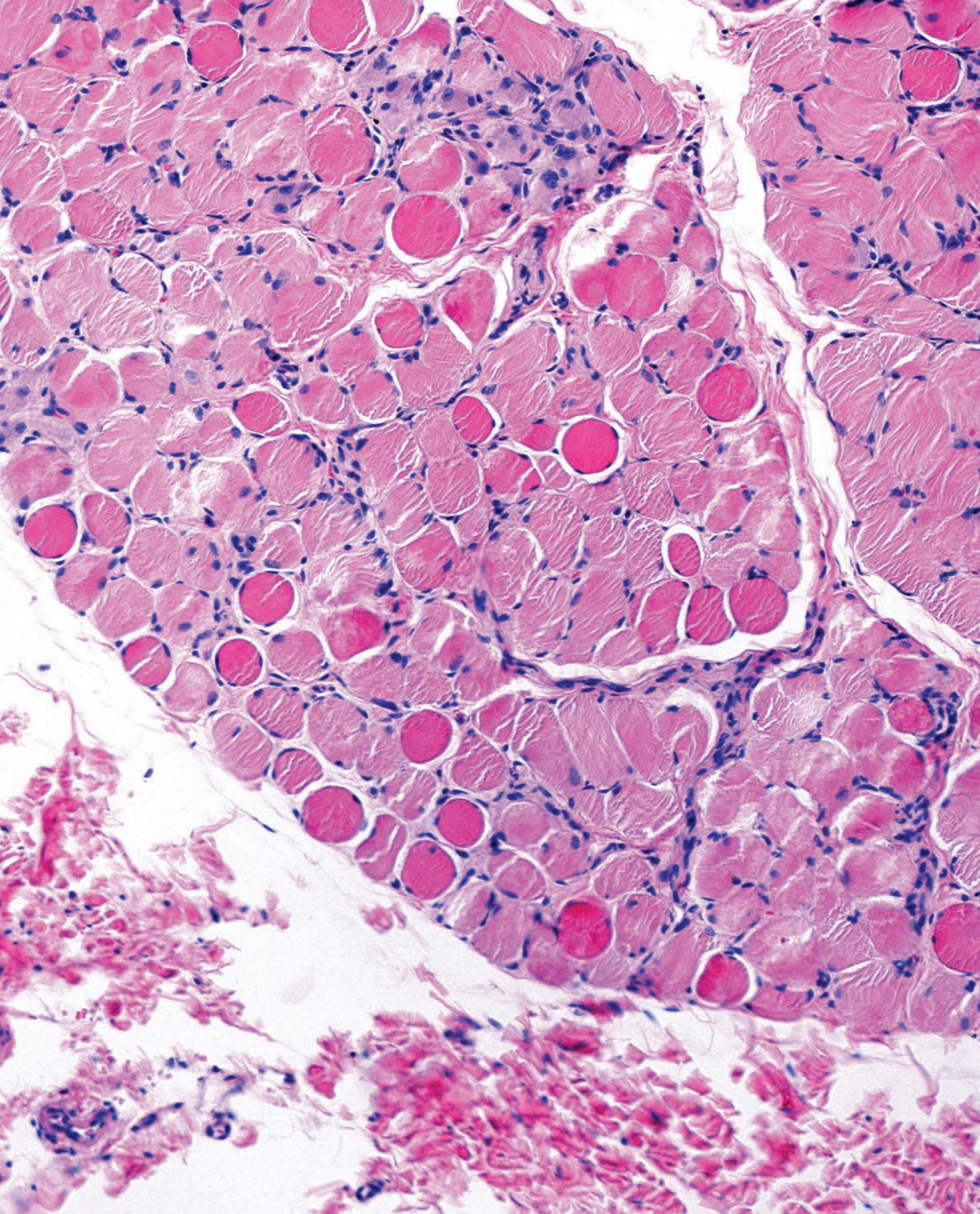
600
hours provided by volunteers, valued at \$25,000 to our organisation

70%
increase in calls from health professionals

55%
increase in calls related to CoughAssist machines

Community Services Program proudly supported by:





Research Advisory Report

The Research Advisory Committee comprises of three board members and CEO Hayley Lethlean. Meeting on three occasions in 2018, we consolidated our current focus areas and implemented approvals for two new focused research initiatives. Our financial commitment into research provisions for Harold and Sylvia Rowell Scholarships, Team Spencer Scholarships and research projects. In addition some investment was allocated internally to enable capacity building for our community wellbeing blog initiative which will launch in 2019.

In the second year of the three year commitment from West Perth Rotary Club, the funds enabled us to invest significantly in our staff to develop and enable greater knowledge in clinical trial awareness.

Another busy year saw ongoing investment into the research projects of Dr Adelaide Withers, Dr Andrew Wilson and Prof Graham Hall. This highly skilled team from Perth Children's Hospital (PCH) and Telethon Kids Institute (TKI) expanded their respiratory research project as successful recipients of a National Health and Medical Research Council (NHMRC) funding grant. MDWA are excited at the opportunities and community outcomes this international collaborative project will bring to paediatric neuromuscular research.

As one of our Harold and Sylvia Rowell Scholarship recipients, Dunhui Li (Oliver) entered his third year of PhD research. You will discover his project focuses on extending 'exon skipping' therapies for Duchenne muscular dystrophy (DMD) beyond the more common mutations.

In 2018 we invested into two new projects both taking place within PCH – a dietitian project and the allocation of funding towards the neuromuscular nurse position to enable Spinraza® administration to 11 SMA patients. We acknowledge and thank the neuromuscular team at PCH for their commitment and dedication to our community and in particular the neuromuscular nurse Jodi Mann who has given selfless hours to these patients and their families. The dietetics program managed by senior dietitian Melanie van der Wilk is a two-year project which will provide enhanced nutrition service to children with neuromuscular conditions at PCH.

As a member of the National Patient Organisation Standing Committee program (NPOSC) Hayley Lethlean represented us once again during 2018. A great outcome from the work undertaken in 2018 was the culmination of a \$1.56M federal funding investment over three years to support the Patient Pathways Program. The program comprises three components - firstly a pilot project testing specialist nurse telehealth case management across 10 disease areas to help patients navigate the health system; secondly the development of a repository of patient experience data to inform future research, clinical care, access to treatments and services and finally an annual conference for health charities in Australia to forge collaboration and reduce duplication.

We welcome you all to read the updates of our research program in detail on the following pages.

Research partners



Harold & Sylvia Rowell PHD Scholarship Recipient, Dunhui Li

MOLECULAR STUDIES ON ANTISENSE OLIGO-INDUCED EXON SKIPPING

Dunhui Li (Oliver) is now in his third year of PhD research, working on strategies to extend 'exon skipping' therapies for Duchenne muscular dystrophy (DMD) beyond the more common mutations.

The dystrophin gene contains 79 blocks of information (exons) that code for the protein dystrophin. The most common DMD-causing mutations occur between exons 43-55, or exons 3-7. The study of dystrophin mutations that cause the less severe condition, Becker muscular dystrophy, has shown that loss of particular exons is compatible with dystrophin function, and lead to exon skipping as a therapy for DMD. Dystrophin function depends upon binding to several partner proteins in order to provide strength and stability to the muscle fibre. Some of the binding interactions involve parts of the protein encoded beyond exon 55, where the gene structure is complex, making it difficult to predict which exons can be skipped to yield a functional dystrophin.

In 2018, Oliver designed and tested antisense oligos to induce Becker muscular dystrophy-like dystrophins that could be used to treat some of the less common DMD mutations. These oligos were tested in cultured cells and then in in mdx (dystrophic) mice. The aim is to generate a map showing exon skipping strategies to treat DMD mutations between exons 55 and 78, with the various dystrophin validated by data from the mice. Results from skipping of dystrophin exons 56 and 57 was inconclusive, while skipping dystrophin exons 58 and 59 reduced the amount of dystrophin but the protein was correctly localized in muscle cells, indicating that the protein has near-normal function. Confirmatory experiments studying the interactions between dystrophin and its binding partners will confirm the functionality of this induced variant.

The strategy being used to extend the range of dystrophin mutations that could be treated by exon skipping, can be applied to other conditions. Oliver has used his experience with DMD to design oligos to treat Parkin-type autosomal recessive juvenile Parkinson's disease (Parkin-ARJP) caused by PARK2 gene mutations, with very encouraging results.

Oliver was selected to do an oral presentation at the 2nd international symposium on "Functional Nucleic Acids" in Perth to showcase the DMD project and also won the Most Outstanding Poster Presentation Prize by a PhD Student at the Symposium of Western Australian Neuroscience. His review paper Precision Medicine through Antisense Oligonucleotide-Mediated Exon skipping, focusing on antisense therapies for Duchenne muscular dystrophy and other inherited rare human conditions was published in Trends in Pharmacological Sciences last November and was cited as one the best reviews published in 2018 by Cell Press.

Harold & Sylvia Rowell Research Project Grant, Annie Robertson & Melanie Van Der Wilk

IMPROVING NUTRITION IN WESTERN AUSTRALIAN CHILDREN WITH NEUROMUSCULAR CONDITIONS

The neuromuscular service at Perth Children's Hospital (PCH) now has a dedicated dietitian thanks to funding support provided by MDWA. This two-year project is an exciting initiative that provides children attending the Neuromuscular Clinic at PCH with an enhanced nutrition service. The grant was originally awarded to Annie Robertson, Senior Dietitian at Perth Children's Hospital. While Annie is on maternity leave the position is being filled until her return by Melanie van der Wilk, also a Senior Dietitian at Perth Children's Hospital. Both Annie and Melanie have over ten years' experience as clinical paediatric dietitians and both have worked with children with neuromuscular conditions (NMCs).

Children with NMCs can face a number of nutrition challenges. In the early years this may range from poor growth to excess weight gain. Older children and adolescents may also struggle with swallowing difficulties, constipation or reflux. And for all children, good nutrition is a critical part of optimising health. The funding received from MDWA has enabled the dietitian to attend all clinics and be available to all children. Families can access nutrition counselling at key milestones such as diagnosis or commencing steroids, or when advice is needed for issues such as faltering growth or weight management. The dietitian is also screening the growth of all children attending clinic to identify any who may benefit from a nutrition consultation. Nutrition screening will provide valuable information on the nutritional status and needs of the children and help with future planning of dietetic services.

The dietitian is also developing evidence based clinical guidelines for the nutritional management of NMCs. Duchenne muscular dystrophy is first in line with a clinical guideline close to being finalised. From this a range of family resources are being developed and those for Duchenne muscular dystrophy will be available soon. Next will be the development of clinical guidelines and family resources for Spinal Muscular Atrophy followed by other neuromuscular conditions. All resources will be available to children coming to clinics at PCH and will also be made available to families via the PCH website.

The next phase of the project will include establishing a nutritional database to help identify the key nutritional issues facing children with NMCs and undertaking nutrition-related research. The ultimate goal of the project is to improve the nutrition of all children attending the neuromuscular clinic and demonstrate the need for an ongoing specialised nutrition service for WA children with NMCs.

Team Spencer MDWA Scholarship Recipient for Spinal Muscular Atrophy (SMA) Research, Dr Adelaide Withers

DEVELOPING CLINICAL PREDICTORS OF DISEASE PROGRESSION IN CHILDREN WITH NMC CONDITIONS - PCH, TKI & STANFORD UNIVERSITY SCHOOL OF MEDICINE, USA

The Neuromuscular Research Team at Perth Children's Hospital (PCH) has continued to grow in size with several new members from Telethon Kids Institute (TKI) joining the team. Recruitment and data collection continues for the pilot project examining predictors of hypoventilation and other markers of respiratory disease progression. Some participants are nearing the end of their initial follow-up period and the team are looking forward to analysing their data.

The team are recipients of a prestigious National Health and Medical Research Council (NHMRC) funding grant. This research is occurring in collaboration with Stanford University, California USA. This is a wonderful opportunity for international collaboration and to raise the profile of paediatric neuromuscular research.

Researchers have continued to measure using the multiple breath washout test (a lung function test) which is not a standard clinical test for children with neuromuscular conditions. It's been found that many participants can perform this test successfully and it's hoped the findings will support use of this test in routine clinical practice as it is much easier than some of the other lung function tests for children, particularly those with neuromuscular weakness.

Investigating home sleep studies as an alternative to sleep studies at the hospital for children with neuromuscular conditions is next on the agenda. Researchers and practitioners understand many families would prefer this as it can be difficult to travel to the hospital for an overnight stay (particularly with equipment) and children may not sleep well in an unfamiliar environment. Currently home sleep studies are not commonly done in children with neuromuscular conditions as some of the monitoring equipment which is essential for detecting hypoventilation is not included with routine home sleep studies. Currently being written is a specific protocol for home sleep studies for patients and the next step will be conducting a trial to validate the use of home sleep studies, comparing them to hospital-based sleep studies.

The team continues to strengthen ties with the neuromuscular community primarily by collaboration with the consumer reference group but also via participation in community information evenings, as invited speakers at meetings and conferences. The research team and in particular Dr Withers remain incredibly grateful for the ongoing support from MDWA and look forward to the continuation of the collaboration between researchers, MDWA, families, patients and the wider neuromuscular community.

Team Spencer Project Grant

SPINRAZA FUNDING AT PERTH CHILDREN'S HOSPITAL

In June 2018 the drug Nusinersen (Spinraza) for the treatment of Spinal Muscular Atrophy (SMA) was added to the Pharmaceutical Benefits Scheme (PBS) in Australia. SMA is a rare genetically-inherited condition that affects the part of the nervous system that controls muscle movement, resulting in the deterioration and eventual loss of cells in the spinal cord called motor neurons.

Nusinersen is an antisense Oligonucleotide that works by helping the body to produce more survival motor neuron protein which in turn reduces the loss of motor neuron nerve cells and improves muscle strength.

Our SMA families here in Western Australia have been watching the positive clinical outcomes of this medication for several years as they have unfolded around the world in clinical trials. Availability through the PBS has been much anticipated by all, including health professionals.

Funding from TEAM Spencer and MDWA has allowed Perth Children's Hospital (PCH) Neurology Department Clinical Nurse Jodi Mann to assist Paediatric Neurologist Dr Maina Kava to immediately start loading doses of Nusinersen in June 2018. They have 11 SMA paediatric patients at PCH and although it is early days they have already seen some amazing improvements in the health of these children, motor function and overall quality of life.

The delivery of Nusinersen is through an injection into the lower back in a procedure known as a lumbar puncture. The medication is administered directly in the child's cerebrospinal fluid. Treatment starts with a series of loading doses day 0, 14, 28 and 63, then maintenance doses every 4 months for the rest of their lives.

They have been able to set up a very successful Nusinersen program at PCH with all doses being delivered in their Day Treatment Unit and under minimal oral sedation. Most certainly the secret behind this success is not only the positive approach the families have to this procedure, but also that Dr Kava and Jodi perform each procedure together and are therefore able to cater to the individual requirements of each child, ensuring continuity of care and most importantly gain trust as this is a repeated invasive procedure.

For Jodi personally as a Paediatric nurse for over 25 years, it has been an absolute privilege to not only get to know and nurse these very special children but to be involved in the delivery of a medication such as this that has completely changed the natural history of what was a very devastating condition.

All of this would not have been possible without the generosity from Rick and Ruth Steven (TEAM Spencer) and MDWA. On behalf of the SMA families and the Neurology Department at PCH, they acknowledge the contributions and are extremely grateful for the support.



Power from the People

People are MDWA's biggest asset. A huge part of our event calendar engages supporters from all walks of life to raise funds and awareness for our organisation and community.

Events range from swinging a rusty golf club to sipping G&Ts with oysters and truffles, to hurtling down a muddy clay bank in Dwellingup on a borrowed mountain bike, and everything you can imagine in between.

These activations are invariably great fun and provide the platform for our people to make a tangible difference whilst having an awesome time.

TOYOTA MUSCULAR DYSTROPHY GOLF CLASSIC

The 11th instalment of the Golf Classic saw a strong field of 124 golfers once again pit their skills against the picturesque Joondalup Resort Golf Course on Tuesday 13th March. The weather made life tough on-course, with temperatures reaching the 40 degree mark, though the generosity of spirit amongst our band of merry golfers never waned.

It was rewarding and exciting to see so many new corporate teams contributing to this event, along with many seasoned veterans, some of whom have played in all previous 10 Golf Classics.

Post golf, there was a wonderful vibe in the poolside marquee where guests enjoyed cocktail food and drinks, presentation of awards and several fundraising activities, superbly hosted by MC Simon Beaumont. Regular auctioneer Tiny Holly kept the audience on their toes and amused with his mock horse racing commentary, which helped raise funds for the cause.

Whilst the heat and humidity made for a tough day, nothing could dampen the enthusiasm of our supporters who helped to raise the most funds ever at one of our Golf Classics, with a net gain in excess of \$68,000.

SUNSMART IRONMAN 70.3 BUSSELTON

Triathlon WA and the SunSmart Ironman 70.3 Busselton event held May 4th-6th once again provided a platform for our committed team of triathletes who went the extra mile to TRIumph over muscular dystrophy.

We thank the ever-present race commentator and MDWA Ambassador Simon Beaumont for constantly plugging the work of MDWA during the race call and bringing awareness of the cause to a broad and fresh community.

Our team of volunteer supporters combined with the purple army who not only completed the challenging course, but also raised funds to see the team generate over \$13,000.





HBF RUN FOR A REASON

2018 saw the biggest ever fundraising result for the HBF Run for a Reason on May 27, with over 65 people participating and raising a total of \$34,000 for us.

Maddy Long and husband Mark rallied a team of 50 family and friends in honour of their baby boy Koen, who was born in 2017 with an extremely rare neuromuscular condition but sadly passed away only 12 days later. Raising over \$20,000, they were not only our teams' top fundraisers, but they were the top fundraisers for the entire event winning two cash prizes that they added to their fundraising tally. We were blown away by their strength, compassion for others, and their dedication to fundraising.



TRUFFLES FOR MUSCLES CHARITY AUCTION

The Truffles for Muscles Charity Auction has become a significant and iconic part of our annual event calendar and in its fifth iteration the auction produced a most spectacular fundraising result from a small but spirited audience on Thursday 28th June.

The warmth in the room was palpable and the event reached fever-pitch when two generous bidders battled it out to purchase a 16-seat corporate suite at Perth Arena which had been kindly provided by Murdoch University.



First-of-the-season Manjimup truffles were up for grabs alongside amazing hospitality experiences, a row of grapes to call your own and a wonderful ocean cruise to the Kimberleys.

As with the previous four auctions, we had unstinting support from Theo at Kailis Bros Leederville who hosted the night, Josh and Rachael from MyattsField Vineyards who donated the wine and Al Blakers, Manjimup Truffles, who donated the hero of the night by the kilo.

Event Ambassador and chef Russell Blaikie provided an incredible recipe - a warm tart of Manjimup kipfler potatoes and truffles with Bokara goat cheese whip. Guests also enjoyed delicious Kailis Bros seafood, washed down with G&T from The West Winds Gin and were treated to macarons made by community member Sarah Kerr.

The event also saw the continuation of our live appeal where guests were encouraged to donate to our Empower Fund.

Truffles for Muscles owes much of its success to the compering skills of our regular MC and Ambassador, Simon Beaumont and his trusty sidekick, the side-splitting auctioneer Tiny Holly, who together helped to generate over \$134,000 while entertaining 100 guests in style yet again.



DWELLINGUP WA 100 MTB CLASSIC

With the sudden and unexpected closure of TriEvents the Dwellingup 100's future was in jeopardy. Thankfully the event lived to see another day due to the mammoth task taken on by Tony Tucknott, John Carney, Single Track Minds, Wembley Cycles and their team who put in blood, sweat and tears to make this event still happen on 18th August.

35 team members who rallied together to raise an amazing \$65,000 were decked out in our purple jerseys which were generously sponsored by Facilitatrix, Fortuna Financial and MACA.

The Kyle Conor Spirit of the Ride award was given to two deserving recipients - Peter Campbell and Tony Tucknott. Not only is Peter the only person to ride all 10 x 100 km events in Dwellingup but for the last 5 years he has raised funds for us, incredibly totalling over \$10,000. As for Tony - we owe this man a lot, his passion for mountain biking is only matched by his passion for flying our flag.

MARQUEE DAY FOR MDWA

With a fast-growing profile and reputation as a Perth racing identity and racehorse owner, community member Amy Evans took on the role of Ambassador for Marquee Day for MDWA on Saturday October 23rd.

Amy not only helped publicise the event, she even formed her own table of guests and provided the room with a few winners from her hot tips during an inspiring interview with guest speaker Brittany Taylor and MC and Ambassador Simon Beaumont.

A full card of high quality racing was coupled with a deluxe lunch and beverage package in the well-appointed Director's Lounge at Ascot Racecourse, providing the perfect atmosphere for another terrific day, raising awareness and critical funds of over \$43,000.

BUSSELTON TOYOTA MD GOLF DAY

The Toyota relationship grew again in 2018 with the determination of Laurie Saltarini from Busselton Toyota helping turn the concept of a rural golf fundraiser into reality on Friday November 23rd.

By negotiating with host club Dunsborough Lakes, Laurie was able to secure 19 teams to get the pilot version of this event off the ground, which lays a terrific foundation for future years.

Dinner and presentations were held in the clubrooms after golf and the 80-strong audience participated in a raffle and silent auction, digging deep to raise over \$16,000.





CAROLS AT SCARBOROUGH

Brought to you by Scarborough Beach Association, Carols at Scarborough was held on Sunday 16th December in the stunning Amphitheatre at Scarborough Beach. For a second year in the row MDWA were the charity partner.

It's estimated that over 5,000 people packed the amphitheatre and beach for a night of community carolling and Christmas spirit with our Ambassador Simon Beaumont hosting the event.

It was fantastic to have so many of the MDWA community come down to join in on the event.

Volunteers were in full force selling candles and songbooks, running a raffle and collecting donations. The night raised just over \$7,500.



DINNERS FOR MDWA

Dinners for MDWA were launched in 2018, an initiative that sees a group of 50 people connected in some way to the organisation buy tickets to attend dinners throughout the year at various restaurants, with part proceeds going directly to MDWA.

Two dinners were held in 2018 – the first at Must Wine Bar and the second at Clarke's of North Beach. All wine was provided at a discounted price by our wine partner MyattsField Vineyards, ensuring more went back into fundraising. \$3,400 was raised between the two dinners.

PAPERCUP PROJECT

MDWA became involved with the Papercup Project in 2018, which saw 100% recyclable paper cups adorned with amazing art and our message – an awareness exercise.

100,000 coffee cups were circulated into various cafes, with 50,000 of them at Engineroom Espresso in North Perth who's owner, Chris Niven, took his support a step further by running his first of many sundowners which raised over \$1,100 for us.

Other cafes have got involved by promoting us on social media, selling special items and even donating their tips.



VOLUNTEER RECOGNITION

We've been so lucky to have the lovely Sue Fidock volunteering with us for the last 12 months on an almost weekly basis. We met Sue over 11 years ago when Toyota WA started sponsoring our Golf Classic.

Sue was in their head office and always worked so tirelessly promoting the day, encouraging dealers far and wide to jump on board and support us.

She's been a regular face at our fundraising events over the last year, is always the first to put her hand up to help, and is reliable and trustworthy. She's whipped our database into shape, packed gift bags like you wouldn't believe and brings a bright happy attitude into the office with her every time she comes. Thank you Sue.



AMBASSADORS

In 2018 we further cemented our ties with our Ambassadors – leading to great outcomes for our organisation and community.

Simon Beaumont continued as our MC extraordinaire at many of our events. Through his knack for storytelling and interviewing, Simon makes our message relatable to everyone and has a huge impact on our fundraising and awareness.

WA cricketing legend Adam Voges attended as many of our events as his schedule allowed and even donned purple with his family to pound the pavement at the HBF Run for A Reason. He also co-hosted a dinner, sought out auction items and threw his profile behind our cause.

Ross Wallman joined our ranks as a new Ambassador after being involved with us since early 2017 via the Hale Mates program. An old Haleian himself, he has injected his cheery personality into the program, acting as a mentor and facilitator for the group – making all the kids feel at ease. He also uses his strong social media presence to raise our profile and has MC'd an event for us as well.

After welcoming her first baby, Renee Baker took a small step back in 2018 but continued to lend her support by promoting the Busselton Ironman and appearing at fundraising events throughout the year.



MAJOR CORPORATE AND COMMUNITY PARTNERS

We are so grateful for the generous support we continue to receive from our partners. Their backing allows us to deliver a holistic service to our community. If you are interested in partnering with us, talk to Mark to find out how you can get involved.

To our major corporate and community partners, we say a heartfelt thank you!





Muscular Dystrophy Association of WA (Inc) | ABN 49 158 959 834

We'd like to acknowledge that Sarah McCloskey allowed us to use the imagery of the mural she painted in Leederville for our staff and community photos. You can buy some of her beautiful work through her website <https://www.hellosarsar.com/>