



**Muscular
Dystrophy WA**

2016 ANNUAL REPORT

MESSAGE FROM THE PATRON



I feel very honoured to have been the Patron of Muscular Dystrophy WA for the past 30 years.

Muscular Dystrophy WA was established in 1967, and together with the Muscular Dystrophy Community, celebrates 50 years of going from “strength to strength”. They are a passionate organisation whose aim is to improve the quality of life and wellbeing of people living with MD and their families.

Professors Steve Wilton and Sue Fletcher joined the Western Australian Neurosciences Research Institute (WANRI) in the early 1990s, and together their research team has been at the forefront in developing a therapy for Duchenne muscular dystrophy.

Their recent breakthrough is creating a new drug to tackle the side effects of muscular dystrophy, which will be safer and more effective than current approaches. The professors should be congratulated on the recent success and achievement.

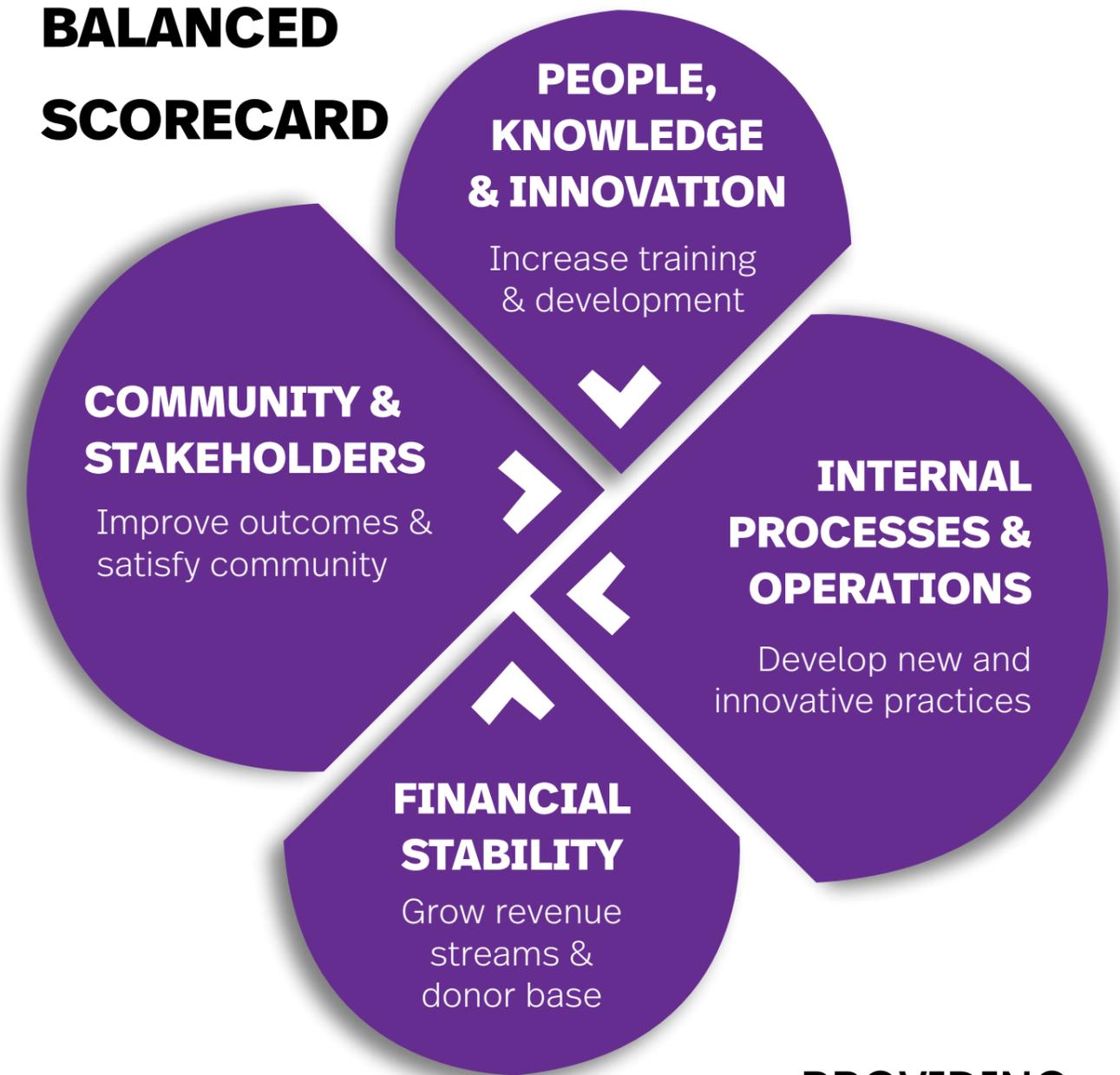
Muscular Dystrophy WA has faced many challenges over the years, but together with their many partners, they have been able to help and support those with muscular dystrophy, to ensure they reach their full potential.

It is a privilege to continue the journey with Muscular Dystrophy WA and help them achieve their mission of “Empowering full lives”.

Mr Stan Perron AM CitWA



THE BALANCED SCORECARD



STRENGTHENING BONDS

RAISING AWARENESS

SUPPORTING RESEARCH

PROVIDING SUPPORT SERVICES

LINKING WITH SERVICES

MEET OUR BOARD



Brad Girdwood
President

Bachelor of Engineering
10 years of business management
2 years' Board service



Brendan Murphy
Vice President

BEng, Mining Engineering (Hons)
Graduate Diploma,
Banking & Finance
Senior site management positions in global & local mining companies both in Australia & overseas
2 years' Board service



Geoff Woods
Treasurer

Bachelor of Business Degree
Fellow of CPA Australia
Chartered Tax Adviser of The Tax Institute
14 years' Board service



Susan Trahar
Member

B App Science (Speech & Hearing)
LLB (Hons)
Graduate Diploma (Legal Practice)
6 years' Board service



Dr Peter W Rowe
Member

Paediatric Neurologist with a dual appointment at Princess Margaret Hospital (PMH) & the State Child Development Centre, Perth (SCDC)
5 years' Board service



Jane Edwards
Member

Background in public relations, marketing & communication, Post Graduate Certificate in Business Administration
Master of Public Relations
2 years' Board service



Libby Oldershaw
Member

Freelance Journalist
Over 25 year's journalism experience
2 years' Board service



Jodie Hatherly
Member

Bachelor of Laws
Bachelor of Arts
Over 22 years as a corporate lawyer in private and public organisations, specialising in oil and gas
Board member since April 2016



Tanvi Haria
Member

Bachelor of Business Administration & Accounting – Fellow of CPA Australia & Councillor, CPA Australia WA Division
Over 25 years of broad commercial & general management experience
Board member since August 2016

MEET OUR STAFF



Hayley Lethlean
Chief Executive Officer

Since January 2015



Piper Marsh
Community Services Manager

Since May 2014



Mark Hullett
Partnerships & Fundraising Manager

Since May 2013



Brianna O'Donnell
Communications Manager

Since April 2005



Toby Gummer
Administration and Information Systems Manager

Since January 2013



Cathy Donovan
Bookkeeper

Since June 2011



Jessica Crute
Community Services Officer

Since April 2016

Empowering **full** lives



President's REPORT

It is with great pride that I present my first report as President of your Board. I wholeheartedly thank our past President Rob Storey and Board members for the incredible working environment they created at what I believe is one of the best run not-for-profits in the country.

Having welcomed two new Board members this year in Jodie Hatherly and Tanvi Haria, we are better positioned now than ever before to deliver our 2016-2020 Strategic Plan and continue serving our community to the highest level possible.

Our CEO, Hayley, has continued to lead our staff in delivering outstanding service to our community, through our commitment to research initiatives, quality social support activities, advocacy, equipment supply and engagement with you all.

This focus enabled us to achieve a number of strategic objectives in 2016, the most critical being to embrace the challenges ahead and reposition our organisation with a greater emphasis on community. Our new brand, launched at the Annual Marquee Race Day in October 2016, marked a new era for Muscular Dystrophy WA.

While tough economic conditions saw declines in some traditional fundraising areas, we have proactively diversified revenue opportunities through good business management. These innovative, new events have demonstrated growth, and increased support and popularity. It is critical we maintain income diversity in this challenging environment, consolidating existing and capitalising on new opportunities. The commitment of our long term donors, event partners and supporters provides a strong foundation for sustainability and for this we are grateful.

Continued collaboration at a national level via the Muscular Dystrophy Foundation (MDF) was ably lead by our Vice President, Brendan Murphy. Our Board unanimously supports the role of MDF in providing a national profile, advocacy and lobbying the federal government for best outcomes for our community.

September 2016 was a momentous month for our Duchenne community and for our Honorary Life Members, Professors Steve Wilton and Sue Fletcher. Steve and Sue have spent more than two decades developing the drug Eteplirsen (now EXONDYS 51), the first treatment for Duchenne muscular dystrophy. We congratulate the whole team on this breakthrough and your ongoing efforts to investigate further treatments across different exons, genes and conditions. Our commitment to supporting research scholarships in this area continues.

2017 promises to be a fantastic year celebrating 50 years of this great organisation. To all our Honorary Life Members and long serving members, I sincerely thank you for your contributions over the years, and I extend particular congratulations to Stan Perron AM CitWA in this, his 30th year as our Patron.

In closing, my sincere thanks to the board, staff and volunteers who enabled the organisation to grow throughout the year. Our biggest thanks goes out to you, the community we serve. The collective community at Muscular Dystrophy WA has a strong and rich history and we remain dedicated and motivated to serving you.

Brad Girdwood, President



CEO's REPORT

***Nothing is impossible,
the word itself says
'I'm possible'***

- Audrey Hepburn.

I think of this quote daily when I look at our strategic plan and focus on what needs to be achieved each day. Because we continue this focus on our six strategic goals, we know we are slowly progressing. Being a small organisation has its challenges, but it also means we can be agile and continually look to new methods, new ideas and opportunities. 2016 has been a year for all three!

We are **better
together**

Strengthening bonds: We know that we do great things if we unite with others who are dedicated to do the same. From collaboration with our corporate partners in fundraising, working alongside inspiring organisations that service our community, to liaising with external contractors, we incorporated positive change across everything we did in 2016.

Linking with services: For our community, like most within the disability sector, the biggest change in 2016 was the continued roll out of the NDIS and WA NDIS. The fundamentals of this scheme is to put the individual with disability first. To achieve real goals for individuals requires preparation, education and knowledge. The Community Services Program, ably lead by Piper Marsh, hosted a number of education workshops throughout the year to keep you all informed. This will remain a priority for future years and we look forward to supporting you all through this journey as the Scheme rolls out across Western Australia.

Supporting research: 2016 was a milestone year for Professors Sue Fletcher and Steve Wilton with their Duchenne muscular dystrophy drug receiving FDA approval. Many milestones have also been achieved for other treatments and late in 2016 the FDA also approved the first treatment for Spinal Muscular Atrophy (SMA) for both adults and paediatrics. These treatments offer so much hope for our wider community, but this also offers a meaningful difference to the lives of those able to access these trials. We will continue working collaboratively and advocating to create a clinical trial environment in Western Australia. This includes working closely with The Telethon Kids Institute, Save our Sons Duchenne Foundation, Centre for Comparative Genomics, The Perron Institute of Neurological and Translational Science and other research institutes locally and internationally.

Raising Awareness: The Community Services Program (CSP) delivered many wins in 2016 with the highest numbers of attendees and support services offered in our history. I encourage you all to read the individual reports and I personally thank Piper and Jess for their dedication to always delivering positive outcomes for the community. The CSP is by far our biggest focus and we will continue to invest more energy and more resources into achieving goals in this area.

Financial sustainability: Income diversity is critical to grow and deliver more specialised services. Despite the economic challenges we face, I congratulate our team in their fundraising efforts, awareness campaigns and ability to deliver events with minimal cost and maximum profit. I particularly acknowledge and thank Mr Stan Perron AM CitWA who has always prioritised our community by offering sound advice and support in his role as Patron over the many years.

To the board, our volunteers and our little team, thank you all for working shoulder to shoulder to remain inclusive, objective and focused to make a difference for the community. We are better together and together everything is possible!

Hayley Lethlean, CEO



TREASURER'S REPORT

I am pleased to present the Association's 31 December 2016 Annual Financial Report, which includes the Financial Statements and Independent Auditor's Report.

We believe in
practical help, not
just hope

The Statement of Comprehensive Income shows a surplus prior to research grants for the year of \$107,244. Last year, there was a surplus prior to research grants of \$116,945. Our net surplus for 2016 was \$4,052.

The reduced surplus was as a result of increased investment in the Community Services Program, investment into strategic rebranding, and reduced income from general donations and bequests.

It must be noted with sincere thanks that our Patron Mr Stan Perron AM CitWA committed a significantly generous donation of \$100,000 to the Association in late 2016. This donation formally launched the 50th Anniversary Campaign for 2017.

In spite of the struggling Perth economy, it has been good to see that three of our more recent fundraising projects, the Annual Golf Day, the Truffles for Muscles Auction and the Marquee Day at Ascot have improved again this year and are generating consistent income for the Association, a total of \$233,894 from all three for 2016.

Due to the continuing low interest rates on Bank deposits, our investments in Growth Equities and Managed Funds in the last 12 months grew modestly from \$1,428,937 at the end of 2015 to \$1,502,666 at 31 December 2016. The philosophy continues to remain confident in the equity markets with the view to them being a good long-term investment option. This provides the Association with short-term income by way of fully franked dividends and Managed Fund income distributions, with the long-term view to higher capital growth in the coming years. Our long-term view remains a minimum of three to five years.

Finally, the Net Assets of the Association increased slightly during the year from \$2,201,675 in 2015 to \$2,205,727 in 2016, after research grants of \$107,244 compared to last year's \$116,945 of research grants.

I thank the Association's dedicated staff and volunteers for their wonderful work ethic and professionalism throughout the year. I also extend a warm welcome our new Board members Jodie Hatherly and Tanvi Haria and look forward to working with them all in 2017.

Geoff Woods, Treasurer

STATISTICS



\$436
provides a suite of counselling services to a newly diagnosed family



7.2%
administration expenses of annual turnover



\$2,205,727
Total Accumulated Funds



\$600,095
Total Fundraising & Donations income



\$107,244
invested into research and scholarships



\$375,147
investment into Community Services Program



\$50
provides one of our community members with a six month supply of masks for their CoughAssist™ Machine



\$8,050
provides one of our community members with the maintenance, insurance and ongoing support and provision of a CoughAssist™ Machine



\$4,052
increase in net surplus position since 2015



\$2,697
allows a child with muscular dystrophy to attend a camp, giving freedom to kids and providing much needed respite to families

OUR PARTNERS

The **Kailis Bros** partnership was founded upon their family relationship with Prof Byron Kakulas. Muscular Dystrophy WA held 40 successful Christmas Auctions with Kailis Bros and this partnership now enables us to deliver the Truffles for Muscles Charity Auction.



With the strength of the **Toyota WA** brand, 2016 saw the ninth successful Golf Classic. During this time, Toyota WA staff, dealerships and their associated businesses have truly embraced the Muscular Dystrophy Community.



With **Telethon's** support in 2016, we've harnessed community's vast knowledge with the view of creating a resource that has the potential to benefit the entire neuromuscular community. Our relationship with Telethon dates back to the late 1960s. We are so grateful they recognise the importance of our Community Services Program and research projects.



Hammond King Touyz (HKT Legal) have been our Honorary Solicitors for more than 40 years with Mr Lionel King offering pro bono legal advice to the Association. Current HKT Principal, Colin Touyz, demonstrates continued commitment by providing ongoing legal expertise.



Lotterywest shows unwavering support of the Community Services Program. Without their support, we would not be able to deliver new and diverse programs at little or no cost to our community.



MyattsField Vineyards' owners and vigneron, Josh and Rachael Davenport, have shown enduring support of Muscular Dystrophy WA for over ten years through the provision of wine, venue and event partnerships. These wonderful people truly go the "extra mile", riding their mountain bikes, playing golf and attending events for the cause.



Scarboro Toyota, long-time supporters of the Toyota WA Muscular Dystrophy Golf Classic, continue their commitment to our community by providing Muscular Dystrophy WA with full-time use of a Toyota RAV4.



The Perron Group and the **Stan Perron Charitable Foundation** have demonstrated unfailing support of the Muscular Dystrophy Community, and are instrumental in the ongoing success of the Toyota WA Muscular Dystrophy Golf Classic.



Clip Media Motion lend their talents and expertise in video production and photography, allowing Muscular Dystrophy WA to utilise high quality promotional assets to raise awareness and, in turn, benefit the community.





COMMUNITY SERVICES PROGRAM

2016 was a very busy and productive year for the Community Services Department as we continued to implement the recommendations from the 2015 Community Services Review.

Everyone deserves the chance to reach their **potential**



This led to a year where we offered a record number of activities and participation rates were up across all programs.

Jessica Crute joined the team in April, not only increasing the capacity of the team but also providing fresh ideas and a different outlook.

Other significant events during 2016 included:

> **Growth of both the WA NDIS and the NDIS trial sites:** As more of the community became involved within these trials, Muscular Dystrophy WA saw a large increase in requests for individual advocacy and representation particularly during the planning phase. This is a trend predicted to continue as the WA NDIS rolls out across WA. To cope with the expected increase in request for support, we are developing a formalised education and advocacy program for 2017 and beyond, to ensure the entire Muscular Dystrophy Community receives the support required.

> **The Peer Support Program** was delivered in conjunction with Save Our Sons Duchenne Foundation and provided two education sessions on planning for the WA NDIS / NDIS plus a workshop to develop people's own self-advocacy skills. Muscular Dystrophy WA and Save Our Sons Duchenne Foundation also developed a WA NDIS / NDIS Planning Resource to assist our community. The resource was launched to a good reception at the 2016 Australian Neuromuscular Conference, in Sydney.

> **Hale Mates**, a new program developed to address the 2015 Review's key recommendation of 'tackling the social isolation experienced by some of our teenage community'. The program was developed in conjunction with Hale School, who have provided the peer mentors, venue and support. The first session was held in February 2017.

Jess and I are proud of the changes we instigated during 2016 and feel that the department is edging ever closer to providing a more holistic and supportive service. As we move through 2017, our 50th year, we will continue to ensure we meet the key recommendations from the 2015 Review while at the same time being responsive to the Muscular Dystrophy Community and the ever changing disability sector. Please always feel free to let us know your thoughts.

Piper Marsh, Community Services Manager

COMMUNITY SERVICES PROGRAM

SUPPORT FOR LIFE PROGRAM

Funded by Telethon and Muscular Dystrophy WA this program looks to harness the collective knowledge of the Muscular Dystrophy Community into a resource, to ensure people with neuromuscular conditions are aware of the entitlements and range of support available to them. 2016 was designated the 'information gathering' phase of the program, which was achieved via a range of strategies including community member interviews, service provider interviews and program research. 2017 will see this information developed into an easily updatable resource, which will be circulated throughout the appropriate services.

SCHOOL HOLIDAY PROGRAM

A key recommendation of the 2015 Review was to increase the number of school-aged activities provided. Muscular Dystrophy WA delivered this by providing a School Holiday Day Program in April and July, in addition to the existing October Camp.

It took a while for families to embrace the day program in April, but 12 children participated across the two days in July. Activities included a visit from Animal Ark, a cartooning workshop, cooking, craft and games.

The 2016 Camp was a roaring success with 15 children attending. They were wonderfully supported by 13 volunteers, three support workers and two staff. Campers participated in a range of activities including a magic show, bubbleology, a quiz night, the traditional camp fire and Master Chef Challenge. A huge thank you to Variety WA for the wonderful American style feast they prepared for our last dinner.

"We had a great time and the kids had a ball. Thanks so much to you all for organising such a great camp again. It's so great for the kids to meet other kids with MD and have that time to get to know each other and bond. They all get on so well, they all have a special connection."

- Mother of a Camper

YOUNG ADULTS SOCIAL GROUP

2016 saw the Young Adults Social Group (YASG) program capitalise on the learnings from the 2015 trial of this program by offering three different events – a Comedy Night at the Charles Hotel, a games night, and to round the year off, a Halloween themed dress up party. 60 people attended across the three events. 2017 will see even more activities added to the YASG calendar.

"It was great! Thanks for organising it! I think the night went quite well. It was really good to meet other people I hadn't met before!"

- YASG participant



COMMUNITY SERVICES PROGRAM

CONVERSATIONS AND CANAPES

65 people from across the community attended this fun-filled night where laughs, good conversation and food were the order of the evening. Music was provided by Em Gel, while many people entertained themselves in the photo booth. A big thank you to MyattsField Vineyards for their support.

"Thank you Muscular Dystrophy WA, what a wonderful evening. The photo booth was a wonderful idea and the food was delicious. I hope we are doing it again next year."

- Attendee

FEMALE CARERS

2016 saw the Mothers Retreat name change to the Female Carers Program. Two vastly different events were provided this year in the hope that they would appeal to a wider audience. The first event in March was a wine and lunch tour of the Bickley Valley. Thanks to MyattsField Vineyards for hosting us and a big thank you to the eight participants who made the bus trip very enjoyable.

The second event was a day of mindfulness training at A Place Just to Be. Ten female carers participated in the workshop, with all reporting how much they enjoyed the experience and how much they had learnt. Thank you to Carers WA for supporting this activity.

"This day reminded me how important it is to take time out for me and even if its only 5-10 minutes a day, so I can continue to do my caring role"

- Wife of husband with MD

MALE CARERS

2016 saw the launch of the Male Carers Program with two events conducted. The first event was generously supported by Little Creatures as the male carers went to Beer School to learn all about brewing and beer. Five carers participated in the day.

The second event was presented by the Special Air Service Regiment (SASR) at Campbell Barracks. The attendees toured the facilities and then enjoyed a superb lunch at the Officer Barracks Club House. Eleven participants enjoyed this once in a life time opportunity. We thank the staff of SASR for facilitating this fantastic experience.

Community Services Program proudly supported by:



COMMUNITY SERVICES PROGRAM

THE DUKE OF EDINBURGH INTERNATIONAL AWARD



Muscular Dystrophy WA celebrated its first Silver Award recipient when Anthony Ambrosini completed the necessary criteria. Completing his Silver involved a commitment to his role as Western Electric Sports Association Website Administrator, vastly improving his fishing skills, improving his performance in electric wheelchair sports and travelling to Sydney for the National Electric Wheelchair Sports Championships. Muscular Dystrophy WA is excited to support Anthony as he works his way towards his Gold Award in 2017.

Two new participants joined the program and signed on for their Bronze Award in 2016 - Noah Ryan and Logan Bayley. Their Awards focus on photography, music mixing, exploring accessible nature trails and volunteering at a nursing home. We look forward to both participants completing their Award in 2017.

ANNUAL COMMUNITY DAY

A number of changes were instigated for the 2016 Annual Community Day. Although the location remained at MyattsField Vineyards, the entertainment and food was a change up from previous years. Children enjoyed an up-close experience with a koala thanks to West Oz Wildlife, while parents sat back, relaxed and listened to WA band The Wire Birds. A delicious paella lunch was provided by Paella Fiesta with the day culminating in the consumption of the perfectly purple lolly bar. Thanks to Lotterywest and MyattsField Vineyards for again supporting this vital event on our calendar, and thank you to the 123 community members who attended and made this day what it was.



464

people attended CSP activities
- an 11% increase from 2015



18%
increase in
calls from other
service providers



40%
increase in
counselling
referrals



1,337

hours of respite
provided, an increase
of 11.4% on 2015



Dramatic increase in
individual advocacy
requests (10.5%),
especially for
WA NDIS & NDIS



285
requests for
information, an
increase of
111% from 2015



32
home visits,
doubled from 2015



834 hours
provided by volunteers,
valued at \$63,856 to our
organisation





RESEARCH ADVISORY REPORT

I am pleased to present the Research Advisory Committee (RAC) Report for 2016. As some of you would be aware, our committee acts as the principal advisory committee to the board in relation to matters of strategic research initiatives, policy and regulation pertaining to research scholarships, research performance, scholarship appointment and assessment.

We are **better
together**

Since our inception in 1967, our pillar of strength has been our commitment and investment into world-renowned research, which has led to advances and support across many facets of our community. A hope for effective treatments and even a cure will always remain a long-term focus. In line with our strategic direction, we remain committed to supporting relevant research into muscular dystrophy and neuromuscular conditions. We continue to create a collaborative environment where research advances that take place both locally and globally, will positively influence the lives of Western Australians living with muscular dystrophy.

In 2016 the RAC met on five occasions and one of our key focuses was to set a Research Strategy for the Association. Following review and deliberation, and as a result of some collaboration through a few international channels, we have finalised the strategy. This defines the priorities for our community and demonstrates how we intend to achieve this. The strategy sets five goals we will work towards to help us achieve our strategic outcomes and our investment in these areas is essential to build upon the successes of the last 50 years. I encourage you all to access our website to review the full Research Policy.

2016 saw our first TEAM Spencer - Muscular Dystrophy WA PhD Scholarship for SMA Research recipient, Loren Price, finalise her study and submit her thesis. Loren is now employed fulltime at the Centre for Comparative Genomics and continues her investigation into molecular therapies.

Our inaugural Muscular Dystrophy WA Harold and Sylvia Rowell PhD Scholarship recipient, Vivienne Travlos, is in the final stages of her data collection for her MyLifeMyVoice study and details are included over the next few pages.

In addition to the scholarships established and underway, we also granted two new scholarships to Iantha Pitout and Oliver Dunhui Li. Iantha is completing her studies in the application of antisense oligomers (AOs) to modifiers of the Survival motor neuron 2 (SMN2) gene in order to reduce the severity of Spinal Muscular Atrophy (SMA). Oliver is investigating molecular studies on alternate splicing with a specific focus on Duchenne muscular dystrophy. An overview of both these students' studies follows within this report.

Susan Trahar, Chair of the Research Advisory Committee

RESEARCH ADVISORY REPORT

TEAM SPENCER MUSCULAR DYSTROPHY WA SCHOLARSHIP FOR SPINAL MUSCULAR ATROPHY (SMA) RESEARCH

IANTHE PITOUT - MODIFYING MODIFIERS: APPLYING ANTISENSE OLIGONUCLEOTIDES TO SPLICING FACTORS OF THE SURVIVAL MOTOR NEURON 2 (SMN2) GENE TO RESTORE FUNCTIONAL SMN PROTEIN

COORDINATING SUPERVISORS: PROFESSOR STEVE WILTON AND PROFESSOR SUE FLETCHER

Ianthe Pitout is the 2016 recipient of the TEAM Spencer Muscular Dystrophy WA Scholarship for Spinal Muscular Atrophy (SMA) Research. This has facilitated her research into manipulating splicing factors that affect the survival motor neuron 2 (SMN2) gene implicated in SMA.

Ianthe uses the antisense oligonucleotide (AO) mediated splicing intervention strategies developed by Professors Wilton and Fletcher that led to their successful therapy for Duchenne muscular dystrophy. Ianthe's research has mainly involved using AOs to upregulate levels of a key splicing factor, in order to indirectly lead to increases in the amount of functional SMN protein produced from the sub-optimal SMN2 gene.

To date, her research shows that the splicing factor upregulation with AOs is a viable mechanism for significantly increasing SMN protein in cell culture. Future work will explore the effects of the splicing factor in a mouse model of SMA. This approach to increasing SMN protein has the potential to be used as part of a combination therapy for treating SMA.

Over the course of her PhD, Ianthe has presented her research findings in both oral and poster format at several conferences. A highlight for her was presenting a late-breaking poster at the World Muscle Society Symposium in Brighton, UK and participating in an exceptional stem cell training workshop in Leiden, the Netherlands. Ianthe uses the skills she learned in the workshop to culture and differentiate SMA patient stem cells, reprogrammed from skin cells, into motor neurons for disease modelling and drug testing.

Ianthe is in the final phases of her PhD and hopes to submit her thesis for examination by the end of June 2017.



RESEARCH ADVISORY REPORT

HAROLD AND SYLVIA ROWELL PhD SCHOLARSHIP

VIVIENNE TRAVLOS - 2014 RECIPIENT

THE MY LIFE MY VOICE TEEN NMD STUDY

SUPERVISORS: ASSOCIATE PROFESSOR SHANE PATMAN, DR JENNY DOWNS AND DR ANDREW WILSON.

Vivienne Travlos has completed data collection with youth aged 13-22 years old from across Australia and New Zealand. All participants and one of their parents completed a comprehensive questionnaire. Selected participants also took part in in-depth interviews.

The description of severity of health issues experienced by these young people builds a solid foundation for future research, testing how to prevent or minimize comorbidities, including fatigue, sleep discomfort, constipation, pain and many more.

Compared with typically developing youth, all but two of the 47 participants who returned the questionnaire reported average to above average wellbeing. Greater wellbeing and life satisfaction were associated with greater sense of belonging and perceived support, not with health issues. Friends made the difference, for both youth and for their parents.

Youth-shared insights are extremely valuable to healthcare professionals. Healthcare professionals should embrace this knowledge to facilitate supportive relationships and invest in harnessing the expertise of peer mentors to optimise healthcare engagement.

Two papers were published in scientific journals in 2016 and further manuscripts are in preparation. Findings have to date been disseminated at three local, three national and one international conference. The papers and conference posters are available on www.mylifemyvoice.org and through the Muscular Dystrophy WA office.

Vivienne is extremely grateful to the Rowell and extended families for their support that helped explore and share the voice of this group of resilient youth living with neuromuscular disorders, who, given the opportunity, can look forward to promising futures.

DUNHUI LI (OLIVER) - 2016 RECIPIENT

MOLECULAR STUDIES ON INDUCED ALTERNATIVE SPLICING

THE CENTRE FOR COMPARATIVE GENOMICS, MURDOCH UNIVERSITY WESTERN AUSTRALIA

COORDINATING SUPERVISORS: PROFESSOR STEVE WILTON, PROFESSOR SUE FLETCHER AND PROFESSOR NORMAN PALMER

In September 2016, Dunhui Li (Oliver) began his PhD research focused on molecular studies on induced alternative splicing, particularly for Duchenne muscular dystrophy (DMD). There are two main aims of his PhD project, i.e. 1) applying antisense oligonucleotide (AO) technology to map the functional domains of the distal third dystrophin gene and 2) screening small chemicals that may improve the efficacy of AOs.

The latter third of DMD gene codes for important structures that are responsible for the binding of dystrophin to proteins that anchor dystrophin to the muscle fibre membrane. Single or multi-exon deletions that do not disrupt the reading frame are very rare downstream of exon 55, and hence there is no 'BMD patient guide' to indicate functionality of in-frame exon deletions. Therefore, Oliver is trying to excise in-frame exons, either individually or as in-frame blocks and assess dystrophin isoform(s) function in PMO treated wild-type mouse diaphragm by examining muscle architecture and function.

Splice switching AOs are showing great therapeutic potential to treat inherited diseases, with EXONDYS 51 recently being approved by the U.S. Food and Drug Administration (FDA) for the treatment of DMD. Although there is an unequivocal increase in dystrophin after treatment, the amounts are modest and there is room for improvement. For this aspect of his project, he is exploring combinatory therapies with small chemicals that can modulate splicing. Riluzole is an FDA approved neuro-protective drug for Amyotrophic lateral sclerosis (ALS) that is known to influence the splicing process. Oliver is now assessing the combination of Riluzole with AOs, to enhance the efficacy of AOs induced exon skipping.

Oliver has generated some promising preliminary results and will present this data at the Australasian Gene and Cell Therapy Society Conference in Sydney (May 2017).



RESEARCH ADVISORY REPORT

UPDATE ON MUSCULAR DYSTROPHY WA EQUIPMENT GRANT

**DR JESSICA TERRILL - PRECLINICAL TESTING OF TAURINE FOR REDUCING
DYSTROPATHOLOGY IN THE MDX MOUSE MODEL OF DUCHENNE MUSCULAR DYSTROPHY**

SCHOOL OF CHEMISTRY AND BIOCHEMISTRY, UNIVERSITY OF WESTERN AUSTRALIA

CHIEF INVESTIGATORS – DR PETER ARTHUR AND PROFESSOR MIRANDA GROUNDS

Glucocorticoids remain the standard treatment to help reduce disease severity in Duchenne muscular dystrophy (DMD), however there are associated side effects.

Repurposed pharmaceutical compounds or nutraceuticals may offer a readily available, cost effective and expedient treatment option for DMD.

Dr Terrill is investigating the potential of the amino acid taurine as a treatment for DMD in the mouse model of the dystrophy (mdx), and has shown that taurine is very effective in protecting dystrophic muscles.

Dr Terrill is now interested in identifying the optimum dose of taurine: too little, and taurine will not be protective; too much, and taurine can cause side effects.

The equipment funded by the Muscular Dystrophy WA grant enables Dr Terrill to accurately quantify the dose of taurine consumed by the mdx mice.

UPDATE ON U.S. FOOD AND DRUG ADMINISTRATION (FDA) ACCELERATED APPROVAL FOR ETEPLIRSEN



DANA MARTIN, VP MEDICAL AFFAIRS AND PATIENT ADVOCACY, SAREPTA THERAPEUTICS

On 19 September 2016, Sarepta Therapeutics in Cambridge, Massachusetts, announced that the U.S. Food and Drug Administration (FDA) granted accelerated approval for eteplirsen (brand name in the United States is EXONDYS 51™), as a once weekly intravenous infusion of 30 milligrams per kilogram for the treatment of Duchenne muscular dystrophy (DMD) in patients who have a confirmed mutation in the DMD gene that is amenable to exon 51 skipping. This indication is based on an increase in dystrophin in skeletal muscles observed in some patients treated with EXONDYS 51.

Professors Steve Wilton, Sue Fletcher and colleagues, currently at Murdoch University and formerly at the Western Australian Neuro-Science Research Institute in Perth, designed the sequence for eteplirsen and have been at the forefront of developing exon skipping therapy for DMD.

To date, more than 150 boys and young men have received eteplirsen in clinical trials. Sarepta has three fully enrolled, on-going, eteplirsen clinical trials in the United States in ambulant and non-ambulant boys and young men from age four up to 21 years of age at trial enrollment. A clinical benefit of EXONDYS 51 has not been established. Continued approval for this indication may be contingent upon verification of a clinical benefit in confirmatory trials. The design of the confirmatory studies and the location of study sites is currently in development.

Sarepta also announced on 19 December 2016, that the European Medicines Agency (EMA) validated the Marketing Authorization Application (MAA) for eteplirsen. This began the formal review process by EMA's Committee for Human Medicinal Products (CHMP) to consider the product for possible conditional approval in the European Union. We will update the community on the status of eteplirsen in Europe as the review period progresses. There are additional clinical trials that are required as part of the EMA process including a pediatric study in boys amenable to skipping exon 51, age six months to four years. The trial is planned to begin enrollment in 2017 at clinical sites in Europe.

A Phase 3, double-blind, placebo-controlled clinical trial in boys age 7 to 13 years with DMD mutations amenable to skipping exon 45 or 53 is currently enrolling patients in the US. Sites in Europe will begin enrolling soon.



POWER FROM THE PEOPLE

SAS REGIMENT PARTNERSHIP

Muscular Dystrophy WA have embarked upon a three year partnership with the Special Air Service Regiment (SASR) which provides two-way mentoring, Campbell Barracks visits for the Muscular Dystrophy WA Community, laboratory visits for SASR staff, and event support through tailored fundraising items.

TOYOTA WA MUSCULAR DYSTROPHY GOLF CLASSIC

1 MARCH, 2016

In consultation with Naming Partner Toyota WA, we took a leap of faith and shifted the Golf Classic from a Friday afternoon to a Tuesday afternoon. This increased the event capacity and allowed more Toyota dealerships and corporate teams to enjoy one of our signature events.

With 33 teams competing, an increase of 65% on the previous year, the weather was kind and the course was in near-perfect condition. Immediately following a great afternoon of golf, the presentation dinner was held in the delightful poolside marquee, with the event generating a net gain of **\$62,327**.

SUNSMART IRONMAN 70.3 BUSSELTON

1 MAY, 2016

Triathlon WA and the SunSmart Ironman 70.3 Busselton event once again played generous host to our team of triathletes who are dedicated to TRI-umph over muscular dystrophy.

The team was once again readily recognised in their sea of red event polo shirts, as other participants and spectators provided a warm welcome during our team photo at the iconic Busselton Jetty.

Event Ambassadors Renee Baker and Beau Waters were on hand to meet the team, and discuss their involvement in the campaign to media and event stakeholders.

Our sincere thanks go to all who swam, rode, ran and fundraised towards an overall team tally of over **\$30,000**.



POWER FROM THE PEOPLE

RIDE FOR SOMEONE WHO CAN'T

3 SEPTEMBER, 2016

One of our most successful longstanding campaigns pits effort and will against valleys and hills, at the Act-Belong-Commit Dwellingup 100 Mountain Bike event. 2016 was the seventh year of our partnership with TriEvents and the Perth Mountain Bike Club, both great advocates of the RIDE For Someone Who Can't Team and Muscular Dystrophy WA.

Our committed riders were joined by an inspirational mother of two young boys, Carly Collins, who became the first person with muscular dystrophy to complete the event. Carly was diagnosed with muscular dystrophy six years ago, and refused to let the condition stop her from taking on the 14km course.

The team once again raised significant funds for the cause, generating well over **\$80,000**.



MARQUEE DAY FOR MUSCULAR DYSTROPHY WA

22 OCTOBER, 2016

Beautiful conditions at Ascot greeted us for the second Marquee Day for Muscular Dystrophy, hosted in the stunning Director's Lounge. Event Naming Partner Arlec Australia enjoyed a birdseye view over the track from their two tables, alongside fellow sponsors Savanna Home Loans and LWP Property Group.

With the luxurious room filled with 140 of our loyal community and supporters, we took the opportunity to unveil our fresh direction and brand, and the fundamentals that support them. The event breezed past with a race every half hour, a hilarious live auction by our man Tiny, and a sumptuous meal washed down with fine wines.

Over **\$32,000** was raised in a festive, fun atmosphere.



POWER FROM THE PEOPLE

DONOR ACKNOWLEDGEMENT

Trudy and David McKenna were introduced to Muscular Dystrophy WA in 2010 by then CEO, John Gummer.

David was one of the first RIDE For Someone Who Can't participants and was integral to the success of the campaign.

Regularly attending events, volunteering their time and supporting auctions, David and Trudy have become part of the muscular dystrophy family, embracing our organisation, our sponsors and our community. Their love of fine wine has led to a special bond with Rachael and Josh from MyattsField Vineyards, the Official Wine Partner of Muscular Dystrophy WA.

Trudy has also assisted in a voluntary capacity at fundraising events and provided administration support in the office. Her bubbly personality exudes enthusiasm for our cause and we are so grateful that these lovely people support so much of our work.

VOLUNTEER RECOGNITION

Volunteering Australia defines volunteering as: time willingly given for the common good and without financial gain. Muscular Dystrophy WA relies heavily on volunteers, we believe giving your time can be just as valuable as giving a monetary donation.

As we head into our 50th Anniversary year, we pay tribute to two wonderful women who have dedicated much of their lives to Muscular Dystrophy WA, who sadly passed away in early 2017.

HONOURING MARIA (MARY) BROERE VALE MARY BROERE 1935 - 2017

Mary and her husband Bob Broere decided to take a proactive approach to their son's Duchenne muscular dystrophy diagnosis and began supporting Muscular Dystrophy WA in the 1980s.

Always hardworking, Mary loved to bake, make jams, sew, knit and crochet, all in aid of fundraising for Muscular Dystrophy WA. Mary took on the role of the then Muscular Dystrophy Research Association Branch Representative for the Armadale area and became the driving force in fundraising within her community.

It was a family affair for the Broeres with Mary's son organising several Squash-a-thons.

Mary offered support to other families impacted by muscular dystrophy and was an active member of our community.

In 1994, Mary was awarded Honorary Life Membership for her exceptional service and long lasting contribution to our Association.

Mary's faith in God helped her through the many difficult times while caring for James and Max. Although her health deteriorated, her faith in God was always strong, and God called her home on 22 January 2017. She was laid to rest in Fremantle Cemetery with this inscription going onto her tombstone, "I shall dwell in the house of the Lord, forever". May she rest in peace.

Muscular Dystrophy WA is ever so grateful to Mary and to her family for their continued support. We were built by people like Mary and we are forever be indebted for her lasting contribution.

HONOURING ELIZABETH RITCHIE VALE ELIZABETH RITCHIE 1929 - 2017

Muscular Dystrophy WA recognises the contribution made by volunteer and Honorary Life Member, Mrs Elizabeth Ritchie (known fondly as Mrs Ritchie). With close to two decades of service, Mrs Ritchie gave her all to the Association.

Mrs Ritchie took an interest in research and built relationships with many of the families. Her drive to raise funds and awareness was inspirational, running countless fundraising stalls and volunteering with our Annual Street Appeals. She always demonstrated her support, even when her health took a turn for the worse.

Mrs Ritchie was passionate about raising the awareness of, and educating people about, muscular dystrophy as a condition. Mrs Ritchie was awarded Honorary Life Membership for her exceptional service and long lasting contribution in 2013.

People volunteer for many different reasons, all selfless, but few impress themselves on our Association the way Mrs Ritchie did.

They say helping others kindles happiness; Mrs Ritchie was happiest when on a fundraising mission. Along with her family, we have no doubt that her passion for Muscular Dystrophy WA was one of the things that kept her going.



Empowering **full** lives



Muscular
Dystrophy WA

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