FIGHT ~~MND~~.

Annual Report 2020/21

Motor Neurone Disease gradually takes away a person’s use of their arms and legs, their ability to eat and swallow, their speech and ultimately their ability to breathe - all in an average time frame of just 27 months.

FightMND was established to find effective treatments and a cure for this horrible and debilitating disease.

FightMND Values

Integrity

Urgency

Efficiency

Boldness

Community


BFT
Play On
BUNNINGS warehouse
FIGHT MND


BF7
Play On
BF7
Play On
BF7
Play On
FIGHT
coles
Michael

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FightMND Board

Bill Guest
Chairman

Pat Cunningham
Vice-Chairman

Neale Daniher
Patron

Gary Nugent
Treasurer

Dr. Trevor Chong

Dr. Judith Slocombe

Lewis Martin

Andrew Dillon

Mark Evans

Camilla Britton
(from May 2020)


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AUSTRALIAN FOOTBALL
BF7
FIGHT MND.
BIG FREEZE

Chairman's Report

I began this year with optimism that we would no longer be facing the challenges that the pandemic threw at us last year. Unfortunately, that was not to be, but I am delighted that once again the FightMND Army came out in force to support our cause. Big Freeze 7 and Sockit2MND both broke previous records and despite cancellations and lockdowns causing ongoing disruptions, Daniher's Drive and community events continued to kick goals.

The launch of the FightMND Club was a highlight of the 2020/21 year, landing in a window between lockdowns and allowing us to meet at the MCG to invite a new cohort to lead the challenge against the Beast. To those donors who have joined the FightMND Club, on behalf of the Board, I thank you for your generosity and commitment and look forward to seeing more of you as a fellow leader of the FightMND Army.

As we look to the future of our organisation and our goals of better treatments and a cure for MND, the strategic plan remains a priority for the Board. We are focussed on charting a path forward that will ensure the organisation can continue to hit targets and ultimately beat the Beast. We are on track to reach all the goals set in 2018 by the end of this year, with a new rolling 5-year strategic plan now in development that will ensure we will ultimately reach our goal of finding a cure. We also remain focussed on continuing to improve our Board operations to ensure robust oversight of operations, fundraising and investments.

The small team at FightMND are passionate, committed and incredibly hard working. I am so proud of their ability to continue to play on, especially through the challenges of the past 18-months.

Leading our team is incoming CEO Dr. Fiona McIntosh who joined the foundation in October 2020. I am delighted to welcome her to FightMND and know she will use her extensive background in consulting and senior management, across both the public and private sectors, to effectively guide FightMND through its next phase of development. I would also like to acknowledge Jamie Howden who stepped down as CEO in October 2020. Jamie played an integral role in furthering FightMND and I thank him for his commitment, enthusiasm, and leadership.

Funding world-leading research remains the number one priority for FightMND and in 2020 we announced investments in 14 new projects. I am proud of the investments we have made in the best MND research projects, supported by the brightest MND researchers, and am motivated by new discoveries, that we will believe, will ultimately lead to a breakthrough for MND.

Care for those living with MND is also a focus. We do this in partnership with the state MND associations and I thank and congratulate them for the work they do to support the 2,000 Australians currently living with MND.

On behalf of the Board, I would like thank both the Federal Government and Victorian State Government for their continued support of FightMND over the past year. Specifically, I'd like to thank Federal Minister for Health, the Hon. Greg Hunt, Treasurer of Australia, the Hon. Josh Frydenberg and Victorian Premier the Hon. Daniel Andrews, for their commitment to the fight against MND. Their support and contribution is instrumental in helping fund vital research projects.

Finally, I'd like to thank our invaluable partners, particularly Coles and Bunnings, who contributed $6.78M and $1.05M respectively to the Big Freeze 7. To our supporters, researchers, volunteers and the FightMND Army thank you for continuing to rise to the occasion and supporting us in the battle to beat the Beast.

It is with some sadness that I conclude this last letter as Chair of FightMND. Although I will remain on the board, I will soon be stepping down to allow for new leadership to further build on the achievements of FightMND to date. I am so proud of the impact FightMND has had during my time as Chair and would like to take this opportunity to thank my fellow board members for their unwavering guidance and support. In particular, I would like to thank Neale Daniher and Pat Cunningham who, with the late Dr. Ian Davis, were the early pioneers of FightMND, choosing to take on a beast that appeared undefeatable. Neale and Pat have shown that with perseverance we can win this battle and their consistent ingenuity, persistence and wisdom has been a guiding force of this incredible foundation.

Bill Guest
Chairman

CEO Report

I am honoured have been appointed to the role of CEO of FightMND in October of 2020. It is a privilege to be tasked with the job of working with FightMND's Board to steer the organisation as we continue on our path toward better treatments and a cure for MND.

My first year has been an amazing ride. With COVID impacting our foundation in so many ways we've had to learn to adapt to a new reality. The obvious impact has been the inability to continue so many of our fundraising campaigns in their traditional format. Working remotely also meant that our ways of working have had to adapt to a "new normal", but I am so incredibly proud of how our small team has risen to the challenge. I'd like to thank them and acknowledge their tenacity, resilience, steadfastness and extraordinary ability to pivot mid-campaign, and still break fundraising records. I'd also like to thank our contractors and volunteers who provide such wonderful support for our team through their busiest times.

The pandemic certainly threatened to derail our biggest campaign, the Big Freeze. We began the Big Freeze 7 campaign with optimism that this year we'd be 'back to normal', marching to the 'G' and watching the slide with a massive crowd of supporters. But with Melbourne going into lockdown just weeks after the campaign launch, the Melbourne v Collingwood game was moved to Sydney and we quickly implemented new plans. We are grateful to the Victorian Government for allowing the slide activation to go ahead and be televised by Channel 7 from an empty MCG, and grateful to the NSW Government that supported the move of the game to Sydney, adding an additional activation at Bondi Icebergs.

Despite these challenges, with the incredible support of the FightMND Army and our amazing partners, Big Freeze 7 smashed the previous records and raised an extraordinary $14.7 million. We'd like to thank the Federal Government for its contribution of $2 million, and Coles which through beanie sales, instore donations and its partnership with the Australian Pork Farmers, contributed an extraordinary $6.78 million to the Big Freeze fundraising total.

Our other campaigns also hit COVID hurdles. Daniher's Drive was cancelled (but still raised an impressive $330,000). The Sockit2MND campaign was cancelled for 2020 but came back in force in 2021, and despite lockdowns, broke its previous record to raise $325,000. Community events once again were hit hard by COVID, however the introduction of a new peer-to-peer campaign, the 27 Challenge added some new flair to our fundraising year, contributing over $250,000 and securing a place on our future fundraising schedule.

Another new initiative for the 2020/21 year was the launch of the FightMND Club. The Club, which recognises donors who make a minimum yearly $10,000 donation to FightMND, was launched at the MCG in May and drew a small crowd of sporting and entertainment personalities, successful business people, and long-time FightMND supporters. Attendees were invited to take the challenge to step up and help lead the FightMND Army. Members of the FightMND Club, across three tiered levels, receive access to a calendar of events providing additional insight into our research initiatives and fundraising campaigns.

At FightMND, we fundraise hard because we are passionate about finding better treatments, and ultimately a cure for MND. In 2020, FightMND proudly committed $10.68M to MND research to support. In 2020, FightMND proudly committed $10.68M to MND research projects that included one clinical trial, seven Drug Development programs and eight IMPACT projects. Research is expensive, and it takes time but each year we get a step closer to our goal of ridding the world of MND.

In October 2020, FightMND also committed a further $2.1 million for the provision of care equipment. This contribution is provided to MND state associations across Australia to enable the purchase and distribution of equipment, benefiting over 2,000 people living with MND.

Our strategic goals remain a key focus and we have implemented new processes and procedures to allow us to mark our progress against these objectives. We are well on track to hitting many of those goals, specifically around funds raised, expense ratio targets and investments made in both cure and care. Trust and enthusiasm for FightMND remain key priorities as is clearly articulating our progress in reaching our goal of a cure.

Our strategic goals also include bringing new leaders into our organisation so that we can continue to build on the achievements to date. This would not be possible without the leaders who have come before and positioned FightMND as an innovator and leader in the fundraising space. I'd like to thank my predecessor, Jamie Howden who did a remarkable job of leading the foundation during his three years as CEO. I'd also like to thank outgoing Chair, Bill Guest who has so ably led FightMND for six years. While Bill will be stepping down as Chair, we are grateful that he will remain on the board, lending his wisdom, expertise and experience to FightMND as we strategically chart our path forward.

A special thank you to our incredible partners who generously give so much to our foundation. Our retail partners Coles and Bunnings, along with Sportsbet, Chemist Warehouse, Channel Seven, Herald Sun and SEN, the AFL, MCC, Melbourne Football Club, Collingwood Football Club and so many others provide support that is truly invaluable.

Finally, FightMND is a success because of the FightMND Army. It is the incredibly committed volunteers who continue to enthusiastically offer up their time and energy. And it is the mums, dads, kids, families, sporting teams, schools, workplaces and everyday Australians who come back year-after-year to buy a beanie, buy socks, make a donation or sponsor a community fundraiser. Because of you, the FightMND Army that stands so strongly by our side, we will win this battle and ultimately beat the Beast.

Dr. Fiona McIntosh
CEO

Research Report

FightMND believes that funding innovative, pioneering research is the key to winning the battle against the Beast. That's why we fund the brightest emerging scientists, whose trail blazing research teaches us more about the disease and brings us another step closer to finding a cure.

Since 2014, our total research investment includes:

Number of projects invested in each state in 20/21:

$47.50 Million
Committed to research initiatives

$13.32 Million
11 clinical trials

$16.68 Million
17 Drug Development projects

$4.5 Million
World-first drug screening platform

$3.74 Million
15 IMPACT grants

$3.11 Million
19 other research grants and initiatives

$2.55 Million
Sporadic ALS Australian - Systems Genomic Consortium (SALSA-SGC)

$2.0 Million
Precision Medicine Program

$1.6 Million
5 research fellowships and scholarships

Total committed to research initiatives

$47.50M

During the 2020/21 financial year, FightMND committed $10.68M to MND research. This included supporting one clinical trial, seven Drug Development programs and eight IMPACT projects.

For more detail see appendix A, page 53.


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FIGHT MND.


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Clinical trials 2020/21 investment

$881,085

Clinical trials test promising new drugs, or drugs already approved for other diseases or conditions, in people with MND. Phase 1 trials are safety studies to assess whether a drug is safe to administer to people, and in particular, people with MND. If a drug is shown to be safe in a Phase 1 study, it can advance to Phase 2 where the impact of the drug on disease progression will be studied.

Phase 1 - Clinical Trial

Safety and preliminary efficacy of oral monepantel in individuals with MND

Grant Recipient: PharmaAust Ltd

Project Leads: Dr Susan Mathers, Prof Dominic Rowe, Dr Richard Mollard

Drug Development projects 2020/21 investment

$6,949,264

Drug Development projects are focused on advancing promising new drugs or therapies through the final stages of testing in preparation for advancement through to testing in MND patients in clinical trials.

Genetics-based Therapies

TDP-43 targeting gene therapy for MND

Project Lead: Prof Roger Chung

Preclinical development of a SOD1 genetic therapy in sporadic MND

Project Leads: Prof Steve Wilton,
Prof Anthony Akkari and Dr Loren Flynn

SMN2 splice-switching oligonucleotide therapy development for MND

Project Lead: A/Prof Bradley Turner

Therapies targeting the structure of motor neurons

Modulating actin dynamics in MND as a novel therapeutic approach

Project Lead: Prof Julie Atkin

Combination therapies - treatments targeting multiple causes of MND

Developing M102 to treat MND

Project Lead: Dr Ning Shan

Therapies targeting the structure of motor neurons

Combination therapy to improve CuATSM outcomes in MND

Project Lead: Prof Justin Yerbury

Therapies targeting the immune system

Complement C3a receptor modulators as disease-modifying drugs for MND

Project Lead: Prof Trent Woodruff

Impact grants 2020/21 Investment

$1,997,542

IMProving and ACcelerating Translation (IMPACT) projects support key areas of research focused on overcoming some of the hurdles and challenges encountered in MND research that contribute to failed drug development or clinical trials.

Outcomes from these projects will include:

- Improvements in drug design and delivery
- Treatments that target disease causing genes
- Improved understanding of the variability in disease characteristics between individuals with MND
- The development of molecular markers to help diagnose MND, or predict if a drug is effective
- Better models for studying MND in laboratory

Drug delivery

Developing blood-brain barrier penetrating peptides

Project Lead: Dr Fazel Shabanpoor

Gene therapies

Development of a dose-escalatable AAV delivery system for MND gene therapies

Project Lead: Prof Roger Chung

Disease heterogeneity

Prion-like strains of TDP43 aggregates in MND

Project Lead: Prof Justin Yerbury

Disease biomarkers

Using biomarkers to address MND heterogeneity and improve detection to benefit clinical trials

Project Lead: A/Prof Mary-Louise Rogers

Identifying biomarkers from extracellular vesicles for early detection, disease progression and therapeutic efficiency in MND

Project Lead: Prof Aaron Russell

Lipidomic signatures in blood as a novel biomarker for MND

Project Lead: Dr Sophia Luikinga

Defining an electrical signature of sporadic MND, and developing a drug screening technology and novel therapy

Project Lead: A/Prof Lezanne Ooi

Disease models

Using 3D spinal cord organoids to model oligodendrocyte neurotoxicity in MND

Project Lead: Dr Samantha Barton

Additional research investments

$856,753

FightMND has also provided further support to strengthen operations of the **Sporadic ALS Australian Systems Genomics Consortium (SALSA-SGC)** and the **Victorian Brain Bank**. Through SALSA-SGC, Australians with MND are contributing to a big-data research resource that combines clinical, lifestyle and biological information. The resource will be a powerful tool for uncovering the causes of MND, advancing clinical trials and developing more effective treatments. The Victoria Brain Bank is an important resource for Australian researchers, providing them with access to well-characterised post-mortem brains and clinical data that may give clues to why MND occurs and improve diagnosis.

A PhD student will be supported by the **Angie Cunnigham PhD Scholarship and Grant-in-Aid** throughout their 3.5-year candidature, enabling a bright and vibrant young researcher to embark on their pursuit of developing a cure for MND.

FightMND will continue to foster and advance MND Research in Australia through its support of the **Australian Summit for MND Research**. In collaboration with MND Australia, FightMND is playing a leading role in facilitating the national summit that will set a pathway that drives MND research in Australia into the future.


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BIG FREEZE

Researcher Profile

Prof Justin Yerbury

University of Wollongong, NSW

Researcher, Professor Justin Yerbury, was diagnosed with MND in May 2016. He first heard about MND 20 years ago when his uncle, cousin and mother were diagnosed in quick succession. It was clear at the time that there was not enough understanding of the molecular basis of the disease and as a result, Prof Yerbury has dedicated himself to increasing the understanding of the origins of MND.

Prof Yerbury is currently working on two projects funded by FightMND, a Drug Development project: “Combination therapy to improve CuATSM outcomes in MND” (see page 26) and an IMPACT project: “Prion-like strains of TDP-43 aggregates in MND” (see page 31).

Prof Yerbury completed his PhD at the University of Wollongong before being awarded an international fellowship from the Australian Research Council to study the biochemical analysis of protein aggregates at the University of Cambridge, UK. Awards and prizes he has received include:

2009	**Bill Gole MND Fellowship**
2011	**Awarded a Vice Chancellors Emerging Researcher Prize**
2012	**Awarded an ARC DECRA Fellowship to build a group around proteostasis defects in ALS**
2015	**NHMRC Career Development Fellowship to continue research into proteostasis defects in ALS**
2018	**Betty Laidlaw prize**
2019	**Wollongong Citizen of the Year**
2020	**Member of the Order of Australia (AM)**

Prof Yerbury was appointed Professor in Neurodegenerative Diseases 10 years after completing his PhD and since being diagnosed with MND in May 2016 has published more than 35 papers. In January 2018 he underwent laryngectomy surgery and is now mechanically ventilated, drastically increasing his survival. He currently uses eye gaze technology in his work.

Professor Yerbury says that the funding provided by FightMND will allow for his IMPACT project to keep moving forward. Without funding it would not be possible to investigate this area so thoroughly. In addition, the funding for his drug development project will allow the team to develop their combination therapy to a point where, if successful, they will be ready to move into clinical trials. It will allow them to test for safety, maximum tolerated dose and effectiveness against CuATSM alone. The process for this testing is labour intensive and expensive and would not be possible without FightMND and the generosity of the Australian public.

Care

The FightMND Care initiative funds vital assistive equipment to help improve the lives of Australians living with MND. Our aim is to provide equitable access to equipment for all Australians living with MND around the country.

As one of our three core objectives, FightMND is committed to ensuring that Australians diagnosed with the disease can live as independently as possible, for as long as possible.

In October 2020, a further commitment of $2.1 million for the provision of care equipment was announced. This contribution by FightMND is provided directly to MND state associations across Australia to enable the purchase and distribution of equipment, benefiting over 2,000 people living with MND.

This new investment takes FightMND's contribution to improving the quality of life of those suffering from MND to $4.58 million since 2017.

The CARE investment process

In consultation with the state associations, the most pressing and urgent needs are identified to ensure the people that require equipment can access it without delay. Mobility, communication and respiratory support devices are just some examples of the types of equipment FightMND's funding has supported this year. Enhancing the quality of life of people living with MND, enabling them to be more easily cared for and to stay better connected with their families and communities remains a key objective of our support.

FightMND acknowledges and thanks the state MND associations that have been providing care solutions to people with MND for many years and is grateful for the opportunity to offer further assistance.

Through the support of the FightMND Army, not only is important MND research progressing but we are also able to support the very tangible and immediate needs of Australians currently living with MND.


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TEXT 'PLAY ON' TO DONATE
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BIG FREEZE
A FIGHTMND EVENT
FIGHT MND.
Coca-Cola

The Big Freeze

During the 2021 campaign, the FightMND Army once again bought the iconic blue beanies, generously donated and helped deliver an incredible, record breaking $14.6 million for Big Freeze 7.

COVID destroyed hopes of a crowd-supported event, with Melbourne forced into its fourth lockdown in late May. This resulted in the AFL hastily changing schedules and the clash between Melbourne and Collingwood, that serves as the backdrop for the Big Freeze, was moved to the Sydney Cricket Ground.

Although we missed the infamous "March to the 'G" and the Army cheering on sliders, thanks to the support of the Victorian Government and the generosity of Network Seven the slide was able to go ahead at the MCG. The move of the game to Sydney also provided an unexpected opportunity to increase awareness of FightMND in New South Wales, helped by an icy celebrity canoe race televised from Bondi Icebergs.

Daisy Pearce faces the slide as Bluey

Our crew of sliders, drawn from Australia's sporting and media worlds, came together to slide into the freezing waters of an icy pool. They fully embraced the Big Freeze campaign, not only working tirelessly in the lead up to the event by sharing the FightMND message across radio, television, print and social media, but by turning up on the day dressed to impress. 'Razor' Ray Chamberlain channelled Elton John in blue sequins, Shane Crawford honoured the day as the Queen, Sarah Jones was the perfect Mary Poppins with a dance to match, and Abbey Holmes slid as 'Dipper', turning up in Dipper's original coat and a rather impressive moustache. Perhaps the most memorable was Gillon McLachlan, who arrived as Meatloaf to exorcise memories of an AFL Grand Final performance that many hope can now be forever forgotten.

Sharni Norder poses as Run DMC

Thanks to Bunnings, we were able to turn a humble MCG changeroom into the 'Slider Hub', decked out with stylish furniture and décor to provide the perfect headquarters for our sliders, and an ideal backdrop for photos and social media posts throughout the day.

This year, the Big Freeze was boosted by the “$2M by Monday” campaign which raised an incredible $2 million in just a few days. With the support of our media partners, ambassadors, sliders and Army, we achieved our goal before the conclusion of the Melbourne v Collingwood game. Contributing also to our record total this year was a $2 million donation by the Australian Government, announced by Minister Hunt during the Big Freeze game.

The success of the Big Freeze would not be possible without the support of our partners. Coles and Bunnings, as our major retail partners, ensured the beanie was promoted and sold Australia-wide through Coles, Coles Express and Bunnings stores. Australian Pork Farmers and Coles also generously donated a portion of the sales of a selection of Australian Pork products, adding to the significant total they raised for the campaign.

Image (left to right): Shane Crawford, Russel Howcroft, Gillion McLachlan, Daisey Pearce, Neale Daniher (centre) Billy Brownless, Sarah Jones, Sharni Norder, Abbey Holmes and Jeff Farmer

Our media partners Herald Sun, SEN and Seven ensured that our message was delivered to a broad audience. Special thanks goes to Network Seven Managing Director, Lewis Martin, who steadfastly supported filming of the slide at the MCG despite the absence of crowds and an accompanying football game.

'The Wiz' Jeff Farmer emerges from the ice

The AFL continues to provide significant support through its partnership with Sportsbet, by supporting the Big Freeze activation each season and through its extensive communication platforms. Melbourne Football Club, Collingwood Club and the entire AFL community continue to offer their unwavering support, buying beanies, encouraging their fans to buy beanies, and helping to raise awareness of, and vital funding for, FightMND. West Coast Eagles, through the Perth Big Freeze match, provided an incredible platform that helped raise awareness and vital funds in Western Australia.

We are also grateful to JC Decaux, oOh! Media and Crown for donating space on their billboards, and to all of Australia's television networks for generously providing time in their advertising schedules.

Thanks to Shout! we were once again able to sell the Big Freeze beanies online, including a kid's beanie that was introduced for the first time this year. We also received incredible support from community, school and workplace fundraisers that collectively sold over 30,000 beanies.

It takes an army to bring a slide to the MCG and we are grateful for the support of the MCC who generously allow us to use the MCG each year. Leisure Pools, Kings Fibreglass, Bells Ice, MSC Signs and the many more who build the slide, bring in a pool, break the ice, test for safety and so much more.

Although we couldn't march this year or fill the MCG, we know the Army was right there with us. From Neale, and all of us at FightMND we say thank you. Because of this support the Big Freeze continues to grow each year bringing us closer to beating the Beast that is MND.

Gillon McLachlan prepares to go down the slide as Meatloaf


FIGHT MND
RUSSELL HONEYCROFT
IT TAK PEOPLE

Community Fundraising

COVID continued to throw challenges at community fundraising with ongoing lockdowns disrupting events and causing numerous cancellations. However, our incredible Army of supporters, many of whom have had a personal experience with MND, rose to the challenge.

DIY Big Freeze

D.I.Y.
BIG FREEZE 7
SUPPORTING FIGHT ~~MND~~.

Despite the upheaval of ongoing COVID restrictions, DIY Big Freeze remained a big source of community fundraising revenue all around Australia. 365 participants sold an incredible 50,000 beanies (raising $1M in beanie sales) and raised an additional $615,000 in donations.

During Big Freeze 7, new branding was established for DIY Big Freeze and a new sub-website was created, highlighting how easy and fun hosting a DIY Big Freeze can be. This also allowed for easy distribution of new fundraising resources (such as posters, info sheets, FAQs and social media guides) to provide support to our incredible community fundraisers.

The majority of our community fundraisers continue to be sports clubs, schools and families with 70% of participants living in Victoria and the remaining 30% drawn from states across Australia.

Special thanks goes to the WA based Boxwood Hills vs Lake Grace Pingrup DIY Big Freeze which in it's fourth year raised $63,376.25, bringing the total raised over four years to $230,000.

Of the schools that participated in DIY Big Freeze 7, Jells Park Primary School was a standout, raising over $25,000.

Other community fundraising such as golf days, walks and swims continued despite the impact of COVID. We are hopeful that 2022 will bring a strong return to community events, especially DIY Big Freeze events.

27 Challenge

The 27 Challenge was launched in Spring of 2020 as a virtual peer-to-peer fundraising campaign, implemented to help mitigate the loss of in-person community fundraising brought by the pandemic. Participants selected their own physical challenges that incorporating the number 27, reflecting the 27-month average life expectancy of someone diagnosed with MND. Groups of friends and families came together to walk, run, cycle, swim and more, drawing fundraising support from their personal networks. Groups of friends and families came together to walk, run, cycle, swim, and more, during Spring of 2020.

Australian Ninja Warrior Michael Fisher (2020) acted as an ambassor to the campaign, enthusiastically enouraging participants to join him in the 27 Challenge via a video that featured him in his Ninja warrior gym.

Michael lost his father to MND in January 2019 after a 4 year battle with the disease.

Fundraisers included Bronwyn Watt who organised a 27 hour spin class in Adelaide that raised over $21,000 and Archie Payne, an 11-year old boy who ran 27 km in Tasmania and raised $3000.

The success of this campaign, which raised a total of $256,000 in it's launch year, provides a wonderful opportunity to continue to grow this event in 2021 and beyond.


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Major Sporting Events

Sadly, Run Melbourne was cancelled in 2020. Regardless, 150 signed up and raised a total of $24,000, with all runners transferring their registration and fundraising pages to the 2021 event. Blackmores Sydney Run festival 2020 was also cancelled, and although 23 people signed up, due to the event not going ahead fundraising was minimal.

Other Notable fundraisers

In late May, Gold Coaster Michael Pell swam 20km in memory of his late Dad who lost his fight against MND in 2011. Despite challenges, like big seas that meant Michael had to move his swim from the ocean to the pool, he successfully raised over $55,000 for FightMND. Massive congratulations and a huge thank you go to Michael.

Sockit2MND

Despite a difficult year for community sport the 2021 Sockit2MND campaign sold a record-breaking 21,776 pairs of socks to raise $326,640. These sales marked a 67% growth on the 2019 campaign (2020 was cancelled).

Congratulations to Gisborne Football Netball Club for being the top club for Sockit2MND in 2021, with 930 socks purchased and over 5000 beanies sold. Special mention goes to Dale Pemberton for coordinating Gisbourne's socks, beanies, and fundraising initiatives.

The Eastern Football Netball League (EFNL) in Melbourne have once again taken the title of the greatest supporters at league level, with over 1000 pairs of socks sold through their clubs. Thanks goes to Troy, Petra and all of the clubs at the EFNL for your continued support in what was most certainly a difficult year for all.

Thanks also goes to Sportspower, who partnered with FightMND for the first time this year and sold socks through their stores, selling over 1000 pairs nationwide.

A partnership with the AFL and AFL Auskick was initiated in 2021 with two AFL games featuring Auskickers wearing Sockit2MND socks at half time: Sydney on Big Freeze Day and Queensland the following week. We look forward to further developing these relationships in the coming year.

To all of the sporting clubs around Australia that supported us doing the 2021 Sockit2MND round we say a massive thank you. See you again next year!

Daniher's Drive

Despite significant enthusiasm for the 2020 Daniher's Drive, due to lockdown restrictions brought by the COVID-19 pandemic, the Drive was disappointingly cancelled. FightMND had received 23 registrations from hopeful Drive participants, each of whom chose to rollover their payment in hope that the 2021 Daniher's Drive will be able to go ahead.

A total of 20 teams participated in Daniher's Drive related fundraising events between January 2020 and October 2020, raising a total of $330,000. Special mention goes to Shady Ladies and the McCool Kids teams which collectively raised over $200,000.

We were also grateful to have continued support from our major Daniher's Drive sponsor, Chemist Warehouse. In October 2020, Chemist Warehouse facilitated an online silent auction and raffle and conducted an in-store donation campaign which collectively raised a total of $231,000 for FightMND.

A big thank you also goes to the Victorian Government for committing $1 million to the cancelled 2020 Daniher's Drive. This takes the total contribution from the State Government to more than $3 million over the past six years. We are grateful for their ongoing support.

DANIHER'S DRIVE
A FIGHT~~MND~~ EVENT

FightMND Club

With the MCG as a backdrop and the Long Room of the MCC filled with FightMND supporters, the FightMND Club was officially launched on 18 May 2021 to acknowledge those who are leading the fight, and to invite future leaders to step up and help beat 'the Beast' that is MND.

In 2007, Pat Cunningham, Neale Daniher and the late Dr Ian Davis, founded FightMND with a vision of a world free of Motor Neurone Disease (MND).

"We believe that in the coming years any person in this country diagnosed with MND should have the opportunity to participate in a clinical trial of investigative new drugs, or evidenced based re-purposed medications"
– the late Dr Ian Davis OAM

Since these leaders began their fight, progress has been made, however, MND still exists, and it is still a killer. The FightMND Club was created to recruit a new cohort of leaders to continue the fight and become part of medical history as a driving force behind better treatments and a cure for MND.

The FightMND Club specifically acknowledges Leaders who contribute a minimum of $10,000 each year, Champions who commit to, or raise between $50,000 and $100,000 and Heroes who provide an annual gift in excess of $100,000.

FightMND Club members are invited to a series of exclusive events throughout the year, with their Club status determining their level of access. Invitations include research tours, the Big Freeze slide rehearsal, Big Freeze game luncheon, Daniher's Drive launch event and invitations to the Annual FightMND Research Update and the FightMND Club Chairman thank you function.

FightMND Club members also receive communication reporting on FightMND news and key research and grant investment projects, prior to public release.

Members are welcomed with a medallion acknowledging their membership status and each year will be gifted a pre-launch Big Freeze beanie. They also receive recognition in the FightMND Annual Report.

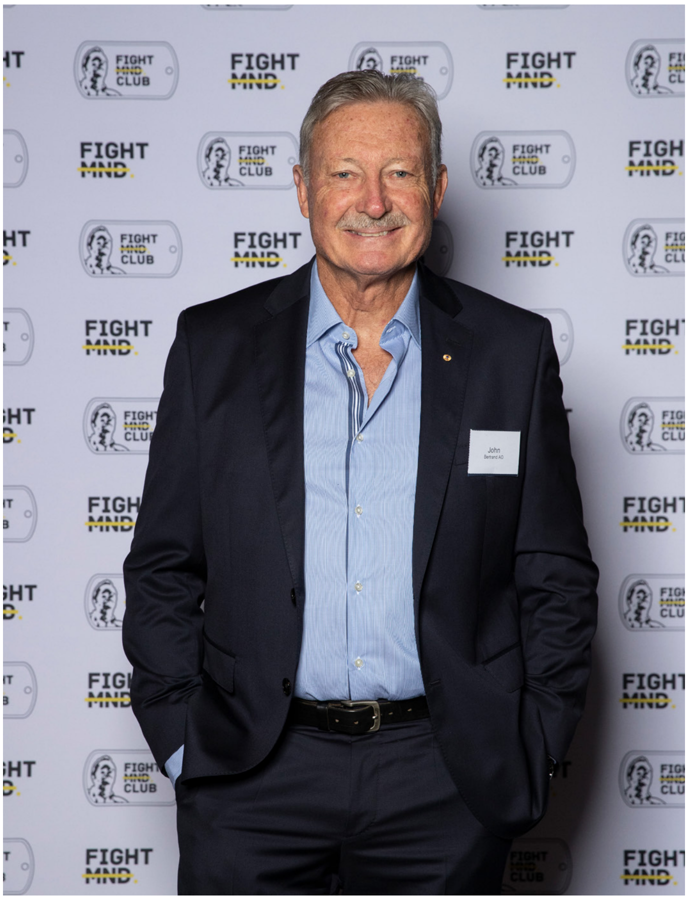
As of 30 June 2021, just weeks after the launch, 40 members had committed to joining the FightMND Club.

For more information, or to join the club visit fightmnd.org.au/fightmnd-club.


Kelly and David Neitz


Judith Slocombe and Janine Allis


Peter Zavecz and Tom Salmon


John Bertrand, AO

Heroes

Chris and Ren Barlow
David and Michelle Laidlaw
Jenny and John Michelmore
Mark and Sarah Laidlaw
Melissa and Mark Duggan
The Trustee for the Bourne Foundation

Champions

Hansen Little Foundation
Rallou Pty Ltd Massey Charitable Foundation
The Hope and Tony Saba Family Foundation

Gift In Wills

Our thanks go to the following people who thoughtfully remembered FightMND in their Wills:

Vivian Rita Pin
Estate of the late Howard Frank Boreham

Leaders

Australian Hotels Association
Betty Clarke
Darren and Joanne Fitzgibbon
Elizabeth and Tony Todaro
Evans Family Foundation
Geoff Steinicke
Greig Novelties South Pty Ltd
Hodges Family Charity Fund
Howard F. Boreham
Jonathan and Terri Lazarus
Lindsay Wood
Lisa Ring (and family)
Mandy Deam Foundation
Maple-Brown Family
Matthew Cook
Michael and Deborah Hayward
Michael Schneider
Natural Selection Group
Paul and Eliza Grant
Philip and Jenny Richmond
Sam and Fiona Woolcock
Shaw and Partners
Sophie and Anthony Duggan
Sovereign Financial
The Calvert-Jones Foundation
The Food Truck Park
The Jamie and Rebecca Gray Foundation
Tom Barr-Smith
Tony Gray
Universal Candy
Wood Family Foundation


BF
FIGHT MND

Financial Report

Total FightMND income for the year was $20.05 million, reflecting the incredible support received from our partners, donors and volunteers. This is down slightly from 2020 due to the cancellation of events as a result of the pandemic.

A total of $10.6 million was invested into medical research and clinical trials to pursue treatment and a cure for motor neurone disease and $1.4 million was provided to state MND foundations for equipment and aids in care for those who suffer from MND.

FightMND continues to manage our cash reserves to address our substantial funding commitments for Cure and Care strategies and we are well on track to fund a new round of grants for 2022. The 2022 Budget was approved by the Board and reflects ambitions of stability in our major fundraising campaigns during uncertain times.

Going forward, FightMND will move to a January to December reporting year. For the year ending 31 December 2022 we expect net fundraising income to be on par with that achieved in the corresponding year in 2021.

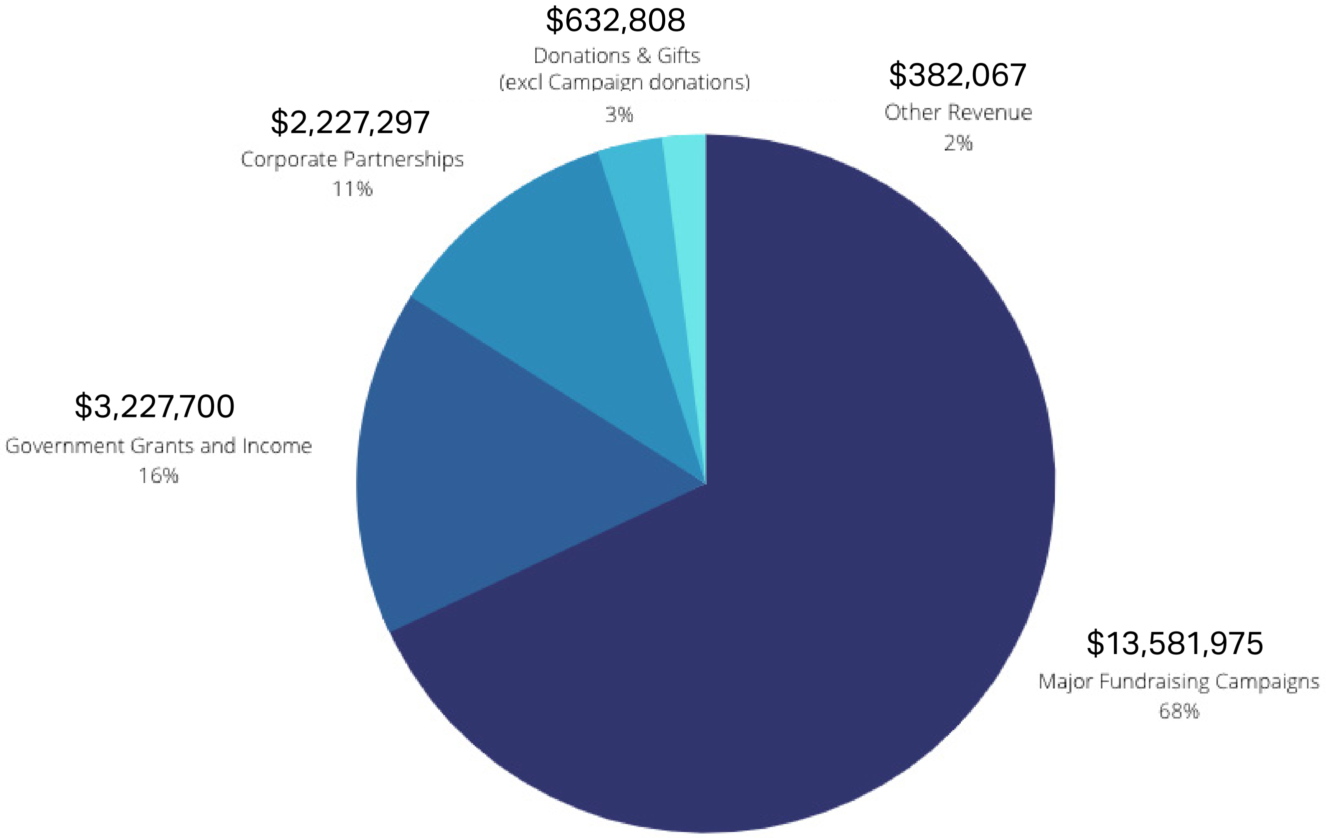
I thank my colleagues for their dedicated commitment and support in overseeing appropriate financial management and governance of the foundation. I also thank our partners, donors and volunteers who have helped us achieve a strong financial outcome for 2021.

Gary Nugent
Foundation Treasurer

NOTE: The summary financial information provided in this section was extracted from the audited general-purpose financial statements and does not include all the information and notes normally included in a statutory financial report. If you require a full set of the financial statements, please contact FightMND via phone 1800 344 486 or email info@fightmnd.org.au

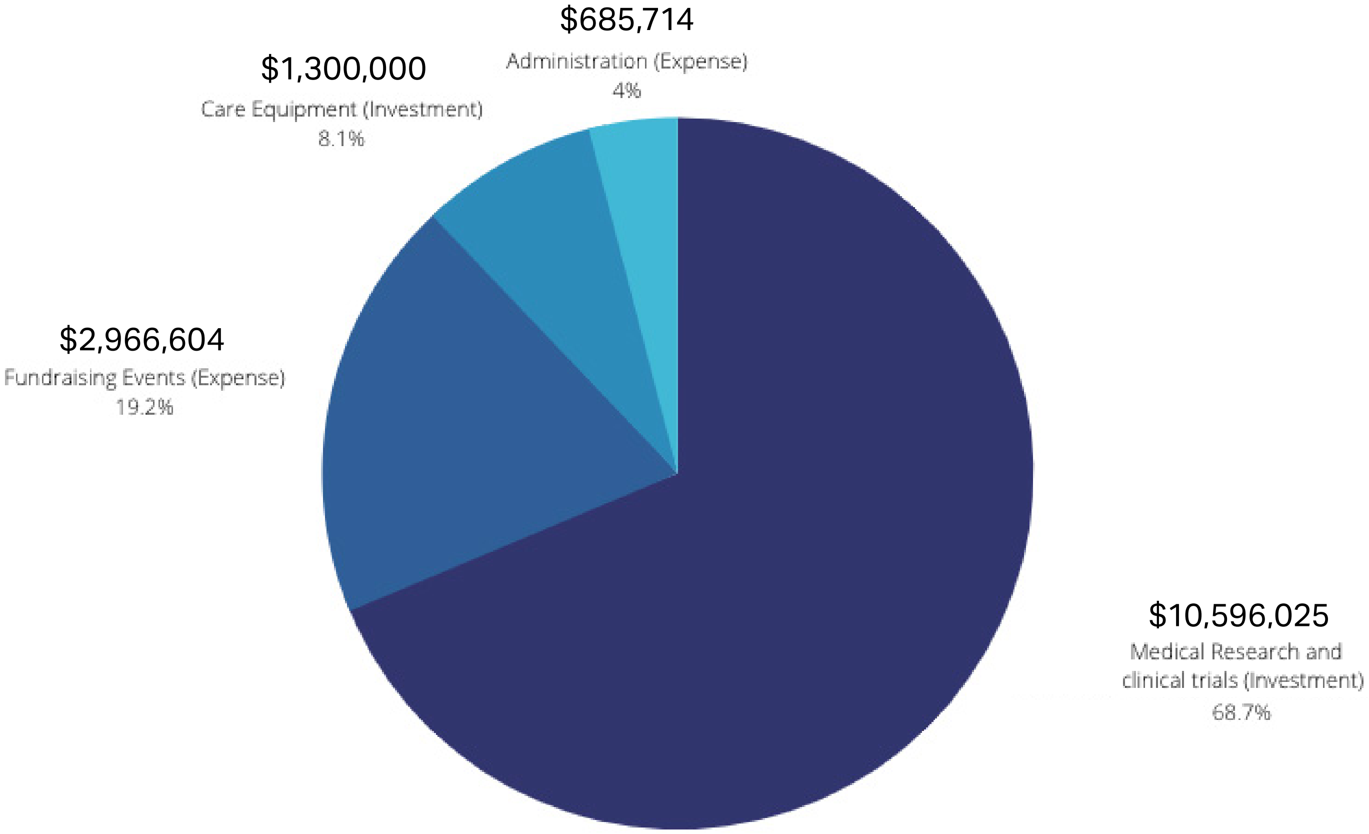
Where our money comes from

Total: $20,051,846



Where our money goes

Total: $15,548,343



Financial Tables

Income Statement		**2021**	**2020**	**2019**
Revenue				
Donations, Gifts and Grants		9,291,364	11,046,229	11,116,749
Fundraising Events		10,344,686	8,941,966	5,072,367
Other Revenue		415,796	1,516,638	765,950
Total Revenue		**20,051,846**	**21,504,833**	**16,955,066**

Expenses		**2021**	**2020**	**2019**
Investment	Medical Research	11,596,025	10,561,067	8,208,106
	Clinical Trials	-1,000,000[*]	881,085	6,758,522
	Care Equipment	1,300,000	1,250,000	2,467,083
Expense	Fundraising Events	2,966,604	2,797,928	2,178,107
	Administrative	685,714	502,515	436,865
Total Expenses		**15,548,343**	**15,992,595**	**20,048,683**

Operating surplus/loss		4,503,503	5,512,238	-3,093,617

[*] The negative clinical trial expense relates to the reversal of a grant awarded where grant conditions were not met and the grant agreement was terminated.

Balance Sheet		**2021**	**2020**	**2019**
Assets	Current Assets	44,671,725	37,158,345	28,190,035
	Non-Current Assets	11,110	12,818	5,214
Total Assets		**44,682,835**	**37,171,163**	**28,195,249**
Liabilities	Current Liabilities	17,909,468	13,025,626	12,368,116
	Non-Current Liabilities	10,160,857	11,604,694	8,798,526
Total Liabilities		**28,070,325**	**24,630,320**	**21,166,642**
Net Assets		**16,612,510**	**12,540,843**	**7,028,607**
Member's Fund		**16,612,510**	**12,540,843**	**7,028,607**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BIG FREEZE
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Thank you

to our FightMND Partners

The support of the FightMND partners is invaluable. Our major Big Freeze retail partners Coles, Coles Express and Bunnings this year contributed more than $7.8 million to our campaign, an enormous achievement.

Shout! once again provided the platform for online beanie sales and Linfox helped to get the beanies to warehouses and shops around Australia. Guests Furniture generously provided space for beanie storage, packing and shipping. Our media partners Network Seven, Herald Sun, SEN and Sportsbet help to share our Big Freeze message as do advertising partners Clemenger, JC Decaux, oOh! Media and Crown.

We are grateful for the support of the AFL, Melbourne Football Club, Collingwood Football Club, West Coast Eagles, and the greater AFL network that keeps the football community engaged in the Big Freeze campaign and ensures the FightMND Army continues to grow.

Our slide would not come together without the support of MSC Signs, Kings Fibreglass, Aquatechnics Pools, Bells Ice and of course the MCC which allows the event to take place at the MCG. Chemist Warehouse and PSC Insurance continue to support us for Daniher’s Drive and we thank Chemist Warehouse for providing significant fundraising opportunities in 2020 despite the Drive being cancelled.

Finally, to the Australian Federal Government and the Victorian State Government, thank you for your ongoing support. FightMND is incredibly grateful for the $1 million donated by the Victorian Government for Daniher’s Drive (2020) and the $2 million donated by the Federal Government during the Big Freeze (2021).

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FIGHT MND

Appendix A: Investment Projects

Project Lead

Dr Susan Mathers

Calvary Health Care Bethlehem, Melbourne, Victoria

Dr Susan Mathers says that one of the favourite aspects of her role is being able to offer involvement with research and clinical trials at the same time as delivering care to people living with MND. At Calvary Health Care Bethlehem she leads a multidisciplinary, state-wide service for people with a progressive neurological disease living in Victoria and bordering states. Over a thousand patients attend this service, including 350 people with Motor Neurone Disease.

Dr Mathers' clinical and research interests focus on the management of progressive neurological diseases, clinical trials and models of care. Dr Mathers is a neurologist at Monash Health, Adjunct Senior Lecturer, School of Clinical Sciences, Monash University and Clinical Director of Neurology at Calvary Health Care Bethlehem, Melbourne.

Project

Phase 1 - Clinical Trial

Safety and preliminary efficacy of oral monepantel in individuals with MND

Monepantel is a drug that is already approved for use in animals as an antiparasitic. It has been shown to have a good safety profile when given to animals, and to humans to treat cancer (in a small study). The death of motor neurons in Motor Neurone Disease (MND) is linked to damage caused when proteins/molecules clump together and accumulate within cells. Monepantel inhibits a cellular pathway called the mTOR pathway in cells. Drugs that inhibit the mTOR pathway have been shown to help clear these protein clumps in models of MND and may slow the progression of MND.

This study works with MND patients across two sites: Calvary Health Care Bethlehem in Melbourne, and Macquarie University Hospital in Sydney.

Impact

The funding provided by FightMND means that the safety and tolerability of oral Monepantel tablets can be tested in people with MND immediately, continuing over the next 12-18 months. The data collected in conjunction with concurrent preclinical studies will determine whether Monepantel should go on to be tested in larger Phase 2 studies.

Principal Investigator

Prof Roger Chung

Macquarie University, NSW

Prof Chung completed his PhD studies in biochemistry in 2003 at the University of Tasmania, and since then has undertaken research in brain injury, nerve regeneration and the study of neurodegenerative diseases such as MND and Alzheimer's disease. One of Prof Chung's major research achievements was the discovery of proteins that promote nerve regeneration following injury, which has been patented and led to the development of therapeutic peptides that are undergoing pre-clinical evaluation for nerve protection and regeneration.

Project

Genetics-based Therapies

TDP-43 targeting gene therapy for MND

TDP-43 is an important molecule in cells that has many functions. In almost all cases of MND, TDP-43 misbehaves and sticks together to form clumps that are thought to be harmful to motor neurons. Investigators in this study have identified the key molecules in a motor neuron that cause TDP-43 to stick together and will use pre-clinical MND models to test if a new gene therapy tool can break up and remove harmful TDP-43, restore health to motor neurons, and stop MND. A positive outcome of this study would be to generate a pathway for gene therapy in future MND clinical trials.

Impact

This funding is essential to support the development of a therapeutically-optimised gene therapy and validating it in pre-clinical disease models. Importantly this cannot be done by one research group alone – experts from different disciplines must collaborate to achieve this and this project includes collaborators based at the Children's Medical Research Institute in Sydney, Flinders University and University of Queensland. The funding provided by FightMND ensures that the scale and duration required to undertake this project is supported, and it can accelerate towards developing a potential therapeutic for MND.

Project

Genetics-based Therapies

Preclinical development of a SOD1 genetic therapy in sporadic MND

This study is working to develop an exciting new genetic drug to treat MND. The drug aims to reduce the production of a damaged molecule called SOD1, which sticks together in motor neurons and contributes to their death. The study will examine if this SOD1-targeted drug delays the onset and slows progression of MND in preclinical models. Evidence obtained from this study will build a strong case for testing the SOD1 drug in a clinical trial for MND patients in the next two to three years.

Impact

This is the first time that FightMND has funded MND research at Murdoch University and the Perron Institute and the first drug development grant FightMND has funded in Western Australia. The funding allows for the necessary safety studies that are required by the US Food and Drug Administration so that the molecule can be progressed into the clinic. It will also allow for the necessary laboratory work to determine how this therapy could help not only people carrying SOD1 mutations, but also those living with sporadic disease.

Principal Investigators

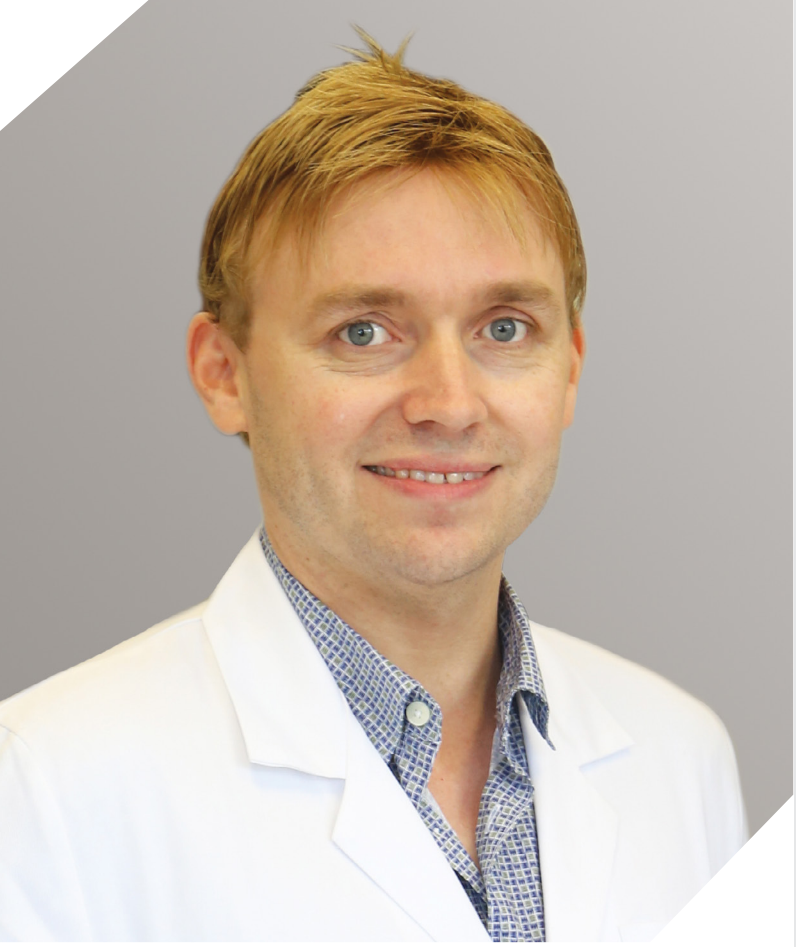
Prof Steve Wilton,
Prof Anthony Akkari
Dr Loren Flynn

Murdoch University, WA

Prof Steve Wilton says that his team has experienced several "favourite" scientific findings, most excitingly, the successful development of the first antisense drugs for Duchenne muscular dystrophy. They have also identified genetic markers that appear to play an important role in sporadic MND and predict that these markers could contribute to understanding patient response to clinical trials. They are hopeful that their discovery of the SOD1 suppression molecule will have broad implications for treating sporadic MND.

Prof Wilton, Prof Anthony Akkari and Dr Loren Flynn collectively have experience in neurodegenerative and neuromuscular disease genetic studies including both basic and clinical research, as well as industry. The team has integrated genetics into drug development and successfully designed antisense therapeutics for Duchenne muscular dystrophy that are now available to patients. Their core capability is in the design of antisense therapies and they have focused their effort on a pipeline of these for MND.

Prof Wilson, Prof Akkari and Dr Flynn are based at Murdoch University at the Centre for Molecular Medicine and Innovative Therapeutics, operating out of the Perron Institute for Neurological and Translational Science on the QEII medical campus, in Perth. The team also work with Black Swan Pharmaceuticals, based in the USA, a Perron Institute collaboration that is working to accelerate MND therapeutics to clinical development.



Principal Investigator

A/Prof Bradley Turner

Florey Institute of Neuroscience and Mental Health, VIC

A/Prof Bradley Turner has always had a fascination for mysteries of the human brain, especially degenerative diseases of the brain. He became interested in “mad cow disease” in his undergraduate years and his honours project was on Alzheimer’s disease which led him to MND. MND shows some clinical and pathological overlap with dementia, suggesting that if we can solve one, we can most likely help the other. His passion for MND research is largely driven by MND patients and their families who battle the disease daily and hope for a cure.

A/Prof Turner heads the MND Laboratory at the Florey Institute of Neuroscience and Mental Health, located at the University of Melbourne. He completed his PhD studies on MND and has been researching MND for 15 years. His research background is cell and molecular biology which means studying the individual cells and biological molecules that make up MND. A/Prof Turner has experience using MND patient donated stem cells to model MND in the laboratory and importantly, for drug discovery and development.

Project

Genetics-based Therapies

SMN2 splice-switching oligonucleotide therapy development for MND

This project will test if a drug called Spinraza is an effective therapy for MND. Spinraza already successfully treats MND in children, which is caused by the deficiency of a molecule called ‘survival motor neuron’, or SMN, in motor neurons. Investigators recently identified that a similar SMN deficiency occurs in adult MND patients. This project will use the latest genetic tools to develop a new form of Spinraza to restore SMN levels in adults affected by MND. Pre-clinical studies in MND models will confirm if the new Spinraza improves motor neuron health, movement and life expectancy. Should this study produce a positive outcome, the next step would be to test Spinraza in a clinical trial for MND.

Impact

The funding provided will allow the fast-tracking rigorous testing of Spinraza in the best available laboratory models of MND. It will also advance Spinraza to maximise its benefit and delivery into the brain by designing, screening and developing a superior formulation of the drug that can be administered into the bloodstream. The goal is to confirm whether Spinraza is effective in laboratory models of MND, so it can be advanced to clinical trial in the shortest time frame possible. This funding also catalyses a new international collaboration between scientists, chemists, neurologists and members of the pharmaceutical industry.

Principal Investigator

Prof Julie Atkin

Macquarie University, NSW

Prof Julie Atkin says that she became interested in MND as she really wanted to work on something that had tangible benefits for human health. Neuroscience really fascinated her and MND is a disease for which there is no effective treatment. Hence there is a real need to find new therapeutic strategies for this terrible illness.

Prof Atkin is a cell biologist/neuroscientist who has worked exclusively on MND for the past 17 years. Her research involves investigating the basic processes that trigger neurodegeneration in motor neurons in MND, and from this, designing new therapeutic approaches. Prior to working in MND, Prof Atkin worked in antibody research and in the biotechnology industry for several years. Prof Atkin is currently based at the Macquarie University Centre for Motor Neurone Disease Research, Faculty of Medicine and Health Sciences.

Project

Therapies targeting the structure of motor neurons

Modulating actin dynamics in MND as a novel therapeutic approach

This study is developing a new treatment for MND that repairs the function of actin. Actin is the most common protein found in humans and is needed to build a frame that holds each cell together. A cell's 'actin' frame can normally be strengthened or broken apart as needed, but in MND a loss of this flexibility makes motor neurons unwell and can lead to their death.

Impact

The funding provided by FightMND will assist investigators to identify the best drug to repair actin function and delay or slow MND in pre-clinical models. A positive outcome of this study would be to have this 'actin-repair' drug ready to test in a future clinical trial for MND patients in Australia.

Principal Investigator

Dr Ning Shan

Aclipse One Inc, PA, USA

Dr Ning Shan says he became interested in MND as he wanted to make a difference. MND is 100% fatal, with patient average life expectancy for this devastating disease being 2-5 years post diagnosis. There is a significant unmet need for better approaches and more effective treatments for fighting MND, as currently approved drugs only marginally influence survival or disease progression.

Dr Shan was trained as a physical chemist and received his PhD degree from the University of Cambridge, UK. He is a pharmaceutical executive with over 17 years of experience in preformulation, formulation, CMC (Chemistry, Manufacturing and Controls), Drug Metabolism and Pharmacokinetics, toxicology and is a subject matter expert in organic solid-state chemistry, with a special focus on pharmaceutical cocrystals. The current focus of Dr Shan's research team is to initiate the first-in-human studies for M102.

Project

Combination therapies - treatments targeting multiple causes of MND

Developing M102 to treat MND

Investigators have identified that the antioxidant and anti-inflammatory properties of a drug called M102, together with its ability to improve a motor neuron's energy level and its communication with other cells and muscles, may be beneficial to those with MND. One of the promising features of this drug is its ability to target and improve many parts of the motor neuron affected by MND, whereas most drugs tested target only one of the causes of the disease.

Impact

The funding provided by FightMND supports an important set of IND-enabling studies for M102 and will accelerate the initiation of its clinical development. It will also support the further development of therapeutic biomarkers that can be applied in the future clinical studies of M102 and potentially other therapeutics. Based on existing pharmacology data, it is believed that M102 could impact, slow down, and potentially reverse the progression of MND.

Principal Investigator

Prof Justin Yerbury

University of Wollongong, NSW

Researcher, Prof Justin Yerbury, was diagnosed with MND in May 2016. He first heard about MND 20 years ago when his uncle, cousin and mother were diagnosed in quick succession.
It was clear at the time that there was not enough understanding of the molecular basis of the disease and as a result, Prof Yerbury has dedicated himself to increasing the understanding of the origins of MND.

Prof Yerbury is currently working on two projects funded by FightMND, a Drug Development project: “Combination therapy to improve CuATSM outcomes in MND” (see page 31) and an IMPACT project: “Prion-like strains of TDP-43 aggregates in MND” (see page 38).

Project

Therapies targeting the structure of motor neurons

Combination therapy to improve CuATSM outcomes in MND

Investigators are using a three-pronged approach to tackle a hereditary form of MND in which a damaged molecule called SOD1 forms clumps and makes motor neurons unwell. The combination approach will use a drug called CuATSM (currently in a Phase 2 MND clinical trial in Australia, funded by FightMND), and two new drugs aiming to block signals instructing motor neurons to die, and prevent harmful SOD1 clumps from forming. These two new drugs are both approved medications, meaning that if effective they can be fast-tracked through to a clinical trial. A successful project could deliver a new potential combination therapy for testing in a clinical trial for MND patients within 24 months.

Impact

This funding will allow development of the combination therapy to a point where, if successful, it will be ready to move into clinical trials. It will be tested for safety, maximum tolerated dose and effectiveness against CuATSM alone. “These are labour intensive and expensive experiments that would not be possible without FightMND’s support,” said Prof Yerbury.

Principal Investigator

Prof Trent Woodruff

The University of Queensland, QLD

With funding from FightMND awarded in 2017, Prof Trent Woodruff has already advanced an immune modulating drug through the final stages of development into a clinical trial for MND. Prof Woodruff notes that this is the best possible outcome for a laboratory aimed at 'translating' research to help people with disease.

Prof Woodruff is a pharmacologist by training, having completed his PhD in 2003, helping to develop a new class of orally active drugs for arthritis. After completing his PhD, Prof Woodruff worked for a biotech company that was formed to commercialise this drug, progressing it through human clinical trials, before licensing it internationally. Prof Woodruff returned to the University of Queensland in 2007 to begin working on a similar drug approach for MND. He now heads a research team aiming to develop and progress new drugs for MND and other neurodegenerative diseases.

Project

Therapies targeting the immune system

Complement C3a receptor modulators as disease-modifying drugs for MND

This project aims to develop a new immune-protective drug to treat MND. The specific part of the immune system targeted by the drug is called C3a and investigators have found that increasing the activity of C3a protects motor neurons, improves movement and prolongs life in preclinical MND models. Safety studies will be performed to ensure the drug is safe for use in patients, and a successful project outcome will be a lead drug engaging the immune system, ready to test in a clinical trial for MND.

Impact

The funding from FightMND is critical in enabling the development of new therapeutic drugs that can target C3a receptors in the central nervous system. "Without this funding, it would not be possible to discover these drugs, and test them in MND models," said Prof Woodruff. This is essential work that must happen before drugs are progressed into human clinical trials.

Project lead

Dr Fazel Shabanpoor

Florey Institute of Neuroscience and Mental Health, VIC

Dr Fazel Shabanpoor is a FightMND mid-career Research Fellow and Head of the Oligonucleotide and Peptide Therapeutics laboratory at the Florey Institute of Neuroscience and Mental Health at The University of Melbourne. Dr Shabanpoor started his research training in pharmacology and chemistry through an Honours degree in 2005. Following completion of his Bachelor of Biomedical Science (Hons) degree, Dr Shabanpoor continued doctoral research training at The Florey. In 2011, he was awarded a NHMRC CJ Martin Fellowship to undertake postdoctoral training in the UK at two medical research laboratories, MRC Laboratory of Molecular Biology in Cambridge and the University of Oxford.

During his tenure in the UK, Dr Shabanpoor acquired a unique set of skills and expertise on using antisense technology for neurodegenerative diseases. In 2014, Dr Shabapoor returned to Australia and established his independent research group at The Florey.

Project

Drug delivery

Developing blood-brain barrier penetrating peptides

In the body, the brain and spinal cord are protected from harmful substances, or pathogens, in the blood stream by a protective barrier called the blood-brain barrier. While the blood-brain barrier is vital to protect the brain, it also creates one of the biggest challenges for treating any neurodegenerative disease - getting drugs across the barrier and into the brain and spinal cord where they are needed.

Impact

The funding provided by FightMND will develop a safe and innovative drug delivery platform based on blood-brain barrier-penetrating peptides to allow for systemic administration of three distinct classes of neurotherapeutics targeting MND. Importantly, the delivery platform will avoid invasive and potentially hazardous methods, such as intrathecal injections, currently used in the clinic to deliver drugs into the central nervous system. The outcomes of this project have a significant potential to provide therapeutic benefit for MND.

Project lead

Prof Roger Chung

Macquarie University, NSW

Prof Chung completed his PhD studies in biochemistry in 2003 at the University of Tasmania, and since then has undertaken research in brain injury, nerve regeneration and the study of neurodegenerative diseases such as MND and Alzheimer's disease. One of Prof Chung's major research achievements was the discovery of proteins that promote nerve regeneration following injury, which has been patented and led to the development of therapeutic peptides that are undergoing pre-clinical evaluation for nerve protection and regeneration.

Project

Gene therapies

Development of a dose-escalatable AAV delivery system for MND gene therapies

Gene therapies can work in a number of ways: by delivering a lost gene; or acting as a gene switch – turning on a gene that has been inactivated, or switching off a gene that is behaving abnormally. While gene therapies have excellent potential for treating diseases, current methods do not have the appropriate level of control – they provide always on, high levels of gene expression – which may have adverse or detrimental effects if prolonged for many years. This project aims to establish a new method for precise control of gene expression. Investigators will perform a proof-of-concept study of their new gene expression control system by combining it with a new gene therapy in development for MND.

Impact

"The funding provided will allow us to test the combination of two technologies (AAV gene therapies and nanoparticle-drug delivery sytems) in a completely new way," says Prof Chung. It will determine whether the combination of these approaches can make a breakthrough in developing an AAV gene therapy that can target motor neurons in MND. If successful, this would have a significant impact towards developing a gene therapy pathway for treating MND.

Project

Disease heterogeneity

Prion-like strains of TDP43 aggregates in MND

The clumping of materials called proteins within motor neurons is a key feature of MND. This project will use new technologies to assess the structure of individual protein clumps to try and define how their shape causes MND and the variability in the speed and severity of MND among patients.

Impact

FightMND's funding will help with the identification of new components of TDP-43 protein clumps that can be specifically targeted with drugs in future studies.

Principal Investigator

Prof Justin Yerbury

University of Wollongong, NSW

Prof Yerbury was appointed Professor in Neurodegenerative Diseases 10 years after completing his PhD and since being diagnosed with MND in May 2016 has published more than 35 papers. In January 2018 he underwent laryngectomy surgery and is now mechanically ventilated, drastically increasing his survival. He currently uses eye gaze technology in his work.

Professor Yerbury says that the funding provided by FightMND will allow for his IMPACT project to keep moving forward. Without funding it would not be possible to investigate this area so thoroughly. In addition, the funding for his drug development project will allow the team to develop their combination therapy to a point where, if successful, they will be ready to move into clinical trials. It will allow them to test for safety, maximum tolerated dose and effectiveness against CuATSM alone. The process for this testing is labour intensive and expensive and would not be possible without FightMND and the generosity of the Australian public.

Project lead

A/Prof Mary-Louise Rogers

Flinders University, SA

After going to the International ALS/MND Symposia in Sydney in 2011, A/Prof Mary-Louise Rogers decided to dedicate her career to developing a cure for MND. She met an inspirational Japanese MND researcher who had MND himself and thought, if he could be inspired to work on a cure, she would like to be working towards treatments for this terrible disease.

A/Prof Rogers works in the Neuroscience area of the Flinders Health and Medical Research Institute in the College of Medicine and Public Health. She gained a PhD in growth factors from the University of Adelaide before moving to Flinders University as a postdoctoral scientist to work on growth factors in the nervous system. In 2011 she became an independent researcher, setting up the only dedicated MND Research Laboratory in South Australia.

Project

Disease biomarkers

Using biomarkers to address MND heterogeneity and improve detection to benefit clinical trials

Key barriers to developing treatments for MND are the highly variable rate of disease progression and severity among patients. A disease biological marker, or 'biomarker', is a measurable marker that changes its appearance with disease progression. Biomarkers can be used to help diagnose patients, predict the rate of disease progression, create sub-groups of patients, or provide a measurable readout that a drug is providing benefit in clinical trials. This research team has previously identified a protein in urine of people with MND called p75ECD, that can track the progression of MND. This was a significant breakthrough, with p75ECD being used as a validated biomarker to track treatment effect in many MND clinical trials. The team has also recently identified another potential urinary biomarker for MND, called Neopterin, that measures immune system activation. Early results indicate that levels of urinary Neopterin are elevated in patients with MND, compared to healthy people. The team will utilise the Ian Davis Flinders University Biomarker Facility, a new high-through-put facility that enables large numbers of patient samples from clinical trials to be assessed for MND biomarkers rapidly and effectively.

Impact

This funding will allow for the validation of these two urinary biomarkers to accurately measure progression of MND, prognosis and benefit of treatment for patients. It will improve the ability to identify and group patients with similar speed and severity of MND to assist with clinical trial patient recruitment and increase the chance of identifying effective treatments in clinical trials.

Project lead

Prof Aaron Russell

Deakin University, VIC

Prof Aaron Russell's interest in MND research has evolved over time as he gradually became aware of friends and family of friends who had been impacted by MND. He also notes that the passion and collegiality of discovery and clinical MND researchers, both in Australia and internationally, has drawn him to this field.

Prof Russell has a PhD in Exercise Physiology/ Molecular Biology from Deakin University and has worked in Switzerland, the USA and Australia. Over the past 20 years his research has focused on understanding the molecular mechanisms regulating skeletal mass during exercise and disease. More recently his attention has shifted to understanding how cells communicate with each other in healthy and diseased conditions, with a specific interest in MND. Prof Russell is based at Deakin University in the School of Exercise and Nutrition Sciences. His research is conducted within the Institute for Physical Activity and Nutrition (IPAN).

Project

Disease biomarkers

Identifying biomarkers from extracellular vesicles for early detection, disease progression and therapeutic efficiency in MND

It is hard to diagnose MND and to know if treatments are reaching their intended targets. This project assesses blood samples from MND patients and a new pre-clinical model of MND, to try to identify ways to accurately detect MND, pinpoint how long a person has lived with MND, and rapidly measure how successful treatments are.

Impact

Funding from FightMND will rapidly speed up the process of identifying new biomarker profiles to predict disease on-set at an early stage. "It is hoped the biomarker profiles can also be used in current clinical trials as a measure of treatment responsiveness, improving the likelihood of current trails being successful," says Prof Russell. Additionally, the biomarker profile observed during the reversal stage may identify new targets for therapeutic manipulation.

Project lead

Dr Sophia Luikinga

Florey Institute of Neuroscience and Mental Health, VIC

Dr Sophie Luikinga says that when she read the news that her application was successful, she could not believe what she was reading, and had to read the email three times over. She is beyond grateful for the confidence and support that FightMND has shown for her project and says it is extremely rewarding to be able to make a difference for people living with this terrible disease.

Dr Luikinga completed her undergraduate science degree in the Netherlands before completing honours in neuroscience at the Florey Institute of Neuroscience and Mental Health in Melbourne. After completing honours, she returned to the Netherlands where she worked on the effects of diabetes on muscle function and perfusion. Her interest in neuroscience was too strong so she took the opportunity to complete her PhD in neuroscience. She is now based at The Florey in Melbourne.

Project

Disease biomarkers

Lipidomic signatures in blood as a novel biomarker for MND

Currently, there is no marker that can accurately diagnose or define MND. This project aims to overcome this barrier by developing a blood test that detects materials called lipids that change in MND. The lipids detected may be able to help diagnose MND, inform on the exact stage of MND, and determine if treatments are successful.

Impact

"Without the funding and support, it is unlikely this project would go ahead," says Dr Luikinga. The investment provided by FightMND will assist the team in investigating the potential of this panel of lipids to function as a reliable biomarker to determine onset of MND, track progression, and ultimately test the effectiveness of drugs in clinical trials. If successful, this could be a future staple test in MND diagnosis and progression.

Project lead

A/Prof Lezanne Ooi

University of Wollongong, NSW

A/Prof Lezanne Ooi was initially inspired by her colleagues at the University of Wollongong to collaborate on an early MND project. It was from there that she developed her MND research program.

A/Prof Ooi has been a neuroscience researcher for twenty years and has worked in neurodegenerative disease research for fifteen years. She is an NHMRC Boosting Dementia Research Leadership Fellow and Group Leader of the Neurodevelopment and Neurodegeneration Laboratory. A/Prof Ooi is a cellular neuroscientist, and her research has spanned electrophysiology, molecular and cellular biology, and pharmacology. She is currently based at The Illawarra Health and Medical Research Institute and School of Chemistry and Molecular Bioscience, University of Wollongong.

Project

Disease biomarkers

Defining an electrical signature of sporadic MND, and developing a drug screening technology and novel therapy

Motor neurons talk to each other using electrical signals. It has been known for some time that patients with MND show alterations in the electrical signals of their motor neurons, and these changes can start even before the onset of symptoms. For this project, MND patients have donated skin cells that are used to recreate their motor neurons in the laboratory. Importantly, the same changes in the electrical signals detected in living patients can also be seen in the recreated motor neurons. This means that patients' cells can be harnessed to investigate the disease process.

Impact

Funding provided by FightMND allows the opportunity to combine electrophysiology and cell biology data with computer science, to study the exact causes of those changes in electrical signals and test novel ways to prevent those changes.

Project lead

Dr Samantha Barton

Florey Institute of Neuroscience and Mental Health, VIC

Dr Samantha Barton says that she has always been fascinated by the inner workings of the brain and understanding how it functions in both development and disease. More recently she has been drawn to understanding the biology of MND. It is such a complicated disease with such a challenging prognosis. I hope with my research background and expertise that I can contribute to the understanding of the underlying causes of MND.

Dr Samantha Barton received her PhD in 2015 before completing her first post-doctoral position at the University of Edinburgh, Scotland. It was in Edinburgh that she learnt how to culture patient induced pluripotent stem cells and differentiate them into key cell types, and make three-dimensional structures like organoids. Dr Barton returned to Melbourne in 2018 where she has established these techniques and continued her research within the Motor Neurone Disease laboratory at the Florey Institute of Neuroscience and Mental Health in Melbourne.

Project

Disease models

Using 3D spinal cord organoids to model oligodendrocyte neurotoxicity in MND

This project will use MND patient's skin cells to grow stem cells and create a pre-clinical MND model, called a 3-dimensional organoid that resembles the spinal cord. The organoids replicate key features of MND that occur in patients and will allow studies to investigate the causes of, and test potential treatments for MND.

Impact

Using patient induced pluripotent stem cells, and deriving complex structures like 3D-organoids, is a very powerful system but is a time and cost heavy endeavour! Investment provided by FightMND will allow further understanding of the role of glia, like oligodendrocytes, in MND onset and progression. Characterising the underlying biology of MND will be of great benefit for people living with MND, as it will uncover new therapeutic targets for treating the cause, rather than the symptoms, of the disease.

Thank you

This year's $8.4 million dollar investment takes FightMND's total investment in MND research to $48.45 million since we began in 2014.

None of this would be possible without the support of our FightMND Army.

To our generous donors, major partners, volunteers and supporters – thank you for taking up the fight with us.