Annual Report

2018-2019

Metabolic
Dietary
Disorders
Association

Embracing the Potential

Reflections

The past 12 months have seen the MDDA continue to adapt and evolve, presenting opportunities for growth and strategic partnerships as advancements in research, treatments and care enable us to embrace the potential for a brighter future for all individuals living with an Inborn Error of (protein) Metabolism (IEM).

With an ongoing focus on achieving positive health outcomes for people living with IEMs, MDDA has successfully delivered on several initiatives and growth strategies. This is a testament to the hard work of the whole organisation, all whom are helping MDDA build on the quality of the support we provide to our community.

MDDA believes wholeheartedly in our vision, a future where all individuals living with an IEM are leading a life at full potential, not limited by choice or resource. It is what drives every initiative we plan, every campaign we work on and every conversation we start or have, whether that be in a social, political or closed environment.

MDDA frequently inform clinicians, government and stakeholders around best ways to implement a patient centred approach and strive towards improved patient health outcomes. We facilitate discussions for education on improving metabolic healthcare services and resources in line with the Health Department's vision of strengthening evidence-based policy advice, improving program management, research, regulation and partnerships with other government agencies, consumers and stakeholders.

Collectively we continue to use our voices and stories to raise awareness to the wider community and provide a detailed picture of living with an IEM and the challenges that affect people's health, wellbeing, income and quality of life.

We are fortunate to have a forward thinking, hardworking and skilled Committee of Management who put in many unpaid hours steering and supporting the organisation and advocating for more resources and better recognition for people with iIEMs. It's been a busy year and without any doubt whatsoever, we would not have been able to achieve any of our successes without an incredible team of dedicated volunteers and supporters. I thank every volunteer in all areas of the organisation who have worked incredibly hard over the last 12 months to deliver and provide meaningful support to our community - through your hard work, people with IEMs are informed and supported. Your participation is one of our most important sources for future planning and strength to our association.

We would like to welcome 2 new staff members to our National Office Team. With thanks to a recent successful competitive grant application, we are very excited by the appointment of our part time Patient Pathways Telehealth nurse, Jo Campbell. Jo is delivering MDDA's new Patient Pathways Program, supporting IEM individuals and families in a national capacity. We also welcome Isabella Hendricks, who joins the team to help drive our digital communications priorities - delivering programs and services to our members, as well as expanding upon our many awareness campaigns and activities. I would like to thank our small, hard-working office team who have been steady and productive and managed a lot with limited resources once again this year.

I am looking forward to building on the work we've completed and continuing to work with, and for, the community of people in Australia living with an IEM.

Monique Cooper
MDDA, President

Contents

Realising our Vision

Our Vision

All individuals living with an IEM are leading a life at full potential, not limited by choice or resource.

Our Mission

The MDDA educates, connects and enables individuals and families living with an IEM – ensuring more informed choices and a better quality of life.

Our Goal

Achieving positive health outcomes for individuals living with IEMs, and requiring life-long metabolic dietary management skills, resources and support.

As the National peak support body supporting IEM families in Australia, we strive towards delivering upon our strategic priorities, and this year has been one of incredible outcomes.

Our Focus / Our Achievements

Our Focus		Our Achievements		
IEM FAMILIES	*Supporting, Educating, Connecting and Enabling*	**26** New Members	**645** Subscribers	**687** Online patient forum participants
		3600+ Voluntary Hours	**3500+** support emails & phone calls	**3** Printed Newsletters
		5 Online eNews	1 National retreat – over **140** attendees	1 State (WA) retreat – over **100** attendees
GOVERNMENT	*Informing, Influencing and Advocating*	**15** Reviews	**125** Resources & and materials developed and distributed	
		7 Advisory responses provided	**2100** Requests for information to the community on health policy matters	
HEALTH PROFESSIONALS	*Collaborating and Educating*	**160+** Medical practitioners attending MDDA education and training sessions	**31** Patient Group meetings attended	
GENERAL PUBLIC	*Informing and Engaging*	**2465** FB MDDA followers (128 New)	2nd year of the Great Protein Challenge **$18,000+** raised, **28,000+** people reached	**11** #teamMDDA fundraising initiatives

Embracing the potential

For many families, the success of MDDA's advocacy efforts in securing access to a new treatment for PKU children in Australia has opened the door to a life of new potential.

Being a new parent is tough enough, let alone with a PKU diagnosis on top of things. Coping day to day can be emotional and worrying, navigating the journey and managing the day to day challenges and milestones is complicated - with a lifelong restrictive low protein medical diet the only means of control and prevention from a life of disability.

That was until one Government decision has helped to lift the burden and provide new hope to families.

Willow and Kennedy's Story

Brooke Dwan and husband Matt welcomed their first child Willow into the world 28th October, 2012.

"We had no idea that a phone call we would receive in 6 days would turn our world around in a way we never imagined."

All newborn babies are tested for PKU through the newborn screening test taken during the first few days of life. Around one in 11,000 newborn babies are diagnosed with PKU.

"I remember him [the doctor] saying brain damage is the side effect if the diet is not adhered to," Brooke recalls on the day Willow was diagnosed, "I couldn't stop crying, I couldn't even continue the conversation. This is when our journey as PKU parents began."

Breastfeeding was replaced with a special medical formula, and as food was introduced, a medically controlled diet low in phenylalanine was introduced.

"This was all that was on offer, no other medicines or treatments, just a restrictive extremely limiting diet to be sustained for life. It was daunting and felt incredibly unfair."

Shortly after Willow was born, Brooke and Matt's second daughter Kennedy was born, and she was also diagnosed with PKU.

"During the pregnancy of our second child we knew that a PKU diagnosis was a 1 in 4 possibility, and on day 5 all those emotions came back to me like the day Willow was diagnosed."

Like many PKU families in Australia, life adjusted as the diet was learned. Eliminating almost all food groups and foods except for some fruit and vegetables, replacing them with synthetically modified foods and supplementing protein requirements with a special medical formula – these are the essential components of a PKU diet.

Like many PKU kids, Willow 7 and Kennedy 4 learnt to lead a life consuming just 8grams of natural protein each a day, with regular weekly blood spot tests being taken to ensure their phenylalanine levels do not exceed beyond safe levels.

A new life with new opportunity

Following a long and successful advocacy campaign led by the MDDA, in May the PBAC listed the treatment known as sapropterin for treatment of children with PKU. Sapropterin is known to be effective for around 30% individuals with PKU, resulting in the ability to better control and lower phenylalanine levels resulting in an ability to safely tolerate a higher level of natural protein and thus provide for greater ability to comply to the PKU diet.

Due to this decision, both Willow and Kennedy were fortunate to be eligible to be tested for their responsiveness to the newly listed treatment.

With a stroke of good fortune, shortly thereafter Brooke and Matt received the most amazing news from their metabolic team that both girls were responsive.

"This next chapter in our lives has only just begun, but we are excited for the future and what this means for our girls to both be provided with this opportunity."

"Whilst it is not a cure, it is the next best thing we could hope for right now and will go a long way in making a difference to our girl's lives."

"We will be forever grateful to the work that has been done by the MDDA to provide such an incredible opportunity for our girls."

Providing hope for a missed generation

"For Leigh, he is saying new sentences, he has more understanding of what is being said, he is asking more questions and understanding the answers better."

These are just some of the remarkable improvements being acknowledged by carers who are witnessing first-hand the impact of introducing a low protein diet and formula to a generation of PKU Australians born prior to newborn screening and dealing with the consequences from a missed or late diagnosis.

"The improvement is very rewarding and can be very emotional at times. To see his life improve so much is so good."

MDDA in collaboration with the Royal Melbourne Hospital and key industry partners have launched the PKU Carers Pathways compendium and outreach program. The program initially designed for carers of 20 late diagnosed or untreated PKU adults with disabilities living in Victoria, will be expanded to all metabolic clinics around Australia.

The carers are generally not family members and have little or no knowledge of PKU or its treatment and management . The majority of the recently identified PKU adults with disabilities are living in shared community housing facilities or Aged Care Homes.

"To receive this compendium and also meet other people that are dealing with PKU and talk to them is such a great help in understanding this condition."

The PKU Carers Pathways Program supplies the carers with a compendium which is a high-quality visual handbook to provide education and guidance on the daily management of PKU. The PKU Carers Pathways Program will also offer an outreach service to provide mentoring and further education to support the carers to provide the best care for PKU Adults with disabilities.

MDDA's intention is to co-ordinate a trained volunteer outreach service to support the carers.

"It is really invaluable and so important, I have found, to connect in with such a supportive community, and if opportunities like this can continue, things can only improve for the people we support. Thank you to the MDDA Team."

Dennis Thompson is Leigh O'Boyle's (late diagnosed PKU) carer and a recipient of the PKU Carer Pathways Program.

As new MDDA members, Dennis is the first carer program recipient, and was pleased to share his story with us at a recent retreat, expressing the differences in Leigh since he has been back on diet and highlighting the importance of this program.

Hope for the late diagnosed

Since the mid 1960's all babies born in Australia have been screened for PKU shortly after birth and commenced on a specialised low protein diet and formula. Life long dietary treatment is required for people with PKU, as the build-up of phenylalanine can lead to brain damage, with learning and behavioural difficulties.

For people born prior to newborn screening, it has been found that introducing a low protein diet with PKU formula (even later in life) can reduce symptoms resulting from late diagnosed or untreated PKU.

Improvements in symptoms can include;

- Decreased aggressive and disruptive behaviour
- Decreased irritability and hyperactivity
- Decreased neurological symptoms
- Decreased episodes of incontinence
- Disappearance of eczema
- Increased ease of bathing and dressing
- Increased verbal communication and appropriate eye contact
- Improved intellectual functioning
- Improved mood
- Increased social awareness

New support initiatives

Paving the path through life's journey with an IEM is filled with joy, celebrations, special moments, but also concern of the unknown, worry of failure and continued search for new information.

MDDA's Transitional Stages Project provides patient support information and resources to parents and families relevant to each stage in their PKU/IEM transitional Journey. From diagnosis right through to adulthood, these resources are designed to provide reassurance, support and access to useful resources and materials for all individuals living with an IEM. Initial phase will focus on PKU in 2018/2019 and then roll out to other IEM conditions thereafter.

Initial focus has been on the newly diagnosed and carers of adults, with work underway to deliver upon more resources for other transitional phases.

Carers of missed or late diagnosed PKU adults

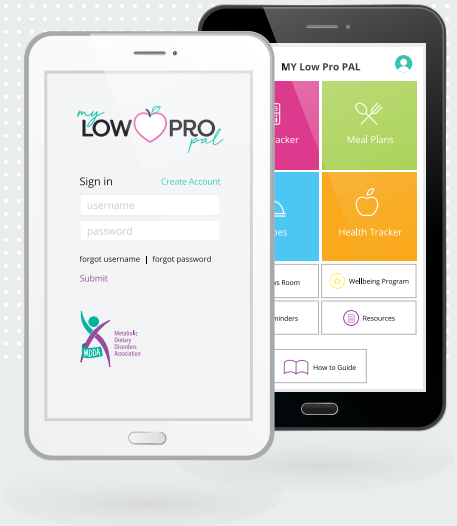
MDDA in collaboration with Royal Melbourne Hospital have created the PKU Carer Compendium for missed or late diagnosed PKU adults living in shared community housing facilities or nursing homes in Victoria. These high-quality visual handbooks and personal compendiums will assist in educating their carer on how PKU needs to be managed on a day to day basis.

The compendium features clinic contact details, treatment of PKU, PKU diet, meal plans, blood spot testing guide, resource list and other useful information.

Stage 2 will engage volunteers to assist with the ongoing delivery of the project with the expectation to roll out the program Australia wide in 2020.

Parents of newly diagnosed babies

MDDA launched our Patient support "PeeKabU" bags, to be provided to parents of newly diagnosed infants providing a range of useful gifts and items designed to help our newly diagnosed families begin their IEM journey.



IEM Dietary Management and Wellbeing App

MDDA have progressed the development of an app that will be an integral comprehensive tool for an individual's day to day management of their IEM and all related clinical, dietary and holistic health priorities.

The features will include comprehensive and interactive meal planners, recipes, product catalogues, ingredient calculators for counting protein and nutritional data, clinical appointment reminders, activity tracker, wellbeing blog, links to IEM educational materials and resources, food diary and access to forums.

MDDA will work with specialist metabolic health professionals throughout the project and apply for recommendation by ASIEM.

Keeping the conversation going

MDDA's online 'patient only' forum provides an ongoing platform for active, inclusive and empowering discussion amongst our IEM patient community. Conversation is diverse and frequent, providing participants with access to a positive and friendly peer-based support network and a safe and private place to discuss topics such as;

- Nutritional information
- Adults returning to diet
- Travel considerations
- Dental health
- Weight management
- Low protein foods
- Meal planning & recipes
- Blood spot testing
- New treatments
- Clinic relations
- IEM Food Grant
- Food labelling & counting
- Maternal PKU concerns
- School camps
- General health & wellbeing
- Dietary compliance
- Lunchbox ideas
- Cooking tips & tricks
- MDDA events and fundraisers
- Clinical trials & Research
- National Disability Insurance Scheme

MDDA signature events

Family Retreats

21-23 September, 2018, South Australia
30-31 March, 2019, Western Australia

Over 140 members came together in South Australia for our National Family Retreat, encouraging us to pursue a journey of new information and enlightenment to uncover the potential to embrace ourselves.

We couldn't stop at just one retreat so we took the program over to Western Australia where over 100 locals experienced the opportunity to connect with the IEM community, giving families a chance to spend time together in a relaxed environment.

"Thank-you for another fantastic Retreat once again this year! We are so grateful for the chance to collaborate with other families with IEMs. We also loved hearing about future treatments."

Our retreats are as much about education and information as they are about social and emotional connections.

"A fantastic weekend with interactions, topics and people. Connecting with individuals helps put a face to PKU. It isn't just a genetic disorder, it's people with lives, dreams and ambitions just like everyone else."

All members young and old make genuine lasting friendships in a safe and supportive environment, forming strong bonds through shared experiences.

MDDA express our gratitude to the health professionals who attended and presented at our retreats. We are incredibly grateful that they see value in participating in sessions and workshops and see them to be informative and worthwhile to our attendees.

"I love coming to the MDDA retreats, the community is fun and friendly and the content is so informative and very relevant to managing my diet."

Thank you to our volunteers whose passion and commitment make the retreats such successful events.

"We would like to thank the MDDA for encouraging us to come to our first retreat. We are leaving with happy and positive mindsets around our PKU challenge and feel so fortunate to have this support."

End of Year Celebrations

Gatherings took place around the country at the end of 2018 to reconnect and celebrate a year of new friendships and achievements. Events took place in Victoria, South Australia, Queensland, New South Wales and Western Australia. These events are an important feature on our annual calendar as they provide an opportunity for families and people of all ages to get together and strengthen bonds.

Informing government health policy

MDDA assists the Australian Government to inform and support the health agenda by improving linkages, networks and cooperation with our members, the health sector, the wider community and the government. MDDA regularly consults with members and people with lived experience to provide opportunity to inform our policy work and responses to government reviews, strategies and health agenda.

Sports Supplements Policy Review

MDDA participated in the Department of Health "Round Table on Sports Supplements". This forum brought together key stakeholders from government, industry and regulatory bodies to review sports supplements regulation following the death of a young women from an undiagnosed Inborn Error of Protein Metabolism (Urea Cycle Disorder) using sports supplements to 'protein load'. MDDA provided input into a range of possible action steps for the Government to consider including legislation review, closer alignment between regulators, the consideration of how sports supplements and foods for special medical purposes are considered in legislative tools versus therapeutic goods.

Life Savings Drug Program Reform

MDDA responded to a written request from the Life Savings Drug Program Secretariat providing our support for the Draft Terms of Reference supplied by the department. We also suggested the addition of another item covering patient access to treatment that should consider the ease of filling prescriptions, including regional patients who do not live close to a specialist metabolic clinic.

Newborn Screening National Program Expansion

MDDA consulted with experts and other stakeholders about the possibility of second tier newborn screening of Tyrosinaemia Type 1. These discussions are ongoing.

National Rare Disease Framework

MDDA together with Rare Voices Australia attended Rare Diseases Day at Parliament House in February to raise awareness about the challenges and inequities people with rare diseases face. MDDA raised the need for all responsive patients to have access to sapropterin, consistent and reliable access to PBS products, Newborn Screening additions for IEMs not diagnosed by the current tests, and actions required by the MDDA in preparation for the review of treatments on the Life Saving Drugs Program.

IEM New Treatment Listings and Reviews

Tyrosinaemia Type 1

We are pleased to see ongoing support for Tyrosinaemia Type 1 patients with new formulations of nitisinone on the Life Saving Drugs Program announced as part of the 2019 federal budget.

Phenylketonuria (PKU)

MDDA provided a written submission to the Pharmaceutical Benefits Advisory Committee (PBAC) in their reconsideration of sapropterin for the treatment of PKU at the November meeting. This submission specifically addressed items raised in the public summary documents from the March 2018 meeting including continuity of access for patients commencing treatment before the age of 18 and the suboptimal health outcomes for adults who have been on treatment since birth. The input of the patient group was acknowledged in the positive recommendation to list for children and adolescents and in particular to continue access beyond 18 for patients who have commenced treatment.

BH4 Deficiency

MDDA were approached by PBAC to provide patient perspective input into a review of the use of sapropterin for patients with BH4 deficiency. MDDA consulted with PBAC representatives to provide input in the Drug Utilisation Subcommittee review of sapropterin for BH4 deficient patients. In order to provide accurate a representative advice we consulted with BH4 deficient patients and their families in Australia to respond to questions raised by the Drug Utilisation Subcommittee.

Government Grants

MDDA is funded under the Health Peak and Advisory Bodies Programme.

Health Peak & Advisory Bodies Programme Grant

MDDA provided written advice to the Health Minister regarding the Health Peak & Advisory Bodies Programme to ensure certainty for patients and patient groups ahead of a 2019 general election. This was a potential gap identified by a number of patient groups and peak bodies and the group were able to work proactively to address this gap ensuring that expert and patient centric advice is available to the department.

CCDR Patient Pathways Program Grant

MDDA were successful recipients of the Patient Pathways Program announced by Health Minister Greg Hunt MP. The program funded by the Australian Department of Health will place a specialist telehealth nurse in our organisation to provide a pathway for support for patients to access a coordinated comprehensive case management service, to talk about their experience so that this information can inform future research, treatment, care, information and support across the health sector. We are working with the Centre for Community-Driven Research to implement this program.

Achieving greater IEM patient health outcomes

MDDA remain in close communication to the nationally recognised Australasian Society for Inborn Errors of Metabolism (ASIEM) to facilitate access to and development of 'world class' best practice management and care for the treatment of IEMs.

National Treatment Guidelines

MDDA requested that ASIEM consider developing consistent national treatment guidelines for other inborn errors of protein metabolism similar to those developed for PKU. These guidelines have provided an invaluable tool for patient empowerment and education and have aligned Australia with activities in other countries. The guidelines also act as an important tool for patients to help asked informed questions about treatment and discuss options with clinicians. In addition to treatment guidelines we have requested the possibility of ASIEM developing clinical best practice guidelines. These guidelines would assist with advocacy to help address a lack of clinical resources in certain states in some Australian clinics and have also been helpful in our advocacy for better access to treatments.

National Sapropterin Testing Protocols for Children

MDDA liaised with ASIEM to provide an endorsed national official statement and factual information to distribute to members regarding Pharmaceutical Benefits Advisory Committee (PBAC) positive recommendation for listing sapropterin for children and adolescents. The statement included information on the testing protocols including; the process, eligibility and timing of testing for children and adolescents for distribution to families to manage expectations once metabolic clinics open the testing process.

Advocacy for Access to Sapropterin for Adults

MDDA facilitated an online discussion forum with the wider adult PKU community on the decision of government not to include access to sapropterin to adults via the Pharmaceutical Benefits Scheme. The purpose of the webinar was to discuss strategies of the patient group to inform government of the clinical benefit to cost ratio in the adult PKU population. Our ongoing advocacy on this matter continues.

Australian Based Clinical Trials

MDDA have liaised with ASIEM and specialist metabolic clinics to explore the possibility of Australian based clinical trials for MMA and PKU. We have sought advice from research and clinical trial organisations about the outcomes of discovery and Phase 1 trials to identify any updates or information that is appropriate for our members.

Maternal PKU

MDDA participated in an educational and social support event held at the Royal Melbourne Hospital Metabolic Diseases Unit meeting with health professionals and patients to talk about the managing their genetic condition while pregnant. It is fundamentally important for women with PKU to adhere to a strict low protein diet due to the high risk of foetal abnormalities.

Patient Outreach Clinics

MDDA continues to liaise with the Victorian metabolic clinics to progress the option of running an Outreach Clinic to IEM individuals living in Tasmania. Currently people from Tasmania attend the Victorian paediatric/adult metabolic clinic yearly or as often as required. An outreach clinic would essentially feature both the health professionals and the MDDA facilitating services to the Tasmanian IEM population.

The co-ordination of MDDA volunteers to attend the remote regional metabolic clinics held yearly in Townsville and Cairns took place once again in April. This service has been requested by the metabolic health practitioners who value the support and social inclusion our volunteers bring to the clinic. In conjunction with the clinic the MDDA jointly hosted a dinner for the IEM community to share experiences. This is usually the only time these IEM individuals living in remote rural areas meet with another person during the year living with the same condition.

Adult Clinical Resources

Resourcing of Adult metabolic clinics continues to be an issue. MDDA are continuing to address with ASIEM and look for further ways we can provide health and wellbeing support to our adult members who without adequate treatment and access to healthcare services, are at a high risk of neurocognitive, mental health and nutritional problems. We have begun discussions with ASIEM and specific clinicians about possible strategies to address a skills/experience gap in this area.

Providing patient centred education and recognition to the health sector

MDDA work with all stakeholders to promote IEM patient centred care and education. Patient care should be respectful and responsive to individual patients' preferences, needs and values. There is evidence that patient centred care has a positive impact, increased adherence to management and improved quality of life for patients.

Newborn Screening Education

Victorian Clinical Directors & Nurse/Midwifes

MDDA presented at four best practice forums in Victoria for newborn bloodspot screening to an audience of public hospital nurse/midwives and clinical directors facilitated by the Dept. of Health Victoria. The presentations involved the patient perspective of diagnosis, the importance of information provided to the family at the time of the blood sample collection and the importance of timeliness in averting adverse outcomes among newborns to do the bloodspot screening in a timely manner.

Department of Health Victoria and the Victorian Clinical Genetic Services (VCGS)

MDDA featured in an educational video on Newborn Screening on the patient perspective of diagnosis and blood spot testing in the home environment. This resource has currently been endorsed for use as an educational tool by the Dept. of Health Victoria and VCGS.

Human Genetics Society of Australasia

MDDA presented at the Human Genetics Society of Australasia annual conference in August on "A Patient Perspective on Newborn Screening" and then provided a patient perspective in a facilitated discussion including clinicians, newborn screening scientific staff and midwives on issues associated with newborn screening including: should NBS be mandatory, giving a NBS diagnosis, the role of preconception and ante-natal screening and the future of screening.

Laboratory Staff

A special moment was shared on Rare Disease Day in February this year with the laboratory staff at the Royal Children's Hospital in Victoria's, Newborn Screening Lab. MDDA facilitated a patient tour and afternoon tea in honour of Rare Disease Day and recognition of the importance work performed behind the scenes by the lab team.

An IEM Patient Centric Approach

IEM dietitians and Health Professionals

MDDA provided educational sessions to metabolic health professionals to assist them with a better understanding and perspective of members living with an IEM. We also provided discussion around best ways to implement a patient centred approach and improved patient health outcomes.

Genetic Counselling Students

As part of our commitment to training and supporting health care workers MDDA hosted two first year Master of Genetic Counselling students from the University of Melbourne for a community-based placement. This placement enabled the training counsellors to understand the complexities of living with a genetic condition and provided the students with an understanding of the vital role that MDDA provide as a community-based support and service organisation that delivers a continuum of care to people outside of the formal clinical setting and provide many of the psychosocial needs of people.

Collaborating with purpose

Rare Voices Australia (RVA)

MDDA attended Rare Voices Australia Rare Disease Summit in November. This summit brought together stakeholders from government, research, clinical, industry and patient groups to discuss best practice and the way forward for rare diseases in Australia.

We participated in working parties on: Supporting Rare Disease Clinical Workforce, Rare Disease Registries, What Makes an Effective Rare Disease Patient Group as well as attending presentations on the PBAC process, the Department of Health's view on access to therapies and an update on the APEC Rare Disease strategies.

MDDA continue to collaborate with Rare Voices Australia on principals and objectives to progress a "National Plan for Rare Diseases".

Genetic Service Network of Victoria (GSNV)

MDDA attended "Bridging Health and Social Care" forum at the Murdoch Children's Research Institute facilitated by Genetic Support Network of Victoria in February.

This forum brought together stakeholders from government, research, clinical, industry and patient groups to discuss the gap between medical services and social and support services. Highlighting the impact across the sector from mental wellness to funding from support services. MDDA participated in working parties on key areas of: Ageing, Transition, NDIS, Mental Wellness, Familial impact and Navigating the Health System.

Global Association for PKU (GAP)

Globally, two thirds of all newborns have no access to screening. Statistically, every hour one unidentified PKU child is born. This means more than 700 PKU patients every month, nearly 9000 every year, all left to the fate of a life with severe mental retardation and physical handicap, that could be prevented by a simple blood spot on a piece of filter paper.

Associations from all over the world came together to form GAP in July 2018, to advance PKU as a global health priority, promote universal access to newborn screening, diagnosis, treatment, care and a cure. Monique Cooper (MDDA President) and Louise Healy (MDDA Vice President) are founding members of GAP. Both Monique and Louise work tirelessly to raise awareness nationally and globally for PKU.

Hotels Industry

The Grand Hotel Warrandyte VIC, is the first establishment in Australia to offer specially designed low protein menu options on their everyday menu. The MDDA appreciate The Grand taking on the task of understanding and catering to the needs of our PKU/IEM community.

The Grand are wonderful supporters who also provide in-kind support and cooking resources for our retreats, host our local events and are always helping out with fundraising and raising awareness.

With the popularity and keen interest on The Grand's menu selection we decided to take the idea nationally, forming Low Pro Eats, the new MDDA foodies program. Designed to provide greater access and choices to people who enjoy hassle free and inclusive low protein dining out experiences. Venues will be recognised nationally as a Low Pro Eats establishments and promoted through our communication platforms.

The Great Protein Challenge continued for the second year on the 1st February and concluded on the 28th February, Rare Disease Day. Through this challenge we together raised over $18,000. Encouraging Australians to eat under 10 grams of protein for one day in the aim of raising awareness and funds in support of people living with a protein Inborn Error of Metabolism.

This year we initiated the dietitians challenge. Thank you to the dietitians and clinicians who participated. We are so grateful for all the amazing efforts of every participant. We have been touched by inspirational and heart warming stories by those that did the challenge as the understanding of rare Inborn Errors of Metabolism continues to grow. We have shown that we are not alone. While we may be small in numbers (we are rare after all), we all have a huge heart and an enormously welcoming community.

The year at a glance

July to September

- Announcement of the Global Association for Phenylketonuria (GAP). Monique Cooper & Louise Healy listed as founding trustees
- Executive Committee members attend NPKUA conference in Atlanta
- 2 graduate students placed with MDDA for work experience through GSNV/Melbourne University
- MDDA welcome volunteer Marta Rachman from Safe Brazil (a support group for PKU Brazil) working with MDDA on special projects
- MDDA presents at Human Genetics Society of Australasia (HGSA) Conference
- SA Family Retreat held at Adelaide Hills Convention Centre
- Member "Blackmores" fun run – raised over $1400

October to December

- Tasmania fundraising event, Caulfield Cup –raised over $4000
- Nicholla Kinscher's Tuppaware fundraiser - NSW
- Member awareness campaign Tasmania ABC media
- End of Year state based celebrations
- MDDA President presents educational talk to Barwon Health midwives
- Listing of sapropterin on the Pharmaceutical Benefits Scheme (PBS) for children and adolescents under 18 years of age (with continued access into adulthood) living with phenylketonuria (PKU).

January to March

- The Great Protein Challenge – raised over $18,000
- Rare Disease Day celebrations – Feb 28
- Newborn Screening and Lab tour at RCH and Queensland Children's Hospital
- Ben runs for PKU - fundraiser
- Great Low Protein Feast held at the Grand Hotel Warrandyte
- In collaboration RVA and MDDA hold parliamentary lunch
- Awareness Day presentation at Community ladies day - VIC

April to June

- WA Family Retreat
- Far North Queensland Clinic
- Member commences 6 weeks weight loss challenge fundraiser
- Webinar held for Adults on Sapropterin
- International PKU/IEM awareness month
- Ben's 2nd run for PKU half Marathon
- Kaitlyn Greer's school fundraiser - WA
- TV Awareness of PBS hope for People with PKU
- Midwife presentation & education
- PKU Mum & Bubs morning tea held at Royal Melbourne Hospital

Financials

MDDA has experienced another financially successful year. Thank you to all our supporters, none of our work would be possible without you, we sincerely thank you for your generosity. Every cent of the income from donations and fundraising go directly to the IEM community through education programs and support.

30 June 2019

Income	
Grants	$229,525
Sponsorships	$162,715
Donations	$18,689
Fundraising	$23,508
Events	$18,488
Other Revenue	$12,464
Total Income	**$465,389**
Expenses	
Operating Costs	$76,360
Secretariat Expenses	$144,126
Member Support	$147,230
Event/Fundraising	$21,382
Total Expenses	**$389,098**
Profit	**$76,291**
Donation and Fundraising contributions to Research fund	$20,000

* Audited Financials available

Thank you to the following #TeamMDDA member driven fundraising initiatives...

- Bianca Albanese: Fun Run (NSW)
- Ben Van Tiggelen: Fun Run x 2 (VIC)
- Senada Suljagic: Birthday Fundraiser (VIC)
- Kaitlyn Greer: School Fundraiser (WA)
- Nicholla Kinscher: Tuppaware Fundraiser (NSW)
- Tracey Scott: Entertainment Books (VIC)
- Stephanie Hutchins: Weight Loss Challenge (QLD)
- Lauren & Justin Penneyston: Caulfield Cup Fundraiser (TAS)
- The Grand Hotel Warrandyte: Raffle (VIC)

#team MDDA

Become a #teamMDDA supporter and raise funds for MDDA as your chosen charity through participating in an activity of your choice. Raise awareness and funds for MDDA whilst doing something you love.

Looking to sponsor a program, initiative or event? MDDA has a number of programs and events looking for financial support. Examples include:

- Low Pro Pop Up Kitchen & Hamper Drive
- IEM Family Assist - retreats & remote clinics
- My Low Pro PAL
- PKU Pathways - outreach support, mentoring
- National PKU & IEM Research & Clinical Care Fund

Make a donation

Donations made to MDDA go towards a number of different initiatives or research projects.

MDDA is seeking funding to support a number of research initiatives in the area of progressing knowledge and potential new treatments/cures for PKU and other IEMs.

Volunteer

Join MDDA and become involved with the committee or project team and be a part of making a difference for all individuals with IEMs whilst making new friends.

Host a Fundraising Event

Hosting a fundraising event can be a fun and rewarding experience and provides a good mechanism for raising awareness and money for IEMs in our community.

Acknowledgements

Sponsorship

Thank you to all of our sponsors for supporting and sharing our vision that all individuals living with an IEM are leading a life at full potential, not limited by choice. We appreciate the support and generosity of our partners and sponsors, without them we would not be able to deliver the current level of programs and resources we offer. Our National Family Retreats and other projects would not be possible without their generous support and contributions. Thank you for your continued collaboration and commitment in supporting the IEM community.

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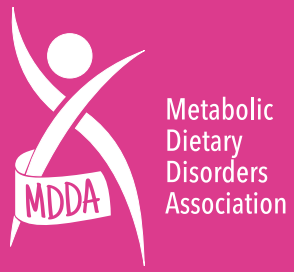

Metabolic Dietary Disorders Association Inc.
Suite 4, 6 Thomas Brew Lane Croydon VIC 3136
PO Box 33 Montrose VIC 3765

Phone 03 9723 0600 | **Freecall** 1800 288 460 | office@mdda.org.au | www.mdda.org.au

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