

2021

Annual Report



Metabolic
Dietary
Disorders
Association

Reflections

I am pleased to present the 2020–21 Annual Report for Metabolic Dietary Disorders Association (MDDA).

The past year has shown that the MDDA & IEM community are adaptable, flexible and resilient. While the COVID-19 pandemic effected every aspect of our organisation for yet another year we continued to provide leadership and support as the national peak body in the areas of advocacy, education, connections and providing a voice for the Australian IEM community.

Notwithstanding lockdowns and restrictions, we turned our focus to embrace the virtual online world to keep engaged with our members and stakeholders. We reimaged the National Conference to a 2-day virtual event, enabling over 200 members to participate. We introduced our Member Connect Hub, a virtual interactive platform bringing members together online. These new initiatives have brought our community together, enabling members to connect and share with their peers, especially important in the current pandemic where many people are feeling isolated. We are learning as we go, supporting one another as we navigate new ways of connecting, supporting, and educating.

I would like to take this opportunity to thank everyone who has contributed in any way this year. The MDDA relies on the support and dedication from our members, volunteers, stakeholders, friends and families to further our support programs, educational programs and events.

Fundraising and donations are an important source of income for us and the pandemic made this a difficult year. To the wonderful members who took on #teammdda challenges and fundraisers – THANK YOU! We appreciate the huge effort and amount of work you did in this challenging year.

Thank you to the many health professionals who willingly and generously supported our MDDA online events this year. We appreciate your time especially during this year where demands on the health system have been enormous. To our generous sponsors and partners, without your funding and sponsorship we wouldn't be able to offer the level of service, support and programs to the IEM community. Thank you BioMarin, Menarini, Nutricia & Vitaflo. We look forward to working with you in the future.

To the MDDA staff and committee, it is through all your hard work and dedication that we strive towards achieving our goal.

The upcoming year will be a busy one for MDDA. Hopefully we will see the re-emergence of in-person events – stay tuned for some exciting announcements via our website. We have several programs and creative initiatives in the pipeline in addition to our business-as-usual work. We will continue to engage closely with the Department of Health and other stakeholders to progress the needs of the IEM community to access the best available treatments and equitable healthcare for all.

I look forward to 2022 and continuing to build and work with, and for, the community of people in Australia with an IEM.



A handwritten signature in black ink, appearing to read 'Monique Cooper'.

Monique Cooper
MDDA, President,
PKU Mum

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

IEM FAMILIES	 32 New Members	 668 Email Subscribers	 1000+ Online patient forum participants
	 3600+ Voluntary Hours	 3120 support emails & phone calls	 3 5 Printed Newsletters eNews
	 2 Member Surveys	 1st National Virtual Retreat – over 200 attendees	 13 PeeKabU bags delivered
GOVERNMENT	 5 Reviews	 40 Resources & materials developed and distributed	
	 4 Advisory responses provided	 120 Requests for information to the community on health policy matters	
HEALTH PROFESSIONALS	 115 Medical practitioners attending MDDA education and training sessions		 21 Group meetings attended
GENERAL PUBLIC	 2.6K Facebook Page followers	 5 #teamMDDA fundraising initiatives	 8 Active FB groups
	322 Instagram Followers	1045+ Members	 29 YouTube Video subscriber and 246 average views

The Power of Shared Experiences

Over the past 12 months members have shared their IEM journeys with those in our community through our MDDA Matters newsletters. These heartfelt, inspiring, stories have helped many realise they are not alone in the day to day challenges they face.

We have heard from individuals and families living with Maple Syrup Urine Disease, PKU teens, kids & bubs, PKU mum and her PKU bub, Tyrosinaemia Type 1 teen, a mother's journey with her toddler and Isovaleric Acidemia, and an Adult PKU story.



Amanda & Tom, Stella, Daisy & Charlie
MSUD



Phoenix & Lee-Anne
PKU Teen



Natalie, Tom, Maddy & Tessa
Tyrosinaemia Type 1 Tween



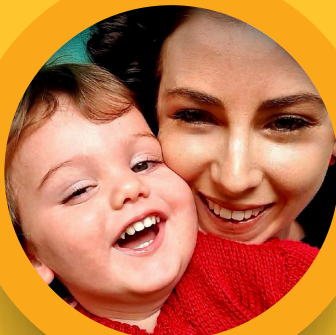
Ben & Morgan, PKU Adult
Annabelle, PKU Baby



Senada, Kenan, Ilyas & Naaila
PKU Child



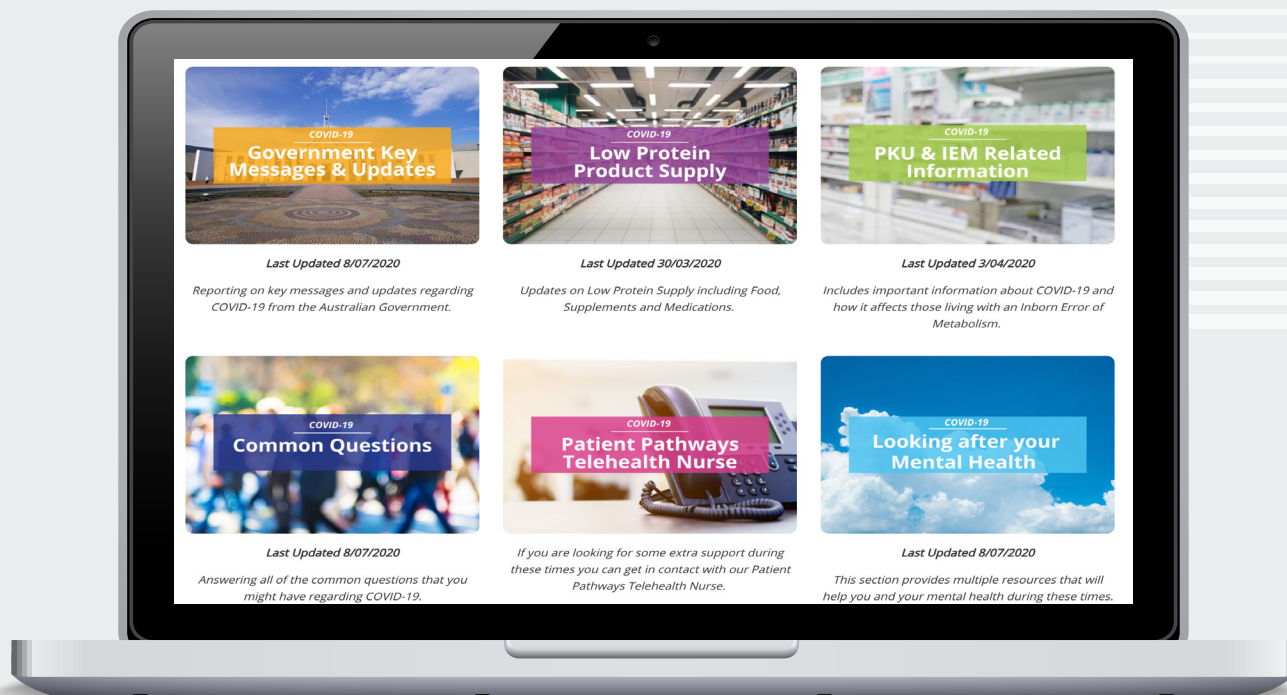
Theresa-Alena
PKU Adult



Shannon & Victor Isovaleric
Acidaemia Child



Olivia
PKU Child



IEMs and COVID-19

Throughout 2020/2021 MDDA experienced the increase in need for services due to the current pandemic. The increase in support and service calls were commonly surrounding the following concerns:

Concerns during uncertain times included

- Continued access to medicines and specialised medical foods
- Logistics related to the new medication dispensing monthly guidelines
- Fear of interrupted imported medicine supplies
- Concerns of supply interruptions to medicines on the Life Savings Drugs Program
- Concerns surrounding vaccination
- Members anxious about the hospital setting, concerned about the risk of acquiring COVID-19
- Anxieties and apprehension of the introduction of telehealth metabolic appointments
- Maintaining access to the Inborn Error of Metabolism Programme
- Members concerned and feeling vulnerable to COVID-19 due to compromised immunity
- Increased stress for some clients in acquiring fresh produce which is a significant component of the low protein diet

MDDA updated our online coronavirus resource to

provide information that is most vital and relevant to IEM patients, families and carers due to the pandemic. The online information includes key messages and updates from Government and the Health Department, including information from specific national metabolic clinics, health professionals and specialist low protein medical food suppliers. Other resources are included with links to common questions, MDDA Pathways telehealth nurse, mental health and other relevant information and services, and how it affects those with an IEM.

MDDA provide direct links to the Department of Health Fact Sheets - COVID-19 National Health Plan resources, the Department of Social Services for referrals and information for people with disability via the Disability Gateway funded by the Australian Government.

We continue to encourage people to keep updated and aware of current developments and notifications from the Federal Government and their local State Government.

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.

Department of Health

MDDA has continued to actively pursue all possible avenues for having pharmacological treatments for PKU subsidised for all Australians. MDDA are pleased to be able to have provided submissions, position statements and other expertise and knowledge to specific government consultation processes to advance IEM care. These included;

- Submission to the Parliamentary Inquiry into approval processes for new medicines and therapies. Our submission highlighted the long wait Australian IEM patients have had to access therapies available overseas, the need to attract clinical trials for the rarer non-PKU IEMs to Australia, the need to invest in early stage research for the very rare IEMs to improve outcomes for rare IEM patients, the need for Australian medicine approval and funding processes to be fit-for purpose to evaluate emerging gene therapies – an important issue for our population with so many potential therapies currently being developed; and the need for approval processes to address the complexity of developing and measuring treatments for rare diseases
- Submission to the review of the Medical Services Advisory Committee (MSAC) guidelines. Our submission highlighted the need for patient involvement and input into submissions and decision making and that patient reported outcome measures need to be considered when evaluating gene therapies, especially if they are for very small patient populations, as is the case for all protein IEMS
- Submission to the Commonwealth's Government Preventative Health Strategy consultation. This submission highlighted the need for Newborn Screening to expand to diagnosis of rare (nonPKU) IEMs at newborn stage; to identify patients with rare lifelong conditions as a priority preventative health population in order to ensure that they have adequate support, including mental health and other clinical support, to help manage their condition throughout life
- Submission to the PBAC for the use of Sapropterin (Kuvan) by maternal women with PKU. While maternal women present a special case due to the need to protect the health of the unborn baby, we are concerned that the application did not address the current inequitable situation for all adult patients in Australia

After many years of advocacy for access to all available treatments it was announced in January 21 that Kuvan (sapropterin dihydrochloride) was recommended by the Pharmaceutical Benefits Advisory Committee (PBAC) for treatment for maternal women living with PKU in Australia. Whilst the outcome is a positive one for maternal women, we are pleased that the PBAC have noted the high clinical need for access to all adults with PKU. MDDA will continue to campaign for access to all safe and approved treatments.

In April 21 MDDA were invited to appear as a witness at a public hearing for The House of Representatives Standing Committee for Health, Aged Care and Sport hearing as part of its Inquiry into the Approval Processes for New Drugs and Novel Medical Technologies in Australia. MDDA had provided a written submission to the Standing Committee. MDDA were asked questions based on our submission with a particular focus on the treatment of rare diseases and conditions where there is high and unmet clinical need. Our Vice President spoke about the long wait for access to new therapies our community experience and the challenges of getting clinical trials for rare IEMs to Australia and the need for reform to resolve these issues. A full transcript of the evidence presented by the VP can be found here: <https://bit.ly/3zxkBLw>



Life Savings Drug Program (LSDP)

MDDA provided a submission and comment to the final Life Savings Drug Program report for Nitisinone the only treatment therapy for the ultra-rare disease Hereditary Tyrosinaemia Type 1 (HT-1). Within the report we collated and incorporated HT-1 family's comments by the request of the Life Saving Drugs Program Expert Panel Secretariat. Comments incorporated into the response included the administration and dispensing of the medicine, acknowledging the LSDP report that the families focus on survival makes issues around side effects less important from a patient perspective, Australia has no capacity to assess plasma succinylacetone levels which may not align with international guidelines and MDDA's position that HT-1 should be added to Newborn Screening Panels inline with other international jurisdictions.

Government Grants

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

Health Peak & Advisory Bodies Programme Grant

MDDA receives funding from the Australian Government to enable MDDA to play our part in informing the Government's health agenda through impartial advice in turn contributing to improved health outcomes for all Australians and their families living with an inborn error of metabolism. This includes consulting and sharing information with our members, the wider health sector, the community and the Government, providing sector knowledge and expertise and providing education and training to health practitioners to improve the quality of health services.

National Disability Conference Initiative (NDCI)

MDDA received funding from the Australian Government under the NDCI. This grant enables MDDA to maximise the inclusion and participation of members and/or their carers to participate in our nationally focused, disability-related, conference held in Australia.

Patient Pathways Program

MDDA are in the second year of funding for the Telehealth Nurse Patient Pathways pilot program funded by the Australian Government co-ordinated by the International Centre for Community – Driven Research. This grant enables MDDA to extend our services to deliver support to patients and connect them to specialist care, support services and opportunities to participate in clinical trials.



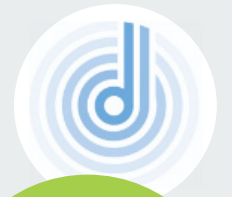
Advancing Care Patient Pathways Program

MDDA's Patient Pathways Program is in its second year continuing to provide an elevated level of patient outreach support through provision of useful resources and information to patients and families specifically targeted to suit and support their current transitional stage of their IEM journey. The Pathways Program provides support, mentoring, educational programs and resources for individuals and families throughout every life stage.

The Program provides a pathway for support for patients to access a coordinated comprehensive case management service, to talk about their experience and collect a repository of patient experience data that can inform future research, treatment, care, information, and support across the health sector.

The program provides support to the community in the following areas:

- Assistance with navigating the healthcare system, accessing services and clinical trials.
- Education and information provision.
- Guidance to develop your ability and confidence to proactively manage their condition.
- Social and emotional support for members and/or their families.
- Support of patient care with external healthcare organisations and facilities.
- Support for ongoing patient care with families and carers following hospital admission, discharge, and accident and/or emergency visits.
- Reconnecting adults to clinic and supporting their Return to care and diet.



Patient Pathways Nurse Supporting MDDA Members - Jo

My role is to provide a central point of communication and engagement to ensure optimal care and coordination of services along a patient's entire health care journey. I am available to assist members to put in action the care plan they have received from their metabolic team and general practitioner.

Each week the I speak with a number of new and existing members. Part of this process is outlining to patients how MDDA can support them living with an IEM and help to direct them to answers to their questions. Some of the common areas include, finding about access to services that they may be eligible for, informing members of different health avenues, assisting members to prepare for upcoming GP and medical specialists' consultations.

- I am available to educate and help patients to better understand their health conditions and enable them to self-manage, participate in decisions about their health care (eg, clinical trials) and improve their own health outcomes.
- I enjoy working with the MDDA team and providing input to our varied programs and resources eg Zest, education resources material and online, including collaborating with the multiple MDDA stakeholders.
- I am passionate about educating and supporting members throughout their challenges and I have been able to work with so many delightful MDDA families and individuals.

The Patient Pathways Program is federally funded by the Australian Department of Health. MDDA are working with the Centre for Community-Driven Research (CCDR) to implement this program.

Number of patients who accessed Pathways nurse

127 Patients

Number of Pathways Nurse led referrals/interventions provided

945

'I have been on a merry go round for the past 19 months. Finally, my doctor ordered a muscle and nerve biopsy. From there he called to say that I have a metabolic disorder. I thought fantastic, they finally know what's wrong with me. Oh how wrong I was. The merry-go-round started again! I did my own investigation and found MDDA, and I requested to join. Then I found my bright star Jo. She directed me to contact the Metabolic Clinic at the Royal Melbourne Hospital via my GP referral. Even though I've had to wait a few months for an appointment, I now have hope I have found the right place, that can help me manage this disorder and get some independence back. Jo was also there to hear me vent many, many times. She kept me going to be honest'

- Sue

MDDA Embracing the Virtual World

MDDA made the difficult decision to cancel all in-person member events scheduled for 2021. We considered members' health, the potential impact and effect that unpredictable state border closures and the resulting imposed restrictions may have on all those attending. It is our hope that in 2022, when there is more certainty, we can resume our in-person events. Whilst we could never replace an in-person event, by using innovative technology, we did feel like we participated in truly unique and invaluable experiences!

Virtual @Home Conference - October 2020



When our 2020 in person National Family Conference was cancelled for a second year, we replaced it with Australia's first ever 2 day Virtual MDDA Conference. The virtual conference took place over the 10-11 October 2020 with more than 40 sessions broadcasted live. Over 200 members participated in the event. The program offered national and international educational and scientific sessions, panel discussions, virtual exhibitions, interactive workshops and networking opportunities. We were able to reimagine the National Family Conference bringing the IEM community together to reconnect and share experiences which were especially valuable in this current difficult time.



"Fortunately, myself with a couple of other PKU teens were given the opportunity to help out at this year's virtual retreat by assisting with the kids club and answering any questions that either other kids with PKU had or parents. It also enabled the younger kids to meet other kids their own age as well as realise that they aren't alone."

Frances – PKU Teen

"Despite the challenges of COVID-19, there is always a way for our community to help each other, share knowledge and provide support to the newer members. Thank you again to all at the MDDA who make such a positive difference to anyone who either directly or indirectly is impacted with an IEM."

Louise – mum of PKU Child



Interactive Workshops were focused on health & wellbeing including;

- Zest Adult Wellness session
- Teens Transitional Workshop focusing on key components of transitional care for IEM teens and their parents/ guardians
- Grandparents Discussion Forum providing an opportunity to share experiences, ask questions and support one another
- PKU Adult Advocacy Workshop discussing tools to use for effective advocacy, setting clear aims and objectives
- New Parents Discussion Forum Support giving those of newly diagnosed infants an opportunity to share their story and connect
- Peekabu Kids Club & Teens Mentoring Session
- Q&A panels
- Rare IEMS Advocacy Panel
- PKU Adults Panel
- IEM Kids & Teens Panel Member Hub to connect online meet new people and build upon peer support networks
- Low Pro Showcase & Low Pro Cooking Masterclasses & Demonstrations

"A massive thank you is in order to all the hard working team behind the retreat, that was able to put on such an amazing event in the midst of the 2020 chaos. Each year's retreat is always such a highly anticipated event in the IEM community, and I think I speak on behalf of everyone in saying we were all so grateful it could go ahead."

Bianca PKU Adult



Mums & Bubs - Maternal PKU Virtual Connect Forum

MDDA, in collaboration with the Royal Melbourne Hospital held a national online event in June 2021 bringing together PKU women who are new or expecting mothers, or those thinking about family planning. The maternal PKU women's journey to motherhood is different to other maternal women. The event provided an opportunity for women to connect, share experiences and hear from metabolic specialists from state metabolic clinics.

MEMBER CONNECT HUB

A welcome place for the IEM community to connect, share and learn

CONNECT →

Launch of Member Connect Hub

How do you keep connections and engagement with your community during a pandemic?

Easy – create the MEMBER CONNECT HUB.

The Hub is an inclusive and interactive virtual platform that is extended to all MDDA members no matter of location as a place to connect and meet with other members, gather in special interest groups and educational forums and attend virtual events and workshops. The Hub provides ability for new and existing members to find people with common interests and conditions and create peer support networks of their own. The Member Connect Lounge is open 24/7 and provides an interactive, easy to use platform where members can attend educational webinar style presentations, attend group chats, 1on1 panel style interviews with audience interaction and join peer support groups regularly online in an informal setting. The LowPro Inspo Virtual Showcase is an interactive resource where members can find latest information of Low Pro foods, products, recipes, cooking demonstrations and low protein food presentations. MDDA updates take place monthly and provide an interactive forum where members can find out what's been happening at MDDA and ask any questions or raise any concerns. This platform also provides much needed connections within the community who are feeling isolated and vulnerable due to the pandemic.

Thank you to our Hub sponsors, Nutricia, Vitaflo and PTC Therapies for all their support.

Key Member Connect Hub Features

- Member Connect Lounge
- Low Pro Inspo Virtual Showcase
- Educational Connect Forums
- Spotlight Series
- Virtual Happy Hours
- Connect Groups
- Teen Workshops/Panels/ Connect Groups



LowPro Inspo

We held our LowPro Inspo Live cooking demonstration, where delicious bowls of Fried Rice were being cooked simultaneously around the country.



PKU Carer's Compendium Launch

Tuesday 11th August 1:30pm AEST



Virtual launch of the PKU Carers Compendium with the Royal Melbourne Hospital - Aug 2020

The event was attended by more than 70 carers representing 37 patients living in supported care. Originally planned to be launched in Victoria and rolled out to other states, using our virtual platform we were able to launch the program and compendium nationally on one day.



All proceeds from the sale of items on the store go towards supporting further MDDA member programs and initiatives.



MDDA Advantage members also gain access to special member offers and discounts.

shop.mdda.org.au

LowPro Inspo is our Low Protein YouTube channel that is designed to provide daily inspiration for people living with protein IEM's. The channel initially focuses on low protein cooking and recipes but will expand to also include other inspirational content from MDDA, our members and affiliated communities.

Whether you're a cooking fanatic, or a novice, there is something here for everyone. From cookies to noodle salad to bread, learn new recipes and new tips to cook something deliciously low pro!

LowPro Inspo is open to everyone, why not join up to our Member Advantage program and also become an inspirator, contributing to our inspiring and growing library of content.

Visit LowPro Inspo on YouTube and click subscribe.



MDDA Marketplace is the hub for all IEM products, merchandise and gifts. Whether you want to don a PeekabU hat, or purchase a compendium, all of our many great products are available online to purchase – with all proceeds going to the MDDA to help us continue to support our Member Advantage Programs.

MDDA CONNECT

MDDA Connect delivers communication and support services across multiple platforms and channels. When you first join our community, MDDA Connect is the way that we connect you in with other members so you can learn new information, stay updated and make lifelong friends. Whether you are a subscriber or a part of our Member Advantage Program, you can benefit from the various features and benefits of Member Connect.

MDDA Connect features include:

- MDDA Community and Member Events
- Patient Community Facebook Forum
- Website & Social Channels
- MDDAs 1800 Number
- MDDA Newsletters



THE GREAT PROTEIN CHALLENGE AUSTRALIA

thegreatproteinchallenge.com.au

The Great Protein Challenge (now a global awareness campaign) is a public health educational awareness and fundraising initiative that helps support kids and adults living with IEMs. It enables people to walk in the shoes of a person living with an IEM for a day to understand the importance, complexity and difficulty of managing their condition.

By being a part of The Great Protein Challenge, you will help raise awareness for all protein IEMs and provide an informative and fun way to engage in the conversation.

Funds raised through the challenge will go straight into continuing to support the community through building further programs and contributing to MDDA's Research foundation.

HOW #LOWPRO CAN YOU GO?



Thank you to our sponsor

BOMARIN

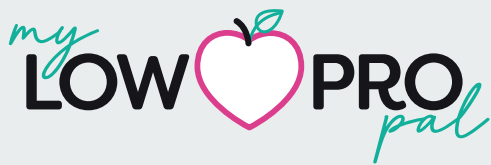


#teamMDDA is a way for you and your family and friends to raise funds in support of the MDDA. You can do so by selecting MDDA as your chosen charity as you participate in an activity of your choice and raise awareness and funds for MDDA whilst doing something you love.

Funds raised via #teamMDDA go straight towards providing greater resources and value back into the MDDA community and research priorities.

By signing up to a #teamMDDA challenge you are joining a group of remarkable individuals who are prepared to go above and beyond to achieve something for a great cause. If doing a fun run, marathon or other outdoor challenge, #teamMDDA running singlets & Tshirts can be ordered (and personalised) via MDDA Marketplace.

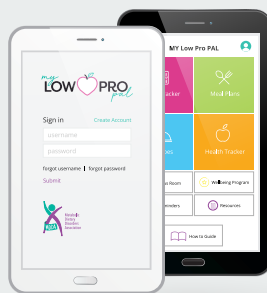
visit www.mdda.org.au/help/teammdda and join the team



To make life a little easier for those managing an IEM, whether it is assisting in protein counting, planning your next meal or helping you to create your next shopping list. **MyLowProPal** is here to help you and motivate you to stay on track and provide a little bit more organisation and wholistic healthiness into your life.

- > Protein counting (per Australian IEM guidelines)
- > Low protein recipes & meal plans (for all ages & protein allowances)
- > Low protein product catalogue
- > Shopping lists
- > Daily food tracker
- > Wellbeing goal setting & monitoring
- > Blood spot monitoring
- > Reminders

... Plus much more



Member Advantage

By joining MDDA's **Member Advantage Program**, you open the door to another level of support, with access to many specifically designed programs, products and services created to help support families and individuals navigate the pathways of managing their IEM – throughout all of the life stages.

Signing up to the MDDA Member Advantage Program is simple, and costs as little as \$35 per year* for a family subscription.

What's more, the first year is free for newly diagnosed families – as we understand more than anyone the importance of being a part of a caring and informed support network.

The MDDA **Patient Pathways Program** is an outreach support system that provides support, mentoring, educational programs and resources for individuals and families living with an IEM throughout every step of their journey. The Pathways Program includes different initiatives and programs that support those with an IEM through every stage of life - from first diagnosis to childhood to adulthood.

Parents of newly diagnosed babies, Food starters, Preschooler, School starters, Tweens, Transitioning adolescents, Adults, Maternal women and Carers of high needs (late diagnosed) PKU patients.

Within this program, MDDA provides a specialist tele-health nurse which provides patients with access to a coordinated comprehensive case management service, to talk about their experiences and collect a repository of patient experience data that can inform future research, treatment, care, information and support across the health sector.

If you would like to book a consultation with MDDA's Tele-health nurse, call 03 9723 0700 or email pathwaysnurse@mdda.org.au.

Visit www.mdda.org.au to find out more



ZONE • ENERGISE • SOAR • THRIVE

Zest is the MDDA IEM Adult Wellbeing and Vitality Program. Zest aims to improve the mental, emotional and physical health of Australian adults living with an IEM. Delivered via a combination of web-based seminars, teleconferences, online forums, weekly goal setting and one-on-one coaching. Zest makes habit-forming life changes in areas identified as limiting individuals from achieving optimal health and management of their IEM.

The initial program includes a 12-week coaching forum with 4 modules...

Zone	Energise	Soar	Thrive
Get into the Zone by focussing on healthy goals and habits.	Be Energised and ready to tackle challenges life throws at you.	Learn various tools and strategies that will help you to Soar.	Gain a support system and new habits to help you Thrive.

To find out more visit www.mdda.org.au/zest

Thank you to our sponsors



Introducing MDDA's Member Connect Hub.

A welcome place for the IEM Community to connect, share and learn.

The Member Connect Hub is an inclusive and interactive virtual platform that is extended to all MDDA members as a place to connect and meet with other members, gather in special interest groups and forums and attend virtual events and workshops. For more information visit mdda.org.au/hub.

Thank you to our sponsors



Achieving greater IEM patient health outcomes



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

We regularly communicate with state based metabolic clinics to facilitate discussions for education on improving metabolic healthcare services, strengthening evidence-based policy advice, improving program management, research, regulation and partnerships with other government agencies, consumers and stakeholders.

National Treatment and Clinical Best Practice Guidelines

MDDA remain in discussions with ASIAM for the consideration to develop consistent national treatment guidelines for other inborn errors of protein metabolism similar to those developed for PKU. In addition, we have requested ASIAM also consider developing clinical best practice guidelines. We continued discussions with ASIAM on the national guidelines for the testing protocol of BH4, trialling and commencing BH4 therapy for PKU children, PKU adolescents and Maternal PKU women. MDDA requested a statement from ASIAM regarding the COVID-19 vaccination efficacy and concerns to distribute to IEM patients.

Member Healthcare Surveys

MDDA conducted 2 member healthcare surveys providing unique information and research opportunities useful to MDDA to design and plan for the IEM community for improved advice to government, health information and education.

Treatment Options

MDDA continue to pursue availability and access to medications and treatments for all IEM patients. Our focus is to ensure that appropriate information is available to families about medications and treatments and to ensure we are in the best position to provide information to policy makers about potential treatment options for metabolic disorders.

Methylmalonic Aciduria (MMA) & Propionic Acidemia (PA) Clinical Trials

MDDA provided updated information to the overseas phase 2 clinical study for patients with methylmalonic acidemia (MMA) and propionic acidemia (PA). Currently there are no targeted pharmacological treatments for MMA or PA that can improve quality of life or extend lifespan. MDDA supported members and provided information to assist with questions on the participation of the trial and the uncertainties of the ongoing trial and the coronavirus impact on the trial site.

Clinical Trials

We are promoting Australia as a good destination for promising clinical trials. We advocate for compassionate access to treatments for those to participate in clinical trials that are effective with pharmaceutical companies currently in the process of trials. MDDA has worked with other companies considering clinical trials in Australia providing advice regarding regulatory requirements and assistance in establishing clinical relationships. We have provided our members with Australian Government clinical trials information to assist them in decision making regarding participation in clinical trials.

Providing patient centred education and resources

Newborn Diagnosis Support

Thirteen “PeeKabU” patient support bags have been distributed this year to newly diagnosed families. Coping with the diagnosis of an IEM can be emotional and worrying time, these bags provide a range of management tools and information including experiences from other families that have been through the process e.g. Dealing with and accepting the diagnosis, How do I learn about my babies IEM? What support is there? Infant feeding, blood sampling, family link outreach and mentoring opportunities etc.

Master of Genetic Counselling Students 2020

As part of our commitment to training and supporting health care workers in June 2020 MDDA began a 3-month host period for three first year Master of Genetic Counselling students from the University of Melbourne for community-based placement. This placement enables 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. Within this program MDDA presented to the 2021 class of first year Master of Genetic Counselling (over 20) students explaining the difficulties and complex treatment of an IEM diagnosis, also providing opportunity for questions.

“We were lucky enough to be given the opportunity to help out with the online retreat where we got to listen to so many of you! All your stories were so interesting and gave us a whole new perspective on what it is like to live with an IEM. It also helped us to see first-hand the importance of support groups and how special the MDDA community is.” Emma, George, Devni

Zest Adult Wellbeing and Vitality Program

MDDA is currently up to the fourth series of Zest, a purposefully designed and tailored IEM Adult Wellbeing and Vitality program. Research has shown us that more than 70% of adults with an IEM struggle to comply with their complex diet and in addition experience a range of serious co-morbidities including higher rates of anxiety and depression, nutrient deficits, sleep problems, higher rates of obesity and health problems associated with obesity. Zest makes habit-forming life changes in areas identified as limiting individuals from achieving optimal health and management of their IEM. The Zest program provides a holistic and tailored approach to these challenges by addressing emotional, physical and social wellbeing. The program is delivered via MDDA’s online virtual events platform via a combination of web-based seminars, teleconferences, online forums, weekly goal setting and one-on-one.

Outreach Far North Queensland Metabolic Clinic

MDDA volunteers attended the remote regional metabolic clinics held yearly in Townsville and Cairns. This service has been requested by the QLD 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 IEM individuals and families living in remote rural areas meet with another person during the year living with the same condition.

Pathways Compendiums

MDDA and the Royal Melbourne Hospital are continuing to work together on the Patient Pathways compendium series with the next instalment “Return to Care”. This compendium is designed for those who may have difficulty adhering to the diet recommendations due the extent of the dietary protein restriction, lack of access or acceptance of modified low protein foods, poor palatability of medical foods, and cost. We expect this resource to be finalised October/ November 2021.

Social Media Channels

We use a range of social media channels to provide opportunities for members to have input into MDDA direction, activities and support services. These social media channels also provide tools for keeping members informed of relevant issues and for promoting optimal health outcomes through a focus on treatment compliance and general wellbeing.

Late Diagnosed PKU Pathways Program

The Late Diagnosed PKU Pathways Program provides primary care support and education through an integrated stepped care approach. The program disseminates information compiled by the IEM community to a range of health services and providers to encourage best methods to implement a patient centred approach to educate and support late/undiagnosed PKU patients. We distributed a survey to primary care givers in community housing and nursing homes to collect data to evaluate how beneficial the compendium is and how we can improve the quality of information.

Online Resources

Resources are updated on our website for Care & Education to help with patient empowerment through education and information provision for health services and the wider community. Examples of online resources are, where to find information on medications, information on artificial sweeteners and which are safe for those with an IEM, information on taking blood samples and the COVID-19 advice resource.



July to September

- ✓ Hosted 3 Master of Genetic Counselling students from Melbourne Uni for community placement
- ✓ Member fundraiser - charity Soccer Day
- ✓ PKU Carers compendium launch with RMH - August
- ✓ Paige our MDDA Treasurer FB birthday fundraiser - July
- ✓ LowPro Inspo live cooking demonstration



October to December

- ✓ Virtual @Home Retreat (10-11 October)
- ✓ 3rd Series of Zest
- ✓ Submission to the Commonwealth's Government Preventative Health Strategy consultation



January to March

- ✓ Jan 29 announced Kuvan recommended by PBAC for treatment for maternal women living in Australia
- ✓ MDDA continue campaign for access for all safe and approved treatments and the needs of adults
- ✓ Submission made on behalf of IEM community, to Medical Services Advisory Committee regarding changes to their guidelines
- ✓ Rare Disease Day celebrations - Feb 29
- ✓ Welks Family Rare Disease Day fundraiser - BBQ breakfast
- ✓ MDDA attend first ever Virtual Rare Disease Day Parliamentary Event



April to June

- ✓ Outreach Far North Queensland Clinic 17-19 May
- ✓ VP Louise Healy witness at a parliamentary inquiry into approval processes for novel therapies hearing, 22 April
- ✓ Member Connect Hub launched 7 June
- ✓ Mums & Bubs Maternal PKU (MPKU) Virtual Connect Forum 8 June
- ✓ Member fundraiser - school free dress day 25 June
- ✓ Member workplace fundraiser for International PKU day - 28 June

Year at a glance

Collaborating with purpose

MDDA share ideas and inspiration, seeking to find solutions to specific challenges and shared goals that are innovative, practical and achievable. We collaborate with a broad range of stakeholders and likeminded organisations.

Rare Voices Australia (RVA)

MDDA continue to collaborate with Rare Voices Australia, our partner organisation and key stakeholder, on implementation of the National Strategic Action Plan for Rare Disease as it relates to the rare IEM community. In particular on equitable access to treatments, measures to encourage and support clinical trials for rare conditions to occur in Australia, providing input in a rare disease workforce project focussed on metabolic conditions and providing condition specific input in the develop of the multi-stakeholder digital rare disease portal funded by the Australian government.

MDDA participated in the Rare Voices Australia Virtual Rare Disease Day Parliamentary Event in March 2021. This event provided the opportunity to engage with stakeholders and parliamentarians about the ongoing implementation of the National Strategic Action Plan for Rare Diseases.



Global Association for PKU (GAP)

MDDA are planning with the Global Association of PKU's (GAP) inaugural Global PKU Patient Conference taking place in September 2021. The first of its kind, this event will bring the global PKU patient community together to celebrate achievements, learn about new developments and move one step closer to advancing PKU as a global health priority, accelerating new knowledge, treatments and a cure.



Royal Melbourne Hospital (RMH)

MDDA in collaboration with the Royal Melbourne Hospital launched the PKU Carer Compendium resource and program for missed or late diagnosed PKU adults living in shared community housing facilities or nursing homes. The virtual launch was held August 2020 with over 65 carer's, health care providers and industry supporters attending. The ongoing program will educate the carers on management of PKU on a day-to-day basis and carers will be able to contact MDDA with any PKU management queries. MDDA and the RMH are currently working on the next in the compendium series "Return to Care" for those who may have difficulty adhering to the diet recommendations due the extent of the dietary protein restriction, lack of access or acceptance of modified low protein foods, poor palatability of medical foods, and the associated costs.



Genetic Service Network of Victoria (GSNV)

We participate in a range of forums and programs led by the GSNV such as the program Fit for Practice placements for students from the Masters in Genetic Counselling and the Rare Disease Day, Who's on Your Team workshop and other workshops. The support and partnership from the GSNV assists MDDA to increase our capacity to support our members.



Community Driven Research (CCDR)

MDDA are working with CCDR in an integrated approach with the aim to help IEM patients navigate the health system and access all services that are available to them, including clinical trials. The program coming to the end of the second year trial has enabled a specialist tele-health nurse in our organisation to provide a support pathway for patients to access a coordinated comprehensive case management service. The telehealth nurse is available to talk to patients about their chronic health experience and collect a repository of patient experience data that can inform future research, treatment, care, information and support across the health sector. The tele-health nurse role has had an immediate impact assisting in the health literacy and collection of quality data about health and healthcare aimed at managing IEM patients with chronic conditions.



Financials

Summary of financial results

30 June 2021

Income 2021	
Conference	\$0
Donations	\$12,311
Event Sales	\$21,000
Fundraising	\$0
Grants	\$179,000
Marketplace	\$197
Subscriptions	\$2,447
Sponsorship	\$86,585
COVID-19	\$86,000
Other	\$32,351
Total Income	\$419,891
Other Revenue & Subsidies	
* MDDA are grateful recipients of COVID -19 Government subsidies	
Expenses 2021	
Operating Costs	\$80,480
Secretariat Expenses	\$221,618
Member Support	\$10,868
Other	\$5,610
Total Expenses	\$318,576

Although our statements show a profit this can be attributed to delayed sponsor income and project work yet to be invoiced. A complete financial report prepared by our accountants Paul Money Partners is available on request.

Thank you to every member who renews their yearly Membership. This is one of the most important contributions you can make to the MDDA. Thank you to every person who had made a donation. 100% of every dollar from a donation is put back into member programs.

Thank you to the following #teamMDDA member driven fundraising initiatives;

- ✓ Chelsea Docherty
- ✓ Leahy Family
- ✓ Dominique Cottee
- ✓ Welk Family
- ✓ Paige Moore



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. Donations over \$2 are fully tax deductible



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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Monique Cooper,
PKU Parent VIC

Vice-President

Louise Healy,
PKU Parent QLD

Treasurer

Paige Moore,
PKU Parent FNQLD

Secretary

Bianca Albanese,
PKU Adult NSW

Health & Wellbeing

Susi Hendricks,
PKU Parent VIC

Executive Officer

Jenny Briant

Administration Assistant

Kerry Tulloch

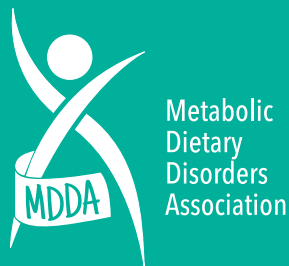
Communications and Marketing

Bella Hendricks

Patient Pathways

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