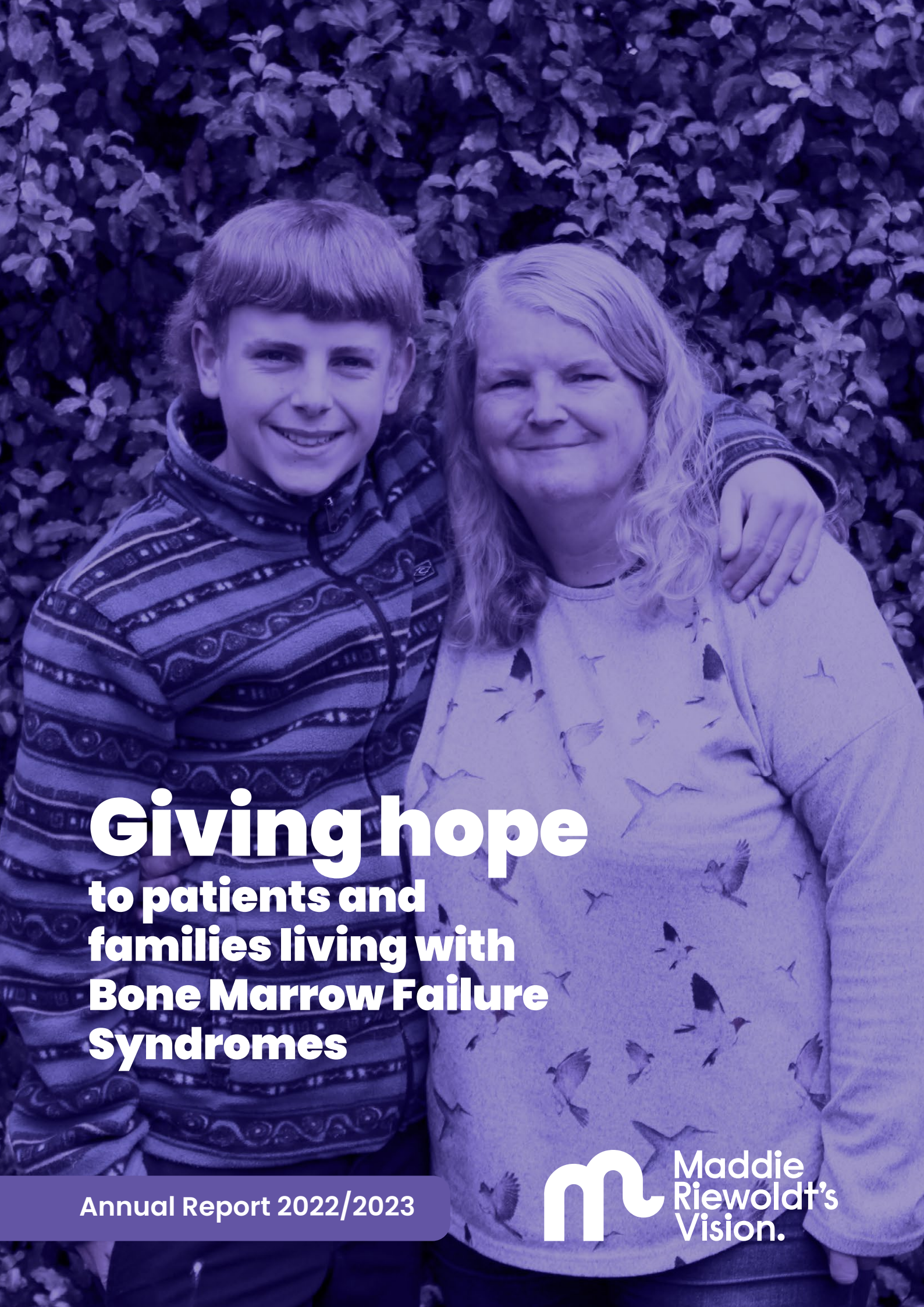


Giving hope

to patients and families living with Bone Marrow Failure Syndromes

Annual Report 2022/2023

Maddie Riewoldt's Vision.

Our vision

Bone Marrow Failure Syndromes are prevented, diagnosed and treated with the best available medical treatments in Australia and patients and families receive the support that they need.

Our mission

Maddie Riewoldt's Vision funds research that accelerates next generation prevention, diagnosis and treatment of Bone Marrow Failure Syndromes while supporting patients and families.

Strategic priorities

Priority 1:
Funding and operation

Continue to build a sustainable funding and operational base.

Priority 2:
Research

Develop and deliver a national research strategy, overseen by the independent Scientific Advisory Committee and supported by the Centre of Research Excellence in Bone Marrow Biology. Fund the most promising bone marrow failure research aligned to our Five Pillars of Research.

Priority 3:
Partnerships

Develop and nurture valuable, long-term partnerships with business, philanthropy, government and communities.

Priority 4:
Reputation and profile

Build a credible and professional national profile leading vital medical research and supporting patients and families across Australia.

Contents

What are Bone Marrow Failure Syndromes?

Bone marrow is an important and complex organ, and is the body's factory for blood cells. Healthy bone marrow works to produce new red and white blood cells and platelets every single day.

Bone Marrow Failure Syndromes are a collection of medical conditions where the bone marrow stops working, or works insufficiently. When our bone marrow fails, our body is unable to produce healthy blood cells, causing life-threatening disruptions to our health.

Bone marrow failure can be acquired or inherited. Inherited bone marrow failure can include additional health challenges, including heart, skeletal malformations, kidney, eye and ear issues, and an increased risk of both solid organ and blood cancer.

Every three days, an Australian is diagnosed with a Bone Marrow Failure Syndrome. Distressingly, most of them are children and young adults and 50% will not survive. Thousands more are living with ongoing complex health issues. Diagnosis is often slow and difficult due to the complexity of the conditions.

While some patients can manage their symptoms with existing therapies, the only established cure is bone marrow transplantation. However, bone marrow transplants don't always work and have a high risk of failure or adverse side effects. When patients don't respond to existing therapies, there aren't many options available.

That's why Maddie's Vision is here – to fund critical research and provide essential support, working towards improved outcomes for patients with Bone Marrow Failure Syndromes.

Maddie's story

Maddie Riewoldt was just 26 years old when she tragically died of a Bone Marrow Failure Syndrome called Aplastic Anaemia. She was diagnosed when she was 21 years old and for five tough years, she put up a brave fight. She spent those years enduring several bone marrow transplants, hundreds of blood transfusions and ongoing treatments.

Maddie fought her battle with inspiring determination and courage. When her fight was ending in February 2015, she asked her family to make her a promise; Maddie wanted nobody else to go through what she did.

Maddie Riewoldt's Vision is her legacy.

We are determined to find better treatments and cures for Bone Marrow Failure Syndromes, so please join us and together we will #FightLikeMaddie.

Chair's report

As another year comes to a close, I am once again amazed and encouraged by the incredible strides that Maddie Riewoldt's Vision has made in the field of Bone Marrow Failure Syndrome research and support. Not only has the organisation improved support options for patients and families, but it has also paved the way for world leading, vital research dedicated to improving diagnosis and treatments.

We are changing the lives of patients and families affected by Bone Marrow Failure Syndromes. By providing them with the support and information they need, we offer hope and make a positive impact on their journey.

The story of young Joel's diagnosis with Aplastic Anaemia, highlighted on page 18, is just one example of the long and lonely road that patients and families often faced without proper support before the establishment of Maddie's Vision. Thankfully, the Maddie Riewoldt's Vision Telehealth Nurse service provides a much-needed source of support for those patients and families grappling with these conditions. Feedback from these patients and families, as shared on page 16, shows just how critical this service is in empowering those affected by Bone Marrow Failure Syndromes. Our Telehealth Nurse, Mei Ling Yeh, has provided patients and families with expert support, information and most importantly, compassion and empathy as they navigate this difficult path.

The research of Alice Maier featured on page 12, is another testament to the importance of patient support. Alice's project focuses on identifying the individual needs of children undergoing a bone marrow transplant – an extremely high-risk treatment for Bone Marrow Failure Syndromes. This important project recognises the impact these treatments have on patients and families beyond treating their bone marrow failure.

Board director Amanda Stevens was an invaluable asset to Maddie Riewoldt's Vision during her tenure as Acting CEO from June to November 2022. I would like to extend my gratitude to her for her leadership during this time and her unwavering support for the organisation.

In November, we were delighted to welcome Amy Coote as CEO of Maddie Riewoldt's Vision. With over a decade of experience in the for-purpose sector, she brings a wealth of leadership and fundraising expertise to the organisation. Since joining us, Amy has already made a significant impact with her expert knowledge and her passion for making a difference to the lives of patients and families. We are excited to see what Amy and the broader Maddie Riewoldt's Vision team will achieve together in the future.

Unfortunately, we said goodbye to Kieran O'Brien in May after over four years of service on the Maddie's Vision Board. Kieran made an enormous impact during his time with us, providing invaluable legal counsel and commercial acumen. We are extremely grateful for his contributions and wish him and his family all the best.

I would like to thank my fellow Board members, our Executive team, and our fantastic community of supporters for their passionate support of Maddie Riewoldt's Vision. Together, we are working towards a future where Bone Marrow Failure Syndromes are prevented, diagnosed and treated with the best available medical treatments in Australia, and all patients and families have access to, and receive, the support that they so desperately need.

Finally, I want to thank the Riewoldt family – Fiona, Joe, Nick and Alex – for their ongoing commitment to Maddie Riewoldt's Vision. Their dedication to honouring Maddie's legacy continues to make a lasting difference to patients, families, and the research community.

Edward Kemp
Chair, Maddie Riewoldt's Vision

CEO's report

I was thrilled to join Maddie Riewoldt's Vision as CEO in November. It is a tremendous honour for me to lead the organisation and continue the Riewoldt family's legacy in memory of Maddie. Since Maddie's Vision was founded we have made a significant impact, but there is still more work to do.

We are committed to discovering improved treatments and cures for Bone Marrow Failure Syndromes. Furthermore, we will continue strengthening vital support for patients and their families affected by these devastating conditions. With our outstanding and dedicated community of supporters by our side, I am confident that we will achieve our goals.

I joined the organisation just in time for the Bloody Good Dinner, an event organised in partnership with the Snowdome Foundation, to raise funds for Bone Marrow Failure Syndrome and blood cancer research. This iconic event raised $710,000 this year thanks to the generosity of guests and event partners, now over $2 million since it started, an outstanding achievement. It was a magical night wherein 300 guests came together with the common goal of raising funds for important research, while enjoying wonderful food and company. We are immensely grateful to the dedicated event committee for providing their time and expertise to create a memorable evening at the Botanical Gardens, and we would also like to thank all the partners and sponsors for their generous support.

When I commenced my role at Maddie Riewoldt's Vision, I was amazed by the vast knowledge and dedication of the researchers at our Centre of Research Excellence in Bone Marrow Biology. They are continuously striving to improve diagnosis, treatment, and eventually find cures for patients with Bone Marrow Failure Syndromes. The newly funded projects for 2022–23 depict a promising future for Bone Marrow Failure Syndrome research. Dr Astrid Glaser's project showcases the exciting advancements in gene editing for treating inherited syndromes like Fanconi Anaemia, which will hopefully pave the way for treatments that are less risky or invasive for patients. Dr Kirsten Fairfax's profile on page 14 illustrates how the initial investment from Maddie's Vision in research and infrastructure is often leveraged to receive other grants and funding opportunities, growing the capacity of Bone Marrow Failure Syndrome research even further. The expansion of Dr Fairfax's team in Tasmania was only possible because of the Alex Gadomski Fellowship, and we are thrilled to see what they achieve next. I want to thank the Gadomski family for their dedication to Bone Marrow Failure Syndrome research in Tasmania, honouring Alex's legacy and making an important impact.

Through our deep relationship with patients and families living with a Bone Marrow Failure Syndrome diagnosis, we are always reminded why we must work so hard – giving them vital support from diagnosis to long term follow up, and hope for a future with better treatments and ultimately a cure.

Connecting with patients and families has been an important and very special part of my role as CEO. Learning about how we can help highlights how essential Maddie Riewoldt's Vision is and why we are tirelessly working to achieve our goals. Thank you for sharing your voice, stories and experiences with us, ensuring we are there for you in the way that you need.

My sincere thanks and gratitude to our wonderful donors, partners, and community for their generosity and support – helping us to #FightLikeMaddie in 2022–23.

Thanks to our hard working, dedicated and passionate Board and Maddie Riewoldt's Vision Executive team for their amazing commitment this year. I look forward to what we will achieve together in the future, to give hope to patients and families living with Bone Marrow Failure Syndromes.

Amy Coote
CEO, Maddie Riewoldt's Vision

Our research

A tangible impact...

Since Maddie Riewoldt's Vision was established in 2015, we have been committed to funding high-quality research projects with the potential to deliver breakthroughs in the prevention, diagnosis and treatment of Bone Marrow Failure Syndromes.

$8.8 MILLION
COMMITTED TO RESEARCH

$22.6 MILLION
FURTHER RAISED FOR RESEARCH THROUGH LEVERAGING MADDIE'S VISION FUNDING BY OUR GRANTEES AND FELLOWS

36
CUTTING-EDGE RESEARCH PROJECTS

2
CLINICAL TRIALS

321
PATIENTS PARTICIPATING IN CLINICAL TRIALS

159
SCIENTIFIC PRESENTATIONS

51
JOURNAL ARTICLES PUBLISHED

13
GRANTS-IN-AID

15
FELLOWSHIPS

2
SCHOLARSHIPS

4
GRANTS

Five Pillars of Research

This year our highly reputable and esteemed Scientific Advisory Committee reviewed and updated our national and international research strategy for our Centre of Research Excellence in Bone Marrow Biology – ensuring we prioritise projects that are most likely to have an impact on improving patient outcomes. Our five key pillars are:

1. **Collection and analysis of clinical data and samples from Bone Marrow Failure Syndrome patients.**

   It is essential to have a repository of patient data and blood and marrow samples to understand more about the patient population and facilitate and accelerate research.

2. **Understanding the biology and environment of blood stem cells.**

   Knowing more about how blood stem cells develop, the factors that affect them, and drugs that can be used to improve their function can enable us to promote the healthy growth of blood cells to support the prevention and treatment of bone marrow failure.

3. **Monitoring and understanding predictors of disease prognosis, treatment response, and disease evolution.**

   Understanding the indicators of disease progression and treatment response can support early intervention with more personalised or tailored treatments for patients.

4. **Improving patient care and multidisciplinary support.**

   Patients and families need access to the best allied health, nursing and multidisciplinary support to receive holistic care that meets their needs.

5. **Clinical trials.**

   Clinical trial platforms are essential to evaluate which treatments and interventions have the potential to improve Bone Marrow Failure Syndrome patient outcomes.

Director of Centre of Research Excellence in Bone Marrow Biology report

Words are immensely powerful determinants of the outcome of any major life event, be it tragedy or celebration. It is in light of our innate recognition of the power that words can hold that we have an inner commitment to find and say (or write) the right word or words so that we may improve a situation by our use of those very words. It can be a tough choice.

I have previously described the heart of Maddie Riewoldt's Vision and the Centre of Research Excellence in Bone Marrow Biology (CRE) as being underpinned by the principles of commonality, co-operation and community. And so it remains in 2023. Those principles have repeatedly been demonstrated by the choice and use of the right words at the right time in (almost) all of our dealings in the last year. The calm and peace that flows from the quiet and knowledgeable advice and support provided by our telehealth nurse, Mei Ling Yeh, has meant so much to so many in time of tragic need. Helping the youngest and most vulnerable members of the Bone Marrow Failure Syndrome community to find the right words to describe their symptoms and distress so we might respond to them more effectively are key drivers of two projects being undertaken by Rachel Edwards (Queensland Children's Hospital) and Alice Maier (Royal Children's Hospital, Melbourne), both recipients of Fiona Riewoldt Nursing and Allied Health Fellowships.

So too, using the right words to consistently communicate and educate each other through the labyrinthine complexities of inherited Bone Marrow Failure Syndromes achieved by a range of CRE-supported projects, including the EMBRACE study led by A/Prof Piers Blombery and Dr Lucy Fox, which has led to an enhanced process of delivery of diagnostic accuracy, genetic counselling and appropriate clinical treatments.

These are but a few examples of how the right words, in the right conversations, at the right time by Maddie's Vision and the CRE, have led, and continue to lead, to better lives for patients and their families.

So many words. But two more before I finish. Two important and yet-to-be-said words to all of the Maddie's Vision family and CRE contributors in 2023: Thank you. Thank you for your tireless efforts as members of the Scientific Advisory Committee or Patient and Family Forum Steering and Organising Committee. Thank you to Jaya Soma, Lou Johns and the Maddie's Vision Executive for keeping our committees on track and action focused through many, many meetings and for keeping our grantees informed and supported. Thank you to all of our grant and fellowship recipients for your outstanding research, publications and presentations. Thank you to all our donors and partners who have given selflessly so that, through research, others may have happier, healthier lives. Thank you to all our patients and their families who place their trust and hope in us and graciously accept our words of support and knowledge.

The CRE in 2023 continues to be a triumph of commonality, co-operation and community, all of which is underpinned by the gift of each other's words.

Professor David Ritchie
Director, Centre of Research Excellence in Bone Marrow Biology
Chair, Scientific Advisory Committee
Board Director

Bone Marrow Biology Centre of Research Excellence.

Patient and Family Forum empowers and educates with the latest information on Bone Marrow Failure Syndromes

The Maddie's Vision Patient and Family Forum connects patients and families with expert researchers and clinicians, providing them with the latest information about Bone Marrow Failure Syndromes. The forum also offers the opportunity to ask questions to ensure all patients and families feel educated and empowered as they navigate diagnosis and treatment.

Our third Patient and Family Forum was held virtually in 2022 and consisted of four specialised sessions run weekly in the evening during November. We were fortunate to have expert clinicians, scientists and allied health professionals present information about the latest treatments, research results and clinical trials, and members of our patient and family community sharing insights and personal perspectives of their journey with Bone Marrow Failure Syndromes.

The opening session contained general topics related to the care of patients with Bone Marrow Failure Syndromes, and the remaining three sessions were dedicated to the specific diagnoses of Fanconi Anaemia, Telomere Biology Disorders and Ribosomopathies. We were delighted to include bone marrow failure experts from both Australia and the USA, including Professor Stella Davies (Director, Division of Bone Marrow Transplantation and Immune Deficiency, Cincinnati Children's Hospital) and Associate Professor Kasiani Myers (Division of Bone Marrow Transplantation and Immune Deficiency, Cincinnati Children's Hospital).

With almost 400 registrants, this event was a resounding success, and all survey respondents rated the event as either meeting or exceeding expectations. Importantly, the forums provided an avenue for patients and families to feel empowered with knowledge and connect with those in similar situations:

> **"...for newly diagnosed families it would be extremely helpful to have [the Patient and Family Forum] every year so they have somewhere to go to connect with others, learn more and not feel like they are on their own."**
>
> Patient and Family Forum feedback

We would like to sincerely thank the Royal Melbourne Hospital Foundation, Alexion and Servier for their generous sponsorship and support of this event.

A huge thanks to the Steering and Organising Committee for their time and expertise dedicated towards ensuring delivery of a successful forum: Dr Lucy Fox (Chair), Dr Pasquale Barbaro, Professor Tracy Bryan, Judy Edwards, Dr Belinda Guo, Lou Johns, Associate Professor Adam Nelson, Professor David Ritchie, Jaya Soma, Amanda Stevens, Dr Parvathy Venugopal and Mei Ling Yeh.

The Royal Melbourne Hospital Foundation

ALEXION AstraZeneca Rare Disease

SERVIER moved by you

Expanding our research portfolio

Maddie Riewoldt's Vision established the first Centre of Research Excellence in Bone Marrow Biology (CRE) in Australia, dedicated to promoting the collaboration of expert researchers around a common purpose and providing the technical and peer support necessary for catalysing innovation across specialisations, ideas and projects in Bone Marrow Failure Syndromes.

The CRE is a virtual centre that provides a centralised contact point for existing researchers, future researchers, commercial entities and community groups.

In the 2022–23 financial year, the CRE expanded its research portfolio to 36 projects, awarding five new grants for cutting-edge projects and critical research infrastructure.

The new projects will extend our research network locally and internationally, enhancing the collaborative strength of research into Bone Marrow Failure Syndromes. This represents an investment of $910,318 over the next two to three years.

Maddie Riewoldt's Vision is honoured to support all our impactful Centre of Research Excellence projects and is delighted to continue building research capacity to find better treatments, cures, and improve health outcomes for patients with Bone Marrow Failure Syndromes.

Dr Vashe Chandrakanthan

Developmental stem cell biologist **Dr Vashe Chandrakanthan**, from the South Australian Health and Medical Research Institute, The University of Adelaide, was awarded the Maddie Riewoldt's Vision Fellowship for his project, "Discovery of novel niche factors to improve long-term stem cell transplantation in Bone Marrow Failure Syndromes". This project focuses on developing a growth factor-based treatment to help the transplanted stem cells to mature and produce healthy adult blood. Dr Chandrakanthan's research has the potential to transform the transplantation management of patients with Aplastic Anaemia and Fanconi Anaemia and improve on current therapy.

Dr Astrid Glaser

The Captain Courageous Fellowship was awarded to **Dr Astrid Glaser** at St Vincent's Institute of Medical Research for her project, "A preclinical trial of next generation gene editing for the prevention of bone marrow failure in Fanconi Anaemia", investigating gene editing techniques with the goal of repairing the underlying changes in a person's DNA that cause inherited Bone Marrow Failure Syndromes. Dr Glaser's fellowship will enable the development of new gene editing tools to repair the DNA mutations that cause Fanconi Anaemia, the most common inherited Bone Marrow Failure Syndrome.

Alice Maier

Alice Maier, a neuropsychologist at The Royal Children's Hospital, Melbourne, is the recipient of the Fiona Riewoldt Fellowship for her project, "Utility of a neurobehavioural assessment for treatment planning, educational, and family support for children undergoing bone marrow transplant for non-malignant disease." Ms Maier's study assesses the impact of a bone marrow transplant on children's development so their treating team and educators can know how best to support young patients before, during and after their transplant. This will provide the treating team with an understanding of the individual needs of the patient and family to help guide treatment planning, promote referrals to support services, and identify educational requirements that can be addressed during and post-transplant.

Dr Daniel North

The Haematology Society of Australia and New Zealand/Maddie Riewoldt's Vision New Investigator PhD Scholarship was awarded to haematology registrar **Dr Daniel North** for his project, "High resolution definition of the mechanisms of poor graft function following allogeneic haematopoietic stem cell transplantation". Dr North's project focuses on understanding poor graft function, a potentially life-threatening complication of transplants, by investigating its causes at a cellular level within the bone marrow environment. By knowing more about why bone marrow transplants fail or result in complications, we can provide better treatments or even prevent them from occurring. Dr North will undertake his PhD project at the MRC Weatherall Institute of Molecular Medicine, University of Oxford, England.

Professor Erica Wood and Professor Zoe McQuilten

Principal investigators **Professor Erica Wood** and **Professor Zoe McQuilten** received a two-year grant extension to manage the Australian Aplastic Anaemia and other Bone Marrow Failure Syndrome Registry. This clinical quality registry collects health information on patients diagnosed with Aplastic Anaemia and inherited Bone Marrow Failure Syndromes in Australia, with the aim of assisting clinicians and researchers to understand and improve current therapies and clinical outcomes for patients, as well as supporting multiple clinical trials by providing a long-term clinical data repository.

To find out more about our ongoing research projects, please visit www.mrv.org.au/research/projects/

From little things, big things grow… The Alex Gadomski Fellowship leverages funding to expand research into Bone Marrow Failure Syndromes

A Fellowship created in the memory of an extraordinary young man, the Alex Gadomski Fellowship, has seen Bone Marrow Failure Syndrome research in Tasmania go from strength to strength.

Dr Kirsten Fairfax was awarded the inaugural Alex Gadomski Fellowship in 2019 for her project, "Determining genetic and epigenetic drivers of gene expression changes in blood cells." Each person has a unique genetic code; this code is long, and current understanding only extends to a fraction of what it does. Dr Fairfax's work aims to understand more about how parts of DNA change the formation of blood cells. This information may be used to form new drug development strategies and clinical diagnoses for Bone Marrow Failure Syndromes.

Dr Fairfax has successfully established the capacity to perform single cell sequencing at the University of Tasmania, facilitating projects in Tasmania that would not have previously been possible. Her team has so far been able to generate sequences of Ribonucleic acid (RNA) from thousands of individual cells from the first seven volunteers who donated bone marrow, with millions more cells from hundreds more samples planned in the coming months to be run on this state-of-the-art platform.

"Establishing the platform has only been possible due to the funding that Maddie Riewoldt's Vision has awarded us, and recent funding awarded by the National Health and Medical Research Council," Dr Fairfax says.

Dr Fairfax has been able to leverage the funding that the Gadomski family has raised in Tasmania to attract nationally competitive government grants, with over a million dollars of government grant funding awarded to the laboratory in the past six months. Dr Fairfax was recently awarded a highly competitive NHMRC Ideas grant to continue the work funded by Maddie Riewoldt's Vision. This grant will use bone marrow samples from Tasmanian patients to understand more about how our DNA code changes the way genes are turned on and off in the bone marrow, and in turn, what causes the immune system to attack the stem cells in the bone marrow.

The increase in government grant funding, and the ongoing support from Maddie's Vision has led to the expansion of the team that is researching into Bone Marrow Failure Syndromes. Dr Fairfax is thrilled with the growth of the team: from just a single researcher in 2019, the team is now recruiting a research assistant, and two additional PhD students, to add to the team which currently includes Ariel Simpson (the Alex Gadomski postgraduate fellow), a new honours student, Caitlin Bidwell (awarded the David Collins Fellowship), and bioinformaticians Thomas Guinan and Alex Barnett.

Dr Fairfax is also one of the chief investigators in a team of researchers that were recently awarded a grant from the Medical Research Future Fund. This grant is led out of St Vincent's Institute in Victoria, by Associate Professor Andrew Deans, and has a primary goal of developing new gene therapies for inherited Bone Marrow Failure Syndromes.

A heartfelt thanks to the Gadomski family and the wonderful Tasmanian community who have gotten behind the Alex Gadomski Fellowship, paving the way for future innovation and discoveries in understanding and treatment of Bone Marrow Failure Syndromes.

Front L–R: Kirsten Fairfax, Ariel Simpson, Freya Patterson
Middle L–R: Pamodha Somasiri, Primrose Mandalawatta, Raj KC
Back L–R: Alex Barnett, Alex Hewitt, Thomas Guinan

Centre of Research Excellence highlights

Medical Research Future Fund stem cells mission grant success

Maddie's Vision is committed to delivering better treatments for Bone Marrow Failure Syndromes and in 2020 established the Gene and Cellular Therapy Subcommittee to advance this aim. We were thrilled when many members of this subcommittee teamed together to successfully obtain a Medical Research Future Fund grant of just under $1 million for funding cutting-edge gene editing research. The grant was awarded in December 2022 and the project commenced in April 2023 – it will run for two years.

The team's project, led by Associate Professor Andrew Deans, titled "PAGETURNER – Pioneering application of Gene Editing to Transplant Using RNA", aims to repair the underlying changes in a person's DNA that cause inherited Bone Marrow Failure Syndromes. The goal of the research is to develop new treatments to correct disease-causing mutations in blood stem cells. This pioneering new technology has the potential to transform the lives of people who require a blood stem cell transplant.

Maddie's Vision is proud to be a supporting partner on the project, and this grant's success is a perfect example of how the Centre of Research Excellence platform has bolstered collaboration and innovation amongst experts in bone marrow failure research. Congratulations to A/Prof Andrew Deans and his team, along with the Maddie's Vision grantees, fellows and CRE members who were involved in this successful application: A/Prof Piers Blombery, Professor Tracy Bryan, A/Prof Wayne Crismani, Dr Kirsten Fairfax, Dr Lucy Fox and Professor David Ritchie.

Centre of Research Excellence inaugural Think Tank

In April, Maddie's Vision facilitated a Centre of Research Excellence in Bone Marrow Biology "Think Tank", an opportunity to encourage cross-fertilisation of projects and map interconnectivity to drive future funding opportunities. The Think Tank reinforced the importance of prioritising collaboration in the CRE to accelerate future Bone Marrow Failure Syndrome research discoveries, with CRE members having access to peers with an exceptionally diverse range of skills and resources. As a result, processes are now in place to provide members of the CRE with improved visibility over each other's work, including available models and platforms, maximising the group's opportunity to draw on each other's expertise and identify potential partnerships.

Collaborative research institutions

Thanks to our collaborative research institutions which form a critical part of the Maddie's Vision community and our Centre of Research Excellence:

ABMTRR Australasian Bone Marrow Transplant Recipient Registry

Australian National University

ANZCHOG Australian & New Zealand Childrens Haematology/Oncology Group

Monash University

ARMI Australian Regenerative Medicine Institute

BIOBANKING VICTORIA Precision Medicine Services

cancer centre for children the children's hospital at Westmead

Centre for Cancer Biology

Children's Health Queensland Hospital and Health Service

Queensland Government

Children's Medical Research Institute Jeans for Genes

HSANZ Haematology Society of Australia and New Zealand

MRC Weatherall Institute of Molecular Medicine

murdoch children's research institute

NIH NHLBI Bethesda Labs

Peter Mac Peter MacCallum Cancer Centre Victoria Australia

QUT Queensland University of Technology

The Royal Children's Hospital Melbourne

The Royal Melbourne Hospital

SVI St Vincent's Institute Medical Research

transfusion research unit

The University of Adelaide

The University of Melbourne

University of South Australia

University of Tasmania

Menzies Institute for Medical Research

The University of Western Australia

Supporting patients and families through the Maddie's Vision Telehealth Nurse Service

As Bone Marrow Failure Syndromes are rare, many patients and families feel overwhelmed, isolated and in need of support when they receive a diagnosis and during treatment.

The Maddie's Vision Telehealth Nurse service is the only available service dedicated to Bone Marrow Failure Syndrome patients, families and carers in Australia. We're delighted that recent feedback about our service has been so positive and that our patient's and families' needs and well-being are understood, evaluated, and fulfilled. Our holistic approach and expertise in Bone Marrow Failure Syndromes make our service unique.

86
patients and families supported through our Telehealth Nurse service.

97%
of patients and families understood their diagnosis well or very well, thanks to the Telehealth Nurse service.

86%
of patients and families understood the treatment process well or very well due to education provided by the service.

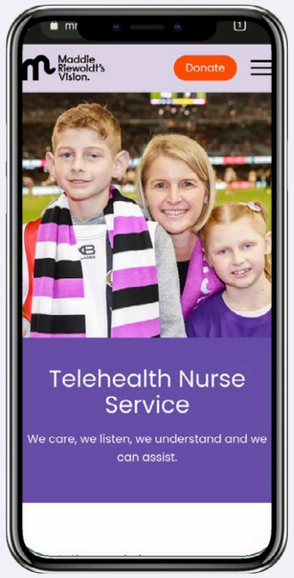
79%
of patients and families understood how to access support resources in relation to their condition as a result of advice provided by the service.

7/10
After receiving supporting from our Telehealth Nurse service, patients and families reported an increase emotional wellbeing from an average of 3/10 to 7/10.

9/10
When asked how well the conversation with the service met their needs, patients and families rated the service with an average score of 9/10.

"We could not be more thankful for the support we received, and continue to receive, after our daughter's life-changing diagnosis. We will be forever grateful."

"The Telehealth nurse has gone above and beyond to support our family."



"The Telehealth nurse service is phenomenal. I relied on the service a lot throughout treatment and regretted that I didn't reach out earlier. I feel that the support is critical, especially before treatment starts."

If you or a loved one is impacted by Bone Marrow Failure Syndromes and need support, contact our Telehealth Nurse via email: telenurse@mrv.org.au or make a booking online at mrv.org.au.

Joel's story

Joel was only six years old when he was diagnosed with Severe Aplastic Anaemia. Despite his mother's medical background as a nurse, it was something the family hadn't heard of before. This was the start of a complex and often lonely journey for the family as they battled through Joel's treatment and relapse.

Joel still remembers being asked by his parents, Ian and Judy, how he got the mysterious bruises that kept appearing on his shins when he started primary school. "I really had no idea where they came from," he recalls.

For an active kid like Joel, bruises from playing weren't cause for concern at the time. However, as the bruises worsened, Joel became increasingly pale and prone to blood noses every night. Judy's instincts as a nurse kicked in, and she decided it was time to take Joel to the GP.

Their GP took one look at the blood test results and sent them straight to the Monash Children's Hospital. "I'll never forget that night," Judy says. After finding out that Joel's haemoglobin was only 43, and his platelets were 11, Judy knew it was serious.

They eventually received a diagnosis of Severe Aplastic Anaemia. "I had no idea what it was at the time," Judy says. "It was just the beginning of our journey."

A lifesaving gift from his sister

Joel needed transfusions to replace the platelet and blood cells he required, but the only hope at a cure would be a bone marrow transplant. Thankfully his sister Emily was a match and was able to donate her bone marrow to help save Joel's life. After receiving his transplant, Joel was isolated at the hospital for six weeks as his immune system was weakened from the treatment.

Judy says it was heartbreaking to watch their active young son confined to a hospital room, missing out on school and time with Emily. "Joel was very brave during this time. He had school online every day for an hour, which we were grateful for."

Unable to see her big brother in person, Emily would talk to Joel on the phone and wave at him through the hospital window.

Despite complications developing during his treatment, Joel was discharged and recovered at home. With his immune system still compromised, Judy did what she could to keep Joel away from crowds while still enjoying outings. "We went shopping at 6 am when it was quiet and went to playgrounds when other children were in class. I'd bring wipes to the playground, and if anyone else came, we'd have to leave quickly. It was hard."

Eventually, Joel could be around his peers and even return to school.

Relapse and making connections

In 2019, Joel began to experience recurrent bruising again. Hoping for the best, Judy took Joel for more blood tests. Unfortunately, they received the heartbreaking news that Joel had relapsed and again had Aplastic Anaemia.

> **"Joel and I cried on the way home because we realised that we were back to square one," Judy says.**

Feeling like she needed extra support from people who understood what they were going through, Judy joined a Facebook group for people impacted by Aplastic Anaemia. She connected with another family in Melbourne with a son living with Aplastic Anaemia the same age as Joel. "We would meet up, and the kids played together. It was just so nice to have friends who have gone through the same thing we have." The families are still close today.

After Joel's relapse, the family started to receive support from the Maddie Riewoldt's Vision Telehealth Nurse service, finally having access to information and support that was vital to Joel's recovery.

At the time, the family was offered a second bone marrow transplant or immunosuppressive therapy by their haematologist. It wasn't an easy decision to make for both Judy and Ian. The Telehealth Nurse service provided support and information to the family in that crucial time.

Joel started immunosuppressive therapy in addition to a medication called Eltrombopag, and his blood cells and platelets slowly improved. Today, Joel is no longer on immunosuppressive therapy, and his blood levels have returned to normal. Joel, Judy, Ian and Emily generously support other families going through a similar journey by helping raise awareness of Bone Marrow Failure Syndromes.

Judy volunteers with Maddie's Vision, offering guidance based on her family's lived experience and perspectives. She recently provided her advice and invaluable expertise as a representative on the Patient and Family Forum committee.

Joel and Emily even appeared in a recent television commercial for Maddie's Vision, airing on Foxtel, helping bring more awareness to these devastating conditions.

After everything Joel went through at such a young age, he feels strongly that more research and awareness are needed: "Future research will be lifesaving! It would be good if there were a cure as anyone can get Aplastic Anaemia. Awareness is so important too because it's so rare, it's been so hard to explain to people what it is."

Our valued community of supporters

Government

The Australian Government Department of Health generously awarded Maddie's Vision with two Medical Research Future Fund grants in 2019 and 2020. These multi-year clinical trials – EMBRACE and RESELECT – continued throughout 2022/2023 to improve patient care. The EMBRACE study (Evaluating Multidisciplinary Bone maRrow fAilure CarE) has provided 310 patients with complex genomic testing leading to improved diagnosis and therefore more personalised treatment. The RESELECT study (REscuing bone marrow function in patients with relapsed acquired aplaStic anaEmia and/or bone marrow failure post aLlogeneic stEm Cell Transplantation) remains active and of high scientific value. Since opening, 11 patients have been recruited on the trial which aims to test the safety and efficacy of combination N-Acetyl-Cysteine and Atorvastatin in improving outcomes for this patient group.

Australian Government
Department of Health

Victorian Cancer Agency (VCA)

The Victorian Cancer Agency (VCA) works to enable cancer research across Victoria. We were thrilled to partner with the VCA via the co-funded VCA/Maddie's Vision International Travelling Fellowship, awarded to Dr Ashvind Prabharan. Dr Prabharan undertook studies at the National Heart, Lung and Blood Institute in Bethesda, USA and has since returned to Melbourne and completed his PhD evaluating the immune microenvironment in poor graft function following stem cell transplant.

VICTORIAN CANCER AGENCY

VICTORIA State Government

Partnerships

Bloody Good Dinner

The Bloody Good Dinner is a joint collaboration between Maddie Riewoldt's Vision and the Snowdome Foundation which, to date, has raised over $2 million for Bone Marrow Failure Syndrome and blood cancer research.

As blood cancer and bone marrow failure are so closely related, both organisations came together to create an event that would really make a difference and connect with people passionate about this important medical research. By bringing supporters from these two organisations together, we had an opportunity to raise significant funds for invaluable research with a truly world-class dining experience.

The 2022 Bloody Good Dinner was a bloody huge success, as 300 guests raised $710,000 for vital research for Maddie Riewoldt's Vision and the Snowdome Foundation!

Guests kicked off the night mingling with drinks and canapes in the beautiful Botanic Gardens, before making their way into the Botanica Marquee, on the banks of the ornamental lake to be welcomed by our MC, Hamish McLachlan. Thanks to Chef Jacqui Challinor, food was abundant, with the guests well-fed all night. Spectacular service from the team at Blakes Feast kept the guest's drinks topped up with a smile. When our guest speaker Grace generously shared her story of her daughter Raya's cancer and the importance of funding research, there wasn't a dry eye in the house. Later in the evening things got quite lively, with our auction attracting some very spirited bidding! Congratulations to our raffle and auction winners who walked away with fantastic prizes thanks to our generous sponsors.

A huge thank you to Pennie Callaghan and the Bloody Good Dinner committee, who volunteered their time and considerable expertise to deliver a truly memorable event.

Thank you to NOMAD Executive Chef, Jacqui Challinor and team, our event partners, the generous sponsors, donors and our dedicated volunteers. Without you, this would just not be possible.

Visit bloodygooddinner.org.au to find out more.

Event partners

Event sponsors

General committee

Pennie Callaghan
(Chair)
Kirstee Macbeth
(Chief Executive Officer, Snowdome)
Amanda Stevens
(Acting Chief Executive Officer, Maddie Riewoldt's Vision)

Annabel Banks
Gian Blundo
Vanessa Cohen
Mitchell Coughlin
Elian De La Rosa Gomez
Carly Duckworth
Lauren Frazer

Jackie Haintz
Meagan Henry
Lou Johns
Stephen Jolson
Christine Lester
Maggie Lynch
Gina McNamara

Elaine Montegriffo
Michelle O'Brien
Billie Paulino
Georgie Stayches
Georgie Tandy
Kasey Thompson
Peta Twisk

St Kilda

While there was no Maddie's Match in the 2022–23 financial year, Maddie Riewoldt's Vision still felt the love and support from our founding partner the St Kilda Football Club. Young Zahra, who has had a big few years after being diagnosed with Aplastic Anaemia and Myelodysplastic Syndrome, visited RESA park to meet the team for a photo. Zahra charmed everyone with her warm and bubbly personality – even at one point politely requesting she be called Elsa, after her favourite Disney character!

Thank you, St Kilda, for being amazing supporters of Maddie's Vision and patients and families impacted by Bone Marrow Failure Syndromes.

Foxtel – lights, camera, action!

The Foxtel Group has generously created a fantastic community awareness advertisement for Maddie Riewoldt's Vision. Airing on over seventeen Foxtel channels, this television campaign raises awareness of Bone Marrow Failure Syndromes and the work that Maddie's Vision does, so that we can continue to fight for better treatments, support options and a cure.

The campaign was written, developed, and produced by Foxtel's in-house creative agency, Foxtel Creative.

Thanks to director Tim Buhagiar, who along with the Foxtel Creative team made something really special. Thanks to St Vincent's Private Hospital East Melbourne and their staff, who provided space to film and fantastic support on the day.

FOXTEL Group

Maddie's Month

For the seventh year, Australians made a difference to people with Bone Marrow Failure Syndromes by including delicious tomatoes and capsicums in their weekly shop at Coles supermarkets.

Our long-term partners at Flavorite raised $100,000 for Bone Marrow Failure Syndrome research in November by donating 10c from every specially marked pack of their tomatoes and capsicums sold at Coles to Maddie Riewoldt's Vision. For this year's campaign, celebrity chef Michael Weldon teamed up with Nick Riewoldt and Chris Millis, Chief Operating Officer at Flavorite, to create a selection of healthy and delicious recipes with the produce that the whole family can enjoy.

Maddie's Month has now raised an amazing $700,000 for Bone Marrow Failure Syndrome research. Thank you to Flavorite for their generous donation and Coles for their support in this year's campaign.

flavorite flavour for life

coles

In memory of Alex Gadomski

Alex Gadomski fought bravely against Aplastic Anaemia and Myelodysplasia before tragically passing away at the young age of 21. To honour Alex's legacy, his family collaborated with Maddie Riewoldt's Vision to create the Tasmanian-based Alex Gadomski Fellowship and Postgraduate Scholarship to support research towards finding a cure for Bone Marrow Failure Syndromes.

The Gadomski family has successfully rallied the community in Tasmania, hosting events and challenges including the Live Life Gala Ball, The 7HOFM Bloody Long Quad, the inaugural Alex Gadomski Fellowship Golf Day and the Alex Gadomski Fellowship High Tea. These events all raise awareness and vital funds for the Fellowship, ensuring that Alex's legacy is harnessed to support innovative medical research.

The family raised an incredible $295,853 for the Alex Gadomski Fellowship in 2022–23. We thank Janet, Paul, Lachlan, and Jordan Gadomski for their fantastic work and contribution to Bone Marrow Failure Syndrome research in Tasmania.

Live Life Gala Ball

The Live Life Gala Ball was again an incredible night out, with the Tasmanian community coming together to raise funds for the Alex Gadomski Fellowship.

7HOFM Bloody Long Quad

Another amazing effort was made by Jordan and Lachlan Gadomski and the team who participated in the 7HOFM Bloody Long Quadrathlon. This inspiring group kayaked 60km, rode 500km, ran 125km and swam 15km to raise funds for the Alex Gadomski Fellowship.

The inaugural Alex Gadomski Fellowship Golf Day

It was a fantastic day out at North West Bay Golf Club for the inaugural Alex Gadomski Fellowship Golf Day.

The Alex Gadomski Fellowship

Philanthropy

Baker Foundation

The Baker Foundation, legacy of the late Thomas Baker, is driving innovation in medical research and general philanthropy. We thank the Baker Foundation for generously supporting our Telehealth Nurse service, enabling the delivery of much needed support to patients with Bone Marrow Failure Syndromes and their families across Australia.

Rae and Peter Gunn Family Foundation

Through the generosity of the Rae and Peter Gunn Family Foundation we celebrate our joint fellowship with the Snowdome Foundation. Thanks to the Foundation, we are able to support Dr Belinda Guo and Dr Katherine Cummins in undertaking their Gunn Family Women in Haematology Fellowships.

Corporate

Coca-Cola Australia Foundation

Coca-Cola employee Darren Beer, father of Hannah Beer, who's fiancé Jack tragically passed away from Aplastic Anaemia in 2021, brought a grant opportunity, the "Coca-Cola Australia Foundation Employee Connected Grant Program" to the attention of Maddie's Vision in 2022. This grant supports local causes and charities connected to Coca-Cola employees in Australia. Following an application process supported by Darren, we were thrilled to be awarded $10,000 to support the work of the Centre of Research Excellence in Bone Marrow Biology. Our heartfelt gratitude to the Coca-Cola Australia Foundation, Darren, and Hannah for their support of Maddie's Vision.

Thank you to our volunteers, donors and partners

Thank you to all the committed and generous people who supported us this year. Whatever you did, and however much you were able to contribute, your invaluable support is making such a difference. You are helping to realise our mission of finding improved treatments and cures for Bone Marrow Failure Syndromes.

Gift for Gab charity walk

The Gift for Gab charity walk in October raised over $26,000 for Bone Marrow Failure Syndromes research and Maddie Riewoldt's Vision. The walk was named after Gabbie, a young woman with Diamond Blackfan Anaemia, by her father Jamahl who with a friend created the first walk five years ago. Jamahl walked a massive 245 kms along the coast from Coffs Harbour to Byron Bay, joined by a team of 20 participants walking 35km each day for seven days! Gabbie was of course waiting for Jamahl and the team at the finish line.

Shaving our head for Angus

At just eight weeks of age, Angus was diagnosed with Diamond Blackfan Anaemia, an inherited Bone Marrow Failure Syndrome. When Angus had to relocate to Sydney for a bone marrow transplant, his high school friends decided to shave their heads to raise awareness of the day-to-day struggles that people with Bone Marrow Failure Syndromes face. More than 15 students shaved their heads for their mate Angus, raising over $8,000. Funds raised will support the Captain Courageous Fellowship – named in honour of Angus – and support research focussed on paediatric inherited Bone Marrow Failure Syndromes.

Measure in love for Larson – a pop-up piano bar

When toddler Larson was diagnosed with a Bone Marrow Failure Syndrome, the Horsham community gathered around him and his parents with a beautiful event to raise awareness and funds for research – the "Measure in Love for Larson Pop-Up Piano Bar". The event raised an amazing $7,000 for Maddie Riewoldt's Vision.

Community supporters

We would also like to recognise the generosity and support of other members of the community who created fundraisers and/or raised awareness for Maddie Riewoldt's Vision in 2022–23: Anna Andrewes, Bond's Buzzy Boys, Alysha and Charlotte Crawford, Brenda Hinshelwood, Horsham Arts Council, Josh Hunt, Pauline Loftus-Hills, Anna McMahon and Liz Miller.

Pro bono support

Thank you to our generous pro bono partners, whose valuable ongoing support, valued at over $175,000, enabled us to use more of our funds towards vital research and support options for patients living with Bone Marrow Failure Syndromes and their families.

The Maddie Riewoldt's Vision team

M Riewoldt Holdings is registered with the Australian Charities and Not-for-profits Commission (ACNC) as a DGR Type 1 organisation (Health promotion charity).
www.acnc.gov.au/charity/charities/f5742f11-cb01-e911-a96e-000d3ad064bd/profile

Executive

Amy Coote
Chief Executive Officer
From November 2022

Meagan Henry
Administration and Operations Co-Ordinator

Lou Johns
Projects Administrator – Centre of Research Excellence in Bone Marrow Biology

Jaya Soma
Project Manager – Centre of Research Excellence in Bone Marrow Biology

Peta Twisk
Marketing and Communications Manager

Mei Ling Yeh
Telehealth Nurse

Board

Edward Kemp
Chair

Kieran O'Brien
Director to May 2023

Michelle O'Brien
Director

Nick Riewoldt
Director

Professor David Ritchie
Director

Amanda Stevens
Director, Acting Chief Executive Officer from June – November 2022

Andrew Yeoland
Director

Scientific Advisory Committee

Professor David Ritchie (Chair)
Dr Pasquale Barbaro
Professor Tony Cesare
Dr Andrea Henden
Dr Victoria Ling
Dr Duncan Purtill
Professor Zoe McQuilten
Professor Kanta Subbarao
Associate Professor Ingrid Winkler

Financial results to 30 June 2023

STATEMENT OF PROFIT OR LOSS AND OTHER COMPREHENSIVE INCOME FOR THE YEAR ENDED 30 JUNE 2023

	Note	2023 $	2022 $
Revenue and other income			
Revenue from contracts with customers	3	4,344	18,552
Interest income	4	40,592	16,480
Donations and grants	4	1,862,121	2,930,524
		1,907,057	**2,965,556**
Less: expenses			
Research grant expenses		(1,452,139)	(1,351,234)
Centre of research excellence costs		(9,385)	(13,518)
Employee expenses – research		(174,665)	(130,922)
Employee expenses – administration		(312,113)	(329,374)
Fundraising costs		(254)	(7,769)
Merchandise costs		(17,709)	(9,414)
Depreciation and amortisation expense		(5,534)	(2,198)
Other expenses		(94,996)	(50,072)
		(2,066,795)	(1,894,501)
Surplus/{deficit) before income tax expense		(159,738)	1,071,055
Income tax expense			
Surplus/{deficit) from continuing operations		(159,738)	1,071,055
Other comprehensive income for the year			
Total comprehensive income		**(159,738)**	**1,071,055**

STATEMENT OF FINANCIAL POSITION AS AT 30 JUNE 2023

	Note	2023 $	2022 $
Current assets			
Cash and cash equivalents	5	3,566,447	4,540,858
Receivables	6	42,815	94,308
Inventories	7	8,391	17,609
Other assets	8	8,470	3,669
Total current assets		**3,626,123**	**4,656,444**
Non-current assets			
Intangible assets	10	41,908	474
Plant and equipment	9	2,831	4,224
Total non-current assets		44,739	4,698
Total assets		**3,670,862**	**4,661,142**
Current liabilities			
Payables	11	48,252	61,213
Provisions	12	20,068	23,507
Deferred grant revenue	13	287,296	848,946
Total current liabilities		**355,616**	**933,666**
Non-current liabilities			
Provisions	12	7,748	10,443
Deferred grant revenue	13		249,797
Total non-current liabilities		7,748	260,240
Total liabilities		363,364	1,193,906
Net assets		**3,307,498**	**3,467,236**
Equity			
Reserves	14	971,071	
Retained earnings	15	2,336,427	3,467,236
Total equity		**3,307,498**	**3,467,236**

Financial results to 30 June 2023

STATEMENT OF CHANGES IN EQUITY FOR THE YEAR ENDED 30 JUNE 2023

	Reserves $	Retained earnings $	Total equity $
Balance as at 1 July 2021		2,396,181	2,396,181
Surplus/(deficit) for the year		1,071,055	1,071,055
Total comprehensive income for the year		1,071,055	1,071,055
Balance as at 30 June 2022		**3,467,236**	**3,467,236**
Balance as at 1 July 2022		3,467,236	3,467,236
Surplus/(deficit) for the year		(159,738)	(159,738)
Total comprehensive income for the year		(159,738)	(159,738)
Transfers	971,071	(971,071)	
Balance as at 30 June 2023	**971,071**	**2,336,427**	**3,307,498**

STATEMENT OF CASH FLOWS FOR THE YEAR ENDED 30 JUNE 2023

	2023 $	2022 $
Cash flow from operating activities		
Receipts from customers and donors	1,162,449	2,700,396
Operating grant receipts	20,000	20,000
Payments to suppliers and employees	(2,151,877)	(2,138,054)
Interest received	40,592	16,480
Net cash provided by/(used in) operating activities	**(928,836)**	**598,822**
Cash flow from investing activities		
Payment for intangible assets	(45,575)	
Net cash provided by/(used in) investing activities	(45,575)	
Reconciliation of cash		
Cash at beginning of the financial year	4,540,858	3,942,036
Net increase/(decrease) in cash held	(974,411)	598,822
Cash at end of financial year	**3,566,447**	**4,540,858**

Abridged financial statements and audited accounts

The financial statements that form part of this Report are abridged. The complete Annual Accounts and Financial Review of Maddie Riewoldt's Vision can be found online at https://www.acnc.gov.au/charity/charities/f5742f11-cb01-e911-a96e-000d3ad064bd/documents/. These statements should be read in conjunction with the accompanying Notes to the Financial Statements.

Support Maddie Riewoldt's Vision

Become a Maddie's Vision regular donor

Regular giving is an incredibly valuable way of providing ongoing support for Maddie's Vision. Whilst we have achieved so much, there is still much more to be done to find better treatments and support options, and we'd love your help! Knowing we have dependable, ongoing sources of funding means that we can confidently commit to more research and support for patients and families.

Fundraise

Whether you're a club, a business, a school or a family, starting your own fundraiser is a fun way to raise awareness and funds for Bone Marrow Failure Syndromes. If you'd like to create your own personal fundraiser, it's quick and easy to set up a page on our website.

In memory

Many families approach us who would like people to make donations instead of sending flowers in memory of someone they love. You can create an "In loving memory" page on our website. You can also make a one-off in memory donation.

Workplaces

Your workplace is a great place to begin your fundraising efforts. Raise funds and workplace morale at the same time! Employers can also facilitate tax effective regular workplace giving as a simple, efficient and ethical way for businesses and their employees to support causes such as Maddie Vision – ask your employer about workplace giving!

Visit mrv.org.au to find out how you can donate or start your own fundraiser today!

MADDIE RIEWOLDT'S VISION
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Instagram: MaddiesVision