

AAR Newsletter - December 2023

Welcome!

Welcome to the Aplastic Anaemia and Other Bone Marrow Failure Syndromes Registry (AAR) newsletter. We look forward to providing you with a quarterly newsletter to inform you on the progress of the registry, important information to note, and inform you of any future activities and upcoming events.

Aplastic Anaemia
and other
Bone Marrow Failure
Syndromes Registry



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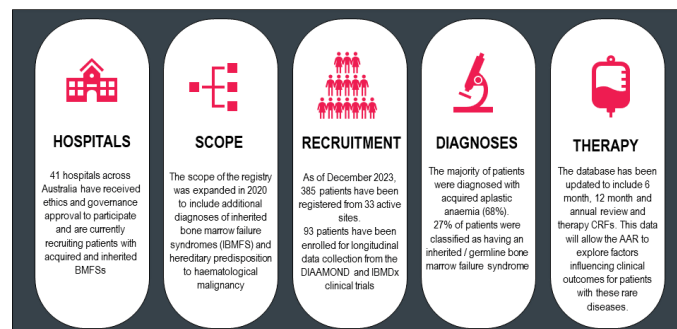
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Registry Progress and Overview

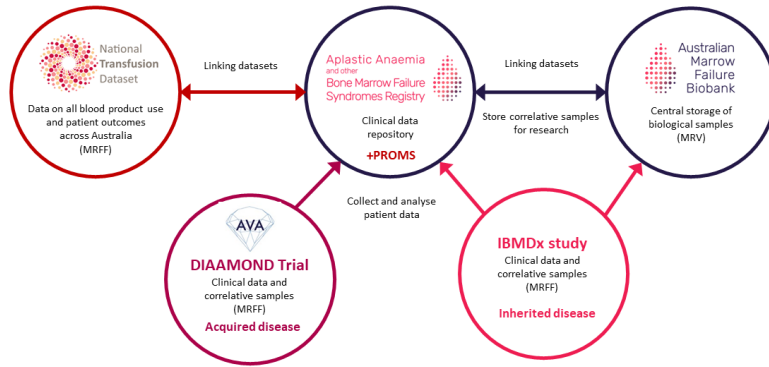
The registry has made some major updates in the last 12 months. Inclusion of a per patient payment for data entry at sites has seen an increase in patient registrations and data capture. We have also made further improvements to the database, incorporating logic so that data entry is more streamlined. Please contact the management team to schedule a training session for your site where we can address common and challenging data entry queries.

The registry was also successful in publishing a [methods paper](#) in Best Practice & Research Clinical Haematology, we extend our appreciation to our contributing sites and the AAR Steering Committee for their ongoing support.



The registry continues to provide infrastructure support and longitudinal data capture for a number of studies. The [DIAAMOND clinical trial](#) is being conducted to see if the addition of a TPO mimetic, avatrombopag, to standard of care in AA patients reduces the need for blood transfusion and supportive care. 49/50 participants have been enrolled, please email the DIAAMOND CRA for further information, sphpm.DIAAMOND@monash.edu

The inherited bone marrow failure and related disorders ([IBMDx](#)) study addresses the challenges associated with diagnosing inherited bone marrow failure syndromes and related disorders, a group of genetically diverse and rare diseases that can manifest throughout a lifetime. By analysing the genomic information of IBMFS patients, this study aims to provide definitive diagnoses and enhance our understanding of these conditions. 107 participants have been recruited and Royal North Shore Hospital will be open to recruitment in January 2024. For further information email IBMDxStudy@petermac.org

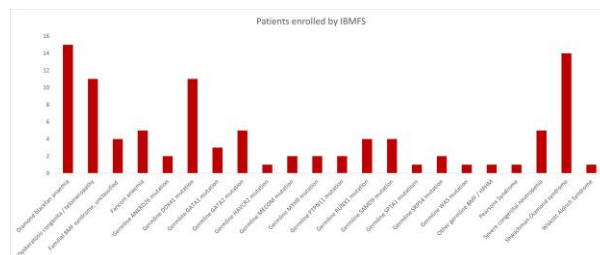
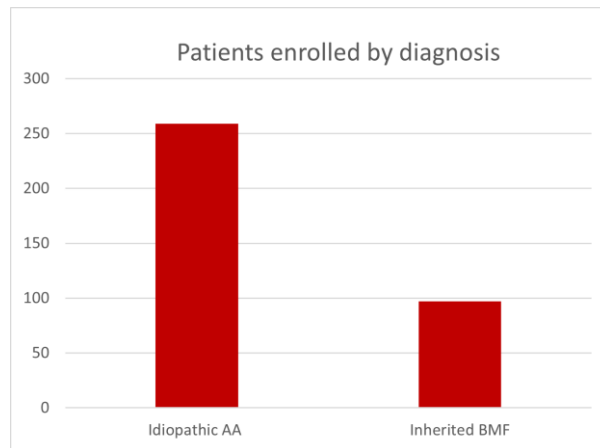


Patient Accrual and Site Payments

Patient accrual has continued to increase and there are now a total of 385 patients enrolled across Australia. **Sites are now eligible for a per patient payment** to offset some of the costs of data entry. If your site has not received and updated contract to permit these payments, please contact the AAR Project Manager.

Data Snapshot

The expanded scope of the registry has resulted in an overall increase in patients enrolled in the registry, with inherited bone marrow failures now contributing to 27% of diagnoses.



Australian Marrow Failure Biobank

In 2020, Monash University including Biobanking Victoria was awarded funding to establish the Australian Marrow Failure Biobank (AMFB).

The Biobank will collect, process and store **matched tissue sample** from patients diagnosed with an inherited bone marrow failure syndrome. The tissue samples will be available to the research community and provide opportunities in the wider field of bone marrow biology, and encourage national and international collaborations. The clinical data will be curated in the AAR and together, the AAR and the AMFB will support research to identify new diagnostic and therapeutic strategies to treat these rare conditions.

HREC approval was obtained in 2023 and we are working with 5 pilot sites to progress governance applications with the goal of commencing sample collection early in 2024. Per patient payments are available in addition to the AAR per patient payments.

If you are an existing AAR site which is interested in participating in the biobank, please contact Lauren on amfbiobank@monash.edu



Welcome to our Consumer Representatives

Consumer representatives can add value to health and medical research through a lived experience in the area of interest and we are excited to add two consumer representatives to our research team. We extend a warm and grateful welcome to Lynda and Cheryl. They have kindly shared their stories and experience living with bone marrow failure below.

Lynda and Daniel

Daniel was diagnosed with Aplastic Anaemia in May of 2020, after our initial relief of it not being a cancer a little research revealed that they have very similar paths. The team at the Royal Children's Hospital helped us navigate what was and may be ahead and after two rounds of immunotherapy a lot of love and over three years later he is on track to not only survive Bone Marrow Failure but thrive in spite of it.

His greatest love is Charlie the family Golden Retriever that came into our lives during his second round of immunotherapy and soccer, which after treatment has become a measure of how well he is doing. What we as a family wish for is a cure for all those suffering from Bone Marrow Failure and in

the mean time for less invasive treatment options and better outcomes. In the future Daniel wants to join the research team as a Haematologist and researcher that will help put an end to those impacted by such conditions.

Cheryll and Matty

Matty is a 14 year old young man living with Fanconi Anaemia CD2. He lives with his grandmother Honey (Cheryll) in Tasmania and has a younger brother Nathan, who he spends every second weekend with Matty and they have a beautiful bond.

He has a fantastic quirky sense of humour and draws people to him wherever he goes. He doesn't let anything stop him and leads a busy full life! He is so hopeful that someone one day will discover a cure for FA so that other kids don't have to endure what he does.



1 - Daniel with Charlie the golden retriever



2 - Matty with Cloud the cockatiel

Maddie Riewoldt's Vision

Maddie Riewoldt's Vision held their third National Symposium on Bone Marrow Failure Syndromes on Saturday October 14th and Sunday October 15th 2023 at the Peter MacCallum Cancer Centre in Melbourne. It was a great opportunity to hear about the latest research into current treatments and research on BMFS.

It was wonderful to meet our Principle Investigators and registry supporters.

Ashleigh Scott (Royal Brisbane & Women's), Tina Carter (Perth Children's) Stephen Ting (Box Hill), Lucy Fox (PMAC), Adam Nelson (Sydney Children's), Erica Wood (Monash University), Sue Morgan (Alfred Hosp). Julie Curtin (Children's at Westmead), Frank Firkin (Chair, AAR Steering Committee), Tatjana Kilo (Children's at Westmead), Lauren Young (Monash University), Melissa Southey (Biobanking Victoria), Zoe McQuilten (Monash University), Juliana Teo (Children's at Westmead), Neil Waters (Monash University), Vanessa Fox (Monash University)



3 - AAR Management group, Pls and supporters at the 2023 MRV Symposium

Maddie Riewoldt's Vision.

Contact Us

If you have any questions about the AAR or would like more information, please contact us

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The AAR acknowledges support from

Participants and sites (The Alfred, Austin Hospital, Box Hill Hospital, Calvary Mater Newcastle, Canberra Hospital, Children's at Westmead, Concord Hospital, Fiona Stanley Hospital, Flinders Medical Centre, Geelong Hospital, Gosford Hospital, John Hunter Children's Hospital, Liverpool Hospital, Mater Brisbane, Monash Medical Centre, Nepean Hospital, Peter MacCallum Cancer Centre, Perth Children's Hospital, Prince of Wales Hospital, Princess Alexandra Hospital, Queen Elizabeth Hospital, Queensland Children's Hospital, Royal Adelaide Hospital, Royal Brisbane and Women's Hospital, Royal Children's Hospital Melbourne, Royal Hobart Hospital, Royal Melbourne Hospital, Royal North Shore, Royal Perth Hospital, Royal Prince Alfred Hospital, Sir Charles Gairdner Hospital, St George Hospital, St Vincent's Hospital Melbourne, St Vincent's Hospital Sydney, Sydney Children's Hospital, Townsville Hospital, Western Hospital, Westmead Hospital, Wollongong Hospital, Women's and Children's Hospital), AAR steering committee, Maddie Riewoldt's Vision and the Registry management team

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