

## AAR Newsletter - August 2024

Welcome!

# Aplastic Anaemia and other Bone Marrow Failure Syndromes Registry



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*Welcome to the Aplastic Anaemia and Other Bone Marrow Failure Syndromes Registry (AAR) newsletter.*

*We are pleased to provide you with updates to the Registry's progress, important information to note, current activities and future events in upcoming editions.*

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## Registry Progress and Overview

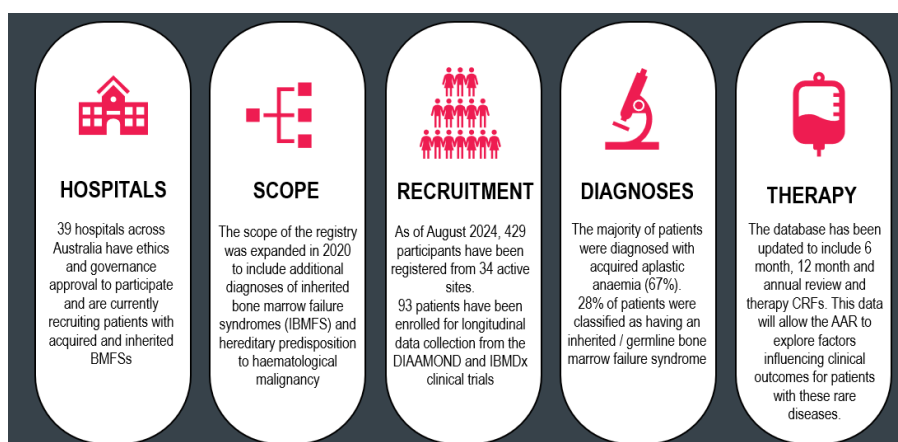
The registry has made some major updates in the past few months.

Sites are now eligible for a per patient payment to offset some of the costs of data entry. The inclusion of a per patient payment for data entry at sites has seen an increase in patient registrations and data capture. If your site has not received an updated contract to permit these payments, please contact the AAR Team ([aar@monash.edu](mailto:aar@monash.edu)).

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 DIAAMOND clinical trial<sup>1</sup> is being conducted to see if the addition of a TPO mimetic, avatrombopag, to standard of care in AA patients is now closed to recruitment and primary analysis is underway. Thank you to all staff who have assisted in this clinical trial.

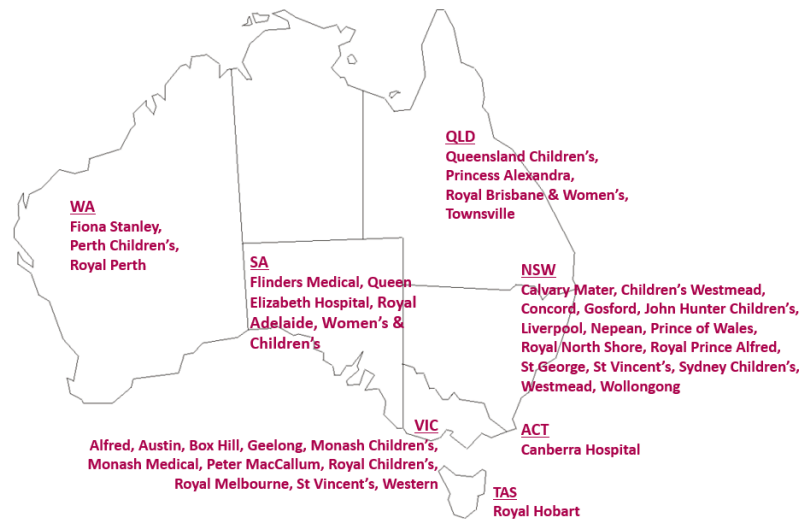
Lastly, a proposed amendment has been submitted for review. This amendment will impact the project protocol, participant information brochure and eCRF. We will be circulating the details of the amendment in our next newsletter - stay tuned!



<sup>1</sup><http://www.anzctr.org.au/ACTRN12619001042134.aspx>

## Participant Accrual

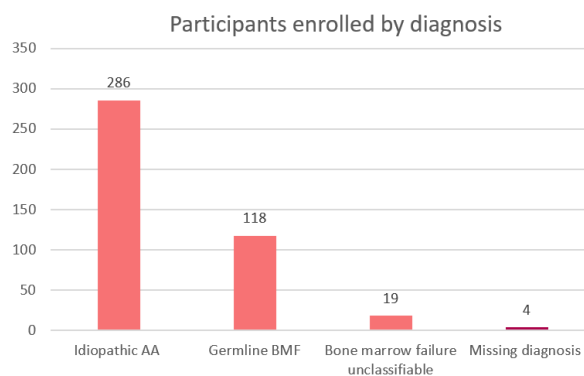
Participant accrual has continued to increase, with over 400 participants enrolled across 34 active sites in Australia. Thank you to all site Principal Investigators and research staff - we appreciate all your hard work and support of the Registry! Your ongoing contribution to a national complete dataset on bone marrow failure syndromes advances and supports scientific studies of these rare diseases.



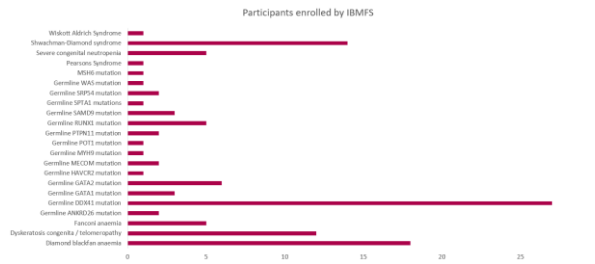
1 - Map of participating sites.

## 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 **28% of diagnoses**.



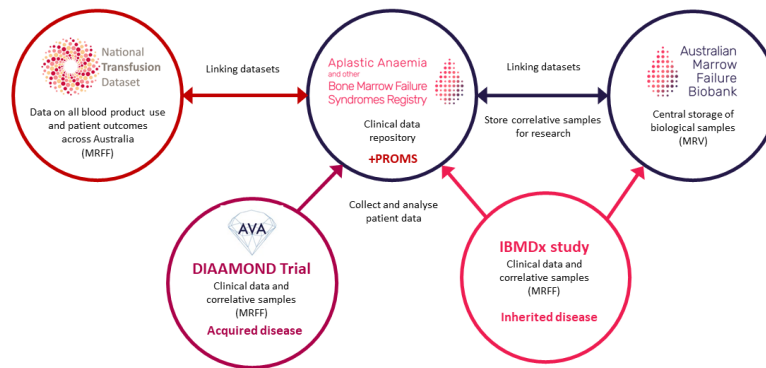
2 - Graph of participants enrolled by diagnosis.



3 - Graph of Inherited BMFS collected on the Registry.

## Associated Projects

The registry continues to provide infrastructure support and longitudinal data capture for a number of studies.



## IBMDx Study

The inherited bone marrow failure and related disorders (IBMDx)<sup>2</sup> 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 understanding of these conditions. **107 participants have been recruited**

For further information please email [IBMDxStudy@petermac.org](mailto:IBMDxStudy@petermac.org)<sup>3</sup>



<sup>2</sup><https://www.anzctr.org.au/Trial/Registration/TrialReview.aspx?id=20239&isClinicalTrial=True>

<sup>3</sup><mailto:IBMDxStudy@petermac.org>

## 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 in mid-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 [amfbiobank@monash.edu](mailto:amfbiobank@monash.edu)**



## Database Changes

The AAR Database has had a few minor changes to facilitate the data entry process. Although listed in the latest database user manual (version 4), the changes are:

- 'Bone marrow failure diagnosis' changed to 'status of diagnosis' to include working diagnosis (if a diagnosis is yet to be determined)
- Additional fields added when abnormal bone marrow cytogenetics is selected in the Investigations form
- Adding an additional field to therapy forms to capture millilitres of red blood cells provided to paediatric participants when transfused
- Specified outcome of bone marrow transplant is collected at 100 days

Please contact the AAR Team if you require a copy of the latest database user manual (version 4, dated 23rd July 2024).



## Contact Us

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

**Email:** [aar@monash.edu](mailto:aar@monash.edu)<sup>4</sup>

**Phone:** 1800 811 326 | 03 9903 0532

**Website:** AAR <sup>5</sup>

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 AAR team.*

Please email us to unsubscribe<sup>6</sup> from future newsletters.



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<sup>4</sup><mailto:aar@monash.edu>

<sup>5</sup><https://aaregistry.org.au/>

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