

# AAR Newsletter - December 2024

Welcome!

## Aplastic Anaemia and other Bone Marrow Failure Syndromes Registry



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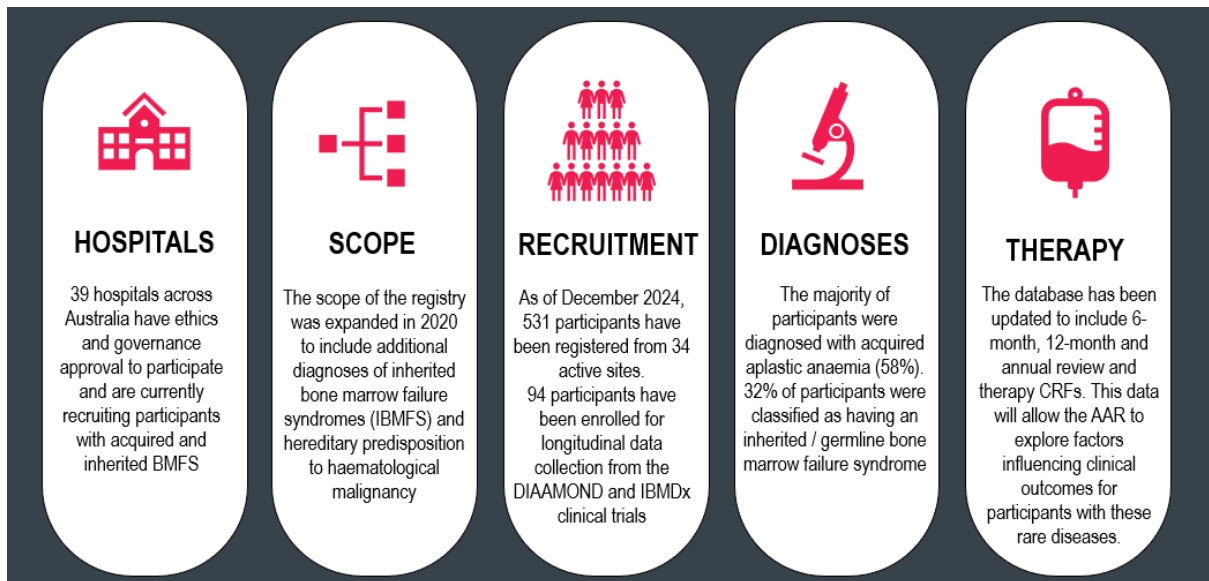
*Welcome to the Aplastic Anaemia and Other Bone Marrow Failure Syndromes Registry (AAR) newsletter for December! We are pleased to provide you with updates to the Registry's progress, important information to note, current activities and future events.*

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### In This Issue

In this edition of the newsletter:

- **Welcome**
- **Registry progress and overview**
- **Project amendments**
- **Data snapshot**
- **Projects and collaborations**
- **Holiday closure period**
- **Contact us**



## Registry progress

Accrual continues to increase, with over 500 participants now enrolled across 34 active sites in Australia. Thank you to all participants and families, site Principal Investigators and research staff - we appreciate all your hard work and support of the Registry!

We are also looking to expand to New Zealand in 2025, so stay tuned for news on that front!



1 - Map of participating sites.

A reminder that sites are now eligible for a 'per patient payment' to help offset the costs of your staff time for each participant recruited. 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)).

## Project Amendments

A number of changes to the project outline and participant information brochure (PIB) have recently been approved by the lead HREC. These include:

- An update to acquired and inherited bone marrow failure syndromes captured on the database.
- Inclusion of blood relatives of AAR participants. For this reason a new PIB has been created to provide to relatives of participants inviting them to part-take in the AAR.
- Update to data collection, use and disclosure.
- Collection of data on pregnancy history and fertility preservation.

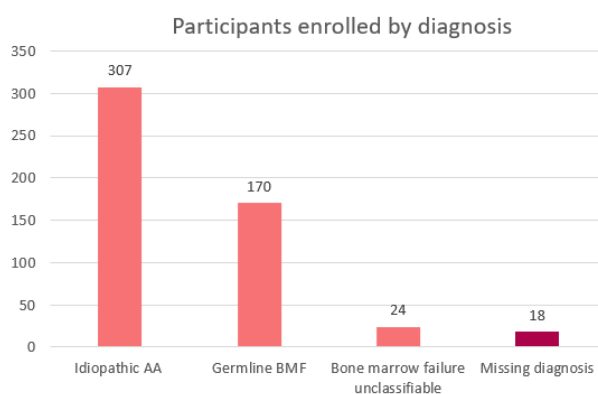
The AAR project team will circulate the approved master copies for the AAR project outline and PIBs in the new year - one for participants and one for relatives of participants.

Please do not hesitate to contact us if you require any assistance with the amendment submission to your RGO. If your site has capacity to send your RGO an amendment submission, we ask that you provide us with a copy of your submission and approval.

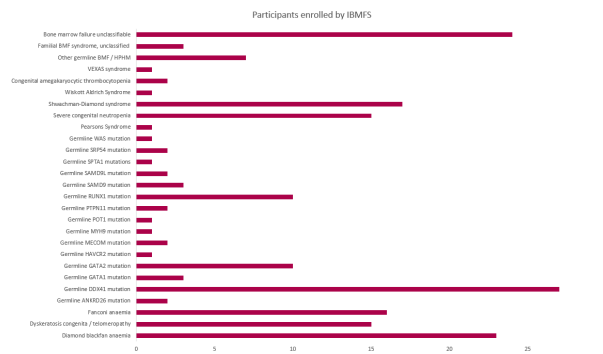
New electronic case report forms (eCRFs) for fertility preservation and for the Australian Marrow Failure Biobank have been added to the database. These are listed in the latest database user manual. Please contact the AAR Team if you require a copy of the manual (version 5, dated 12 December 2024) and/or if you would like to receive further data entry training at your site.

## Data Snapshot

The expanded scope of the registry has resulted in an overall increase in participants, and inherited bone marrow failure syndromes now account for 32% of diagnoses. Please note that 18 participants are missing a diagnosis, so we would appreciate it if sites could check this information is completed on all your participants. Thank you!



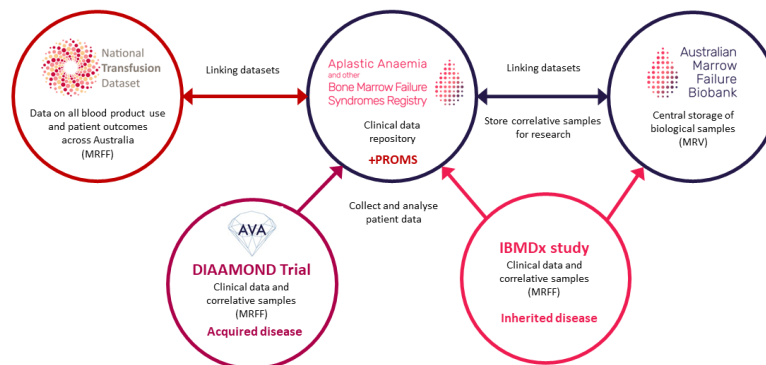
2 - Graph of participants enrolled by diagnosis.



3 - Graph of inherited BMFS collected on the AAR.

## Projects and collaborations

The registry provides infrastructure support and longitudinal data capture for the DIAAMOND Trial, the IBMDx study and the new Australian Marrow Failure Biobank. Please see below an update on each study. If you have an idea or a suggestion for a project using the AAR data, please get in touch – our contact details are below.



## DIAAMOND Trial

The MRFF-funded DIAAMOND trial of avatrombopag in severe aplastic anaemia completed recruitment in early 2024 and the first results were presented at the recent American Society of Hematology congress in San Diego.

Links to the ASH abstracts:

<https://ash.confex.com/ash/2024/webprogram/Paper209378.html>

<https://ash.confex.com/ash/2024/webprogram/Paper208841.html>

Work is now underway drafting the first manuscript from the study.

More information on the trial is available at: <https://aaregistry.org.au/clinical-trials-diamond>



### *IBMDx Study*

The MRFF-funded inherited bone marrow failure and related disorders (IBMDx)<sup>1</sup> study addresses the challenges associated with diagnosis of these genetically diverse and rare diseases.

For further information please email [IBMDxStudy@petermac.org](mailto:IBMDxStudy@petermac.org)<sup>2</sup>



### *Australian Marrow Failure Biobank*

Thanks to support from Maddie Riewoldt's Vision, and in partnership with Biobanking Victoria, the AMFB will collect, process and store matched tissue sample from participants with an established or newly presenting diagnosis of a bone marrow failure syndrome.

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<sup>1</sup><https://www.anzctr.org.au/Trial/Registration/TrialReview.aspx?id=20239&isClinicalTrial=True>

<sup>2</sup><mailto:IBMDxStudy@petermac.org>

We are excited to announce that Peter MacCallum Cancer Centre and Concord Hospital are approved to contribute participant samples to the biobank. Perth Children's Hospital will commence sample collection in January 2025 and we are finalising governance approval at 3 additional pilot sites early next year.

For more information on the AMFB please refer to the AMFB page on our website:

<https://aaregistry.org.au/australian-marrow-failure-biobank>

If you are an existing AAR site interested in participating in the biobank, please contact [amfbiobank@monash.edu](mailto:amfbiobank@monash.edu)<sup>3</sup>



### *International Telomere Biology Disorder Collaboration*

The AAR has contributed de-identified data on Australian participants with a telomere biology disorder (TBD) to the Clinical Care Consortium of Telomere-Associated Ailments (CCCTAA) Database. If your site would like to contribute to the database, please speak to your patients and families with TBDs about enrolment in the AAR. We anticipate that this important work will foster numerous future research opportunities.

### *International ANKRD26-Related Thrombocytopenia Collaboration*

The AAR and other international institutions are collaborating on a study led by the University of Wisconsin-Madison of clinical outcomes in patients with germline ANKRD26 variants. Please reach out to your patients with germline ANKRD26 variant for enrolment to the AAR and contributions to this international collaboration. Sites will be eligible for per patient payments for all newly enrolled participants.

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<sup>3</sup><mailto:amfbiobank@monash.edu>

## Holiday closure period

As we approach the end of the year, the AAR project team would like to say a big 'thank you' for your continued support throughout the year!

Our team is taking a short break from **Saturday 21st December to Wednesday 1st January 2025, reopening on 2nd January.**

We wish you a safe and happy holiday season and look forward to working with you in 2025!



## Contact Us

If you have any questions about the AAR or would like more information, or would like to propose a research project using AAR data, please contact us.

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

**Phone:** 1800 811 326 | 03 9903 8267

**Website:** [AAR](https://aaregistry.org.au/)<sup>5</sup>

The AAR acknowledges support from the:

- *Participants*
- *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, The Royal Melbourne Hospital, Royal North Shore, Royal Perth Hospital, Royal Prince Alfred Hospital, St George Hospital, St Vincent's Hospital Melbourne, St Vincent's Hospital Sydney,*

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

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

*Sydney Children's Hospital, Townsville Hospital, Western Hospital, Westmead Hospital, Wollongong Hospital, and Women's and Children's Hospital*

- *AAR Steering Committee: Frank Firkin, Xavier Badoux, Ashish Bajel, Paddy Barbaro, Merrole Cole-Sinclair, Cecily Forsyth, John Gibson, Devendra Hiwase, Anna Johnston, Zoe McQuilten, Tony Mills, Lynda Morakis, Ashvind Prabahran, Cheryl Opperman, Fernando Roncolato, Jeff Szer, Stephen Ting, Shahla Vilcassim, Neil Waters and Erica Wood.*
- *Maddie Riewoldt's Vision*
- *AAR Working Group: Lucy Fox, Vanessa Fox, Robyn Sutherland, Lauren Young and Prislene Singh*



4 - Members of the AAR Steering Committee, Investigators and Staff attending the 2023 National Symposium on Bone Marrow Failure Syndromes. Photo credit: Maddie Riewoldt's Vision.

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



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<sup>6</sup><mailto:aar@monash.edu>