Friday 23rd May 2025 - 2.30pm – 7.30pm

Opening Plenary and Welcome Reception Sponsored by Zouki	
Opening Plenary	
2.30 – 2.45pm	Open & Welcome – Professor David Ritchie (Director, Maddie's Vision Centre of Research Excellence in Bone Marrow Biology)
Keynote presentation **Chair: Professor David Ritchie**	
2.45 – 3.30pm	**Keynote Presentation:** Dr Marcin Wlodarski (St Jude Children's Research Hospital, United States) *Bone Marrow Failure Syndromes – diagnosis, therapy and surveillance*

3.30 – 4.15pm: Afternoon tea

Patient and Family Presentations **Chair: Dr Lucy Fox**	
4.15 – 4.30pm	Ross Forsyth
4.30 – 4.45pm	Anna McMahon
Maddie Riewoldt's Vision – 10 Years of Impact **Chair: Amy Coote**	
4.45 – 5.30pm	Amy Coote (Maddie's Vision CEO) *Ten Years On: A Legacy of Impact. A Future of Hope.*
	Mei Ling Yeh (Maddie's Vision Tele-Support Service Nurse) *Maddie's Vision National Support Services - Progress and Impact*
Welcome Reception:	
5.30 – 7.30pm	Canapes and drinks served on Level 13

END OF DAY 1

Saturday 24th May 2025 - 9am – 5.30pm

9.00 – 9.15am	**Symposium Welcome:** Amy Coote (Maddie's Vision CEO) & Professor David Ritchie (Chair Maddie's Vision SAC & CRE)
Session 1: The Bone Marrow Niche **Chair: Dr Ashvind Prabahran**	
Sponsored by Novartis	
9.15 - 9.55am	Dr Marta Derecka (St. Jude Children's Research Hospital, United States) *Transcriptional control of bone marrow niche in normal and pre-malignant hematopoiesis*
9.55 - 10.15am	Professor David Ritchie (Royal Melbourne Hospital) *Is poor graft function allo-immune aplastic anaemia?*
10.15 - 10.35am	Dr Vashe Chandrakanthan (University of Adelaide) *Bioengineered adult human endothelial cell derived haematopoietic stem cells re-establish haematopoiesis in failed bone marrow*
10.35 - 10.55am	Dr Raymond Yip (Walter and Eliza Hall Institute of Medical Research) *Spatial transcriptomics analysis of bone marrow microenvironment*

10.55 – 11.30am: Morning tea

Session 2: Acquired Aplastic Anaemia and Bone Marrow Transplant **Chair: Professor David Ritchie**	
Sponsored by Sobi	
11.30 - 11.50am	Professor Zoe McQuilten (Monash University) *Efficacy and safety of avatrombopag in addition to immunosuppressive therapy (IST) for patients with previously untreated sAA*
11.50 - 12.10pm	Dr Ashvind Prabahran (Monash Health) *Safety and Efficacy of First Line Immunosuppression with ATG and Ciclosporin in Older Patients with Aplastic Anaemia*
12.10 - 12.30pm	Rachel Edwards (Children's Health Queensland Hospital and Health Service) *Understanding the Symptom Burden of Paediatric Bone Marrow Transplant: Insights from Patient Reported Outcome Measures*
12.30 - 12.50pm	Alice Maier (The Royal Children's Hospital, Melbourne) *The Role of Neuropsychology in Haematopoietic Stem Cell Transplant Planning for Bone Marrow Failure Syndromes*

12.50 – 1.50pm: Lunch

Session 3: Emerging Bone Marrow Failure Conditions and Novel Approaches to Therapy **Chair: Professor Tracy Bryan**	
Sponsored by Takeda	
1.50 - 2.10pm	Dr Marcin Wlodarski (St. Jude Children's Research Hospital, United States) *MDM4 haploinsufficiency leads to P53-mediated bone marrow failure*
2.10 - 2.30pm	Professor Graham Lieschke (Monash University) *Evaluating new genes and mutations for bone marrow failure disorders*
2.30 - 2.50pm	Dr Sean Harrop (Peter MacCallum Cancer Centre) *Heterozygous germline TET2 loss of function variants associated with an ALPS-like phenotype*
2.50 - 3.10pm	Dr Miles Horton (Walter and Eliza Hall Institute of Medical Research) and Dr Thomas Lew (Peter MacCallum Cancer Centre / Royal Melbourne Hospital) *Prime editing to correct genetic drivers of haematologic diseases with minimal off-target toxicity and tuneable in vivo selection*
3.10 - 3.30pm	Dr Ashley Yang (Children's Medical Research Institute, Westmead) *Disease modelling and gene therapy for telomere-related bone marrow failure*

3.30 – 4.05pm: Afternoon tea

Session 4: Focus on Fanconi Anaemia **Chair: Associate Professor Adam Nelson**	
4.05 – 4.25pm	Dr Lorna McLeman (St. Vincent's Institute of Medical Research) *Novel gene editing approaches for Fanconi anaemia*
4.25 – 4.45pm	Associate Professor Wayne Crismani (St. Vincent's Institute of Medical Research) *New tools to identify cases of Fanconi anaemia*
Virtual Keynote presentation chair: Associate Professor Adam Nelson	
4.45pm - 5.30pm	**Virtual Keynote Presentation:** Dr Paula Río (Centro de Investigaciones Energéticas, Medioambientales y Tecnológicas, Madrid, Spain) *Hematopoietic stem cell gene therapy: Overcoming barriers in Fanconi anemia*

END OF DAY 2

Sunday 25th May 2025 – 9am – 1.10pm

Keynote presentation chair: Associate Professor Piers Blombery	
9.00 – 9.45am	**Keynote presentation:** Dr Marcin Wlodarski (St Jude Children's Research Hospital, United States) *Somatic gene rescue in SAMD9 and SAMD9L syndromes: natural gene therapy or sword of Damocles?*
Session 1: Somatic Mosaicism in Inherited Bone Marrow Failure Syndromes **Chair: Associate Professor Piers Blombery**	
9.45 – 10.05am	Associate Professor Chris Hahn (Centre for Cancer Biology, University of South Australia) *ERG deficiency syndrome – a new autosomal dominant bone marrow failure syndrome displaying somatic genetic rescue*
10.05 – 10.25am	Dr Parvathy Venugopal (Centre for Cancer Biology, University of South Australia) *Somatic Genetic Rescue in Bone Marrow Failure – Implications and Opportunities*
10.25 – 10.45am	Dr Lucy Fox (Peter McCallum Cancer Centre) *Donor-derived somatic genetic rescue after allogeneic stem cell transplantation from a SAMD9-affected sibling donor*

10.45 – 11.20am: Morning tea

Session 2: Bone Marrow Failure Diagnostics – Somatic and Germline Characterisation **Chair: Dr Lucy Fox**	
11.20 – 11.40am	Associate Professor Piers Blombery (Peter MacCallum Cancer Centre) *Whole genome sequencing for diagnosis in bone marrow failure – the IBMDx study*
11.40 – 12pm	Dr Vahid Pazhakh (Peter MacCallum Cancer Centre) *Pursuing diagnosis after whole genome sequencing, insights from the IBMDx study*
12 – 12.20pm	Associate Professor Tina Carter (Perth Children's Hospital) *A review of paediatric and adolescent patients diagnosed with Inherited Bone Marrow Failure Syndromes (IBMFS) in Western Australia at Perth Children's Hospital.*
12.20 – 12.40pm	Emma Murphy (Peter MacCallum Cancer Centre) *Sensitive genomic characterisation of the clonal haematopoietic landscape in individuals with germline GATA2 haploinsufficiency*
12.40 – 1pm	Dr Simon Wu (Peter MacCallum Cancer Centre) *The genomic profiles of patients with UBA1 variants across haematological malignancies*
1pm – 1.10pm	Closing comments and questions

END OF DAY 3