Saturday 14th October – 9am – 5pm

9.00 - 9.15 am	**Symposium Welcome:** Amy Coote (MRV CEO) & Professor David Ritchie (Chair MRV SAC & CRE)
Keynote presentation chair – Associate Professor Wayne Crismani	
9.15 – 10.00 am	**Keynote presentation:** Professor Rodrigo Calado (University of São Paulo, Brasil) *A new era in the treatment of aplastic anaemia: novel drugs and strategies*
Session 1: Future treatments including gene editing **Chair: Associate Professor Wayne Crismani**	
Sponsored by St Vincent's Institute of Medical Research	
10.00 – 10.15 am	Ariel Simpson (PhD candidate, University of Tasmania) *Analysing CRISPR base-editing efficiencies for Bone Marrow Failure targets*
10.15 – 10.30 am	Dr Lorna McLeman (St Vincent's Institute of Medical Research) *Precision gene editing for Fanconi Anaemia I: A tale of Men*
10.30 – 10.45 am	Dr Astrid Glaser (St Vincent's Institute of Medical Research) *Precision gene editing for Fanconi Anaemia II: A tale of Mice*
10:45 – 11:00 am	Associate Professor Adam Nelson (Sydney Children's Hospital) *Metabolic alterations in Fanconi Anaemia and potential therapeutic strategies*

11.00 – 11.30am: Morning tea

Session 2: Gene discovery and stem cell biology **Chair: Professor Tracy Bryan**	
Sponsored by Novartis	
11.30 – 11.45 am	Professor Graham Lieschke (Australian Regenerative Medicine Institute) *Evaluating new severe congenital neutropenia disease genes in zebrafish models*
11.45 – 12.00 pm	Dr Katharine Goodall (Murdoch Children's Research Institute) *Developing curative therapy for complex immune disorders caused by hypomorphic RAG1 mutations*
12.00 – 12.15 pm	Dr Kirsten Fairfax (University of Tasmania) *Immunogenetics and its implications for bone marrow failure syndromes*
12.15 – 12.30 pm	Dr Vashe Chandrakanthan (The University of Adelaide) *Bioengineered Haematopoietic Stem Cells and Stromal Cells Re-establish Haematopoietic Stem Cell Niche in Failed Bone Marrow*
12:30 – 12:45 pm	Ryan Collinson (PhD candidate, University of Western Australia) *Loss of TCF3 is associated with progression to bone marrow scarring and failure in myeloproliferative neoplasms*

12.45 – 1.45 pm: Lunch

Session 3: Inherited haematology: elements of diagnosis and management **Chair: Dr Lorna McLeman**	
Sponsored by AstraZeneca	
1.45 – 2.00 pm	Dr Lucy Fox (Peter MacCallum Cancer Centre) *The evolution of the genetic haematology service at Peter MacCallum Cancer Centre and Royal Melbourne Hospital*
2.00 - 2.15 pm	Dr Sharon Savage (National Cancer Institute, USA) *Cancer screening approaches in Fanconi anaemia*
2.15 – 2.25 pm	Dr Diva Baggio (Peter MacCallum Cancer Centre) *Approach to managing healthy individuals with germline predisposition to haematologic malignancy: The example of blood donation*
2.25 – 2.35 pm	Dr Eliska Furlong (Perth Children's Hospital) *A demonstrative case of personalised genomics in diagnosis and management of a child presenting with hypocellular myelodysplastic syndrome.*
2.35 – 2.45 pm	Dr Michelle Tan (Peter MacCallum Cancer Centre) *A novel germline SAMD9 variant in a paediatric patient with bone marrow failure, with longitudinal molecular analysis of a somatic rescue event*

2.45 – 3.15 pm: Afternoon tea

Session 4: Disease management and collaborations **Chair: Dr Lucy Fox**	
Sponsored by Alexion	
3.15 – 3.30 pm	Rachel Edwards (PhD candidate, Queensland Children's Hospital) *Optimising symptom management in young people receiving Bone Marrow Transplantation: Symptom-PROMPT*
3.30 – 3.45 pm	Alice Maier (Royal Children's Hospital, Melbourne) *Utility of a neurobehavioral assessment for treatment planning, educational, and family support for children undergoing bone marrow transplantation for non-malignant disease*
3.45 – 4.00 pm	Dr Ashleigh Scott (Royal Brisbane and Women's Hospital) *Lower dose antithymocte globulin as frontline treatment for adult acquired aplastic anaemia*
4.00 – 4.20 pm	Professor Erica Wood and Professor Melissa Southey (Monash University) *The Aplastic Anaemia and Other Bone Marrow Failure Syndromes Registry (AAR) and Australian Marrow Failure Biobank (AMFB)*
4.20 – 4.35 pm	Associate Professor Stephen Ting (Monash University) *An update of the DiAAMOND-Ava-First and DiAAMOND-Ava-Next Bayesian Optimal Phase II Trials studying the Efficacy and Safety of Avatrombopag in*

	Combination with Immunosuppressive Therapy in Treatment Naïve and Relapsed/Refractory Severe Aplastic Anaemia.
4.35 – 4.45pm	Dr Sharon Savage (National Cancer Institute, USA) *International telomere biology disorder registry collaboration: CCCTAA*
4.45 – 5.00pm	Closing comments and questions – Dr Lucy Fox (Peter MacCallum Cancer Centre)

Sunday 15th October – 9am- 1pm

Session 5: Telomere Biology Disorders: functional genomics **Chair: Associate Professor Piers Blombery**	
Sponsored by Link Healthcare	
9.00 – 9.45am	**Keynote presentation:** Dr Sharon Savage (National Cancer Institute, USA) *Genotype-phenotype relationships in the Telomere Biology Disorders*
9.45 – 9.55am	Telomere Biology Disorder case study – Dr Lucy Fox (Peter MacCallum Cancer Centre)
9.55 – 10.10am	Professor Tracy Bryan (Children's Medical Research Institute, Westmead) *The role of functional genomics in diagnosis of Telomere Biology Disorders*
10.10 – 10.25am	Dr John Mackintosh (Prince Charles Hospital, Brisbane) *Telomere Shortening and Associated Gene Mutations in Adults with Pulmonary Fibrosis: Data from a Tertiary Australian Pulmonary Fibrosis Centre*
10.25 – 10.40am	Dr Ashley Yang (Children's Medical Research Institute, Westmead) *Development of a disease model and gene therapy for telomere-related bone marrow failure*

10.45 – 11.15am: Morning tea

Session 6: Disease mechanisms **Chair: Professor David Ritchie**	
Sponsored by Gilead	
11.15 – 11.30am	Associate Professor Rachel Koldej (Royal Melbourne Hospital) *Dissecting immune dysregulation in acquired Bone Marrow Failure Syndromes to identify new therapeutic leads*
11.30 – 11.45am	Dr Ashvind Prabahran (Royal Melbourne Hospital) *Comprehensive Evaluation of Immune Microenvironment in Poor Graft Function following Allogeneic Stem Cell Transplantation*

11.45 – 12.00pm	Associate Professor Amee George (Australian National University, ACT) *Identification of biomarkers and therapies which modulate the canonical nucleolar surveillance pathway in Diamond Blackfan Anaemia*
12.00 – 12.15pm	Dr Stephen Ma (Austin Health) *The under-recognized phenotype of germline GATA1 disease in females*
12.15 – 12.30pm	Case study – Professor Rodrigo Calado *Uncommon inherited aplastic anaemia*
12.30 – 1.00pm	Closing comments and questions