

PRELIMINARY PROGRAM *Program is subject to change

Thursday 7 November 2024

pre-existing AAV immunity

Thursday 7 November 2024		
08:00 Registration desk open		
08:00 – 08:40 Arrival tea and coffee		
08:45 – 09:00 Conference opening		
Room: Leighton Hall		
Conference opening Welcome to Country		
Session 1 Advanced Therapeutics		
Presentations		
The Kids Advanced Therapeutics odyssey – charting progress and for	ging ahead	
Panel session		
Panel: Driving investment to build and accelerate capability across advanced therapeutics translation		
Morning tea and poster presentations		
Session 2 Precision therapy for cancer therapeutic and innovative applications		
Room: Leighton Hall		
Keynote presentation		
International Keynote Speaker		
The Molecular Characterisation Initiative: precision diagnosis for paed		
Prof Elaine Mardis, Institute for Genomic Medicine at Nationwide Children's	Hospital	
Presentations series		
Setting the Australian scene: Zero Childhood Cancer Program (ZERO): Utilising precision data to drive therapeutic recommendations and to innovate discoveries		
Translational Tumour Biology – utilising Cas13 technology to identify t	herapeutic targets using a molecular biology approach	
Innovation driving precision drug targeting discoveries - translating ideas from genetic disease into cancer		
Panel session		
Transitioning precision therapy research to standard clinical care		
Lunch and poster presentations		
Session 3.1 Functional genomics and precision therapy / cell and gene	Session 3.2 Innovation and commercialisation opportunities (Part 1)	
therapy advancements: from bench to bedside		
Room: Leighton Hall	Room: Tyree Room	
Presentation series		
Cell therapy pipeline, past, present, and future success		
The world of avatar technology for cystic fibrosis and beyond		
Case study: Rett syndrome – from diagnosis to treatment		
The next steps in developing better AAV delivery systems to tackle		

Panel session	
Barriers, challenges, and opportunities to gene and cell therapy research translation and commercialisation	
Afternoon tea and poster presentations	
Session 4.1 No one left behind: truly engaging families in precision medicine	Session 4.2 Innovation and commercialisation opportunities (Part 2)
Room: Leighton Hall	Room: Tyree Room
Presentation series	Presentations
Co-designing and co-imagining the future of precision medicine – optimal patient-centred care	NAT Network – introducing the non-animal technology network
Psychosocial Enabling Platform – purpose, current and future activities	Biobanking – activities across NSW
Psychosocial Precision Medicine – Addressing the Social Determinants of Health	Clinical trials commercialisation
Panel session	Session presentation
Doing a better job of communicating with families: addressing challenges and driving solutions	Functional Genomics Enabling Platform Showcase: Facility, Laboratory Services, and Equipment to Deliver New Research Capacity
Rapid presentations: Applications of psychosocial research	
Closing address	
Room: Leighton Hall	
18:00 – 22:00 Conference dinner	
The Lounge, UNSW Sydney	

Friday 8 November 2024

08:00 Registration desk open		
08:00 – 08:40 Arrival tea and coffee		
Session 5.1 Precision medicine informed by real world data	Session 5.2 N=1 clinical trials in paediatric advanced therapies	
Room: Leighton Hall	Room: Tyree Room	
Keynote presentation	Panel session	
 International Keynote Speaker Practicalities of using real-world electronic health data for research: insights from the UK, Great Ormond Street Hospital clinical informatics research program Prof Neil Sebire, Great Ormond Street Hospital Institute of Child Health Presentation series Advanced genome and transcriptome analytics - using comprehensive molecular profiling techniques accelerating the uptake of precision medicine Advanced clinical data analytics: creating an ecosystem to access, extract and interpret clinical eMR and administrative data Kids Link: utilising data linkage to advance data-driven healthcare to strengthen our health system Panel session Harnessing real world data for patient benefit 		
Morning tea and poster presentations		

<u>Session 6</u> Health Systems Implementation and Evaluation and precision therapy - delivering outcomes-focused sustainability (for children) in the era of precision

Room: Leighton Hall

Keynote presentation

International Keynote Speaker

The need to evaluate health services innovations at scale: challenges and opportunities in the US and Australia

Prof Gary L Freed, University of Michigan

Presentations series

Health systems implementation and health economics

What is PEACH and PEACH-E, current update, next steps

Panel session

Thinking outside the silos: innovations to optimise the health system for children

Lunch	
Session 7.1 The road ahead in genomics and genetic testing	Session 7.2 Workshop
Room: Leighton Hall	Room: Tyree Room
Presentation series	Health Systems Implementation and Economics (HSIE) & Psychosocial Workshop
The future direction for genomics testing: a global perspective on improving access to genomics knowledge and technology	
Genomic testing - what is the roadmap for Australia	
Rare Diseases NSW	
Panel session	
Challenges of sustained translation in genomic screening and testing: battle scars, lessons learned, and priority actions to move forward	
Afternoon tea and poster presentations	
Panel session	
Room: Leighton Hall	
Reflections and next steps for paediatric precision medicine	
Conference close	