



****PROGRAM SUBJECT TO CHANGE****

Thursday 7 November 2024

08.00	Registration desk open	
08.00 - 08.45	Arrival tea and coffee	
08.45 - 09.00	Conference Opening Room: Leighton Hall Conference Opening, Luminesce Alliance Chair Ms Kathryn Greiner AO Welcome to Country, La Perouse Local Aboriginal Land Council	
09.00 - 10.45	SESSION 1 - Advanced Therapeutics Chair: Adj A/Prof Paula Bray, Sydney Children's Hospitals Network	
9.00	Keynote presentation NSW Health Research Strategy Update Dr Kim Sutherland, OHMR, NSW Health	
9.30	Perspectives from Family Members Interview Session	
9.45	Presentation The Kids Advanced Therapeutics Odyssey - Charting progress and Forging Ahead Dr Michelle Lorentzos, Sydney Children's Hospitals Network	
10.00	Panel Session - Interactive Q&A discussion session Opportunities and Challenges for Advanced Therapeutics: clinical translation, access, regulation, commercial and funding landscape Facilitator: Ms Lani Attwood, Sydney Children's Hospitals Network Panel members: Dr Michelle Lorentzos, Sydney Children's Hospitals Network Prof Ian Alexander, Children's Medical Research Institute Dr Paul Fennessy, Paul Fennessy Advisory Vanessa Fitzgerald, NSW Health	
10.45 - 11.15	Morning tea and Poster Presentations	
11.15 - 13.00	SESSION 2.1 - Precision Therapy for Cancer Therapeutic and Innovative Applications Sponsor logo Room: Leighton Hall Chair: Prof Michelle Haber, Children's Cancer Institute	SESSION 2.2 - Conference Abstracts Presentations Room: Tyree Room Chair: Dr Laura Fawcett, Sydney Children's Hospitals Network
11.15	Keynote Presentation The Molecular Characterisations Initiative: precision diagnosis for paediatric cancers Prof Elaine Mardis, Institute for Genomic Medicine at Nationwide Children's Hospital	11.15 Treatments for those who have none platform: from Western Australia to nationwide Dr May Aung-Htut, Murdoch University, Centre for Molecular Medicine & Innovative Therapeutics

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11.50	<p>Setting the Australian scene: what is ZERO? Utilising precision data to drive therapeutic recommendations and to innovate discoveries</p> <p>Prof Michelle Haber, Children's Cancer Institute</p>	<p>11.30 Testing gene replacement therapy in retinitis pigmentosa and Leber congenital amaurosis</p> <p>Dr To Ha Loi, Children's Medical Research Institute</p>
11.55	<p>Translational Tumour Biology – utilising Cas13 technology to identify therapeutic targets using a molecular biology approach</p> <p>A/Prof Paul Ekert, Children's Cancer Institute</p>	<p>11.45 Pre-natal AAV gene therapy for HBSL disease in Dars1 transgenic mice</p> <p>Miss Elena Venuti, UNSW Sydney</p>
12.10	<p>Innovation driving precision drug targeting discoveries – translating ideas from genetic disease into cancer</p> <p>Dr Antoine de Weck, Children's Cancer Institute</p>	<p>12.00 Gene Therapy for Brittle Bone Disease</p> <p>A/Prof Aaron Schindeler, Sydney Children's Hospitals Network</p>
12.25	<p>Utilising high-throughput drug screening for therapeutic and innovative outputs</p> <p>Dr Gabor Tax, Children's Cancer Institute</p>	<p>12.15 PeptAlde: Accelerating Antimicrobial Peptide Discovery Using AI Ensemble Techniques</p> <p>Ms Lauren Lu, Children's Medical Research Institute</p>
12.40	<p>Panel discussion: Transitioning precision therapy research to standard clinical care.</p> <p>Facilitator: Prof Elaine Mardis, Institute for Genomic Medicine at Nationwide Children's Hospital</p> <p>Panel members:</p> <p>A/Prof Paul Ekert, Deputy Director – Research Themes, Children's Cancer Institute</p> <p>Prof David Ziegler, Sydney Children's Hospitals Network</p> <p>Prof Ian Street, Director, Therapeutic Innovations for Kids (THINK), Children's Cancer Institute</p> <p>A/Prof Ness Tyrrell, Children's Cancer Institute</p>	<p>12.30 Modelling PROM1 retinal disease in a mouse model and human retinal organoids</p> <p>Dr William Yates, Children's Medical Research Institute</p> <p>12.45 Using behavioural theories to implement novel communication resources for healthcare professionals</p> <p>Miss Amber-Marie Firriolo, Behavioural Sciences Unit (UNSW)</p>
13.00 – 14.00	Lunch and Poster Presentations	
14.00 – 15.30	<p>SESSION 3.1 - Functional Genomics and Precision Therapy: Cell And Gene Therapy Advancements: From Bench to Bedside</p> <p>Room: Leighton Hall</p> <p>Chair: Prof Roger Reddel</p>	<p>SESSION 3.2 - Innovation and Commercialisation Opportunities: Paediatric Cancer Drug Discovery Innovation and the Road to Commercialisation</p> <p>Room: Tyree Room</p> <p>Chair: Prof Richard Henry, UNSW Sydney</p>
14.00	<p>Cell therapy pipeline, past, present, and future success</p> <p>A/Prof Anai Gonzalez-Cordero, Children's Medical Research Institute</p>	<p>Why is novel drug discovery needed for paediatric cancer?</p> <p>Dr Emily Mould, Children's Cancer Institute.</p>
14.15	<p>The world of avatar technology for cystic fibrosis and beyond</p> <p>Dr Shafagh Waters, UNSW Sydney</p>	<p>Collaborative drug discovery models: how can drug discovery for paediatric cancers be accomplished collectively?</p> <p>Prof Ian Street, Children's Cancer Institute</p>
14.30	<p>Case study: Rett syndrome – from diagnosis to treatment</p> <p>A/Prof Wendy Gold, University of Sydney</p>	<p>What are the challenges in finding novel drugs for paediatric cancers?</p> <p>A/Prof Antoine De Weck, Children's Cancer Institute</p>
14.45	<p>Overcoming the challenges posed by pre-existing immunity to AAV viral vectors</p> <p>A/Prof Grant Logan, Children's Medical Research Institute</p>	

14:45	<p>Panel Session</p> <p>Barriers, challenges, and opportunities to gene and cell therapy research translation and commercialisation</p> <p>Facilitator: A/Prof Wendy Gold, University of Sydney</p>	<p>14.45 Panel Session</p> <p>Overcoming the roadblocks and achieving a sustainable model of paediatric cancer drug discovery and commercialisation.</p> <p>Facilitator: Prof Richard Henry</p>
15:00	<p>Panel members:</p> <p>A/Prof Anai Gonzalez-Cordero, Children’s Medical Research Institute</p> <p>Mr Brett Anslow, Consumer Stem Cell Academic TBC</p> <p>Commercialisation experience TBC</p>	<p>Panel members:</p> <p>A/Prof Greg Arndt, Head of Drug Discovery Biology, THINK, Children’s Cancer Institute</p> <p>Prof David Ziegler, Sydney Children’s Hospitals Network</p> <p>Dr Julia Warning, NSW Health</p> <p>Brandon Capital Partners representative</p> <p>Industry representative TBC</p>
15.30 - 16.00	Afternoon tea and Poster Presentations	
16:00 – 17:30	<p>SESSION 4.1 No one left behind: truly engaging families in precision medicine</p> <p>Room: Leighton Hall</p> <p>Chair: Prof Claire Wakefield, UNSW Sydney</p>	<p>SESSION 4.2 Innovation and Commercialisation Opportunities</p> <p>Room: Tyree Room</p> <p>Chair: TBC</p>
16:00	<p>Co-designing and co-imagining the future of precision medicine – optimal patient-centred care</p> <p>Kris Pierce, Child Unlimited</p>	<p>16:00 NAT Network – Non-animal technology network</p> <p>TBC</p>
16:15	<p>Psychosocial Enabling Platform – purpose, current and future activities</p> <p>Dr Kate Hetherington, UNSW Sydney</p>	<p>16:15 Health biobanking in NSW- maximising efficiency and research support</p> <p>Prof Jennifer Byrne, NSW Health Pathology</p>
16:30	<p>Psychosocial Precision Medicine – Addressing the Social Determinants of Health</p> <p>Prof Sue Woolfenden, Sydney Local Health District and University of Sydney</p>	<p>16:30 Clinical trials commercialisation</p> <p>Anne O’Neill, Director, Enterprise, International Partnerships and Clinical Trials, NSW Health</p>
16:45	<p>Panel Session</p> <p>Doing a better job of communicating with families: addressing challenges and driving solutions</p> <p>Facilitator: Prof Claire Wakefield</p> <p>Panel members:</p> <p>Prof Les White, NSW Health</p> <p>Kris Pierce, Child Unlimited</p> <p>Seaneen Wallace, Sydney Children’s Hospitals Network</p> <p>Lived experience researcher TBC</p>	<p>16:45 AAV development and translation</p> <p>A/Prof Leszek Lisowski, Children’s Medical Research Institute</p>
17:10	<p>Rapid Fire Presentations (5 minutes each)</p> <p>Adapting SIBS-ONLINE for Australian siblings of patients with genetic epilepsy</p> <p>Dr Lauren Kelada, UNSW Sydney</p> <p>Partnering with consumers to advance research and impact for inherited retinal diseases</p> <p>Dr Eden Robertson, UNSW Sydney</p> <p>Trio germline sequencing in childhood cancer: Healthcare professionals’ perspectives from PREDICT</p> <p>Miss Jacqueline Hunter, University of Melbourne/University of New South Wales</p>	<p>17:00 Functional Genomics Enabling Platform Showcase: Facility, Laboratory Services, and Equipment to Deliver New Research Capacity</p> <p>Mr Josh Studdert, Children’s Medical Research Institute</p>
17:30	Close	
18.00–22.00	Conference Dinner – The Lounge, UNSW Sydney	



Friday 8 November 2024

08:00 **Registration desk open**

08.00 - 08.45 **Arrival tea and coffee**

08.45 - 10.30 **SESSION 5.1 Precision medicine informed by real world data**

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Room: Leighton Hall

Chair: TBC

08.45 **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

09.15 **Advanced genome and transcriptome analytics - using comprehensive molecular profiling techniques accelerating the uptake of precision medicine**

A/Prof Mark Cowley, Children's Cancer Institute

09.30 **Advanced clinical data analytics: creating an ecosystem to access, extract and interpret clinical eMR and administrative data**

Prof Tom Snelling, University of Sydney

09.45 **Kids Link: Utilising data linkage to advance data-driven healthcare to strengthen our health system**

Prof Natasha Nassar, University of Sydney

10.00 **NCI Australia's Precision Medicine Infrastructure and Clinic Genomics Services**

Dr Warren Kaplan, NCI

10.10 **Panel Session**

Harnessing real-world data for patient benefit

Facilitator: TBC

Panel members:

Prof Neil Sebire, Great Ormond Street Hospital Institute of Child Health

A/Prof Mark Cowley, Children's Cancer Institute

Prof Tom Snelling, University of Sydney

Prof Natasha Nassar, University of Sydney

10.30 - 11.00 **Morning tea and Poster Presentations**

SESSION 5.2 Kids Advanced Therapeutics Pipeline
N=1 clinical trials in paediatric advanced therapies

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Room: Tyree Room

Chair/Facilitator: Adj. Associate Professor Paula Bray, Sydney Children's Hospitals Network

The session will explore the opportunities and challenges of N=1 clinical trials in paediatric advanced therapies, including clinical translation, patient access, ethical considerations, and scientific innovations

Panel members:

A/Prof Leszek Lisowski, Children's Medical Research Institute

Prof Adam Jaffe, Sydney Children's Hospitals Network

Dr Richard Webster, Sydney Children's Hospitals Network

Dr May Aung-Htut, Monash University, Rare diseases specialist

A/Prof Sarah Garnett, Sydney Children's Hospitals Network

Consumer TBC

11.00-13.00	<p>SESSION 6.1 HSIE and Precision Therapy - Delivering outcomes focused sustainability for children in the era of precision medicine</p> <p>Room: Leighton Hall</p> <p>Chair: Adj A/Prof Cathryn Cox, Sydney Children's Hospitals Network</p>	<p>11:00 - 13:00</p> <p>SESSION 6.2 Conference Abstracts Presentations</p> <p>Room: Tyree Room</p> <p>Chair: Dr Radoslaw (Radek) Szmyd, Children's Medical Research Institute</p>
11.00	<p>The need to evaluate health services innovations at scale: challenges and opportunities in the US and Australia</p> <p>Prof Gary L Freed, University of Michigan</p>	<p>11.00 Harnessing liquid biopsy to unveil somatic RAS-MEK pathway variants in extracranial AVMs</p> <p>Dr Smadar Kahana-Edwin, The Children's Hospital at Westmead</p>
11.30	<p>Health systems implementation and health economics -Child Health services research and innovations</p> <p>Prof Raghu Lingam, UNSW Sydney</p>	<p>11.15 Cryptico: A Computational Approach for Exploring Cryptons in Search of Novel Therapeutics</p> <p>Dr Pablo Acera, Children's Cancer Institute</p>
11.45	<p>What is PEACH and PEACH-E? Current Update and Next Steps</p> <p>Prof Karen Zwi & Ms Seaneen Wallace, Sydney Children's Hospitals Network</p>	<p>11.30 A Data Lakehouse for Childhood Cancer Precision Medicine</p> <p>Mr James Bradley, Children's Cancer Institute</p>
12.00	<p>Panel Session</p> <p>Thinking outside the silos: innovations to optimise the health system for children</p> <p>Facilitator: Adj A/Prof Cathryn Cox, Sydney Children's Hospitals Network</p> <p>Panel members:</p> <p>Prof Raghu Lingam, UNSW Sydney</p> <p>Prof Gary L Freed, University of Michigan</p> <p>Prof Sue Woolfenden, University of Sydney</p> <p>Dr Helen Goodwin, NSW Health</p> <p>Dr Sophy Shih, Kirby Institute</p>	<p>11.45 CBL mutations in CNS and solid tumours - a new therapeutic opportunity?</p> <p>Dr Lauren Brown, Children's Cancer Institute</p>
12.30	<p>Abstract Presentations</p> <p>The consumer group landscape: identifying advocacy gaps and improving precision medicine outcomes</p> <p>Miss Tiana Kittos, Child Unlimited</p> <p>Paediatric service navigation to address life challenges: a mixed-methods study</p> <p>Ms Rebecca Bosward, Sydney Local Health District</p>	<p>12.00 Identifying non-coding lncRNA dependencies in high-risk MLL-rearranged acute myeloid leukaemia</p> <p>Mr Steven He, Children's Cancer Institute</p>
		<p>12.15 Individualized-tumour-informed panel approach enables ultra-sensitive ctDNA-based minimal disease monitoring for paediatric cancers</p> <p>Dr Wenhan Chen, Children's Cancer Institute</p>
		<p>12.30 Inferring the life history of NTRK-fusion paediatric tumours</p> <p>Dr Piyushkumar Mundra, Children's Cancer Institute</p>
13.00 - 14.00	Lunch	<p>12.45 PREDICT and Validate: Investigating Germline Alterations in Paediatric Cancer Predisposition [RAPID]</p> <p>Miss Safaa Al Haj Hussein, The Children's Hospital At Westmead, The University Of Sydney</p>
14.00 - 15.30	<p>SESSION 7.1 The road ahead in genomics and genetic testing</p> <p>Room: Leighton Hall</p> <p>Chair: TBC</p>	<p>SESSION 7.2 - Health Systems Implementation and Economics (HSIE) & Psychosocial Workshop</p> <p>Room: Tyree Room</p> <p>Facilitator: Prof Raghu Lingam, UNSW Sydney.</p>
14.00	<p>The future direction for genomics testing: a global perspective on improving access to genomics knowledge and technology</p> <p>Tiffany Boughtwood, Australian Genomics</p>	<p>Summary: Health systems and interventions are complex, with complex humans at the core as both providers and receivers.</p>
14.15	<p>Genomic testing - what is the roadmap for Australia</p> <p>Dr Emma Palmer, Sydney Children's Hospitals Network</p>	<p>Prof Lingam will provide an engaging session on health systems implementation research, exploring frameworks for the development and evaluation of complex health interventions.</p>
14.30	<p>The State of Rare Diseases in Australia</p> <p>Prof Adam Jaffe, UNSW Sydney</p>	<p>Ms Wallace, Dr Ostojic alongside two research advisors will explore the intricacies and key considerations of 'co-design' and involvement of consumers in the planning, design and evaluation of health interventions.</p>

14.45	<p>Abstract Presentation: A collaborative implementation science approach to genomics and precision medicine multidisciplinary teams</p> <p>Dr Alan Ma, University of Sydney/Sydney Children's Hospitals Network</p>	<p>Prof Raghu Lingam, UNSW Sydney</p> <p>Seaneen Wallace, Sydney Children's Hospitals Network</p> <p>Dr Katarina Ostojic, University of Sydney</p>
15.10	<p>Challenges of sustained translation in genomic screening and testing: battle scars, lessons learned, and priority actions to move forward</p> <p>Facilitator: Prof Adam Jaffe, UNSW Sydney</p> <p>Panel members:</p> <p>Tiffany Boughtwood, Australian Genomics</p> <p>A/Prof Ness Tyrrell, Children's Cancer Institute</p> <p>Prof Claire Wakefield, UNSW Sydney</p> <p>Adj A/Prof Paula Bray, Sydney Children's Hospitals Network</p> <p>Dr Emma Palmer, Sydney Children's Hospitals Network</p> <p>NSW Health Representative TBC</p>	<p>Consumer research advisors - Mr Jack Calderan (Young Adult Research Advisor) and Ms Alunya Wilkinson (Parent Research Advisor)</p> <p>Prof Gary L Freed, University of Michigan</p>
15.30 - 16.00	Afternoon tea and Poster Presentations	
16.00 - 16.30	<p>Panel discussion: Reflections and next steps for paediatric precision medicine</p> <p>Facilitator: Kathryn Greiner AO</p> <p>Panel members:</p> <p>Prof Michelle Haber</p> <p>Prof Roger Reddell</p> <p>Adj A/Prof Cathryn Cox</p> <p>NSW Health Representative TBC</p> <p>Prizes</p>	
16.45	Conference close	

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