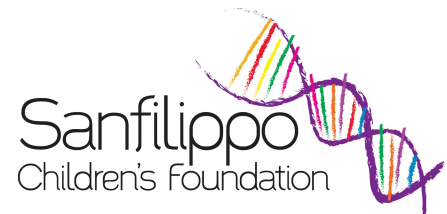# 2023 Solving Sanfilippo Symposium
19th & 20th May, 2023
South Australian Health & Medical Research Institute (SAHMRI)
North Terrace, Adelaide, South Australia *(and online)*

PROGRAM	
DAY ONE - Friday 19th May - Please arrive by 8.45am for a prompt 9am start	
9:00-9.30	**Welcome**
	Welcome - Kerren Hosking, CEO, Sanfilippo Children's Foundation
	Kellie & Jamie Campbell - Mikayla's story
	Global Roadmap for Sanfilippo Syndrome Therapeutics - implementation update. Dr Lisa Melton, Head of Research, Sanfilippo Children's Foundation
9.30-10.30	**Diagnosis, Prognosis & Monitoring - Chair, Prof Kim Hemsley**
	Dr Nicholas Smith, The University of Adelaide & Women's & Children's Hospital - Clinical development of therapies for Sanfilippo: the case for early diagnosis & treatment
	Associate Professor Karin Kassahn, The University of Adelaide & SA Pathology - NewbornsInSA: metabolic and genomic NBS pilot
	Discussion - Pathway to faster diagnosis of Sanfilippo and access to trials
MORNING TEA 10.30-11.00	
11.00-12.15	**TREAT: targeting the underlying neuropathology - Chair, A/Prof Cedric Bardy**
	Professor Alexey Pshezhetsky, University of Montreal - (Recorded) - Targeting amyloidogenesis and neurodegeneration in Sanfilippo disease
	Dr Karissa Barthelson, Flinders University - Identifying common disease mechanisms between Sanfilippo syndrome & Alzheimer's disease using RNAseq
	Kleopatra Pericleous, SA Pathology & The University of Adelaide - Investigating neuropathology in MPS IIIA using mouse primary neuronal cultures
LUNCH	12.15-1.00
1.00-1.15	**Symposium address: Senator Marielle Smith, Senator for South Australia**
1.15-2.15	**MANAGE: symptoms and behaviour - Chair, Prof Sandra Orgeig**
	Mia Holloway, Dementia Support Australia - Practical insights into childhood dementia and how Dementia Support Australia can help
	Dr Jasneek Chawla, The University of Queensland (Online) - Sleep disturbance in neurodevelopmental and neurodegenerative disorders: from understanding to intervention
	Discussion - Addressing the gaps in symptom management for families
2.15-3.45	**STOP: treating Sanfilippo at its root cause - Chair, Dr Adeline Lau**
	Professor Yves Bleriot, Poitiers University, France (Online) - Pharmacological chaperone therapies for Sanfilippo type B

	Associate Professor Cedric Bardy & Dr Zarina Greenberg, South Australian Health & Medical Research Institute - Sanfilippo Brain in a Dish project - Characterising Sanfilippo type A patient-derived iPSC neurons for drug screening
	Associate Professor Anthony Cook, University of Tasmania - Tackling the heterogeneity of childhood dementia
	Discussion - Drug discovery, drug repurposing and advancing clinical translation
AFTERNOON TEA 3.45-4.15	
4.15-5.15	**GROUP DISCUSSION**
	A Global Roadmap for Sanfilippo - Advancing the implementation of the roadmap in Australia: avenues for collaboration & funding
5:15-5.30	**REFLECTIONS from family**
	NETWORKING DINNER - Dinner and drinks served in the SAHMRI Auditorium
DAY TWO - Saturday 20th May - Please arrive by 8.45am for a prompt 9am start	
9.00-10.30	**TREAT: inflammation and neuroprotection - Chair, A/Prof Michael Lardelli**
	KEYNOTE - Professor Nicola Allen, Salk Institute, USA (Online) - The role of astrocytes in neurological disease
	Dr Paul Austin, The University of Sydney, & **Dr Adeline Lau**, Flinders University - Targeting microglia in Sanfilippo syndrome with photobiomodulation
	Professor Kim Hemsley, Flinders University - Targeting purine metabolism as a treatment approach for Sanfilippo syndrome
	Dr Emma Parkinson-Lawrence & Professor Sandra Orgeig, University of South Australia - Is inflammation the key to lungs being more susceptible to pathogen insult?
MORNING TEA 10:30-11.00	
	Dr Lynda Polgreen, Lundquist Institute at Harbor-UCLA Medical Center, USA (Online) - Clinical advances in anti-inflammatory treatment - Anakinra trial update
	Discussion - Anti-inflammatories in Sanfilippo syndrome and related conditions
12.00-12.15	**SYMPOSIUM WRAP-UP: parent & clinician reflections**
12.15-1.00	**LUNCH**
1.00-2.30	**PARENTS NETWORKING & LABORATORY TOUR**

With thanks to our hosts:

SAHMRI
South Australian Health &
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