2ND ASIA-PACIFIC LYSOSOMAL CONFERENCE

FEBRUARY 14th - 16th 2019

Airport Holiday Inn Auckland NEW ZEALAND

SCIENTIFIC FAMILY PROGRAM

Thursday 14th February 2019

1:00–2:00 pm Registration open

Coffee and tea available on arrival

2:00 pm	Official Welcome – Powhiri	
2:20 pm	Welcome	**John Forman, LDNZ; David Palmer, Scientific Chair**
2:30 pm	**Key Note Presentation:** An overview of LSD's and their challenges – Looking back and looking forward	Hans Aerts, Netherlands
Session 1	**Lysosomal Biology and Pathophysiology of Lysosomal Storage Diseases**	**Chair: David Palmer, New Zealand**
3:00 pm	Membrane lipids and storage compounds regulate lysosomal sphingolipid catabolism and trigger a secondary accumulation of lipids in lysosomal disease	Konrad Sandhoff, Germany
3:40 pm	Lysosomal proteins, proteomics and disease	David Sleat, USA
4:10 pm	Carbohydrate-mediated lysosomal protein trafficking, and modifications to improve therapies	Antony Fairbanks, New Zealand
4:40 pm	Identifying therapeutic targets to treat Niemann–Pick type C disease	Andrew Munkacsi, New Zealand
5:10 pm	**Close of Day**	
6:30–8:30 pm	**Welcome Reception**	

Day Two 15th February 2019

Session 2	**Disease Models and Therapy Studies**	**Chair: David Sleat**

8:30 am	Studies on sheep models of Batten disease	Nadia Mitchell, New Zealand
9:00 am	Development of prognostic tools for use in children with Sanfilippo syndrome	Kim Hemsley, Australia
9:30 am	Neuropathological assessments of animal LSD therapy trials	Jon Cooper, USA
10:00–10:20 am	**Morning Break**	
Session 2	**Disease Models and Therapy Studies**	**Chair: Kim Hemsley**
10:20 am	Gene therapy for the gangliosidoses - from the bench to the bedside	Heather Gray-Edwards, USA
10:50 am	Pathogenesis and treatment of skeletal disease in MPS children	Sharon Byers, Australia
11:20 am	GAG reduction in MPS IV	Mireille Tallandier, France
11:40am	Gene therapy with haematopoietic stem cells	Koji Eto, Japan
12:00–1:00 pm	**Lunch**	
1:00–3:00 pm	**Family Workshops – Professionals/Industry/others are encouraged to attend a workshop of your interest and share your knowledge with the families, but also get to hear the reality of living with these diseases.**	
	MPS	**Chair: Kaustav Bhattacharya, Australia**
	Fabry	Chair: TBA
	Batten	**Chair: Heather Adams, USA**
	Pompe	Chair: TBA
	Sialidosis/Mucolipidosis/MLD/Others	Chair: TBA
Session 3	**Lysosomal diseases in other parts of the world**	**Chair: Hans Aerts**

1:00 pm	Spectrum of lysosomal storage disorders: 13 years experience from a single tertiary care centre in Kerala	Dr Sheela Nampoothiri, India
1:20 pm	Targeting DHHCs to enhance PPT1 stability – potential applications in CLN1 disease	Ivanhoe Leung, New Zealand
1:40 pm	Newborn screening for lysosomal storage diseases by tandem mass spectrometry: update in Taiwan's experience	Yin-Hsiu Chien, Taiwan
2:00 pm	**TBA**	TBA
2:20 pm	Understanding the role of neurogenetics in translational research of neuronal ceroid lipofuscinoses in Argentina	Ines Noher de Halac, Argentina
2:40 pm	A novel therapeutic approach for treatment of CNS manifestations in patients with mucopolysaccharidosis	Kazunori Tanizawa, Japan
3:00–3:20 pm	**Afternoon Break**	
Session 4	**Existing and Emerging Therapies**	**Chair Nadia Mitchell, New Zealand**
3:20 pm	Platform AAV-based gene therapy approaches to treat lysosomal storage diseases	Steven Gray, USA
3:50 pm	Pathogenesis, enzyme replacement and gene therapies for glycoprotein and glycolipid storage diseases	Alessandra d'Azzo, USA
4:20 pm	Gene therapy for lysosomal diseases	Brian Bigger, England
4:50 pm	Development of pharmacological chaperone therapy for lysosomal storage diseases	Katsumi Higaki, Japan
5:20 pm	Gene therapy for Fabry disease	Mark Thomas, Australia
5:40pm	**End of Formal Presentations**	
6:00 - 7:00pm	**Poster Viewing Session, evening on your own to network and meet up with friends**	

Day Three 16th February 2019

Session 5	**Access to Medicines**	**Chair: David Palmer**
8:00 am	New Zealand issues of access to therapies	John Forman, New Zealand
8:30 am	Carrier testing for couples planning pregnancies	Jim McGill, Australia
9:00 am	Being diagnosed with MPS, accessing therapy and considering new therapies	Samantha Prior, Australia

9:20 am	Cost of treatment and cost of other therapies	TBA
9:40am	**Morning Break**	
Session 6	**Diagnosis, Clinical Management Care and Support**	**Chair: Jon Cooper, UK**
10:00 am	**Key Note Presentation:** Diagnostic and advances using tandem mass spectroscopy	Jim McGill, Australia
10:30 am	Longitudinal assessment of children with neurodegenarative disorders: clinical and research applications	Heather Adams, USA
10:50am	Viewing Sialidosis through a different lens	Dan Peach, New Zealand
11:00 am	Clinical Presentations of lysosomal storage disorders	Kaustav Bhattacharya, Australia
11:25 am	Realities of living with Morquio syndrome	Rachel Lodewyk, New Zealand
	Realities of living with Hunter syndrome	Kirsty Taylor, New Zealand
11:40 am	Lysosomal diseases: A New Zealand paediatrician's experience	Rosie Marks, New Zealand
12:10–1:10 pm	**Lunch**	
Session 7	**Diagnosis, Clinical Management Care and Support - Continues**	**Chair Steven Gray, USA**
1:10 pm	Transition to Adult Health Services – What are the issues in New Zealand	TBA
1:30 pm	Transition to Adult Health Services – What are the issues in Australia	TBA
1:50 pm	Transition the solution in United Kingdom	TBA
2:10 pm	Cardiac issues in lysosomal storage diseases	
2:30 pm	Pain management and palliative care in lysosomal storage diseases	**Ross Drake, New Zealand**
2:50 pm	**Afternoon Break**	
3:10 pm	Spinal complications in lysosomal diseases – What are the issues and how to treat	TBA
3:30 pm	Patient registries and their importance: A New Zealand example	Richard Roxburgh, New Zealand
3:50 pm	Newborn screening - What's happening in New Zealand	TBA
4:10 pm	What is best for the children, families and caring for parents	Vanessa Ede-Scott, Australia

4:30 pm	Wills trusts and enduring powers of attorney – Why do we need to do this	Perpetual Trustees
5:00 pm	**Close of Day**	
6:30 pm–late	**Awards Dinner 6.30 for 7 pm to be seated.**	