Amicus Therapeutics

SYMPOSIUM INVITATION

Recent Advances in the Treatment of Lysosomal Disorders: New Options and the Growing Body of Evidence

This symposium is intended for Health Care Professionals.

FRIDAY, 24 OCTOBER 2025
17:45 - 18:45

AGENDA

17:45-17:50
Dr. Uma Ramaswami: Welcome & Introduction from the Chair

17:50-18:10
A/Prof Mark Thomas: "Evolving Evidence in Fabry Disease: What the Long Term Data Reveals."

18:10-18:30
A/Prof Michel Tchan: Pombiliti and Opfolda: Trial Data to Real World Insights and Experience

18:30-18:45
Panel: Combined Q&A Session

SPEAKERS

Dr. Uma Ramaswami is a Consultant in Inherited Metabolic Disorders and Clinical Lead for the Lysosomal Storage Disorders Unit at the Royal Free Hospital, London. Uma is an Honorary Associate Professor in Genetics and Genomic Medicine at University College London. Widely published in LSD research, she has had a pivotal role in developing UK national treatment criteria and guidelines for LSDs, including Fabry Disease. Uma is interested in utilising technology such as telecommunication and apps to monitor and improve health care. She is passionate about improving early diagnosis and long-term care for patients with rare metabolic conditions and is the Co-Clinical Lead for the UK Paediatric Familial Hypercholesterolaemia Register.

Dr. Mark Thomas trained in Sydney and London, working fulltime at Royal Perth Hospital as a staff nephrologist since 1988, with current outreach clinics in Esperance & TJ Aboriginal community. He is an active teacher with wide clinical research interests including kidney disease in Aborigines, diabetic nephropathy, polycystic kidney disease, and Fabry disease, running the Statewide service, and involved in the world's first gene transplant programs. He has published several book chapters, 71 peer-reviewed articles and 126 scientific abstracts, with adjunct university professorships at UWA and Curtin. He also loves bushwalking, snorkeling and his 8 grandchildren.

Dr. Michel Tchan is a Clinical and Metabolic Geneticist specialising in the care of adults with genetic disorders and inborn errors of metabolism. He has served as Head of the Department of Genetic Medicine at Westmead Hospital, Sydney, since 2014, and holds the academic title of Associate Professor at the University of Sydney. Dr Tchan leads the New South Wales statewide Adult Genetic Metabolic Disorders Clinic. His clinical practice encompasses metabolic disorders, adult neurogenetics, renal genetics, and general adult clinical genetics. His research interests focus on the clinical aspects of lysosomal storage disorders, neurogenetic movement disorders, renal genetic conditions, and the development and evaluation of novel therapies for genetic diseases. He has acted as Principal Investigator in multiple early- and late-phase clinical trials, including studies of olipudase alfa for Niemann-Pick disease type B, cipaglucosidase alfa for Pompe disease, and sepiapterin for phenylketonuria. Dr Tchan has authored over 100 peer-reviewed publications including in leading journals such as The Lancet, Nature Genetics, Genetics in Medicine, and the Journal of Inherited Metabolic Disease.

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LYSOSOMAL DISEASE SUMMIT

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