

AAV Gene Therapy as a Potential Treatment Modality for Neuronopathic MPS II

Please join our expert faculty

for an educational symposium to understand:

- 1 The natural history of neuronopathic MPS II and the need for early treatment
- 2 The role of potential biomarkers as an early indicator of treatment effects in neuronopathic MPS II
- 3 The potential of gene therapy as a treatment modality for MPS II

Speakers



Maria Fuller, PhD

Clinical Scientist, Genetics and Molecular Pathology, SA Pathology, Adelaide, South Australia



Paul Harmatz, MD

Medical Director of the Pediatric Clinical Research Program in Mucopolysaccharidoses (MPS) and Related Disorders, UCSF Benioff Children's Hospital, Oakland, California



Dawn Phillips, PhD

Senior Director, Clinical Outcomes Research, REGENXBIO



Raymond Wang, MD

Director of the Multidisciplinary Lysosomal Storage Disorder Program at Children's Hospital of California, Orange, California



Friday, February 24, 2023



11:30 – 11:45 am | Registration and Lunch

11:45 am – 12:45 pm | Live Symposium



Orlando 1 | Hilton Orlando

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