



5TH INTERNATIONAL SYMPOSIUM ON UREA CYCLE DISORDERS

MAPPING PROGRESS: _____
CURRENT CONCEPTS AND NEW INSIGHTS
IN UREA CYCLE DISORDERS

SEPTEMBER 22-24, 2021

All times are Eastern Time Zone (New York, United States)

Time (ET)	Lecture Title/Event	Summary	Planned Speaker
Day 1: Overview and International Perspectives: Clinical Experience (Wednesday, September 22, 2021)			
Session Co-Chairs: Matthias Baumgartner, University Children's Hospital, Zurich, Switzerland, Gerard Berry, Boston Children's Hospital, United States, & Cynthia Le Mons, National Urea Cycle Disorders Foundation, Pasadena, United States			
1100-1110	Welcome		Andrea Gropman, MD and Cynthia Le Mons, Symposium Co-Chairs
1110-1120	Session Introduction		Gerard Berry, MD, Session Scientific Program Chair
1120-1135	Overview of UCD and Australian Experience	Overviews and perspectives on the UCD clinical experience and recent research from around the globe.	Kaustuv Bhattacharya, MD, Sydney Children's Hospitals Network
1135-1150	Status of UCD Treatment in Japan		Kimitoshi Nakamura, MD, PhD, Kumamoto University Graduate School of Medical Sciences, Japan
1150-1205	Overview of UCD in Middle East		Sarar Mohamed, MD, Prince Sultan Military Medical City and Alfaisal University, Riyadh, Saudi Arabia
1205-1230	The Challenge of Understanding and Predicting Phenotypic Diversity		Stefan Kölker, MD, University Children's, Heidelberg, Germany
1230-1245	Overview of UCD in South America		Soledad Kleppe, MD, Hospital Italiano de Buenos Aires, Argentina
1245-1300	Urea Cycle Disorders Consortium: Knowledge Gaps and Unknowns		Susan A. Berry, MD, University of Minnesota, Minneapolis, United States
1300-1330	Discussion Panel	Open discussions with all speakers from session	Moderators: Matthias Baumgartner, MD, Gerard Berry, MD, Cynthia Le Mons
1330-1430	Poster Session	Junior Investigator/trainees present their research	Lead: Sandesh Nagamani, MD
Day 2: Biomarkers and Outcomes: Clinical Trial Readiness (Thursday, September 23, 2021)			
Session Co-Chairs: Lindsay Burrage, Baylor College of Medicine, Houston, United States & Andrea Gropman, Children's National, Washington, DC, United States			
1100-1110	Session Introduction		Andrea Gropman, MD and Lindsay Burrage, MD, PhD, Session Scientific Program Chairs
1110-1140	Impact of Medical Management on the Clinical Long-Term Outcome in Individuals with Urea Cycle Disorders	Impact of medical management on the cognitive outcome, intersects with age and stage of diagnosis	Roland Posset, MD, University Children's Hospital, Heidelberg, Germany
1140-1200	Neurocognitive Outcomes Related to Treatment of UCD	Clinical outcomes and clinical trial readiness	Greta Wilkening, PsyD, Colorado Children's Hospital, Aurora, United States
1200-1220	How Can Animal Models Inform Us About the Pathophysiology of Hyperammonemia and Impact on the Brain?	Discussion of decades of research on ammonia and how it impacts brain plasticity	Olivier Braissant, PhD, University of Lausanne, Switzerland
1220-1240	EEG and Neuroimaging Studies: What Have We Learned and Early Biomarkers for Brain Injury in Clinical Trials	Biomarkers/early signs/monitoring using noninvasive neuromonitoring	Andrea Gropman, MD, Children's National, Washington, DC, United States



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1240-1300	Preclinical Studies on Blood Brain Barrier in UCD	Blood-brain barrier and its insights for the pathogenesis of neurocognitive deficits	Jordan Kho, PhD, Baylor College of Medicine, Houston, United States
1300-1320	FDA: Scientific and Regulatory Insights into the Use of Biomarkers in Rare Disease Trials	Review of FDA's process for biomarker selection and qualification	Patroula Smpokou, MD, Deputy Director, Division of Rare Diseases & Medical Genetics, Office of Rare Diseases, Pediatrics, Urologic, and Reproductive Medicine, Office of New Drugs, Center for Drug Evaluation and Research, US Food and Drug Administration
1320-1340	Chronic Liver Involvement in Urea Cycle Disorders	Chronic liver involvement in urea cycle disorders	Carlo Dionisi-Vici, MD, Bambino Gesù Children's Hospital, Rome, Italy
1340-1400	Biomarkers for Liver Disease in Urea Cycle Disorders	Use of non-invasive imaging to evaluate liver fibrosis	Lindsay Burrage, MD, PhD, Baylor College of Medicine, Houston, United States
1400-1430	Discussion Panel	Open discussions with all speakers from the session	Moderators: Lindsay Burrage, MD, PhD and Andrea Gropman, MD
1430-1530	Meet the Professor	Networking	Lead: Sandesh Nagamani, MD
Day 3: Diagnosis and Therapies (Friday, September 24, 2021)			
Session Co-Chairs: Sandesh Nagamani, Baylor College of Medicine, Houston, United States & Oleg Shchelochkov, National Institutes of Health, Bethesda, United States			
1100-1110	Session Introduction		Sandesh Nagamani, MD and Oleg Shchelochkov, MD, Session Scientific Program Chairs
1110-1130	"Hi, my name is Zoey"	A family's perspective on diagnosis and treatment	John Zalusky, Zoey Zalusky's family, United States
1130-1150	Newborn Screening for Proximal UCD – Are We There?	The international perspective on the expanded newborn screening of UCDs	Orna Staretz-Chacham, MD, Soroka Medical Center
1150-1210	Dietary Interventions in UCDs	Management of Nitrogen Homeostasis	Debra Hook, RD, University of California, Davis, United States
1210-1230	Transplant Decision Making in Urea Cycle Disorders: How can you decide?	Transplant decisions	George Mazariegos, MD, Children's Hospital Pittsburgh, United States
1230-1250	N-Carbamylglutamate in the Treatment of Hyperammonemia	Studies in small molecules for UCD management	Nicholas Ah Mew, MD, Children's National, Washington, DC, United States
1250-1310	The Dawn of Genomic Medicine in the Treatment of UCDs	The dawn of novel genomic medicine in UCDs	Ian Alexander, BMedSci, MBBS, PhD, Children's Hospitals Network and Children's Medical Research Institute, Sydney, Australia
1310-1340	Discussion Panel	Open discussions with all speakers from session	Moderators: Sandesh Nagamani, MD and Oleg Shchelochkov, MD
1340-1410	Closing Reception	Networking Roundtables	Hosts: Andrea Gropman, MD and Cynthia Le Mons