6TH INTERNATIONAL SYMPOSIUM ON UCD

ADVANCEMENTS IN UNDERSTANDING: GLOBAL PERSPECTIVES AND INNOVATIONS IN UREA CYCLE DISORDERS

SEPTEMBER 2, 2025, 8 AM – 4:45 PM

OKURA HOTEL, KYOTO, JAPAN

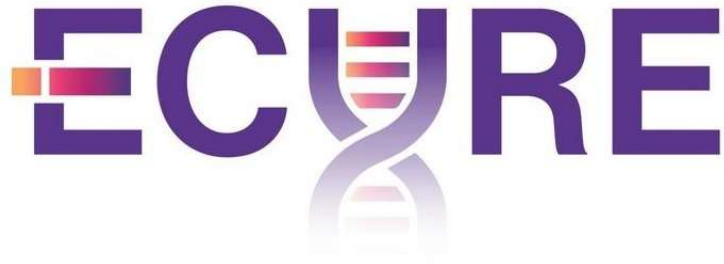| Session 1: Disease models and mechanisms		
Moderators: Matthias Zielonka, Andrea Gropman		
0800-0820	Andrea Gropman and Tresa Warner	The Journey to the Current UCD Landscape
0820-0840	Ljubica Caldovic	Cellular Models for UCD Research
0840-0900	Nicola Brunetti-Pierri	Using Animal Models to Understand UCD
0900-0920	Rebecca Ahrens-Nicklas	Personalized Therapeutic Genome Editing for UCD
0920-0935	Open discussion	
0935-0950	Break	
Session 2: From phenotypic diversity to precision medicine		
Moderators: Kimihiko Oishi, Stefan Kölker		
0950-1010	Andrea Gropman	Brain Imaging as a Prognostic Biomarker for UCD
1010-1030	Greta Wilkening	Unraveling the Neuropsychological and Quality of Life Complexity of UCD
1030-1050	Matthias Zielonka	Early Prediction of Disease Severity: A Tool for Precision Medicine in UCD
1050-1110	Aimee Dudley	Large-Scale Functional Assays for Understanding Variant Impact in UCD
1110-1130	Johannes Häberle	Diagnostic and Clinical Trial Applications Of *In Vivo* Ureagenesis Measurement
1130-1145	Open discussion	
1145-1240	Lunch	
Session 3: Innovative vs established therapies: Advantages and limitations		
Moderators: Carlo Dionisi-Vici, Nicholas Ah Mew		
1240-1300	Tresa Warner and Barbara Yu	Innovative Therapies: Patients' Expectations and Outcomes
1300-1320	Yoko Nakajima	An East-West Comparison Of UCD Dietary Management
1320-1340	Akinari Fukuda	Metabolic Management Versus Whole Liver Or Stem Cell Transplantation
1340-1400	Cary O. Harding	Gene Therapies for UCD
1400-1420	Gerald Schwank	Current Progress and Challenges in Gene Editing for UCD
1420-1440	Sonam Gurung	RNA-Based Therapies for UCD: An Update
1440-1455	Open discussion	
1455-1510	Break	
Session 4: Challenges of newborn screening for UCDs		
Moderators: Shirou Matsumoto, Giancarlo la Marca		
1510-1530	Shawn McCandless	Promise and Challenges of Newborn Screening for UCDs
1530-1550	Yoicho Wada	Proteomic Approach to Screening for UCD
1550-1610	Stefan Kölker	Newborn Screening for All UCD? Results of a Pilot Study
1610-1630	Ines Thiele	Opportunities in Machine Learning-Supported Newborn Screening
1630-1645	Open discussion and wrap up	

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The symposium is presented by:

Urea Cycle Disorders Consortium

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European registry and network for Intoxication type Metabolic Diseases

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