



2019 OHF International Hyperoxaluria Workshop

Joseph B. Martin Conference Center
At Harvard Medical School

Day 1 - Friday, June 21, 2019

7:00 am – 8:00 am	Continental Breakfast / Registration
8:00 am – 8:20 am	Welcome Michelle Baum, MD Michael Somers, MD Kim Hollander, OHF Executive Director
8:20 am – 8:30 am	Onward & Upword Charles & Susan Gambale
8:30 am – 11:20 am	What Have We Learned from Patient Registries Session Chair: Michelle Baum, MD
8:30 am – 9:00 am	Rare Kidney Stone Consortium John Lieske, MD
9:00 am – 9:30 am	OxalEurope Registry Update Sander Garrelfs, MD
9:30 am – 9:50 am	PH3 – The Israeli Experience and Beyond Yaacov Frishberg, MD
9:50 am – 10:00 am	Health-Related Quality of Life (HRQoL) in Primary Hyperoxaluria (PH) Frank Modersitzki, MPH
10:00 am – 10:30 am	Panel: Yaacov Frishberg, MD, Sander Garrelfs, MD John Lieske, MD, Frank Modersitzki, MPH
10:30 am – 11:00 am	Break/Poster Viewing
10:45 am – 11:20 am	Kidney Health Initiative/White Paper Dawn Milliner, MD
11:00 am – 11:20 am	Development in Rare Disease Aliza Thompson, MD U.S. Food and Drug Administration (FDA)
11:20 am – 12:20 pm	Genetics Session Chair: Michelle Baum, MD

11:20 am – 11:35 pm	Use of Genetic Screening for Stone Patients Friedhelm Hildebrandt, MD
11:35 am – 11:50 pm	Genetic Predictions Peter Harris, PhD
11:50 am – 12:20 pm	How Can We Make Genetics More Accessible Around the World? Panel: Peter Harris, PhD, Friedhelm Hildebrandt, MD, Bernd Hoppe, MD, Jaap Grothoff, MD and Yaacov Frishberg, MD
	Oxalosis
12:20 pm – 1:00 pm	Session Chair: Sally Hulton, MD
12:20 pm – 12:35 pm	State of the Art Assessment for Systemic Oxalosis Bernd Hoppe, MD
12:35 pm – 12:50 pm	Echocardiogram and Systemic Oxalosis David Sas, DO
12:50 pm – 1:00 pm	Phenotypic Expression of Primary Hyperoxaluria: Comparative Features of Types I, 2 and 3 Prince Singh, MD
1:00 pm – 1:55 pm	Lunch
	Enteric Hyperoxaluria
1:55 pm – 3:10 pm	Session Chair: Dawn Milliner, MD
1:55 pm – 2:00 pm	Living with EH A Patient Experience
2:00 pm – 2:10 pm	Burden of Disease in Enteric Hyperoxaluria Annamaria Kausz, MD, Allena Pharmaceuticals
2:10 pm – 2:30 pm	How Are We Advancing Basic and Clinical Research on Enteric Hyperoxaluria? Felix Knauf, MD
2:30 pm – 2:50 pm	Oxalobacter Formigenes Colonization and Urinary Oxalate Excretion John Knight, PhD
2:50 pm – 3:00 pm	The Magnitude of Oxalate Excretion in Enteric Hyperoxaluria Matthew D'Costa, MD
3:00 pm – 3:10 pm	Insight into the Peroxisomal Transport Machinery: How to Re-Route Mitochondrial AGT to the Perixisome Ruth Belostosky, PhD
3:10 pm – 3:30 pm	Break / Poster Viewing
	Controversies in PH Treatment/Timing of Dialysis and Transplant
3:30 pm – 5:00 pm	Session Chairs: Michael Somers, MD, Jaap Grothoff, MD

3:45 pm – 5:00 pm Panel: Jaap Groothoff, MD, Yaacov Frishberg, MD, Sally Hulton, MD, Bernd Hoppe, MD, David Sas, DO, Michael Somers, MD

5:00 pm – 6:30 pm **World Reception/Poster Viewing**

Day 2 - Saturday, June 22, 2019

8:00 am – 8:30 am **Continental Breakfast / Registration**

8:30 am – 9:15 am **Patient Experiences and Grant Opportunities**

8:30 am – 8:45 am "Water with Friends" Study
George & Josie Tidmore

8:45 am – 9:00 am I Got Off at the Wrong Stop
Patient Story: Natalie Berra

9:00 am – 9:15 am The Future of OHF
Kim Hollander, OHF

American Society of Nephrology/OHF Career Development Grant
Lama Nazzal, MD

9:15 am – 11:15 am **Pathways in Oxalate Metabolism**

Session Chair: Ross Holmes, PhD

9:15 am – 9:25 am Endogenous and Exogenous Oxalate Synthesis
Sonia Fargue, PhD

9:25 am – 9:35 am Unraveling the Clinical Pathophysiology of Primary Hyperoxaluria Type 1 by Means of Stable Isotopes
Sander Garrelfs, MD

9:35 am – 9:50 am Oxalate Metabolism and Intestinal Microbiome
Lama Nazzal, MD

9:50 am – 10:15 am **Break / Poster Viewing**

10:15 am – 10:30 am Inactivation of Hydroxyproline Dehydrogenase Lowers Urinary Oxalate and Glycolate Excretion in Mice
Todd Lowther, PhD Abstract

10:30 am – 10:45 am Metabolomics of Intestinal Bacteria for Novel PH Therapeutics
Casey Chamberlain

10:45 am – 10:55 am P2X7 Receptor Stimulation is not Required for Oxalate Induced Kidney Injury
Hannah Luz, PhD

10:55 am – 11:05 am Inflammasome Activation in the Primary Hyperoxalurias
Bernd Hoppe, MD

11:05 am – 11:15 am Mapping Metabolomic Profile in PH1 and PH3

Cristina Martin-Higuera, PhD

Future Treatment in Research Part 1

- 11:15 am – 12:15 pm Session Chairs: John Knight, PhD, Eduardo Salido, MD
- 11:15 am – 11:25 am Mitochondrial Import Inhibitors
Carla Kohler, PhD
- 11:25 am – 11:35 am Development of a Small-Molecule Therapy for PH1 Based on the Combined Administration of B6 Vitamers and Pharmacological Chaperones
Barbara Cellini, PhD
- 11:35 am – 11:45 am Abstract
- 11:45 am – 11:55 am Disease Models and Advanced therapies for Primary Hyperoxaluria by cell reprogramming and genome editing
Juan Rodriguez-Madoz, PhD
- 11:55 am – 12:05 pm In vivo glycolate oxidase disruption by AAV-mediated delivery of paired *S. aureus* Cas9 nickases to treat primary hyperoxaluria
Neara Zabaleta, PhD
- 12:05 pm – 12:15 pm Abstract
- 12:15 pm - 1:15 pm **Lunch**
- Future Treatment in Research Part 2**
- 1:15 pm – 3:05 pm Session Chairs: John Knight, PhD, Eduardo Salido, MD
- 1:15 pm – 1:25 pm Identifying Small Molecules to Correct Abnormal Protein Trafficking of AGT in Human Cell Model of PH1 to Avoid Need for Liver Transplantation
Jordan Whatley, MD
- 1:25 pm – 1:35 pm Crisper/CAS9 Mediated Gene Knockout to Address PH
Kyle Wood, MD
- 1:35 pm – 1:45 pm Advanced Therapies for the Treatment of PH1: Cellular Reprogramming and Gene Editing
Virginia Nieto-Romero
- 1:45 pm – 1:55 pm Dual GO/LDH Inhibitor for Treatment of PH1
Maria Dolores Moya-Garzon
- 1:55 pm – 2:05 pm Questions and Answers
John Knight, PhD, Eduardo Salido, MD
- 2:05 pm – 2:55 pm **Open Poster Session / Break**

Industry Pipeline

2:55 pm – 4:45 pm	Session Chair: Michelle Baum, MD
2:55 pm – 3:05 pm	Alnylam Pharmaceuticals Tracy McGregor, MD
3:05 pm – 3:15 pm	Dicerna Pharmaceuticals Ralf Roskamp, MD
3:15 pm – 3:25 pm	Bastian Dehmel, MD Oxthera AB
3:25 pm – 3:35 pm	Intellia Therapeutics Sean Burns, MD
3:35 pm – 3:45 pm	Novome Biotechnologies Lauren Popov, PhD
3:45 pm – 3:55 pm	PHOx Therapeutics Jonathan Fox, MD, PhD
3:55 pm – 4:05 pm	Allena Pharmaceuticals Louis Brenner, MD
4:05 pm – 4:15 pm	Captozyme Ira W. Klimberg, MD
4:15 pm – 4:45 pm	Panel: Allena Pharmaceuticals, Alnylam Pharmaceuticals, Captozyme, Dicerna Pharmaceuticals, Intellia Therapeutics, Novome Biotechnologies, Oxthera AB, PHOx Therapeutics,
4:45 pm – 5:00 pm	Summary of Workshop and Proposal for Next Meeting
5:00 pm	Program Ends – Professional Dinner Cruise