UNIVERSITY OF MINNESOTA
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YOU'RE INVITED TO WORLD*Fair* ™ 2024!

The Advanced Therapies Program* cordially invites you to the 10th Annual WORLD*Fair* ™ Patient and Healthcare Provider Meeting, a gathering of individuals interested in lysosomal diseases. See attached tentative program for more details.

Saturday, October 12, 2024

8:00 AM – 12:00 PM

University of Minnesota Landscape Arboretum

3675 Arboretum Drive, Chaska MN 55318

This a free informational meeting for patients diagnosed with lysosomal diseases, family members and caregivers, and health care providers. Enjoy a self-guided tour and access to the beautiful public garden and grounds after the meeting!

Contact:

Information about the meeting: Please contact Dr. Jeanine Jarnes PharmD at: utzx0002@umn.edu or 952-905-0070
Information about the University of Minnesota Landscape Arboretum: arb.umn.edu and 612-624-2200

The Advanced Therapies Program is a collaboration of the professionals of **University of Minnesota and **Fairview Pharmacy Services** to provide cutting-edge care for lysosomal conditions and other rare diseases requiring special knowledge and care.*

WORLD*Fair*™ 2024 PROGRAM OVERVIEW

8:00 AM – Doors Open

8:00 AM to 9:00 AM Registration, Visit Exhibit Hall, Complementary Continental Breakfast

9:00 AM to 9:45 AM General Plenary Session

"Living as an Adult with a Lysosomal Disease"

Speaker: Dr. Chester Whitley, PhD, MD, Advanced Therapies, Department of Pediatrics, University of Minnesota

"Family Planning Considerations for Adults Living with a Lysosomal Disease"

Speaker: Andrea Atherton, MS, Genetic Counselor, Regional Medical Director, Ultra Rare Medicines, Amgen Rare Disease Medical Affairs

"*CDC Newborn Screening Accuracy Project for MPS Diseases*"

Speaker: Dr. Nishitha Pillai, Advanced Therapies, Department of Pediatrics, University of Minnesota

9:45 AM to 10:00 AM Break, Exhibit Hall Open

10:00 AM to 12:00 PM – Breakout Sessions

Fabry Disease Breakout Session

Session Moderator: Dr. Sofia Shrestha, PharmD, Advanced Therapies, Fairview Pharmacy Services and Department of Pediatrics, University of Minnesota

- 20-Year Data on Fabrazyme® Therapy

  Speaker: Sanofi
- Updates on Elfabrio®:

  Speaker: Dr. Raphael Schiffmann MD, MHSc, FAAN, Research Neurologist, Texas Neurology, Dallas, Texas

  a. What have we learned during the past year?

  b. What about use of Elfabrio® in Pediatrics?
- Updates on Galafold®:

  Speaker: Amicus Therapeutics

  a. What have we learned during the past year?

  b. What about use of Galafold® in Pediatrics?
- Managing Gastrointestinal Symptoms in Fabry Disease

  Speaker: *To be announced*

Gaucher Disease Breakout Session

Session Moderator: Dr. Chester Whitley, PhD, MD, Advanced Therapies, Department of Pediatrics, University of Minnesota

- Cyndi Frank, Co-Founder & Co-President of Gaucher Community Alliance

  Title of Presentation: "*My Journey: Diagnosis, Ceredase®, Cerezyme®, Cerdelga®*"
- Update on VPRIV®: Effects of VPRIV® on Bone Health & Gaucher Disease Management

  Speaker: Takeda
- Update on Cerdelga®: Effects of Cerdelga®: on Bone Health & Gaucher Disease Management

  Speaker: Sanofi

Pompe Disease Breakout Session

Session Moderator: Dr. Nishitha Pillai, MD, Advanced Therapies, Department of Pediatrics, University of Minnesota

- Updates on Pombiliti® plus Opfolda®

  Speaker: Amicus Therapeutics

  a. What have we learned during the past year?

  b. What about use of Pombiliti® plus Opfolda® in Pediatrics?
- Updates on Nexviazyme®

  Speaker: Sanofi

  a. What have we learned during the past year?

  b. Update on use of Nexviazyme® in Pediatrics?
- Diagnostic Odyssey

  Speaker Lori Koehler shares her daughters experience and diagnostic odyssey leading to a diagnosis of late-onset Pompe Disease
- Gastrointestinal Symptoms in Pompe Disease

  Speaker: *To be announced*

Mucopolysaccharidosis Diseases Breakout Session

Session Moderators:

Dr. Elizabeth Braunlin, MD, PhD, Department of Pediatrics, University of Minnesota

Dr. Paul Orchard, MD, Department of Pediatrics, University of Minnesota

- Interim analysis of a phase 1/2 study of weekly intravenous tividenofusp alfa in Mucopolysaccharidosis Type II

  Speaker: Dr. Kwangchae Yoon, PharmD, Senior Medical Science Liaison, Denali Therapeutics

- Updates on Cardiovascular Findings in MPS Conditions

  Speaker: Dr. Elizabeth Braunlin, MD PhD, Cardiologist, University of Minnesota, Department of Pediatrics

- "*Updates on ERT for MPS Conditions: What we have learned*"

  Speaker: Stephanie Dolce, RN, MSN, BioMarin Clinical Coordinator

- Lenti-Viral Gene Therapy for Mucopolysaccharidoses

  Speaker: Dr. Paul Orchard, MD, University of Minnesota, Department of Pediatrics

- UX110 (ABO-102) Gene Therapy for the Potential Treatment of Sanfilippo Syndrome Type IIIA (MPS IIIA)

  Speaker: Ultragenyx

Cystinosis Diseases Breakout Session

- Updates on Therapies for Cystinosis

  Speaker: Dr. Paul Grimm, MD, Nephrologist, Professor of Pediatrics Nephrology, Stanford Medicine Children's Health, Stanford, California

- Managing Gastrointestinal Symptoms in Cystinosis

  Speaker: Andrea Atherton, MS, Genetic Counselor, Regional Medical Director, Ultra Rare Medicines, Amgen Rare Disease Medical Affairs

- Living as an adult with Cystinosis

  Speaker: Rebekah Palmer, Professional Advisory Council and Co-Founder of Next Generation Cystinosis

12:00 PM – Meeting Ends Exhibits are available throughout the day (8:00 AM – 12:00 PM)