



UNIVERSITY OF MINNESOTA

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Crookston Duluth Morris Rochester Twin Cities

YOU'RE INVITED TO **WORLDFair™ 2024!**

The Advanced Therapies Program* cordially invites you to the 10th Annual **WORLDFair™** 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.*



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WORLDFair™ 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: Chiesi Pharmaceuticals
 - 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
 - a. 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 tividinofusp 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 Vimizim®
Speaker: BioMarin
- Lenti-Viral Gene Therapy
Speaker: Dr. Paul Orchard, MD, University of Minnesota, Department of Pediatrics

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 Pompe Disease
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)

ATTENDEES

- Patients, Parents, and Caregivers
- Lysosomal Disease Clinicians
- Patient Advocates
- Research Professionals
- Pharmaceutical Representatives
- Everyone with an interest in lysosomal disease



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