## Conference Agenda
**June 8 – 11, 2024**

### Day 1
**Saturday, June 8, 2024**

<table>
<thead>
<tr>
<th>TIME</th>
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<tbody>
<tr>
<td>11:00-1:00 PM</td>
<td>Registration</td>
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<tr>
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<td>(Sonoran Room Foyer; West)</td>
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<tr>
<td>1:00-1:10 PM</td>
<td>Welcome &amp; Introduction</td>
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<tr>
<td></td>
<td>Sean Kassen &amp; Andy Lieberman</td>
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<td>(Sonoran Room)</td>
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<td>1:10-2:00 PM</td>
<td>Keynote Presentation: Phyllis Hanson (40+10)</td>
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<td><em>Beyond Repair: ESCRTs Enhance Membrane Resilience</em></td>
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<tr>
<td></td>
<td><strong>Session #1</strong></td>
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<td><em>Lysosomal Biology and Cholesterol Trafficking</em></td>
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<td></td>
<td><em>(Sonoran Room)</em></td>
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<td></td>
<td>Andy Lieberman: session leader</td>
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<td>2:00-2:30 PM</td>
<td>Sandra Maday (20+10)</td>
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<td><em>Lysosomal pathways in synaptic signaling between neurons and astrocytes</em></td>
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2:30-3:00 PM  Roberto Zoncu (20+10)

Regulation of Lysosomal Membrane Homeostasis in Niemann-Pick type C

3:00-3:15 PM  Break (15)

3:15-3:45 PM  Yvette Wong (20+10)

Regulation and function of lysosomal inter-organelle contact sites in health and disease

3:45-4:15 PM  Suzanne Pfefer (20+10)

Loss of neuroprotection in the striatum in NPC and other neurodegenerative diseases

4:15 - 4:35 PM  Mike Henne (15+5)

SNX-RGS proteins as lipid transporters that influence lysosome phosphatidic acid (PA) and LBPA metabolism in Niemann Pick disorder

4:35 - 4:55 PM  Albert Lu (15+5)

Uncovering the role of SNX13 in an NPC1 bypass cholesterol egress pathway

4:55 - 5:10 PM  Hijai-Regina Shin (10+5)

Lysosome at the center of lipid sensing and growth regulation

5:15 - 8:00 PM  Reception & dinner
## Day 2
### Sunday, June 9, 2024

<table>
<thead>
<tr>
<th>TIME</th>
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<tbody>
<tr>
<td>7:00-8:00 AM</td>
<td>Breakfast</td>
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**Session #2  Cholesterol trafficking and signaling**  
*(Sonoran Room)*  
**Eamonn Dickson: session leader**

8:00-8:30 AM  
**Emily Eden (20+10)**  
*The importance of lysosomal lipids in inter-organelle communication.*

8:30-9:00 AM  
**Arun Radhakrishnan (20+10)**  
*Role of NPC1 in microbial infection*

9:00-9:20 AM  
**HY Yang (15 + 5)**  
*Phosphatidylserine regulates plasma membrane repair through tetraspanin-enriched macromdomains*

9:20-9:40 AM  
**Xiaochun Li (15+5)**  
*Structural Insights into Lysosomal Transporters*

9:40-9:55 AM  
**Daniel Wustner (10+5)**  
*Role of Niemann Pick type C2 protein in cellular cholesterol elux and inflammation*

9:55-10:10 AM  
**Break (15)**
Session #3  
**Cholesterol trafficking and signaling**  
(Sonoran Room) HY Yang: session leader

10:10-10:40 AM  
Judith Storch (20+10)  
*Understanding the mechanisms and utility of using lysobisphosphatidic acid (LBPA)/bismonoacylglycerophosphate (BMP) as an NPC disease therapeutic.*

10:40-11:00 AM  
Eamonn Dickson (15+5)  
*NPC1-dependent remodeling of ion channels at membrane contact sites contribute to neurodegeneration*  

11:00 - 11:20 AM  
*Rajat Rohatgi (15+5)*  
*Lipid trafficking pathways in Niemann-Pick Type C disease*

11:20 - 11:40 PM  
*Chris Patzke (15+5)*  
*Platform to Analyze Mutations Causing Niemann Pick Type C Disease in Human Neurons*

11:40 - 11:55 AM  
Valentina Pallottini (10+5)  
*A possible new therapeutic drug target for Niemann-Pick type C1 disease: the BET proteins*

11:55 – 12:45 PM  
Lunch

Session #4  
**Biomarkers and Characterization**  
(Sonoran Room) Elizabeth Berry-Kravis: session leader

12:45 - 1:00 PM  
Frank Pfreger (10+5)  
*Progressive pathologic changes in the retinal pigment epithelium of NPC1-deficient mice*
1:00 - 1:15 PM  Wenping Li (10+5)
Progranulin alterations in the Niemann-Pick type C1 I1061T mouse model

1:15 - 1:35 PM  Jorge L. Rodriguez-Gil (15+5)
Molecular and Cellular Basis of NPC1 Lung Disease

1:35-1:55 PM  Denny Porter (15+5)
Identification and Characterization of Serum Protein Biomarkers in Niemann-Pick disease, type C1

1:55-2:15 PM  Stephanie Cologna (15+5)
The growing biomarker landscape in NPC1

2:15-2:25 PM  Critical Path Institute (10)

2:25-5:00 PM  Afternoon Break

5:00-6:30 PM  Poster Session
(Terrace Level)  Foyer

6:30-7:30 PM  Dinner
(Murphy Room)
Day 3
Monday, June 10, 2024

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<tr>
<th>TIME</th>
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<tr>
<td>7:00-8:00 AM</td>
<td>Breakfast</td>
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<td><strong>Session #5</strong> Therapeutic Approaches &amp; Modeling (Sonoran Room)</td>
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<td>Stephani Cologna: session leader</td>
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<td>8:00-8:30 AM</td>
<td>Elsa Rodrigues <em>(20+10)</em></td>
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<td>Unveiling <em>CYP46A1 as a Therapeutic Target in Niemann-Pick Type C Disease: Promising Advances and Potential Challenges</em></td>
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<td>8:30 – 8:50 AM</td>
<td>Mark Schultz <em>(15+5)</em></td>
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<td>Exploring mRNA therapeutics in Niemann-Pick type C1 disease</td>
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<td>8:50 - 9:10 AM</td>
<td>Andy Lieberman <em>(15+5)</em></td>
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<td><em>Model systems for the identification of Niemann-Pick C1 proteostatic therapies</em></td>
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<td>9:10 - 9:30 AM</td>
<td>Kevin Vaughan <em>(15+5)</em></td>
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<td><em>Overcoming Cholesterol Transport Defects in Niemann Pick Type C Disease.</em></td>
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<td>9:30 – 9:50 AM</td>
<td>Marco Segatto <em>(15+5)</em></td>
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<td><em>Modulation of the neurotrophin receptor p75 reduces cholesterol accumulation in Niemann-Pick type C cells</em></td>
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<td>9:50 - 10:05 AM</td>
<td>Coffee Break</td>
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Session #6  Therapeutic Approaches & Modeling  
(Sonoran Room)  Suzanne Pfeffer: session leader

10:05 - 10:25 AM  Charles Vite (15+5)
  *The feline NPC1 colony at the University of Florida – new collaborations.*

10:25 - 10:45 AM  Jian Xiong (15+5)
  *Modulating Bis(Monoacylglycerol)Phosphate for enhanced lysosomal function -- A novel therapeutic strategy for Niemann Pick C1?*

10:45 - 11:00 AM  Thomas Grewal (10+5)
  *Targeting TBC1D15-mediated Rab7 inactivation to overcome cholesterol accumulation in NPC1 disease*

11:00 - 11:15 AM  Ben Calvarey (10+5)
  *Understanding the Spatial Covariance of NPC1 Fold Design through Machine Learning to Address Clinical Disease*

11:15-11:35 PM  Solomon Mbua (15+5)
  *The International Niemann-Pick Disease Registry – An update and progress in North America*

11:35-12:25 PM  Lunch

Session #7  Pharmaceutical & Clinical Data  
(Sonoran Room)  Denny Porter: session leader

12:25-12:55 PM  Elizabeth Berry-Kravis (20+10)
  *Long-term Open Label Treatment of Neurological Symptoms in*
Niemann-Pick Type C1 with Intrathecal Adrabetadex Through Expanded Access

12:55-1:25 PM  Fred Maxfield (20+10)
Testing chaperone modulation in NPC1 mutant cell lines

1:25-1:55 PM  Ross Fredenberg (15+5)
Update on NPC1 Stabilizer Drug Development Program

1:55-2:15 PM  Melissa Wasserstein (15+5)
A multi-tiered approach to screening newborns for complex disorders

2:15-2:35 PM  Scenic Biotech (15+5)
Discovery of a novel Niemann Pick type C (NPC) disease modifier and development towards its clinical application

2:35-2:55 PM  Intrabio (15+5)
Evaluation of the safety and efficacy of N-acetyl-L-leucine for NPC

2:55-5:00 PM  Afternoon Break

5:00-6:30 PM  Poster Session
(Terrace Level)  Foyer

6:30-8:00 PM  Reception and Dinner
(Murphy Room)  Special remarks from NPC family
Day 4

Tuesday, June 11, 2024

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<tr>
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<tr>
<td>7:30-9:00 AM</td>
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