**Scientific Conference on Niemann-Pick Type C Disease**  
Sponsored and Hosted by:  
*Ara Parseghian Medical Research Foundation*  
Co-Sponsored by:  
*Office of Rare Diseases, NIH*

*The Westin La Paloma Resort & Spa – Tucson, Arizona – June 1-2, 2007*  

**Friday, June 1, 2007**

7:15-8:00 Continental Breakfast - La Paloma Country Club

8:15-8:30 Cindy and Michael Parseghian, Welcoming Address - Murphey Room

**NPC1 Protein and Biochemistry**

8:30-8:50 Frances Sharom, Ph.D., University of Guelph - “Purification of human NPC1 and characterization of its binding to sterols and lipids”

9:00-9:20 Suzanne Pfeffer, Ph.D., Stanford University - “Functional analysis of NPC1 protein”

9:30-9:50 Kevin Vaughan, Ph.D., University of Notre Dame - “The Role of Dynein-Driven Motility in the Trafficking of NPC1 Membranes”

10:00-10:10 Haruaki Ninomiya, M.D., Tottori University - “Proteosomal degradation of NPC1”

10:15-10:30 Coffee break

**NPC1 Function and Neurobiology**

10:30-10:40 Jeffrey Krise, Ph.D., University of Kansas - “Evaluating NPC-1’s functional role in both sensing and facilitating the intracellular trafficking of lysosomal stores of amine-containing molecules”

10:45-10:55 Hongyuan Yang, Ph.D., University of New South Wales - “Ncr1p, the yeast ortholog of the human NPC1 protein, regulates the exit of sterols from the yeast vacuole”

11:00-11:20 Barbara Karten, Ph.D., Dalhousie University - “The role of NPC1 in synaptic and neuronal function”

11:30-11:50 Matthew Scott, Ph.D., Stanford University - “In Vivo Studies of NPC Disease: Why do we need new mice?” (Manuel Lopez to present)

12:00-1:45 LUNCH – La Paloma Country Club (SAB to meet over lunch in 19th Hole)
NPC2 Function

2:00-2:20 Peter Lobel, Ph.D., Robert Wood Johnson Medical School - “Molecular Characterization of Niemann-Pick C2 Disease”

2:30-2:50 Ann Stock, Ph.D., Robert Wood Johnson Medical School - “Structural and functional characterization of NPC2”

3:00-3:20 Judith Storch, Ph.D., Rutgers University - “The role of NPC2 in lysosomal cholesterol transport”

3:30-5:00 Poster Session – Finger Rock Room III

6:00-6:45 Cocktails (cash bar) – La Paloma Country Club

6:45 Buffet Dinner – La Paloma Country Club

Saturday, June 2, 2007

7:00-7:45 Continental Breakfast – La Paloma Country Club

Therapeutic Strategies for NPC Disease

8:00-8:20 Frederick Maxfield, Ph.D., Weill Medical College of Cornell University - “Chemical Compounds that Reduce Cholesterol Accumulation in NPC Cells”

8:30-8:50 Stephen Sturley, Ph.D., Columbia University - “Intervention Points in NPC Disease, Insight from NPC-Protein Interactions”

9:00-9:20 Daniel Ory, M.D., Washington University - “Mechanism of neurosteroid protection in a mouse model of NPC Disease”

9:30-9:50 Charles Vite, D.V.M., Ph.D., University of Pennsylvania - “Effect of allopregnanolone administration on disease markers in NP-C cats”

10:00-10:20 Joyce Repa, Ph.D., UT Southwestern - “Liver X Receptor agonists increase cholesterol loss from the CNS and attenuate microglia inflammatory responses to enhance the survival of Npc1 - null mice”

10:30-10:45 Coffee Break

10:45-11:05 Steven Walkley, D.V.M., Ph.D., Albert Einstein College of Medicine - “Combinatorial Approaches to Therapy for NPC Disease”
11:15-11:35  Silvana Zanlungo, Ph.D., Pontificia Universidad Catolica de Chile - “Signaling pathways leading to neuronal death in NPC: Finding new therapeutic targets”

11:45-12:00  Discussion/Conclusion

12:15-1:30  Lunch – La Paloma Country Club

12:15-4:00  SAB Meeting over lunch – La Paloma Country Club, 19th Hole

1:30-3:00  Poster Session – Finger Rock Room III

3:00-6:00  Open afternoon for conference attendees

6:00-6:45  Cocktails (cash bar) - La Paloma Country Club

6:45  Final Buffet Dinner - La Paloma Country Club