

# Promising Therapies for Niemann-Pick Type C Disease

June 3–4, 2010

*Hilton Rockville Hotel and Executive Meeting Center  
Rockville, Maryland*

## **Day 1, June 3, 2010**

- 8:00 a.m.                   **Welcome and Opening Comments**  
*Stephen Groft, ORDR and Story Landis, NINDS*
- 8:15–10:00 a.m.       **Session 1—Framework for Drug Discovery**  
*Session Chair: Elizabeth Neufeld*
- 8:15–8:30 a.m.       **Overview of existing therapies for NPC disease: strategies and challenges for discovery of new therapies**  
*Dan Ory*
- 8:30–8:45 a.m.       **High-throughput screens for discovery of small molecules with NPC disease-modifying potential**  
*Yiannis Ioannou*
- 8:45–9:00 a.m.       **Evaluation of candidate compounds in animal models of NPC disease**  
*Steve Walkley*
- 9:00–9:15 a.m.       **Discovery of biomarkers for NPC disease**  
*Dan Ory*
- 9:15–9:30 a.m.       **NIH observational study: a critical step towards implementation of clinical studies**  
*Forbes Porter*
- 9:30–9:45 a.m.       **Case study for NPC clinical trial: n-acetyl cysteine (NAC)—basic science and clinical trial plan**  
*Forbes Porter*
- 9:45–10:00 a.m.       **NINDS Clinical Trials**  
*Petra Kaufmann*
- 10:00–10:15 a.m.     **General Discussion**  
*Moderator: Elizabeth Neufeld*
- 10:15–10:30 a.m.     **BREAK**

- 10:30–12:15 p.m.      **Session 2—Specific Therapies in the Drug Discovery Pipeline**  
*Session Chair: William Sly*
- 10:30–10:45 a.m.      **Compound discovery through screening of FDA small molecule library**  
*Christopher Austin/Wei Zheng*
- 10:45–11:00 a.m.      **Sphingomyelinase therapy**  
*Fred Maxfield*
- 11:00–11:15 a.m.      **Calcium modulators and anti-inflammatory therapies**  
*Fran Platt*
- 11:15–11:30 a.m.      **Imatanib (Gleevec) and other candidate compounds**  
*Silvana Zanlungo*
- 11:30–11:45 a.m.      **New imino sugar compound therapy**  
*John Marshall*
- 11:45–12:00 p.m.      **Miglustat and related glycosphingolipid synthesis inhibitors—basic science and clinical trial experience**  
*Marc Patterson*
- 12:00–12:15 p.m.      **General Discussion**  
*Moderator: William Sly*
- 12:15–1:30 p.m.      **LUNCH**
- 1:30–6:00 p.m.      **Session 3—Therapeutic Potential of Cyclodextrin**  
*Session Chair: William Gahl*

**Part I—What Is Known About Cyclodextrin and Its Clinical Potential for NPC?**

- 1:30–1:50 p.m.      **Cyclodextrin and the CNS in mice**  
*Cristin Davidson*
- 1:50–2:10 p.m.      **Cyclodextrin and its impact on NPC mouse model**  
*John Dietschy*
- 2:10–2:30 p.m.      **Cyclodextrin in the NPC cat model**  
*Charles Vite*
- 2:30–2:50 p.m.      **Cyclodextrin and the blood-brain barrier**  
*David Begley*
- 2:50–3:10 p.m.      **Pharmacological and cell culture studies of cyclodextrin**  
*Lina Abi-Mosleh*
- 3:10–3:30 p.m.      **Cyclodextrin and cell culture studies**  
*Jean Vance*

3:30–3:45 p.m.      **BREAK**

**Part II—Panel Discussions, Moderator: John Curd**

3:45–4:30 p.m.      **Cyclodextrins—Industry Roundtable (5-minute presentations, each followed by general discussion)**  
*Rick Stratton or Lajos. Szente, CTD; J.D. Pipkin, Cydex; Marcus Brewster, JNJ*

4:30–5:15 p.m.      **Experience on the FDA compassionate use of cyclodextrin in children**  
*Caroline Hastings*  
*Chris Hempel, Hugh Hempel (patient’s family perspective)*

5:15–6:00 p.m.      **General Discussion**  
*Moderator: William Gahl*

7:00 p.m.              **Welcome Reception**

**Day 2, June 4, 2010**

8:00–10:30 a.m.      **Session 4—Therapy Development and Partnerships**  
*Session Chair: Jonathan Jacoby*

8:00–8:20 a.m.      **NINDS Translational Program**  
*Bill Matthew*

8:20–8:40 a.m.      **TRND**  
*Christopher Austin*

8:40–9:00 a.m.      **FDA Orphan Drug Development**  
*Elizabeth McNeill*

9:00–9:20 a.m.      **Pharmacometrics**  
*Jogarao Gobburu*

9:20–9:40 a.m.      **FDA ORD**  
*Anne Pariser*

9:40–10:00 a.m.      **General Discussion**  
*Moderator: Jonathan Jacoby*

10:00–10:30 a.m.      **Patient Advocacy Panel and Partnerships**  
*Karen Quandt, NNPfD; Phil Marella, DART; Susan Green, NPC-UK; Cindy Parseghian, APMRF; Chris Hempel, A&C Fund; Bryan Hadley, Hadley Hope*

10:30–10:45 a.m.      **BREAK**

10:45–noon	<b>Session 5—Where Do We Go From Here?</b>
10:45–11:45 a.m.	<b>Moderators: Dan Ory and Steve Walkley</b> Broad discussion aimed at the goal of developing action points, defining resources and resource needs, fostering collaboration, and setting future goals
11:45–noon	<b>Concluding Remarks</b> <i>Danilo Tagle, Stephen Groft</i>
Noon	<b>Meeting adjourns</b>