

Saturday, May 30
Board room at resort

2:00pm Welcome and Introduction

2:15 pm - 6:00 pm Session 1: Presentations

* each presentation will be 15 minutes followed by a 5 minute question and answer period

2:15 – 2:30	Yanjanin
2:35 – 2:50	Walsh-Vockley
2:55 – 3:10	Giugliani
3:15 – 3:30	Imrie
3:35 – 3:50	Vanier
3:55 – 4:10	Break
4:10 – 4:25	Runz
4:30 – 4:45	Porter
4:50 – 5:05	Patterson
5:10 – 5:25	Garver
5:30 – 5:45	Rolfs

Discussion, leave for dinner

6:00 pm - 8:00 pm Cocktails and dinner at Country Club

Sunday, May 31
Board room at resort

7:30 am Continental breakfast at Country Club

8:00 am - 9:00 am Session 2: Presentations

8:00 – 8:20	Brown
8:20 – 8:40	Stadlober-Degwerth
8:40 – 9:00	Vaurio

9:15 am – 11:45 am Session 3: Standardizing Evaluations and Care of NPC Patients

- Review of Severity Scale
- Standard Evaluation of NPC patients
- Discussion of NPC Registry/Collaborative
- Historical scoring and possibility of factor analysis

12:00 - 1:00 pm Buffet lunch at Country Club

1:15 pm - 4:45 pm **Session 4: Clinical Trials in NPC**
1:15 – 2:15 Clinical Trials: identification of researchers and sites with interest and resources to institute NPC clinical trials
 - Independent trials
 - Component of collaborative trial

Break

2:30 – 3:30 Standard of Care Guidelines

Break

3:45 – 4:45 Open Discussion

6:00 pm - 8:30 pm **Buffet dinner at Country Club**

Yanjanin	NIH observational NPC trial
Walsh-Vockley	Patient Support and Advocacy: the Role of the Genetic Counselor at the NNPDPF
Giugliani	NPC in Brazil and South America
Imrie	Clinical support for NPC-the UK team
Vanier	French NPC cohorts
Runz	Towards a common NPC-patient registry
Porter	Development and characterization of an NPC severity scale
Patterson	Use of an NPC severity scale in evaluation of miglustat therapy
Brown	Pilot study to evaluate the feasibility of remote video data capture in assessing lysosomal storage disease severity
Stadlober-Degwerth	Cognitive assessments in adult Niemann-Pick Disease, type C
Vaurio	Neurocognitive testing for NPC
Rolfs	Frequency of NPC in psychiatric patients-genetic and clinical characteristics