Final Program

"Outcome Measures and Infrastructure for Phase III Studies in Batten Disease (JNCL)"

December 6-7, 2013. Rochester, New York

DAY ONE - December 6, 2013

theme	time	topic	speaker
background on NCLs, Rare Disease Research	8:15-8:30	Welcome to meeting – Introduction & Orientation	Adams
	8:30-8:50	Natural history of JNCL and other NCLs	Mink
	8:50-9:20	Experimental therapeutics in rare diseases	Griggs
	9:20-9:40	Development and validation of clinical trial endpoints	Adams
challenges	10:20-10:50	Patient registries in rare diseases	deBlieck
	10:50-11:20	Widely used clinical endpoints for neurologic disease – applications for JNCL	Augustine
	11:20-11:50	Clinical endpoints and what matters to families	Bletsoe, Sikorra

Afternoon:

state of the science in JNCL	1:00 – 1:30	Biostatistical methods and research design for rare diseases – features of statistically robust endpoints	McDermott
	1:30- 2:00	Preclinical endpoints in CLN3 *with emphasis on preclinical data that may guide selection of endpoints / outcomes in clinical trials.*	Pearce
	2:00-2:30	Review of Mass General Biorepository for NCLs *with emphasis on Biorepository data that may be relevant as endpoints*	Staropoli
	2:45-3:15	International NCL Registry *with emphasis on registry data that may be relevant as endpoints*	Schulz
	3:15-3:45	Review of Hamburg NCL rating scale – reliability, validity, *with emphasis on rating scale data that may be relevant as endpoints*	Schulz
	3:45-4:15	Review of UBDRS – reliability, validity, *with emphasis on rating scale data that may be relevant as endpoints*	Mink

Evening:

	6:00 – 8:30 p.m.	**Poster Reception & Dinner**	

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DAY TWO - December 7, 2013

8:45-10:45 *Moderators will lead discussions, ensure balanced participation, and maintain focus.*	***Working Groups – part 1:*** *Each group will examine the information presented on Day 1 and other relevant information, to propose a set of outcomes / endpoints with potential merit for JNCL Clinical Trials, and/or infrastructure required (e.g., registries). Discussion of outcome measures/clinical endpoints should consider reliability, validity, and relevance of potential endpoints, and propose a roadmap for further testing/development of such endpoints.*			
	Rating Scales Session Chair: Mink	**Biomarker Endpoints** Session Chair: Pearce	**Registries** Session Chair: Kwon	**Other Endpoints** Session Chair: Augustine
	C. Leonard & L.B. Fersternberg to 'float' among the 4 workshops			
11:00-12:20	**MODERATOR: ERIKA F AUGUSTINE, MD** **Group Summaries: Initial recommendations from each working group, feedback from all participants** *Feedback from all participants will be be used at the second working group session* Each group has 20 minutes (10 to present, 10 for discussion/feedback)			

Afternoon:

1:30-3:00	**MODERATOR: GARY D CLARK, MD** ***Working Groups – part 2*** *Groups re-convene in order to address comments from morning feedback and develop final summary*			
	Rating Scales Session Chair: Mink	**Biomarker Endpoints** Session Chair: Pearce	**Registries** Session Chair: Kwon	**Other Endpoints** Session Chair: Augustine
	C. Leonard & L.B. Fersternberg to 'float' among the 4 workshops			
3:30-4:50	**Group Summaries – e.g. action items, research priorities, existing & required infrastructure** Each group has 20 minutes (10 mins presentation, 10 mins discussion)			
4:50-5:10	**Executive Summary**			

Evening:

	Group Dinner	