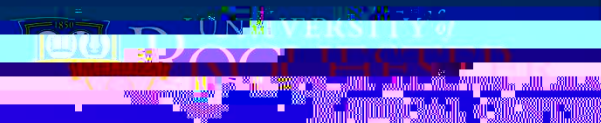
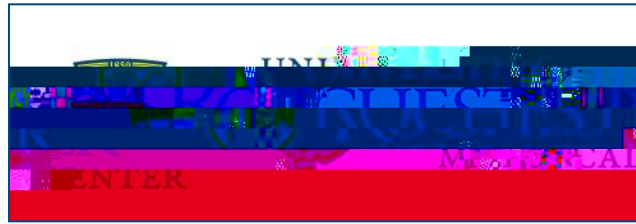
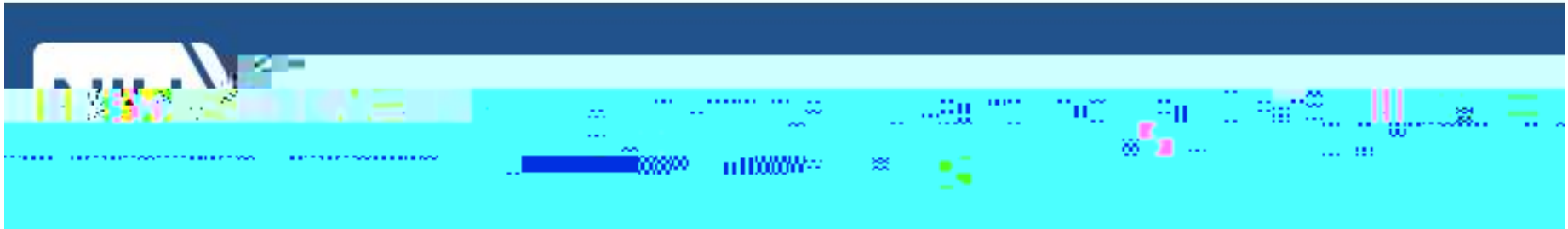




Outcome Measures and Infrastructure for Phase III Studies in Batten Disease (JNCL)

December 6-7, 2013
Rochester, NY USA





- Dan Parr, Magda Ramzy, Alyssa Thatcher, Sara Defendorf (Admin. Support)
- All participants, from near and far!
- Parents – Lori Sikorra (California, USA) & Ellen Bletsoe (Thrapston, UK)
- Advocacy Groups – BDSRA (Chris Leonard) & BBDF (Lisa Beth Furstenberg)
-

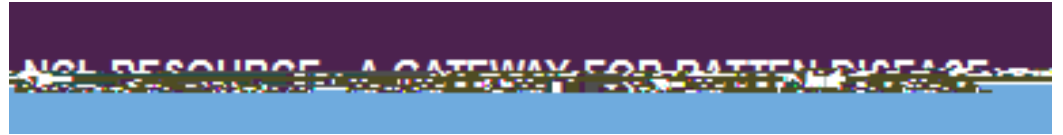
12th Hamburg, Germany (2009)
13th. London, England (2012)
14th. Córdoba, Argentina (2014)

Örebro, Sweden
(2006)

Beyond Batten (2011)

South Beach, CA, USA (2012)

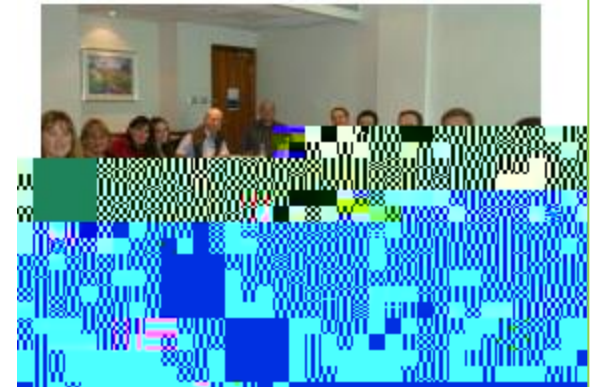
Annual, Hamburg, Germany



<http://www.ucl.ac.uk/ncl/meetingspast.shtml>

1980: International Symposium on Human and Animal Models of Ceroid-lipofuscinosis, Røros, Norway

1st
Worldwide
Meeting of
Batten
Disease
International
Alliance
(BDIA)





- “need to develop new tools to more rapidly assess the effects of potential therapies” ¹
- Sensitive, responsive, “well-defined and reliable endpoint measures [are] particularly important in rare diseases where sample sizes are limited” ², in order to assess a clinically meaningful change in response to intervention.

To bring together clinical research experts in JNCL and rare diseases to focus on establishing common ground for outcomes and infrastructure in support of



THEMES

Background on the NCLs

Rare disease clinical research

Experimental therapeutics, Clinical trial endpoints

Challenging ourselves...

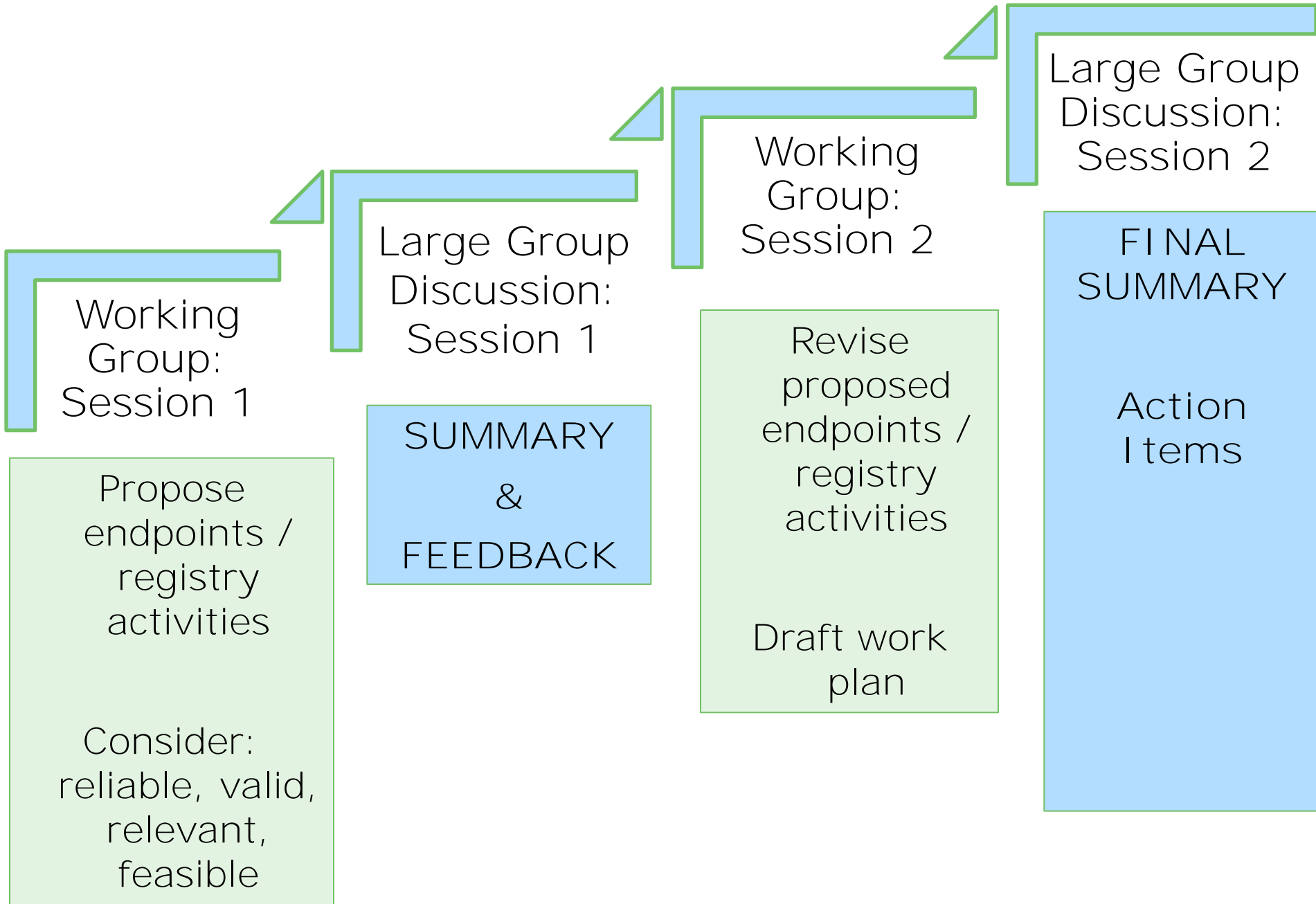
Registries, Clinical endpoints, What matters to parents

State of the Science in JNCL, *with focus on clinical trial applications*

Poster Presentations







1. A set of potential clinical endpoints in JNCL and a plan for their development and validation for future Phase III studies.

2. A plan for expansion of NCL Patient Registries, to incorporate clinical trial endpoint work.

3. Enduring and evolving content
 - Manuscript of proceedings
 - Website (with PDFs of Day 1 talks and reference materials to support experimental therapeutics and Phase III trials in JNCL. We hope these materials will be useful for clinical trials in the other NCLs as well.
 - Maintain interactions among participants at future meetings

1. Strimbu K & Tavel JA. What are biomarkers? *Curr Opin HIV AIDS* 2010. 5(8) 463-466
2. Gliklich RE, Dreyer NA, editors. *Registries for Evaluating Patient Outcomes: A User's Guide*. 2nd edition. Rockville (MD): Agency for Healthcare Research and Quality (US); 2010 Sep. Available from: <http://www.ncbi.nlm.nih.gov/books/NBK49444/>
3. McDowell I. *Measuring Health: A guide to rating scales and questionnaires*, Third Edition. 2006. Oxford Univ. Press.
4. *Robert Temple, FDA; Biomarkers Definitions Working Group 2001; Institute of Medicine 2010*