

CC-CHOC Rare Diseases Breakout Report

May 1, 2009

Baltimore

Major topics

- **IRB protections is a major issue in rare diseases research**: Is there “overprotection”? Are family/patient protection values and perspectives consistent with those of the IRBs?
 - Opportunity for dialog within CTSA setting
 - Collaborative research
 - Discussions with the OHRP

Major topics, cont'd

Collaborate in sharing consenting and research database procedures:

- support more efficient longitudinal follow-up of patients
- for patient consent/IRB approval of sample sharing in interinstitutional collaborative networks.

Major topics, cont'd

- Database harmonization across societies/ research groups to better support rare diseases research
- National volunteer registry proposed within CTSA has different potential implications for rare and common diseases – rare diseases research needs to be considered (perhaps liaison should be identified)

Major topics, cont'd

- Provide value of CTSA to greater pediatric research community:
 - Session at PAS for next year re CTSA: propose one topic on ethics/regulatory in rare diseases; collaborate in proposing session with 3 topics?
 - Training and education of young investigators a major priority