CTSA Consortium Child Health Oversight Committee (CC-CHOC)

Panel:

Bonnie Ramsey, Chair
Shari Barkin, Chair-Elect (via teleconference)
Lisa Guay-Woodford
Ron Sokol

Child Health Emphasis in the CTSA

- **Defined by NIH Reform Act of 2006 - SEC. 106. Enhancing the Clinical and Translational Science**
  - IN GENERAL- In administering the Clinical and Translational Science Award (CTSA), the Director of NIH shall establish a mechanism to preserve independent funding and infrastructure for pediatric clinical research centers by:

    (1) allowing the appointment of a secondary principal investigator under a single CTSA, such that a pediatric principal investigator may be appointed with direct authority over a separate budget and infrastructure for pediatric clinical research;

    or

    (2) otherwise securing institutional independence of pediatric clinical research centers with respect to finances, infrastructure, resources, and research agenda.
  - REPORT - As part of the biennial report under section 403 of the Public Health Service Act, the Director of NIH shall provide an evaluation and comparison of outcomes and effectiveness of training programs under subsection (a).
- **Focus on child health should move beyond the Reform Act and become integrated into overarching goals of CTSA Consortium**

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CC-CHOC Mission Statement

- Provide a unique national forum to identify collaborative opportunities to facilitate child health clinical and translational research
  - Building partnerships (e.g., PAS, NICHD)
  - Building infrastructure (e.g., shared resources)
  - Developing standard approaches (e.g., optimizing outcome measures)
  - Identifying and overcoming barriers (e.g., multi-center IRB review)
- Set priorities and enhance child health research across CTSA Consortium

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CC-CHOC Key Accomplishments (1)

- Supporting development of improved pediatric outcome measures
  - In partnership with NICHD, CC-CHOC coordinated successful applications for 18 projects from 17 CTSA sites worth $ 8.5 M
  - Linked to Best Pharmaceuticals for Children Act of 2002 (BPCA)
- Establishing collaborative infrastructure for product development
  - Workshop in February 2009 gathered specifications from various stakeholders
  - Follow up workshops planned to address topics identified as priorities
  - Resource inventory underway with existing networks and studies such as the National Children's Study and others to leverage expertise and infrastructure

CC-CHOC Key Accomplishments (2)

- Minimize barriers to multi-site research projects
  - Identified IRB inconsistencies across institutions as a barrier in 2007
    - Sponsored two workshops, September 2007 and April 2009
    - Developed a proposal for a federated IRB model
    - Presented proposal to SACHRP (Secretary's Advisory Committee on Human Research Protections)
    - Initiated pilot program to facilitate multi-center IRB review
- Developed a national ethics consult service (Pediatrics Research Ethics Working Group)
- Coordinated with other CTSA workgroups and key function committees

CC-CHOC Key Accomplishments (3)

- Advance general CTSA goals
  - Integrating active child health subcommittees of Drug and Device Development, Pediatric Research Ethics Workgroup, T2 Research, Metrics of Success, Rare Diseases and Life Span Research into CTSA Workgroups and Key Function Committees
  - Leading on new initiatives such as the multi-center IRB review

CC-CHOC Key Accomplishments (4)

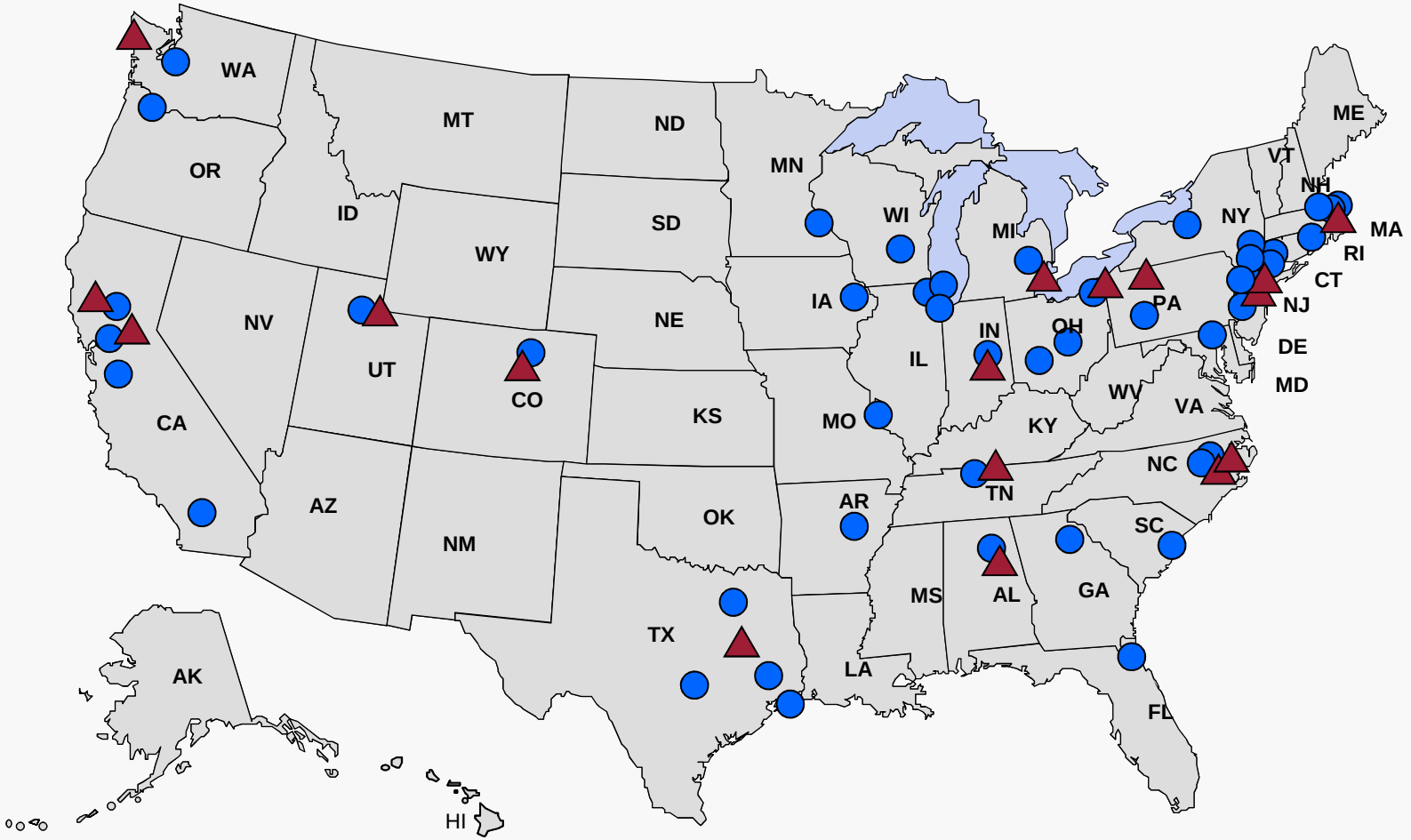
- Form external alliances to promote CTSA goals
  - Pediatric Academic Societies Alliance with Topic Symposium and Platform Sessions (May 2009)
  - Distributed Biobanks for Rare Disease Research (2008 admin supplement; collaboration with Translational workgroup; interim report posted on wiki)

    https://www.ctsawiki.org/wiki/display/Peds/Rare+Diseases+Workgroup

CHOC Alliances

- Alliance organization with Pediatric Academic Societies
  - 2010 Meeting – May 1-4, Vancouver, BC
  - Annual CC-CHOC Face-to-Face Forum/ R13 submitted
  - Three symposia
    - CTSA's: Changing the Environment for Pediatric Translational Research
    - Regulatory Challenges in Pediatric Research
    - Translating Research into Practice (joint with Academic Pediatric Association)
  - Two abstract sessions
    - Focus on young investigators
    - Focus on bioinformatics
- Society for Clinical and Translational Science National Meeting 2010
  - Child Health Symposium: Child health life span research

BPCA Awards – A National Partnership with NICHD



Participating Institutions

- BPCA awardees
- CTSAs (Sept 2009)

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NICHD BPCA Funded CTSA Multisite Projects*

Topic Areas:8-neonatology, 5-neurology, 4-hypertension/hypotension, 3-"other"; >1 topic area possible

1	Duke	Development of a PK algorithm to improve neonatal outcomes
2	UTHSC	Advanced MRI to assess neonatal care and outcome
3	Columbia	Targets and Barriers for Hydroxyurea use in Sickle Hemoglobinopathies
4	Utah	Improving Management of the Neonatal Abstinence Syndrome
5	U Pitt	Cardiac Outcome Measures for Pediatric Muscular Dystrophy
6	UNC	Outcome Measures for chronic lung disease of prematurity
7	U-CO	Small volume fentanyl PK/PD & PG in neonates
8	UC-Davis	Outcome Measures for Trials in Children with Autism
9	Vanderbilt	Wireless Home-Based Tools for studying sleep in Autism
10	U-MI	Pediatric Cardiac Intensive Care Data Standards Repository
11	Stanford	Methadone vs. Morphine PD/PD in infants after cardiac surgery
12	UAB	Nasal Potential Difference Studies Utilizing CFTR Modulators
13	Indiana	Predictors of Vincristine-induced peripheral neuropathy
14	CWRU	Efficacy Outcomes Measures in Antihypertensive Trials in Children
15	CWRU	Effect of BMI on Exposure-Response Relationships to Lisinopril in Children
16	U-Wash	Advancing Patient Reported Outcomes (PROs) in children with Cystic Fibrosis
17	AECOM	Pediatric hypertension outcome measures
18	Tufts	Improving BPD predictors and outcomes for clinical trials

*** Each project must include ≥ 3 CTSA sites**

R13: Linking CTSAs to Create Effective Collaborations to Improve Child Health

- Three year conference grant submitted
- Alliance with the Pediatric Academic Societies (PAS) National Meetings
- Four main objectives:
  - Build and share expertise of CC-CHOC investigators across CTSA sites
  - Expand reach of expertise to both CTSA and non-CTSA child health researchers attending the PAS meeting
  - Provide an annual F2F forum to promote multi-site collaboration involving both CTSA and non-CTSA institutions
  - Promote fellow/junior faculty career development in clinical and translational science

R13: Content Areas and Format

Content:

Key research interest areas that encompass the breadth of translational science in child health and enhance the collaborative infrastructure necessary to conduct quality clinical research focused on child health

1) Year one: Informatics
2) Year two: Community engagement
3) Year three: Life span research

Format:

- Workshop: Experts providing latest information, tools, and approaches
- Symposia: Overview of skill sets to reach both CTSA and non-CTSA child health researchers (minimum of 2/yr)
- Abstract sessions: Encourage junior and senior researchers to submit their work focusing on specific topic area.
- CTSA Child Health Research Fellow Award (4/year)

Metrics of Success: Measuring Impact

Eight Starting Process Measures:

1. CTSA grant funding for child health research
2. IRB-approved and funded child health research
3. Number of child health trainees/faculty who have received mentored grants
4. CTSA child health research pilot grants
5. Active child health studies in CRC
6. Child health research conducted in collaboration with other CTSA's
7. Child health research investigator participation in institutional leadership
8. Institutionally unique approaches for added value in child health research

Metrics of Success: Next Steps

- Distributed metrics to all CC-CHOC members in August 2009
- CC-CHOC members asked to collect initial "baseline" metrics data by January 2010
- CC-CHOC Operations Group will collate data to establish report, institutional benchmarks established
- Aggregate data to be presented at F2F CC-CHOC meeting in May 2010

Two Year Goals

- Multi-center IRB model implemented and tested on several trials
- Institution specific metrics of success benchmarks established
- Established prioritization models for pediatric drugs and devices
- Establishment of a virtual biorepository for utilization by rare disease groups
- Contribute child health core competencies to the SGC standard curriculum

Seven Year Goals

- National infrastructure including data management tools, support services and policies for networks to conduct multi-center trials
- More emphasis on life span research with collaborations across all ages (child to adult health) to focus on prevention of adult disorders and better defining antecedents of adult disease (e.g. obesity)
- Additional metrics of success that include the impact of research on improved child health
- Increased number of young faculty developing careers in pediatric clinical and translational sciences
- Strong emphasis in child health across all CTSA's

20 Year Goals

- Robust child health research community with senior faculty in leadership positions and junior faculty eager to develop their careers in clinical translational science
- Personalized medicine with focus on prevention of childhood disorders and childhood antecedents of adult disease (e.g., obesity, hypertension, cancers). Research studies spanning the entire lifespan from children to adults (already done in CF)
- Established minimally invasive, cheap and valid outcome measures that are age-specific, thereby increasing the speed and efficiency of pediatric drug/device development
- More precise understanding of what is a “normal” child both in terms of genotype and phenotype
- Better health for children worldwide as a result of successful research discoveries moved into action

CC-CHOC Strengths 2009-2010

- CC-CHOC well-aligned with CTSA strategic goals and key functions
- Creative, energetic, and collaborative group; opportunities to pilot promising initiatives on a smaller but still nationwide scale
- Active partnerships already in place

Challenges for CC-CHOC

- CC-CHOC actively engaged in consortium activities, but organizational structure can promote silo building
- Child health is not always considered as a key participant in relevant CTSA consortium activities
- Promoting lifespan research across CTSA consortium has not been a priority and requires better collaboration of child health and adult investigators

Questions for Discussion

- How do we ensure child health representation and perspective across CTSA consortium committees ? How is representation best tracked and coordinated?
- Should we reconsider mechanisms for interactions between the CC-CHOC and CCSC?
- How do we generate more interest among adult investigators in lifespan research and other CC-CHOC activities?

THANK YOU

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