

Pediatrics Oversight Committee of the CTSA

May 5, 2008

Pediatric Oversight Committee (POC)

- March 22, 2007. Initial meeting, 12 CTSA sites
Elected J. Puck, UCSF, as Chair for 2 year term
Identified distinctive features of pediatric research
Recommended to PI Committee inclusion of a pediatric researcher on other CTSA committees
- Quarterly phone meetings; working groups
- Sept, 2007, Pediatric IRB Web Conference
- Oct, 2007. Pediatric Oversight Committee enlarged with new CTSA sites. *Fulfills legislative mandate to incorporate pediatric component into CTSA Programs.*
- Future: June 23-24, 2008, Face-to-face meeting in Bethesda

Pediatric IRB Web Conference

September 11, 2007

- Presented and discussed 5 cases
- Addressed issues raised by cases
 - Variability in IRB review, implications for multi-center studies
 - Definition of minimal risk
 - Definition of who has a disorder or condition (allowing minor increase over minimal risk in research)
- Formulated outcomes
 - Publish case summaries and the points they illustrate
 - Encourage investigators with multi-site studies to post on the secure CTSA Wiki for pediatrics
 - Constitute a national group of experts in pediatric ethics for prospective consultation about study designs, consent forms in planned pediatric research

Research Involving Infants and Children -- “not just little adults”

45cfr46 section	Topic	Rules
46.401	Application	Children in DHHS supported studies
46.402, 403	Definitions	Children: legally too young to consent for medical treatment Assent: children understand and agree Permission: parent/guardian permits participation IRB duties: explicit review of childrens involvement
46.404	Minimal risk	Research approvable if risk of study participation is not greater than everyday life
46.405	> min risk, but prospect of direct benefit	Intervention anticipated to benefit individual subjects; expected benefit from being in the study at least as great as from available alternatives
46.406	Minor increase over min risk; no direct benefit	Study experiences commensurate with those inherent in expected medical care; likely to gain generalizable knowledge of subject's condition
46.407	Not otherwise approvable	DHHS Secretary's expert panel: i) it is a chance to understand, prevent or treat a serious children's health problem? ii) is this the only way? iii) is it ethical?

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CTSA Meetings by Phone Conference September 2007, January & April 2008

- Working groups on high priority issues
 - » Ethics in Pediatrics; build on momentum from web conference [Alex Kon, UC Davis; Lainie Ross, U Chicago; Steve Hirschfeld, NICHD]
 - » Identifying drug and device companies that support pediatric research and development [Jennifer Li, Duke; Carole Marcus, U Penn & CHOP; Steve Hirschfeld]
 - » Rare diseases [Robert Steiner, OHSU; Jennifer Puck, UCSF; Gail Pearson, NHLBI]
- Prioritize additional issues
 - » Sharing resources for training (e.g. specialized courses) and research (e.g. consent templates)
 - » Conducting joint pediatric and adult studies

Infants and Children in Research

- **Pediatric Research Ethics Consultation Group**
 - Norman Fost (U WI, Madison)
 - Eric Kodish (Case Western)
 - Alex Kon (UC Davis)
 - Robert Nelson (U Penn)
 - Mary Purucker (NCCR)
 - Lainie Ross (U Chicago)
 - Jon Tyson (U Texas)
 - David Wendler (NCC)
 - Benjamin Wilfond (U Washington)
- **Provide expert consultation to pediatric researchers facing challenges relating to IRBs**
- **Determine sources of variability in IRB review of multi-center studies**

Pediatric Drugs and Devices

- Facilitate establishment of partnerships in order to pursue multicenter pediatric drug and device studies.
- Pediatric Device Stakeholders' Meeting at Natcher Auditorium on the NIH campus in Bethesda on July 23, 2008.
- One-day workshop in Bethesda in late 2008 for POC members, NIH and FDA experts, drug and device company representatives, CTSA Public-Private Partnerships Steering Committee. Address barriers.

Rare Diseases

- **Rare Diseases Workgroup Vision**
Rare diseases are prominent in pediatric research, should gain from CTSA reorganization -- no single center has enough patients for optimal clinical studies
- **Collaborative efforts**
With NIH NCRR, ORD, Clinical Research Network, National Newborn Screening Coordinating Center, other networks
- **Facilitation of multi-institutional collaborations**
 - IRB standardization
 - Common structures for natural history studies
 - Templates for registries, databases
 - Centralized biorepositories
 - Specialized laboratory testing sites
 - Normative data and samples, matched healthy controls
- **Pilot CTSA-wide strategies with specific diseases**
 - Conditions for which newborn screening exists or is proposed
 - Other rare diseases that are of interest to CTSA investigators

Demonstration of POC Strength

- Dec, 2007, NICHD invited 'Capability Statements' to inform an upcoming plan for a National Newborn Screening Translational Research Network Coordinating Center.
- Response by members of the CTSA community: Nancy Green, Columbia; Jennifer Puck UCSF; Bob Steiner, OHSU
- Distributive model, based on Children's Oncology Group
- POC members asked to suggest likely participants
 - 27 experts at 14 CTSA sites responded: metabolic disease, hemoglobinopathies, other screened disorders, public health and ethical issues of newborn screening
- Proof of concept: CTSA ad hoc networks can be a platform to support and integrate multi-disciplinary and multi-institutional research
- POC will work with contract awardee to include CTSA input

POC Assistance from NCRR

- NIH Intramural liasons, Gail Pearson, NHLBI; Steven Hirschfeld, NIAID; Mary Purucker, NCRR
- CTSI PI Liasons: Mike McCune, UCSF; Pam Davis, Case Western
- Secure, electronic Wiki Space for posting Workgroup news, protocols, studies open for enrollment, etc.
- Contractor assistance with conference calls and scheduling
- Face-to-Face meeting in Bethesda, June 2008

Critical Challenges

- Funding climate for research is poor.
- CTSA funding for transformation and infrastructure is limited.
- Despite legislative mandate, pediatric investigators need to remain proactive
- Support for building multi-institutional administrative support structures has been lacking; a new supplemental funding opportunity for this has just been announced
- Mechanisms for communication between CTSA committees are needed. PI liaisons and pediatric members of other committees are potential links.