CTSA Pediatrics Oversight Committee (POC)
Face-to-Face Meeting
June 23-24, 2008
Meeting Summary

POC Face-to-Face Meeting

Attendees: J. Puck, UCSF (Chair); S. Hirschfeld, NICHD (NIH Coordinator); G. Pearson, NHLBI (NIH Coordinator); M. Purucker, NCRR (NIH Coordinator); S. Alexander, Stanford Univ.; B. Alving, NCRR; S. Barkin, Vanderbilt Univ.; T. Beck, NCRR; C. Boyce, NIMH; E. Collier, NCRR; J. Davis, Tufts Univ.; D. DiMichele, Weill Cornell; F. Dunston, Emory Univ.; L. Epstein, Northwestern Univ.; D. Gipson, UNC; N. Green, Columbia, Univ.; L. Guay-Woodford, UAB; J. Gurney, Univ. of Michigan; D. Hagler, Mayo; D. Hale, UT-San Antonio; L. Haverkos, NICHD; W. Hay, Univ. of Colorado-Denver; A. Hayward, NCRR; R. Higgins, NICHD; A. Hoberman, Univ. of Pittsburgh; M. Hostetter, Yale Univ.; L. Immergluck, Emory Univ.; R. Jacobson, Mayo; S. Kashyap, Columbia Univ.; F. Kaskel, Einstein; H. Keyserling, Emory Univ.; A. Kon, UC Davis; J. Li, Duke Univ.; B. Lubin, CHORI; C. Marcus, Univ. of Pennsylvania; D. McCloskey, NCRR; M. Moxey-Mims, NIDDK; J. Murray, Univ. of Iowa; E. Neufeld, Harvard Univ.; V. Pemberton, NHLBI; A. Philipps, UC Davis; C. Quinn, UT-Southwestern; B. Ramsey, Univ. of Washington; S. Rivkees, Yale Univ.; L. Ross, Univ. of Chicago; S. Shurin, NHLBI; W. Smoyer, Ohio State Univ.; R. Steiner, OHSU; P. Szilagyi, Univ. of Rochester; J. Thoene, Univ. of Michigan; S. Vasan, Rockefeller Univ.; D. Wara, UCSF; N. White, Washington Univ.; P. Zeitlin, Johns Hopkins Univ.; B. Zemel, Univ. of Pennsylvania; H. Aden, BAH; B. Ecken, BAH; J. Goldstein, BAH; P. Hashemi, BAH; R. Samavedam, BAH

I. Welcome and Introductions

Dr. Puck welcomed attendees and introduced Bonnie Ramsey as Chair-Elect.

The CTSA institutions provided brief presentations on accomplishments, goals, current barriers and potential solutions. These presentations are posted on CTSAweb.org. Common goals at CTSA institutions include a focus on training and mentorship, multidisciplinary collaboration, community engagement, reducing barriers for investigators to participate in multicenter studies, broadening the scope of research to include practice-based studies, obtaining support for pilot studies, and bridging the expertise at various clinical sites. Challenges include weak internal and external communication, delays in multicenter studies, lack of support staff trained in pediatric skills, paucity in discipline to evaluate programs and reallocate resources, shortage of statistical support, cultural divide between bench scientists and clinical researchers, lack of integration between the CTSA, the university and the Children’s medical center, and lack of patient numbers to achieve enrollment targets.

Proposed solutions include Web-based dialogue (i.e. Wiki based interactions), K30 program and K12 grants, forming a local advisory committee to address local barriers, complementary collaborations, sharing resources with other programs to optimize efficiency, and conducting laboratory work across an entire state or region.

II. The CTSA Collaborative Vision

Dr. Alving, Director, NCRR, shared her vision for pediatric research within the CTSA consortium. She emphasized the need for pediatricians to collaborate with other committees and to demonstrate their additional value. She also noted that rare

diseases, while a staple of pediatric research, are also adult diseases, citing the example of sickle cell disease and noting that pediatric and adult medicine must work hand-in-hand to be fully successful. She further urged POC members to redouble their efforts to educate their adult colleagues about the special needs in research protocols for children.

Dr. Hayward, Director of the NCRR Division that oversees the CTSA Program, described the state of the CTSAs and their relationship to NIH extramural research funding. He noted that 38 of the expected 60 CTSAs are now funded, and that 41% of NIH's extramural funds spent in 2008 go to these 38 institutions. That percentage is expected to increase to over 50% when the CTSA program is fully implemented. Dr. Hayward highlighted the CTSA Consortium Oversight Committee's (CCOC) strategic planning process, which will be the focus of their October 2008 meeting.

In mentioning the large number of pediatric hospitals and research centers, Dr. Hayward emphasized the power of speaking together through mechanism of the CTSA Pediatrics Oversight Committee. He described the activities of the Community Engagement Steering Committee, and suggested that this group may have particular relevance for POC activities. Dr. Hayward emphasized that a key opportunity for the POC is to develop consensus definitions, common vocabularies and data standards specific to pediatric research. He also described the two regional activities initiated by CTSA sites that are close together geographically as another model in which the CTSAs can collaborate and include non-CTSA organizations.

III. Discussion of POC Mission and Goals

Dr. Puck summarized previous POC activities, as posted on CTSAWeb.org and the CTSA Wiki. She described the three POC Working Groups: Ethics, Drugs and Devices and Rare Diseases. She mentioned that although 8 of 10 CTSA committees have pediatric representation, regular communication between these committees and the POC has not been established. The addition of two new CTSAs with PIs who are pediatricians will assure rapid communication between the POC and CCOC. It was noted that there is a specific need for pediatric representation on the Evaluation Steering Committee and Community Engagement Steering Committee.

As a result of the discussions, members identified needs for the following:

- Clear metrics of success for pediatricians in research
- Core principles of operation for pediatric T2 research
- Lifespan approaches to solve problems that affect both children and adults
- An Operations Group
- Information on NIH-funded trials and clinical research networks at current CTSA sites
- Boiler plate language and templates to assist pediatric protocol development for multi-institutional studies
- Adding all types of pediatric clinical research (not just interventional trials) to the clinicaltrials.gov website and augmenting some of the data fields to identify pediatric research as a means of improving the ability to catalog pediatric studies
- Developing formal liaison relationships with national organizations such as SPR and PAS
- Applying for R13 (conference) grants to support POC-related meetings
- Community-based clinical research (e.g., the SEPA program)

New workgroups were formed by consensus to address needs (see their reports below).

IV. Policy Update from the American Academy of Pediatrics
Mr. Del Monte provided a policy update from the American Academy of Pediatrics' (AAP) Department of Federal Affairs. He offered insight on the two tracks which bills travel - authorization and appropriation. Recent examples of bills that have been passed and been authorized include the NIH Reform Act of 2006 and the FDA Amendments Act of 2007. However, sufficient funds have not been appropriated to support the requirements in the legislation, resulting in unfunded mandates. Mr. Del Monte noted that approximately 80% of appropriations are spoken for before budget discussions about new legislation commence. Mr. Del Monte also described AAP's Strategic Plan, which focuses on access to care, quality and finance. He also noted that AAP has boiler plate language available for messages to Congress in support of pediatric research among other issues.

V. Pediatric Research DVD Update
Ms. Pemberton described a web site and related documentary-style video being developed by NHLBI, NCRR and the National Marfan Foundation aimed at explaining common issues in pediatric research to parents in lay terms. In developing the content, parents, clinical experts, researchers and children were interviewed on thoughts and experiences about participating in a clinical trial. A test server has been set up to facilitate the final round of review by outside experts. There is a main feature film that is approximately 10 minutes long, which can be looped to run continuously. The web site and video material are expected to be launched publicly later in the summer. The videos will be freely available.

In terms of dissemination, suggestions included the following:

- Links to patient advocacy groups
- Contact the Genetic Alliance
- Produce videos in multiple languages (Spanish translation is already planned)
- Place main feature film on YouTube for mass consumption
- Loop various elements of the DVD content in waiting rooms
- Provide a more welcoming phrase in lieu of "minority concerns in research"

VI. Workgroup Updates and Breakout Sessions
Prior to the breakout sessions, members from the three pre-existing workgroups provided updates on accomplishments as well as aims for the breakout session.

Dr. Steiner provided notes on the previous evening's Rare Diseases Workgroup informal session. The workgroup had an initial conference call with NIH followed by a teleconference with rare disease experts to identify gaps to be addressed. These included adding to the limited number of investigators looking into rare diseases, improving registries, producing a white paper and submitting an application for an administrative supplement. The workgroup has consulted with Dr. Groft from the Office of Rare Diseases to further define their charge.

Dr. Li declared that the mission of the Pediatric Drugs and Devices Workgroup is to foster collaborative translational and clinical research so that safer and more effective drugs and devices can be developed for children. The goals of the workgroup are to:

- Initiate a process for centralized pediatric CTSA IRB review, harmonization of contracts, intellectual property, and budgeting to promote CTSA as a clinical trials network
- Identify industry partners
- Develop a warehouse for consent documents and case report forms
- Establish a consultative service for study design
- Develop a post-marketing pediatric device registry among institutions
- Hold an interactive meeting among CTSA institutions, NIH, FDA, industry, tentatively scheduled for December 11-12, 2008.

Dr. Ross noted that the ethics component of the POC held a web conference on 9/11/2007, which gave rise to the Pediatric Research Ethics Consultation (PRECG) Group. The PRECG has merged with the POC Infants and Children in Research Workgroup to become the Pediatric Research Ethics Workgroup (PREW). Membership will be open to all POC members. PREW members decided that it would be an added benefit to participate in meetings of the other POC workgroups.

During the breakout session, workgroups met to advance their agendas. Summaries may be found at CTSAWeb.org or on the Wiki.

VII. Report from CTSA Steering Committees

A. Community Engagement (Donna Jo McCloskey)

Dr. Purucker introduced Dr. McCloskey, the NIH Coordinator for the Community Engagement Steering Committee (CESC). The CESC mission is to ensure the successful implementation of a broad community engagement plan among the CTSA sites by sharing knowledge, expertise and resources. Areas of focus include community outreach, access and dissemination of translational research. The CESC has three workgroups.

1. Community-Based Academic and Practice Partnership Workgroup to identify existing community-based programs with the hope that the information will be used by each of the CTSAs
2. Education Workgroup to align with another CTSA Steering Committee, the Education Steering Committee to formulate educational competencies for use in developing curricula for masters and Ph.D. in translational research. The CESC saw the need to incorporate a basic skill set that would be needed for community engagement translational research.
3. Regional Workshops Workgroup to establish and host workshops in five regions between September 5 and October 17, 2008, to share best practices and identify resources in each region. The ultimate goal is to gather the information for publication. The kick-off meeting for the regional workshops was on May 9th in Bethesda, which hosted 400 attendees.

B. Informatics

Dr. Collier provided an overview of the Informatics Steering Committee (ISC), for which she serves as the NIH Coordinator. The ISC serves as a forum for agreement on best practices to facilitate the exchange of data and information. There are interest and project groups listed on CTSAWeb.org and on CTSA Wiki. The interest groups explore broad themes and the project groups have more specific timelines and deliverables.

Dr. Collier noted that the ISC submitted two applications for an administrative supplement. The first is a web portal/resource, aimed at addressing the needs of various CTSA groups, including Informatics, Translational, Clinical Research Ethics Educational Materials and Public Private Partnerships. The second is a recruitment registry for patients. The ISC will hold a Face-to-Face meeting October 15-17, 2008 in Bethesda.

C. Science Education Partnership Award (SEPA)
Dr. Beck offered a brief presentation on SEPA (http://www.hallofhealth.org/sepa), which encourages and supports partnerships between scientists and clinicians with educators, community organizations, museums and science centers to increase the participation of a diversified generation of young people in clinical and basic research careers and improve K-12 and the public understanding of, benefit from and support for, NIH-funded medical research. SEPA has an R25 educational funding mechanism and offers five-year awards ($1.3M) for Phase I and II research, which is approximately $250,000 per year.

VIII. Report from Clinical Research Management Task Force
Dr. Ramsey reported on the key items at the Clinical Research Management Task Force Workshop, which was taking place simultaneously with the POC Face-to-Face meeting. The Task Force meeting focused on IRBs and contracts. Meeting representatives included contract officers, IRB members and PIs.

With respect to IRBs, common barriers include submission quality of applications, lack of screening for PI-initiated studies and lack of corporate memory. Recommended tools include having senior consultants available to help young investigators who are new to the process, pooling resources, conducting a pre-scientific review, and outsourcing to central IRBs. Requests for action include unifying data management support, standardizing template language, utilizing case studies, and developing metrics that address efficiency and quality measures, as well as PI satisfaction and subjects' understanding consent forms.

The planned next step is to develop best practices through the CTSA, apply and test them, have a silent period and re-examine the situation.

On the topic of contracts, common barriers include the tension between contract officers and PIs stemming from communication gaps, struggles with industry pressures, lack of master service agreements and staff burnout. Recommended tools include using central budgeting tools, matching workload to FTE and educating sponsors. PIs indicated unanimous desire for a tracking database to check where contracts are. Participants also cited the need for education for contract officers and PIs using case studies. A recommendation was for a few institutions to pilot standardized contract language across CTSA for publications and intellectual property.

IX. Day 1 Summary and Operations Group Discussion
Dr. Puck offered a review of the events that took place on Day 1. This included the formation of three new workgroups and an Operations Group.

A. Purpose of an Operations Group
    1. Keep momentum between POC quarterly conference calls/face-to-face meetings

2. Spread out the work load to include a larger group than the volunteer working group leaders, but a manageable few compared to the overall 38 (soon to be 60) CTSA pediatric representatives
3. Ensure input to pediatrics from the standing committees under the CTSA Consortium Oversight Committee (i.e. the PIs)
4. Collate information from the working groups for the broader membership
5. Facilitate/coordinate R13 meeting grant applications
6. Help Chair and Chair-Elect plan in-person meetings

B. Constitution of the Operations Group
1. The "Operations Group" is different from the working groups. The working groups are not indefinitely constituted and not a part of the governance structure. Their existence may vary from year to year, and they can be constituted and dissolved by the POC as needs arise and are met.
2. The POC Operations Group is analogous to the Operations Group of the CCOC. It will serve as an Executive Committee. NIH will have membership in a 1 to 2 ratio to the site members.

C. Selection of members
1. Although we initially asked for volunteers, because the group will have a decision-making role, the membership should be by election by the POC voting members (or their delegates).
   a. Nominations are to be solicited through Friday 6/27. Members can nominate themselves or others. Nominees thus far include:
   b. Shari Barkin (Vanderbilt), Carrie Byington (Utah), Jonathan Davis (Tufts), Leon Epstein (Northwestern) Nancy Green (Columbia), Daniel Hale (UT-San Antonio), Bill Hay (Colorado-Denver), Robert Jacobson (Mayo), Rick Kaskel (Einstein), Carole Marcus (UPenn), Ellis Neufeld (Harvard)
2. Membership – after discussion, the following membership plan was moved by Dr. White, seconded, and adopted by the POC by unanimous vote of the members present:
   a. 7 members at-large from the POC, 3 of whom are NIH Coordinators
   b. POC Chair and POC Chair-Elect will serve as voting members
   c. Not more than one elected member will be from each site, and different regions of the country should be broadly represented. However, a single site could have a pediatric PI for the overall CTSA plus an elected member of the Operations Group.
   d. Pediatric PIs of overall CTSAs will serve *ex officio*.
3. Terms
   a. At-large members will serve two-year terms.
   b. Members elected in 2008 will draw straws for one vs. two year term to allow half the group to turn over annually.
      - First election to be held electronically during the week of 6/30/08.
      - Plan for monthly conference calls. Mr. Hashemi will help organize.

D. Action Items
1. Review the CCOC manual of operations for the constitution of their operations group so as to make the pediatrics group equivalent.

2. Establish how POC can have involvement in the Strategic Planning process which is about to be undertaken by CCOC. Also, determine strategies on how pediatric feedback will get to the CCOC.
3. Check on current lobbying efforts of Federation of Pediatric Organizations (FOPO), APS/SPR, NACHRI, and ASPDC.
4. With new working groups, begin to collect some of the data discussed during our meeting:
    a. Clinical Trials Networks and multicenter studies open at CTSA sites, with possible addition of fields to Clinical Trials.gov (later delegated to a new workgroup; see below)
    b. Establish which groups and which leaders will plan R13 applications
5. Planning for next face-to-face meeting in Spring 2009 (with PAS in Baltimore)
6. Establish routine feedback mechanism from the six CTSA workgroups and six CTSA steering committees. Not all have pediatric representation yet, and those that do are not necessarily feeding back.

X. Workgroup and Other Reports

A. Rare Diseases Workgroup

Dr. Steiner provided a brief report on the goals and priorities, as well as a list of challenges and possible solutions for the Rare Diseases Workgroup. The goal of the workgroup is to have collaborative participation in clinical trials for rare diseases. The priorities include the following:

1. Template/example protocols, informed consent forms (ICF)
2. Universal Natural History Registry
3. CTSA-wide networks for natural history, intervention studies rare diseases
4. Uniform protocol, ICF for registries, biobanks and investigational new drug applications for infants with rare disorders who may die early.

B. Pediatric Drugs and Devices Workgroup

Dr. Marcus stated that the workgroup has been energized by additional membership. The goals of the workgroup are to facilitate collaborative translational and clinical research so that safer drugs and devices are developed for children and to partner with industry to increase research resulting in approval of drugs and devices for children. The workgroup has the capacity facilitate regulatory processes, establish registries and provide consultative services. The workgroup plans include the following:

1. Obtain more information about existing networks
2. Poll constituents about device needs
3. Develop small working groups
4. Interact with FDA
5. E-mail and conference calls
6. Establish metrics for evaluating progress
7. Consider timing of industry conference

C. Pediatric Research Ethics Workgroup

The PREW will meet bimonthly on the 4th Monday at 2pm ET. Dr. Ross stated that the PREW is happy to examine protocols that are deemed best or sub-optimal practices.

D. Metrics of Success Workgroup
The Metrics of Success Workgroup was born out of comments about the sizeable NIH investment and the sensitivity to measurement of success, as well as the NIH Reform Act which articulates the importance of funding. The workgroup aims to create consistent, transparent that hold institutions accountable as a larger part of advancing CTSAs. Dr. Barkin reviewed the pediatric dashboard which lists 10 common items that arose from discussions. The next step is to begin to establish benchmarks and to monitor incremental change. The workgroup also intends to set up teleconferences and nominate two members to serve on the Evaluation Steering Committee.

E. Pediatric-Adult Lifespan Research Workgroup
Dr. Hay noted that this group has not met yet. He noted that funding for pediatric research has decreased in percentage of total NIH spending from 13.1% in 1993 to 10.1% this year. He also reviewed Dr. Zerhouni's future paradigm to preempt diseases, which argues that disease burden is reduced by preclinical intervention.

F. T2 Research Workgroup
Dr. Gipson noted that the goal of this workgroup upon formation is to determine a definition for T2 research. She added that this is a great opportunity to educate the pediatric health community about T2 research by taking T1-related outcome studies and prove that it works.

G. Clinical Research Networks Workgroup
Drs. Wara and White stated that immediate goals of the workgroup are to identify multicenter research groups working with pediatric subjects at the 38 CTSA sites, assist with patient recruitment and help with organization.

XI. The Way Forward: Strategic Thinking for Pediatric Research
Dr. Pearson introduced Dr. Shurin, Deputy Director, NHLBI. Dr. Shurin serves on the NIH IC Directors' Advisory Board that helps Dr. Alving and NCRR oversee the CTSA program.

Dr. Shurin noted that the CTSA was created to solve problems identified by investigators. Examples include a lack of infrastructure to complete research, high costs, high time burden, and regulatory barriers. CTSA goals include transforming the environment for clinical and translational science, increasing the efficiency and speed of clinical and translational science, bridging effective strategies and treatments into clinical practice and creating an environment to overcome impediments. Clinical research by its very nature is collaborative. She urged the need for members to listen productively, ask the right questions and understand others' perspectives.

Since the CTSA program will ultimately be a $500M per year investment on behalf of the NIH, the program carries high visibility in the biomedical research community as well as in Congress. For that reason, it is vital that infrastructure and other resources not be duplicated. Dr. Shurin urged avoiding parochialism, focusing on investment in science rather than infrastructure and improvement in public health, encouraging institutions to partner on investments, supporting faculty and building assets.

A big problem faced by NHLBI and other Institutes at NIH is dealing with inadequate recruitment in expensive clinical trials. CTSAs' insight on whether specific trials are helpful in the first place, and a more efficient way of conducting trials would be useful. It is imperative to identify fundamental issues in clinical trials and use CTSA as a leverage point to resolve them.

XII. Summary: Next Steps and Action Items

Dr. Puck provided a brief summary of next steps as well as action items. She thanked POC members for their participation.

Subsequent to the meeting, Dr. Hirschfeld sent to POC members a listing of clinical research networks at CTSA sites funded by several NIH institutes as compiled in 2007; and a listing of NICHD-funded networks that have a presence at CTSA sites as of June 24, 2008.

Additionally, Mr. Hashemi submitted a list of action items, follow-up items and resources to POC members.

#	Action Items	Owner	Due Date
1	Set up workgroup teleconferences and contact Mr. Hashemi (hashemi_paymon@bah.com) to inform of regular meeting date	Workgroup Chairs	
2	Create/update and submit workgroup descriptions, and send to Mr. Tim Newman (help@ctsaweb.org) and Mr. Hashemi (hashemi_paymon@bah.com)	Workgroup Chairs	
3	Close nominations and conduct Operations Group elections	Mr. Hashemi	