

Pediatrics Oversight Committee (POC)
Meeting Summary
January 4, 2008
12:00 p.m. – 1:30 p.m. EST
Web Conference

Adobe Connect Recording URL: <https://webmeeting.nih.gov/p75543885/>

Call-in number: 1-866-519-2804

Passcode: 700880

Attendees: J. Puck, Chair (UCSF); S. Hirschfeld, NIH Coordinator (NICHD); G. Pearson, NIH Coordinator (NHLBI); A. Sawczuk, NIH Coordinator (NCRR); C. Boyce (NIMH); T. Carpenter (Yale Univ.); S. Cohn (Univ. of Chicago); E. Collier (NCRR); M. DeBaun (Washington Univ.); F. Dunston (Emory Univ.); M. Dwyer-White (Univ. of Michigan); K. Edwards (Vanderbilt Univ.); P. Giardina (Weill Cornell); L. Haverkos (NICHD); R. Higgins (NICHD); D. Hirtz (NINDS); L. Immergluck (Emory Univ.); R. Kavey (NHLBI); A. Kon (UC Davis); J. Li (Duke Univ.); B. Lubin (CHORI); C. Marcus (Emory Univ.); M. Moxey-Mims (NIDDK); J. Murray (Univ. of Iowa); V. Pemberton (NHLBI); A. Philipps (UC Davis); B. Ramsey (Univ. of Washington); L. Friedman Ross (Univ. of Chicago); R. Steiner (OHSU); P. Szilagyi (Univ. of Rochester); B. Talbot (NCRR); A. Tarantal (UC Davis); S. Vasani (Rockefeller Univ.); P. Zeitlin (Johns Hopkins Univ.); K. Kidd (NCRR); E. Davis (BAH); M. Greene (BAH); P. Hashemi (BAH)

Welcome New Members

Dr. Puck welcomed POC members to the meeting, including in particular those from the twelve new CTSA sites. She invited more members to join from the original sites, where only 1 member has been designated up until now and stressed the importance of having at least one member from each CTSA institution on every call. While there is currently no limit on the number of POC members from each CTSA site, members are expected to be active in the POC agendas and workgroups. Only 1 member per site will be a voting member.

Dr. Sawczuk, a member of the NCRR scientific staff, introduced herself. She will be collaborating with Drs. Hirschfeld and Pearson as the NIH Coordinators responsible for the activities of the POC, and will facilitate communication with the NCRR leadership.

Presentation on CTSA Wiki

Dr. Kidd delivered an overview of CTSA Wiki (http://www.ctsawiki.org/wiki/download/attachments/10846451/Kameha+Kidd_Peds_1.0_4.08.ppt). As a collaborative online work environment, the CTSA Wiki allows members to share, develop and collect content and information. The goals of the Wiki are to expedite information sharing and facilitate collaboration to advance the goals for CTSA Consortium. The three phases of the CTSA Wiki process were explained. Support

services are now available. On average, it takes an online community at least a year to embrace the new technology.

Dr. Kidd demonstrated how to enter the CTSA Wiki, access the dashboard functions, and navigate within the Wiki in order to maximize efficiency. She also described the functions of spaces, pages, and child pages, as well as how to organize information from the home page and subsequently link to subpages. She also demonstrated how to enable email alerts that notify users about new or modified documents. Presently, 24-hour summary alerts are limited to the entire Wiki or to specific spaces. Further training with smaller groups on how to use the Wiki is available; working group leaders and others who are interested can coordinate with Dr. Kidd to schedule an in-depth Wiki training session. Additional help is available from Dr. Kidd (kiddka@mail.nih.gov) and Mr. Newman (newmantim@mail.nih.gov).

Report from CTSA Consortium Oversight Committee Meeting, December 18, 2007

Dr. Puck briefed POC members on her presentation to the CTSA Consortium Oversight Committee that took place on December 18, 2007 (<http://www.ctsawiki.org/wiki/download/attachments/10846451/POC+Report+to+CTSA+CCOC+20071218.ppt>). She reviewed background information about the POC, starting with the initial face-to-face meeting in March 2007, and discussed the POC's current workgroups and initiatives. She emphasized that the POC's mission is to address pediatric research issues that bridge across CTSA institutions.

At the last POC call on September 25, 2007, the committee prioritized topics to concentrate on, and workgroups have been or are being established to address the top three: ethical issues in pediatrics, rare diseases, and pediatric drugs and devices. Dr. Puck also described the response of investigators at many CTSA sites to an NICHD request for capacity statements for a proposed national coordinating center for newborn screening. As evidence of the value of the CTSA network, she highlighted how quickly extramural POC members were able to enlist 27 experts from 14 sites to contribute expertise and commitment to this effort.

Infants and Children in Research Workgroup

Leaders: Drs. Alex Kon (UC Davis), Lainie Friedman Ross (U Chicago), and Steven Hirschfeld (NICHD)

This was originally called a Pediatric IRB Workgroup or a Pediatric Ethics Workgroup; the current name has been selected to avoid confusion. The leaders reported on activities. The goal of this workgroup is to strive for uniformity and clarity in ethical and regulatory aspects of pediatric research, in part by providing evaluation of protocols and ethics consultation services through a national consultation panel. The workgroup members function as liaisons with the CTSA Clinical Research Ethics Workgroup, the CTSA Regulatory Subcommittee and the POC.

The major issues identified at the September 11, 2007, conference on pediatric research challenges for IRBs were variability and inconsistency among IRBs in multi institutional studies regarding acceptability of protocols, stipulations requested, definitions of minimal risk and of minor increment over minimal risk and acceptance of definitions of disease or condition. These definitions are critical to interpretation of what is permissible research involving children under the national regulations within 45 cfr 46, sections 404-407 (<http://ohsr.od.nih.gov/guidelines/45cfr46.html>). To address these issues, a three part plan was devised consisting of publishing the workshop findings, providing prospective ethics consultations through an expert panel and encouraging IRBs to post their findings and recommendations for multi-site studies on the CTSA Wiki.

The publication is in preparation; the case discussants have provided their comments and final drafts are being written. The set of manuscripts will be submitted to a journal during the first quarter of calendar 2008. The consultant panel known as the Pediatric Research Ethics Consult Committee has been organized and is ready to provide consultations on ethical issues for multi-site protocols. Requests should be submitted to Mr. Hashemi, who will forward them to the workgroup leaders and the panel.

The panel members are:

Dr. Lainie Friedman Ross-University of Chicago

Dr. Eric Kodish- Cleveland Clinic

Dr. Robert Nelson- University of Pennsylvania

Dr. Jon Tyson-University of Texas, Houston

Dr. David Wendler, National Institutes of Health

Dr. Ben Wilfond- University of Washington

Dr. Alexander Kon-University of California, Davis (co-chair)

Dr. Steven Hirschfeld-National Institutes of Health (co-chair)

A designated expert will provide a written opinion that will be reviewed by the panel. This opinion will then be provided to the requestor so that it can be appended to a protocol when it is submitted for IRB review. For further details, contact the workgroup leaders.

A question was raised as to whether the Pediatric Advisory Committee (PAC) at the Food and Drug Administration (FDA) has an overlapping function. The FDA PAC provides general non-binding comments on research in response to questions raised by the FDA, whereas the CTSA consult service will provide comments on individual studies.

An area for posting comments on infants and children in research from the expert panel will be established on the CTSA Wiki. Once this is in place, an e-mail will be sent to the POC with instructions and sample text to be forwarded to local IRBs about the resource and also about the ethics consultation panel.

Rare Diseases Workgroup

Leaders: Robert Steiner (OHSU), Jennifer Puck (UCSF), Steven Hirschfeld (NICHD), Gail Pearson (NHLBI)

Dr. Steiner noted that the workgroup leaders have had a few preliminary calls and hope to schedule a preliminary call with specific rare disease research experts later in January (<http://www.ctsawiki.org/wiki/download/attachments/10846451/Steiner+Report+1-3-07.ppt>). The purpose of this call will be to obtain input on pressing rare disease research issues that can benefit from use of CTSA. He explained the potential for collaboration with various federal entities, including NCCR, the NIH Office of Rare Diseases and the FDA.

He cited a number of challenges in conducting rare disease research, including insufficient numbers of patients at a single study center, limited funding for many rare diseases, limited numbers of researchers trained to study rare diseases, infrastructure needs. Some successful networks already exist for studying particular diseases, such as cystic fibrosis, and may serve as models for studying other conditions. Objectives for this workgroup are identifying gaps in the systematic study of rare diseases and determining how the CTSA Consortium can help address those gaps.

Pediatric Drugs and Devices Workgroup

Leaders: Jennifer Li (Duke), Carole Marcus (U Penn, CHOP), Steven Hirschfeld (NICHD)

Drs. Li and Marcus reported that the workgroup is in an early phase. One of the priorities for the Pediatric Drugs and Devices Workgroup is to interface with industry and to promote the CTSA network as an attractive place for industry to do early translational and clinical work in pediatric populations. Issues of introduction of new products or exclusivity rights will be addressed. Currently, many industry sponsors are conducting clinical studies outside of the US due to real and perceived difficulties with domestic IRBs and intellectual property issues. This POC workgroup would like to hold a face-to-face workshop with CTSA members, industry partners and FDA regulators to explore these issues in the context of pediatric research.

Some CTSA members cited confirmed the need for improvement and cited specific issues related to intellectual property rights. Dr. Sawczuk reported that the CTSA is examining intellectual property issues through several venues, including a workshop taking place in Spring 2008, organized by the Public Private Partnerships Steering Committee and the Regulatory Workgroup.

A follow up phone call for the POC Drugs and Devices workgroup will be held later this month and POC members with an interest in joining the workgroup are encouraged to contact Mr. Hashemi to sign up. Other contributors (pediatricians or non-pediatricians with relevant expertise and interest) from CTSA sites are also welcome to join.

Funding of POC and Pediatric Activities

The question of funding for CTSA pediatric activities was broached. Dr. Puck expressed the need to be creative about funding, since the POC will not have resources at its disposal apart from the CTSA funds given to each site. It was also suggested that projects could be leveraged by R01 grants; it is expected that the POC, functioning within the CTSA Consortium, will provide the infrastructure to make collaborative projects fundable.

Pediatricians on CTSA Committees

Dr. Sawczuk shared a table of pediatric representation on CTSA Committees (<http://www.ctsawiki.org/wiki/display/Peds/Pediatrics+Oversight+Committee-+Home>). All members who know of updated or additional pediatric representation on committees should contact Dr. Sawczuk (sawczuka@mail.nih.gov) or Mr. Hashemi (hashemi_paymon@bah.com). Also, POC members should observe the gaps and look for pediatricians to volunteer to serve on these committees, forwarding their names to the individuals above.

Pediatric Implications of FDA Reauthorization Act of 2007

Dr. Hirschfeld provided a brief description of the FDA Amendments Act of 2007 (<http://www.ctsawiki.org/wiki/download/attachments/10846451/FDA+Reauth+Act+20071129.ppt?version=1>). Special attention was called to items with pediatric implications, including a new medical devices act meant to stimulate development of devices for pediatric use, the renewal of the Pediatric Research Equity Act (a mandate to perform studies for drugs and biologics in children), and an incentive program and mechanism for studying off-patent medications contained in the Best Pharmaceuticals for Children Act.

New requirements for postmarket studies and other new requirements for listing clinical trials in the clinicaltrials.gov database were also mentioned. Awareness of the new requirements for listing studies in registries will be necessary to comply with the law.

Committee Membership and Chair-Elect Nominations

The POC will develop a nominations committee leading to the election of a CTSA Chair-elect at the next face-to-face meeting.

Committee Face-to-Face Meeting in Bethesda, 2008

A face-to-face POC meeting is planned for late spring or early summer of 2008. The meeting will avoid other major pediatric meetings. POC members will need to request funding to attend these meetings from their own CTSA's. Non-voting members and those unable to travel to Bethesda are encouraged to participate in the face-to-face meeting through web and teleconference functions if they are unable to participate in person.

Workgroup sessions may be planned in conjunction with this meeting to maximize productivity.

While the Pediatric Academic Societies (PAS) Annual Meeting in Hawaii, May 3-6, 2008, could be an opportunity for POC members to meet informally it will not be possible to hold an official meeting there due to cost and other constraints.

Action Items

1. Additional POC membership is encouraged from the original 12 CTSA sites, and each site should have alternate POC member(s) to assure participation from all sites at each future conference call. Names of new members should be submitted to Mr. Hashemi at (hashemi_paymon@bah.com).
2. Dr. Sawczuk, our POC liaison to other CTSA committees should keep Drs. Pearson, Hirschfeld and Puck informed of activities by other CTSA committees that may synergize with POC activities. POC members should explore the CTSA Wiki to learn what is going on throughout the CTSA structure.
3. Drs. Pearson, Hirschfeld and Mr. Hashemi will create the mechanism for committee members to provide input on the rankings of proposed new activities and workgroup creation. They will also recruit a nominating committee.
4. Mr. Hashemi will work with the committee members to determine a regular standing meeting date and time on a quarterly basis for at least the next year.
5. Workgroup leaders are to schedule advanced Wiki training sessions for their members once they have some experience with the tool.
6. Committee members or others at CTSA sites who would like to join a workgroup or suggest new workgroups should contact Dr. Pearson (pearson@nhlbi.nih.gov) or Mr. Hashemi (hashemi_paymon@bah.com).
7. Mr. Hashemi will poll POC members by email and form a posting site on the CTSA Wiki to determine who is attending the PAS Annual Meeting in Hawaii from May 3-6 and how these individuals can get together. (<http://pediatrics.hawaii-convention.com/>).
8. A draft agenda and timing for the 2008 POC face-to-face meeting will be generated by Drs. Pearson, Hirschfeld, Puck and the workgroup leaders.
9. A nominating committee for POC Chair-elect will be generated by email with the POC membership by Mr. Hashemi and Dr. Pearson.

Key Decisions

1. Dr. Friedman Ross will be the new Chair of the POC Infants and Children in Research Workgroup.

Next Meeting

The next meeting of the Pediatrics Oversight Committee will be scheduled as noted above.

The meeting adjourned at 1:31p.m. EST.