

**CTSA Pediatrics Oversight Committee (POC)
Consortium Meeting
April 14, 2008
Meeting Summary**

Conference Number: 1-866-285-7778

Participant Code: 7334287

Time: 12:00 PM – 1:30 PM ET

Attendees: J. Puck, UCSF (Chair); S. Hirschfeld, NICHD (NIH Coordinator); G. Pearson, NHLBI (NIH Coordinator); M. Purucker, NCRR (NIH Coordinator); A. Sawczuk, NCRR (NIH Coordinator); A. Al-Uzri for R. Steiner, OHSU; S. Arslanian, Univ. of Pittsburgh; C. Boyce, NIMH; E. Collier, NCRR; K. Edwards, Vanderbilt Univ.; J. Glade-Bender, Columbia Univ.; J. Gurney, Univ. of Michigan; L. Haverkos, NICHD; L. Immergluck, Emory Univ.; S. Kashyap, Columbia Univ.; H. Keyserling for B. Stoll, Emory Univ.; A. Kon, UC Davis; J. Li, Duke Univ.; B. Lubin, CHORI; C. Marcus, Univ. of Pennsylvania; J. McNamara, NIAID; M. Moxey-Mims, NIDDK; J. Murray, Univ. of Iowa; M. Nesin, NIAID; V. Pemberton, NHLBI; J. Perez-Fontan, UT-Southwestern; B. Ramsey, Univ. of Washington; K. Rauen, UCSF; J. Read, NICHD; L. Ross, Univ. of Chicago; J. St. Geme, Duke Univ.; S. Vasan, Rockefeller Univ.; P. Hashemi, BAH

I. Welcome and Introductions

Dr. Puck welcomed members and offered a preview of the agenda items. [Note added to minutes: Although not present at this phone meeting, Dr. Mike McCune (PI, UCSF CTSA) and Dr. Pam Davis (PI, Case Western CTSA), have been assigned to the POC as liaisons. Both are eager to participate and assist us in taking advantage of CTSA opportunities to enhance pediatric research. We hope to have them on future meeting calls.]

II. Workgroup Updates

A. Infants and Children in Research and the Pediatric Research Ethics Consultation Group

Dr. Kon provided a brief history of the Pediatric Research Ethics Consultation Group (PRECG). He noted that the variability of IRBs and protocol review at different institutions was a key theme during the 1st POC Face-to-Face meeting. The first POC workgroup, now called the Workgroup on Infants and Children in Research, was formed to address ethical and regulatory issues in pediatrics and held a highly successful web conference on September 11, 2007. As an outcome of this meeting, the PRECG was formed to provide expert consultation to pediatric researchers facing challenges relating to IRBs. In addition to Dr. Kon and Dr. Hirschfeld, PRECG members include Norman Fost (UW-Madison), Eric Kodish (Case Western), Robert Nelson (UPenn), Mary Purucker (NCRR), Lainie Ross (Chicago), Jon Tyson (Texas), David Wendler (NCC), and Benjamin Wilfond (UWashington), all highly regarded experts in pediatric regulatory and IRB issues.

The PRECG will have a two-pronged approach:

- 1) To provide consultation services to pediatric investigators and research ethics consultants at individual CTSA sites. While respecting issues of confidentiality, the PRECG will provide independent assessments of protocols submitted to them. They will use their collective experience to assist in judging level of risk. They will also help assess whether research

subjects who may be exposed to greater than minimal risk without expectation of direct benefit can be considered to have a “condition” under federal regulations.

2) To determine the sources of variability in IRB review of multicenter studies.

The PRECG urges POC members to submit problematic protocols (or sufficiently detailed summaries) along with a copy of the IRB responses, where possible. Progress depends on actual cases being available to consider. Names or identifying information should be redacted as needed prior to submitting in order to protect confidentiality, intellectual property or other privacy concerns. Before submitting, permission should be obtained from the investigators and sponsors of the protocol.

Several POC members suggested protocols they know of that might be excellent for PRECG review, covering such topics as adolescents, genome-wide association studies and rare diseases. An e-mail will be sent to all POC members asking investigators to submit protocols to Paymon Hashemi (hashemi_paymon@bah.com) for review by the PRECG.

B. Pediatric Drugs and Devices

Dr. Li reported that the Pediatric Drugs and Devices Workgroup had begun discussions to determine what can be accomplished. The goal of this workgroup is to facilitate the establishment of partnerships in order to pursue multicenter pediatric drug and device studies.

The workgroup proposes to hold a one-day, invitation-only workshop in Bethesda in late 2008. Those invited would include interested POC members, NIH and FDA staff, drug and device company representatives and members of the CTSA Public Private Partnerships Steering Committee. The goal of the workshop would be to determine barriers that prevent drug and device companies from working with pediatric researchers. To date, a preliminary agenda consisting of think-tank sessions, case presentations, lessons learned and policy updates has been drafted.

There will also be a Pediatric Device Stakeholders' Meeting at Natcher Auditorium on the NIH campus in Bethesda on July 23, 2008. This event will bring together representatives from industry and academia with the regulatory and funding agencies of HHS to discuss a pediatric device research plan. This meeting may provide an opportunity to identify potential partners in product development. There is no fee to attend the meeting.

Dr. Ramsey noted that her Cystic Fibrosis Network conducted a survey with 30 industry partners that identified specific problems. She will send the survey to POC members.

C. Rare Diseases

In Dr. Steiner's absence Dr. Puck reported that the Rare Diseases Workgroup is in the process of scheduling future conference calls, and plans to meet during the informal session of the POC Face-to-Face meeting on June 22. The group wishes to establish new pathways to take advantage of CTSA structure in advancing multi-institutional collaborations to study rare disorders. Test cases are needed to pilot this process. The workgroup asked POC members to identify specific rare diseases championed by an investigator at a CTSA site with ideas for a multicenter study. While some rare disease research is already well-supported by established networks, diseases ripe for assistance from the workgroup may be as yet less organized. One

focus of the group will be diseases for which newborn screening has been instituted or is proposed, and for which outcomes research is needed.

POC members suggested additional diseases and will poll investigators at their sites. Paymon Hashemi will solicit additional suggestions from POC members in a follow-up email, and the contacts will be forwarded to the workgroup. The list of rare diseases under review will be posted and updated on the POC Wiki page under the Rare Disease Workgroup.

III. Nomination Process, CTSA Chair-Elect

Dr. Pearson provided a brief description on the nomination process for the POC Chair-Elect, who will assist Dr. Puck starting in June, 2008 and then become the POC Chair in mid-2009. The term for the POC Chair will be two years.

- Only non-Federal members of the POC are eligible to be nominated.
- All POC members are eligible to nominate the Chair-Elect.
- Nominations should be sent to Paymon Hashemi (hashemi_paymon@bah.com) by April 25, 2008.
- Mr. Hashemi will forward the names of the nominees to Drs. Pearson and Hirschfeld, who will verify that Chair-Elect nominees are willing to participate and send the resulting slate of names to Mr. Hashemi by May 4, 2008.
- NCCR will place the nominees' names into the Adobe Connect system for voting from May 5 to May 9, 2008, and POC members will be notified when the election "opens".
- The Chair-Elect will be announced on May 12, 2008 and will begin his/her term at the June Face-to-Face meeting.

IV. Participation on CTSA Wiki

Dr. Puck invited POC members to visit the CTSA POC Wiki space (<http://www.ctsawiki.org/wiki/display/Peds/Pediatrics+Oversight+Committee+Home>). On the Wiki, members are able to view meeting summaries and rosters. Mr. Hashemi will create discussion pages to increase collaboration amongst members.

V. Planning Face-to-Face Meeting to be held June 23-24

Dr. Puck provided a brief description of a proposed schedule. The current plan is for individual workgroups to meet informally on the evening of June 22 from approximately 6:00 – 9:00pm ET, followed by the Face-to-Face meeting which will start on the morning of June 23 and adjourn during the early afternoon on June 24. The leadership will examine strategies to maximize participation for POC members who will be attending the Clinical Research Management Taskforce Workshop, which runs concurrently with the POC Face-to-Face Meeting. The meeting location and other details will be provided to POC members soon.

Members were informed that Drs. Barbara Alving, Director, NCCR, and Anthony Hayward, Director, Division of Clinical Research Resources, will be in attendance. Also, the POC plans to invite Dr. Steven Groft, Director, Office of Rare Diseases, NIH, to help assess ways in which CTSA's can work with his Office to improve research in rare diseases. Suggestions for other guests should be communicated to Paymon Hashemi.

Although it is important for CTSA investigators to get to know one another and learn more about pediatric programs at the CTSA sites, there will not be time for individual presentations at this

meeting. Instead, each POC voting member should prepare a brief introductory statement prior to the meeting. These will be posted on the WIKI and will be available at the meeting.

Members suggested that some time should be dedicated to the following topics for discussion:

- How pediatric research is being assimilated into the CTSA's
- Best practices for accomplishing CTSA goals with resource constraints. Dr. Ramsey volunteered to speak about the experience at the University of Washington

VI. Next Meeting

The next POC meeting will be the Face-to-Face Meeting in Bethesda starting with the informal session on the evening of June 22 and the formal meeting on June 23 and 24. The conference call adjourned at 1:30pm.

VII. Action Items

#	Action Items	Owner	Due Date
1	Submit protocol for multicenter adolescent CMV vaccine study to Mr. Hashemi	Drs. Edwards & Nesin	April 28
2	Submit protocol for multicenter genome-wide association study (GWAS) in children to Mr. Hashemi	Dr. Murray	April 28
3	Submit industry-sponsored studies upon receipt of permission and de-identification of company names to Mr. Hashemi	Drs. Al-Uzri, Kashyap, Lubin & Ramsey	April 28
4	Identify interested members of the Public Private Partnerships Steering Committee to participate at the proposed Pediatric Drugs and Devices Workshop in late 2008.	Dr. Sawczuk	April 28
5	Obtain survey of 50 industry partners to POC members from Dr. Ramsey	Mr. Hashemi	April 28
6	Submit brief description of rare disease work on map kinase pathway syndromes and Malignant Vascular Anomaly Syndromes	Dr. Rauen, Dr. Glade-Bender	April 28
7	Invitation to members to submit other ideas for rare diseases research	Mr. Hashemi	April 25
8	Create a discussion page for each of the POC Workgroups	Mr. Hashemi	April 14