

**Best Pharmaceuticals for Children Act (BPCA)
Pulmonary Therapeutic Area Working Group Conference Call and Webcast
October 7, 2011
2:00 p.m.–3:00 p.m. ET**

Participants

John Alexander, M.D., M.P.H.
John T. Berger, III, M.D.
Carol Blaisdell, M.D.
Louis Chicoine, M.D.
Jonathan M. Davis, M.D.
Oluchi Elekwachi, Pharm.D., M.P.H.
James M. Greenberg, M.D.
Sabrina Heidemann, M.D.
Nadia Hejazi, M.D.
Abraham Karkowsky, M.D., Ph.D.
Matthew M. Laughon, M.D., M.P.H.
Heber C. Nielsen, M.D.
Mary Purucker, M.D., Ph.D.
Hanna Phan, Pharm.D., B.C.P.S.
Michael Reed, Pharm.D., F.C.C.P., F.C.P.
George Z. Retsch-Bogart, M.D.
David Siegel, M.D.
Perdita Taylor-Zapata, M.D.
Teri Moser Woo, Ph.D., R.N., C.N.L., C.P.N.P.

Presentation

Dr. Taylor-Zapata presented and briefly discussed the following:

- BPCA Pediatric Therapeutic Area Outreach (“big picture”)
- The instructions and template for the working groups.

BPCA Pediatric Therapeutic Area Outreach. These slides summarized the working groups’ mission and charge and projected outcomes, which include interfacing, publications, and research initiatives. The BPCA program would like to see publications and research initiatives come out of these discussions, but these are not required outcomes. Funding is not guaranteed for research projects recommended by the working groups.

Instructions for Working Groups. In preparation for the next call and the BPCA annual meeting in December, working group members are asked to develop the following:

- A paragraph on scientific gaps in therapeutics in their field
- A needs assessment list—a list of what is needed to address the gaps
- A brief list of references, including the most relevant articles/journals related to scientific advances and evidence-based treatment in their respective field

- A “blueprint” for how to close the gaps.

Dr. Taylor-Zapata said that the working group would be divided into subgroups to prepare these paragraphs, which need to be completed in the next couple of weeks. The template provided in the instructions is meant to help working group members develop the paragraphs, which should identify the gap, what is missing, and what needs to be done to close the gap.

Discussion Points Document

Dr. Nielsen reviewed the Discussion Points document, which had been sent by e-mail to all the working group members. This document includes columns for gap area, disease, initial discussion points, names of interested members, and potential recommended discussion points. In the document, the highlighted names associated with particular gap areas are those members who had indicated that they were interested in taking responsibility for organizing recommendations for that area.

Dr. Nielsen found that some areas were well represented in terms of highlighted names, and others were not. To make sure all gap areas are covered, he listed interested members for each disease. The diseases and number of interested members were as follows:

- Pulmonary hypertension—11 interested members
- Asthma—8 interested members
- Cystic fibrosis—3 interested members
- Croup—1 interested member.

Some people identified themselves as interested in more than one area. Dr. Nielsen thought it would be best to ask the people who signed up for a particular disease to consider themselves a subcommittee and to ask three or four people to head the subcommittee. The subcommittee leaders’ tasks will be to get more complete input from subcommittee members and to put together the template sheets for the gap areas.

Proposed subcommittee leaders are as follows.

- Pulmonary hypertension subcommittee: Drs. Chicoine, Berger, and Laughon, and Dr. Allison Chung.
- Asthma subcommittee: Dr. Christopher Newth, Dr. Kimberley Benner, and Dr. Thomas Green. Croup can be combined with the asthma subcommittee; Dr. Newth is a leader for both asthma and croup.
- Cystic fibrosis subcommittee: Dr. Greg Kearns, Dr. Hanna Phan, and Dr. Retsch-Bogart.

Discussion

Dr. Blaisdell asked whether she had missed an e-mail that defined the subcommittees. Dr. Nielsen said that all the information he had came from the Discussion Points document that was sent to everyone. He used that document to identify the people interested in a particular disease and created the list of subcommittees and leaders. He can provide a list of subcommittee

members that can be disseminated, if it would be helpful. Not every working group member is assigned to a subcommittee, however.

Dr. Taylor-Zapata said that the Discussion Points document was sent out on October 6, but an updated version will be distributed that regroups everyone according to Dr. Nielsen's plan.

Dr. Reed asked whether working group members who had missed the opportunity to indicate interest in a subgroup should now state what they want to work on. He had sent an e-mail indicating his interest in working on croup and cystic fibrosis, but apparently his e-mail did not get through. Dr. Nielsen said he had not received Dr. Reed's email and noted that Dr. Woo, who was also not on his list, had volunteered to be in the asthma group. He said that the subcommittee leaders should be in touch with volunteers to ask for input for the templates or to request that they prepare a paragraph, for example.

The goal is to pull all the information together and get it to Dr. Taylor-Zapata to disseminate to the working group before the last conference call to be held the first week of November. Dr. Nielsen will be responsible for taking the information and creating a collated summary that will go to the BPCA annual meeting in December.

Dr. Nielsen said he would send Dr. Taylor-Zapata and Brandy Weathersby of Circle Solutions the information he had, including the names of additional volunteers from this conference call. This information can be sent to the working group; everyone will have a chance to say what area they want to be involved in. Within a few days, the subcommittee leaders will know who is in their group. Any subcommittee leader who is not able to be involved should contact Dr. Nielsen as soon as possible.

Dr. Retsch-Bogart asked for clarification about the next step for subcommittee leaders. Dr. Nielsen said the next step is to contact other people and pull in more information for the templates. The leaders could send out a group e-mail or have a conference call for their group to discuss what needs to be done. The leaders can ask volunteers to provide a paragraph to put in the template or ask them to complete the template for the gap area they chose. The leaders will need to distill the information received into a reasonable number of template sheets.

Dr. Taylor-Zapata asked everyone to identify their area(s) of interest by contacting her or by responding to Ms. Weathersby's October 6 e-mail. Dr. Taylor-Zapata and Ms. Weathersby can pull together the subgroups and make e-mail groups to connect the subgroups together.

Dr. Blaisdell asked whether federal staff should be involved in the subcommittees. Dr. Taylor-Zapata said that would be fine. Dr. Blaisdell said she could share what was learned from a recent National Heart, Lung, and Blood Institute (NHLBI) workshop on pulmonary hypertension. Dr. Nielsen said that insights from that workshop would be helpful and suggested that federal staff could be involved at the level and degree they think appropriate. He noted that there is a cross-cutting issues section at the end of the Discussion Points document that he had not addressed. NIH staff can help the working group be aware of any cross-cutting issues, such as funding sources, applicable to each subcommittee.

Dr. Davis said he thought he was the leader for the novel agents gap area and would love to have Dr. Blaisdell involved. One of the challenges is trying to find drugs that are already used in adults that can be tested in infants, and there are novel agents developed for neonates, such as nitric oxide, that are applicable. He hoped that Dr. Blaisdell would be able to share information about novel agents that were discussed at the NHLBI workshop or have been studied. Dr. Blaisdell said she would be happy to share information.

Dr. Nielsen noted that the pulmonary hypertension subcommittee will address the disease and its related gap areas, including the one Dr. Davis volunteered for. The subcommittee leaders can turn to Dr. Davis because he is willing to take responsibility for a novel agents subgroup.

Dr. Blaisdell said that, regarding asthma, the main issues were inhalation delivery and allergic reactions. If federal content experts are needed related to inhalation delivery, someone from the NHLBI's lung division could help.

Dr. Siegel mentioned two areas of interest: (1) the relationship of obstructive apnea due to obesity to pulmonary hypertension and (2) myocardial toxicity from medications used to treat asthma in the pediatric intensive care unit. Dr. Nielsen asked that Dr. Siegel contact the appropriate subcommittee leaders with this information.

If working group members have questions, they can contact Dr. Taylor Zapata, Ms. Weathersby, or Dr. Nielson.

Next Steps:

- Dr. Nielson will send Dr. Taylor-Zapata and Ms. Weathersby the information he compiled about the subcommittees for dissemination to the working group.
- Working group members who have not already done so should let Dr. Taylor-Zapata or Ms. Weathersby know right away about the area they wish to work on.
- Subcommittee leaders will contact volunteers by e-mail or telephone, collect input for the templates, and submit the templates to Dr. Taylor-Zapata before the first week of November.
- Dr. Taylor-Zapata and Ms. Weathersby will send out updated information to the working group and will make arrangements to connect subcommittee members by e-mail so they can work together to complete the templates.
- Circle Solutions will poll for a time for the final working group conference call to be held the first week of November.