Best Pharmaceuticals for Children Act (BPCA)
Hematology Working Group Conference Call and Webcast
October 4, 2011
10:00 a.m.–10:35 p.m. ET

Participants

Marcia L. Buck, Pharm.D., F.C.C.P., F.P.P.A.G.
Allan Doctor, M.D.
Beth Durmowicz, M.D.
Oluchi Elekwachi, Pharm.D., M.P.H.
Jonathan Goldsmith, M.D.
Gordon L. Klein, M.D., M.P.H.
Kathleen Neville, M.D., M.S.
Victor Santana, M.D.
Kristin Snyder, M.D.
Perdita Taylor-Zapata, M.D.
Courtney Thornburg, M.D., M.S.
Surendra Varma, M.D., F.A.A.P.
Anne Zajicek, M.D., Pharm.D.

Presentation

Dr. Taylor-Zapata presented and discussed the following:
- The BPCA Pediatric Therapeutic Area Outreach ("big picture")
- The instructions and template for the working groups
- The Hematology Working Group's revised discussion points.

Dr. Taylor-Zapata said that the working group had some good discussions during the last couple of conference calls, and now is the time to do some paperwork. She wanted to revisit the big picture slides, the instructions and template, and the discussion points document so that the group can prepare for the BPCA annual meeting, to be held December 8–9, 2011, in Bethesda, MD.

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 goal of interfacing—communication among working group members—is to develop long-lasting collaborations committed to improving scientific knowledge and availability of drugs used in children. The BPCA program would like to see publications and research initiatives come out of these discussions, but these are not required outcomes.

Instructions for Working Groups. In preparation for the next call and the annual meeting, working group members are asked to develop the following by the end of October:
- 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.

A template was provided for working group members to use to complete these tasks.

Previously, working group members signed up for a specific area(s) of interest in the "discussion points" document that was distributed after the second call. Working group members are now asked to consider and review the available published literature and the existing U.S. Food and Drug Administration (FDA) drug label regarding the drug of interest. Working group members are asked to provide their assessment regarding the scientific questions that need to be addressed.

Group members can be specific about the hypothesis that needs to be tested, the population that needs to be included in the study, the endpoints that need to be measured, and the general study design, but should refrain from providing directive specifics that sound like study proposals, grant proposals, and the like. The immediate outcome of these efforts is to help group leaders with developing final recommendations to be presented at the annual meeting in December.

Discussion Points Document. This revised document, which was sent to working group members on October 3, 2011, lists the gap areas identified during previous discussions. For each gap area, the document includes the disease/condition, initial discussion comments, drugs of interest, names of working group members who expressed an interest in this area, and recommended discussion points. The lead person for each gap area is asked to develop the paragraph about scientific gaps, needs assessment list, reference list, and blueprint, as described in the instructions and template.

Dr. Thornburg said that the template provides a guide to the information needed for each topic area. When she received discussion points from interested members, she tried to assign each person to one topic of interest and avoid assigning people to multiple areas. For example, in the pathophysiology area related to pediatric stroke, more evidence is needed that children and adults are different and that children need specific studies for drugs of interest. Group members who are assigned to one topic and also listed as an interested member in another topic area can provide their thoughts to the leader in that topic area. When two people are leaders for a topic area, they can work together to complete the tasks in that area. Dr. Thornburg reviewed the topics and the working group members assigned to each topic.

Dr. Klein asked about the logistics of turning in the material called for in the template. Dr. Thornburg said the leaders could send completed templates to her, Dr. Taylor-Zapata, and/or Brandy Weathersby of Circle Solutions. They will review the material submitted and put everything together to present during the next conference call. Dr. Klein asked how he should contact other working group members assigned to his area. Dr. Taylor-Zapata said contact information would be e-mailed to him and the other topic leaders. The material indicated in the template should be sent in by October 31, 2011. Circle Solutions will poll for the next call, which she hopes will occur during the first week of November. During that call, working group members will discuss how to present the information at the annual meeting.

Dr. Doctor asked about the mechanism by which proposed studies will be funded and executed. Dr. Taylor-Zapata explained that questions will go to the National Institute of Child Health and Human Development's Pediatric Trials Network (PTN), which will prioritize what the network can do. Some studies could be done as a contract if they are not within the scope of the PTN. However, there is no guarantee about funding for research based on the working group's recommendations. Information about the PTN is on the BPCA Web site at http://bpca.nichd.nih.gov/clinical/network/index.cfm.

Dr. Taylor-Zapata encouraged working group members not assigned to a topic area to fill out a template for a topic of interest and send it in by the end of October. She noted that registration information for the annual meeting will be sent out next week.

FDA Meeting Announcement

Dr. Durmowicz announced that the Pediatric Oncology Subcommittee of the Oncologic Drugs Advisory Committee, FDA, is meeting on Wednesday, November 2, 2011, to discuss the development of anticoagulant products in pediatric patients. The meeting is public and will be held at the FDA White Oak Campus in Silver Spring, MD. Members of the Hematology Working Group may be interested in attending. Details about the meeting will be sent to the working group. [Note: The link to the *Federal Register* notice for the meeting is www.regulations.gov/#!documentDetail;D=FDA-2011-N-0002-0111.] Dr. Snyder said that she is part of the planning committee for the meeting, which will certainly be relevant for the working group. A transcript will be available about a month after the meeting; however, an early draft of the transcript is usually available sooner for review. Dr. Snyder will find out whether the transcript will be available in time for the annual meeting.

Next Steps:

- Topic leaders will work with others assigned to their area to complete the information in the template. They will send completed templates to Dr. Thornburg, Dr. Taylor-Zapata, and/or Brandy Weathersby by October 31, 2011.
- Contact information for interested working group members will be e-mailed to the topic area leaders.
- Details about the FDA committee meeting to discuss anticoagulant drugs in pediatric patients will be e-mailed to the working group members.
- Dr. Snyder will find out whether the transcript of the November 2, 2011, FDA committee meeting will be available in time for the annual meeting.
- Ms. Kistler will poll the working group about the time for the next conference call in the first week of November.