

**Best Pharmaceuticals for Children Act (BPCA)  
Antipsychotics Safety Therapeutics Working Group Conference Call  
April 23, 2010  
10:00 a.m.–10:45 a.m. ET**

**Participants**

Matthew Bacho  
Jeffrey Blumer, M.D., Ph.D.  
Felicia Collins, M.D., M.P.H.  
Judith Cope, M.D., M.P.H.  
Julie Dopheide, Pharm.D.  
Gilman Grave, M.D.  
James Korelitz, Ph.D.  
Merle Paule, Ph.D.  
Merrily Poth, M.D.  
Adelaide Robb, M.D.  
William Rodriguez, M.D., Ph.D.  
Perdita Taylor-Zapata, M.D.  
Benedetto Vitiello, M.D.  
Anne Zajicek, M.D.  
Julie Zito, Ph.D.

**Purpose**

The purpose of this conference call and webcast was to review the following:

- 2009 BPCA annual scientific meeting recommendations and comments
- Progress in response to the working group’s recommendations
- Current plans for 2010 prioritization and meeting.

An open forum for questions and discussion concluded the call.

**Presentation**

Dr. Taylor-Zapata reviewed the 2002 BPCA and the 2007 BPCA and described the role of the National Institutes of Health (NIH) in the BPCA program. The focus of 2007 BPCA activities shifted from prioritizing drugs to identifying the needs of pediatric therapeutics. The NICHD has primary responsibility for implementing the BPCA program, including organizing and developing a prioritization process, awarding and monitoring clinical trials, developing investigational new drugs, collecting data for potential label modification, and drafting and submitting label modifications for specific ages and indications. The NIH develops—in collaboration with the Food and Drug Administration (FDA), Institutes, experts, parents, and others—an updated priority list of needs in pediatric therapeutics, identifying primarily off-patent therapies that “most urgently” require study in pediatric populations. The guiding principles of the revised BPCA prioritization framework are (1) a well-defined process, (2) well-

defined objective criteria, (3) legitimacy and fairness, and (4) expert involvement. There are four stages of the prioritization process: (1) gather nominations, (2) prioritize therapeutic areas, (3) prioritize pediatric needs, and (4) gather public comment. The main goal of the BPCA program is to advance the science of pediatric pharmaceuticals and improve pediatric labeling. The BPCA program uses global and specific outreach approaches to achieve this goal.

A request for information has been posted asking for research nominations concerning pediatric needs and gaps in therapeutic areas. A preliminary list of nominations will be published in the *Federal Register* in July or August 2010. The list will be finalized in late 2010 or early 2011.

## **2009 BPCA Annual Scientific Meeting Recommendations and Comments**

The Antipsychotics Safety Therapeutics Working Group made the following recommendations:

- Funding is needed urgently to implement the working group's recommendations.
- A deficit exists in needed data and studies for understanding pediatric antipsychotic therapeutics, particularly use over the long term.
- The FDA should make short-term data available for secondary studies by investigators from the field.
- The working group should draft a review article summarizing current knowledge and recommended directions.
- The working group, the NIH, and the FDA need to collaborate to identify the relevant variables to be included in electronic medical records.
- The working group needs to learn more about the FDA's Adverse Event Reporting System so that recommendations can be developed regarding its use for monitoring serious adverse events (AEs) associated with pediatric antipsychotic therapeutics.
- A design needs to be developed for studies of risk factors/predictors of AEs and the effects of long-term use of antipsychotic medications.
- Animal models need to be explored to address issues of toxicity, particularly with long-term use.
- The working group has an important purpose and would like to continue its activities.

## **Progress in Response to Recommendations**

In response to these recommendations, the following progress has been made:

- With regard to needed data and studies for understanding pediatric antipsychotic therapeutics, particularly use over the long term, several working group members presented at the FDA Advisory Committee meeting in November 2009 and discussed the needs and issues. The FDA is working with pharmaceutical companies to collect more long-term data.
- With regard to developing a design for studies of risk factors/predictors of AEs and the effects of long-term use of antipsychotic medications, (1) the NICHD is considering future funding opportunities in the area of AEs and (2) the NIH has been in contact with a number of pediatric networks in order to identify patients taking antipsychotic medications and gather information on long-term use. The NICHD has begun discussions with an American Academy of Pediatrics network that is considering this effort.

- With regard to drafting a review article summarizing current knowledge and recommended directions, Dr. Taylor-Zapata asked for follow-up from working group members and is willing to offer assistance in drafting the article.

## **Current Plans for 2010 Prioritization and Meeting**

The 2010 BPCA annual scientific meeting is tentatively scheduled for November. Antipsychotics Safety Therapeutics Working Group members will be invited. BPCA program activities in 2010 will focus on three therapeutic areas: endocrinology, gastroenterology, and neurology. New working groups will be formed to address these areas. Working group members are invited to participate in 2010 teleconferences and to become stakeholders who evaluate new therapeutic areas under consideration for BPCA funding.

## **Open Forum**

The following topics and issues were noted and/or discussed:

- A request for access to data that the FDA holds for independent analysis—A national working group meeting is being planned for September 2010 to look at ways to improve collection of AE data (sponsored by the Critical Path Institute).
- Development and drafting of the review article—Dr. Zito and three colleagues have begun a literature review and are summarizing the working group's recommendations.
- Inclusion of the American Academy of Child and Adolescent Psychiatry and Medicaid as resources to identify patients taking antipsychotic medications and gather information on long-term use
- A request for FDA follow-up to the November 2009 meeting of the Pediatric Advisory Committee.

In addition:

- Dr. Paule volunteered to join the neurology working group.
- Dr. Poth volunteered to join the endocrinology working group.
- Drs. Dopheide and Poth volunteered to help Dr. Zito with drafting the review article.

## **Action Items:**

- Dr. Zito will send her contact information and contact information for Dr. Safer to Dr. Rodriguez, who will pass the information on to AE working group meeting organizers.