

**Best Pharmaceuticals for Children Act (BPCA)
Neurology Disease Therapeutics Working Group Conference Call and Webcast
June 10, 2010
2:00 p.m.–2:35 p.m. ET**

Participants

Virginia Elgin, M.D., FAAP
Margaret C. Grabb, Ph.D.
George Ricaurte, M.D., Ph.D.
Philip Sheridan, M.D.
Karen Sirocco, Ph.D.
Perdita Taylor-Zapata, M.D.
Steven Weinstein, M.D.

Purpose

The purpose of this conference call and webcast was to follow up on the working group's discussion during its April 28 conference call and discuss potential future activities.

Presentation

Dr. Taylor-Zapata briefly discussed the following:

- Information about BPCA activities, the three 2010 working groups, and the BPCA annual prioritization meeting. The working groups' recommendations will be presented at the annual meeting (to be held on November 9 and 10, 2010, in Bethesda, MD).
- The recommendations made by the Neurology Disease Therapeutics Working Group during its April conference call
- Follow-up activities conducted since the April call, including a database search.

Recommendations from April Conference Call

During its April conference call, the working group recommended that the NICHD should:

- Consider attention deficit-hyperactivity disorder (ADHD) and related disorders as part of the neurology area
- Acquire data on:
 - The general frequency of neurological conditions in the pediatric population
 - The number and type of drugs most frequently prescribed for pediatric neurology disorders
- Obtain more information on the long-term effects of drugs:
 - The ineffectiveness or even deleterious effects of long-term drug use in pediatric populations
 - Acute versus chronic medication use
 - Behavioral effects (that is, suicidality and drug abuse) of long-term medication use

- Conduct more comparative studies of drug use in pediatric versus adult populations as well as more pharmacokinetic/pharmacodynamic studies
- Gather more information on the use/extrapolation of animal models
- Address specific drug-related issues:
 - Therapeutics for autism/autism spectrum disorder
 - Developmental anesthesiology, including neonatal and perinatal anesthesiology
 - Therapeutics for traumatic brain injury.

Follow-Up Activities

Dr. Taylor-Zapata described some follow-up activities conducted since the April call. The NICHD, through a contract with the Lewin Group, was able to obtain information from a search of the Ingenix database of pharmacy claims for about 20 million people across the United States. The database includes about 4 million children. The search identified the top 20 neurologic therapeutics used in children. Categories of commonly used drugs include narcotic analgesics, stimulants, antiemetics, antianxiety agents, antidepressants, and antipsychotics.

Previous research in this area under BPCA includes:

- ADHD—claims database information on frequency of diagnosis and frequency of medications from 2000 Medicaid database and 2004 commercial database
- Migraines—literature reviews (2007)
- Pediatric anesthetic toxicities—literature reviews (2008)
- Fragile X and autism research—BPCA co-funded NICHD-sponsored research on new therapeutic approaches for these disorders.

Dr. Taylor-Zapata provided information about the Traumatic Brain Injury (TBI) Clinical Trials Network, which is part of the National Center for Medical Rehabilitation Research. The contact person for the TBI network is Dr. Beth Ansel. Dr. Taylor-Zapata discussed a National Institute of Neurological Disorders and Stroke (NINDS) meeting in May 2010 on headache. She attended the meeting and participated in the pediatric headache breakout group. Each breakout group will put together its recommendations for future study. The NINDS contact person is Dr. Linda Porter.

Dr. Taylor-Zapata listed some potential next steps and asked for comments from the group. The working group will meet in a breakout session on the first day of the BPCA annual meeting (November 9), and a representative will present the group's recommendations on the second day. Details about the annual meeting will be sent to everyone. Requests for specific information, such as additional literature reviews or database searches, should be sent to her.

Open Forum

Working group members asked about the following:

- The length of studies supported under BPCA. Dr. Taylor-Zapata said that studies are typically 3- to 5-year trials. The BPCA legislation is up for renewal in 2012.

- The funding mechanism for the BPCA studies. Dr. Taylor-Zapata said the BPCA funding for studies prioritized under the NIH prioritization process for off-patent drugs is drawn from 19 NIH Institutes and Centers. In addition, there is a large component of BPCA studies conducted by pharmaceutical companies for patented drugs through the Food and Drug Administration Amendments Act (FDAAA).
- The need for a fundamental understanding of the pharmacokinetic differences between children and adults to guide practitioners. Dr. Sheridan noted that the FDA will publish a paper about how extrapolations from adult data are made in the approval of drugs for pediatric use. He will send the group a preprint once the paper is accepted.

Next Steps:

- The NICHD will ask the Lewin Group to conduct further searches of the Ingenix database to gather more specific information about the top neurologic drugs used in children.
- Dr. Taylor-Zapata will send the group the past literature reviews on the topics of migraines and pediatric anesthetic toxicities.
- Dr. Taylor-Zapata will send the group information about the BPCA annual meeting.
- Group members will email Dr. Taylor-Zapata with other ideas for activities.