

**Best Pharmaceuticals for Children Act (BPCA)  
Neurology Disease Therapeutics Working Group Conference Call and  
Webcast**

**April 28, 2010**

**10:00 a.m.–10:45 p.m. ET**

**Participants**

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Lucy Civitello, M.D.  
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Margaret C. Grabb, Ph.D.  
David Jett, Ph.D.  
Roger Packer, M.D.  
Merle G. Paule, Ph.D.  
James Riviello, M.D.  
Christopher Sarampote, Ph.D.  
Philip Sheridan, M.D.  
Perdita Taylor-Zapata, M.D.  
Steven Weinstein, M.D.

**Purpose**

The purpose of this conference call and webcast was to:

- Provide an overview of BPCA
- Describe the role of therapeutic area working groups
- List the needs in neurology (questions to experts).

**Presentation**

Dr. Taylor-Zapata reviewed the background of BPCA legislation and the work being carried out in the BPCA program. The *Eunice Kennedy Shriver* National Institute of Child Health and Human Development (NICHD) is the lead agency responsible for establishing and conducting pediatric drug development activities, which are carried out by its Obstetric and Pediatric Pharmacology Branch. The drug development program involves a prioritization process to identify gaps in pediatric therapeutics that need further study and clinical trials of primarily off-patent drugs that have been prioritized. As of November 2009, 106 therapeutics have been discussed with experts and 76 drug–indication pairs have been identified and listed as priority. As of March 2009, 16 therapeutic categories, 35 diseases/conditions, and 48 therapeutics—drugs, biologics, or delivery systems—have been listed as priority.

Each year, the NICHD identifies three new areas for focus. For 2010, those areas are neurology, endocrinology, and gastroenterology. Therapeutic area working groups for these three areas have

been formed. The groups will meet two or three times a year. Minutes of meetings will be posted on the BPCA Web site and distributed to working group members. The NIH asks the working groups for recommendations of drugs (drug classes) or other areas of research that impact therapeutics and need further study in pediatrics. The Neurology Disease Therapeutic Working Group's recommendations will be presented at the annual BPCA prioritization meeting in November 2010 and could lead to publications, workshops, or studies. Working group members will be invited to participate in the annual meeting.

The current BPCA-related interests in neurology are (1) treatment of seizures with lorazepam, (2) treatment of attention deficit/hyperactivity disorder (ADHD) with methylphenidate, and (3) limited pharmacoepidemiology data (for example, the 2004–2005 commercial database and the 2000–2001 Medicaid database). Potential BPCA-related interests in neurology are (1) treatment of migraines with eletriptan and (2) treatment of seizures with zonisamide.

With regard to the needs in neurology under the BPCA program, Dr. Taylor-Zapata noted the following:

- The BPCA program mandate is to identify needs in therapeutics.
- The NICHD is fully aware that the National Institute of Neurological Diseases and Stroke is the lead institute in neurology research.
- The NICHD became interested in the needs in this area due to three drugs (lorazepam, eletriptan, and zonisamide) that were listed as priority for study under the BPCA program.

The questions posed to the Neurology Disease Therapeutics Working Group were as follows:

- Would the study of eletriptan for the treatment of migraines be feasible/practical?
- Has the issue of drug efficacy in the treatment of adolescent migraines been resolved? Have the right outcome measures been used?
- Is the issue of high placebo responder rates in headache studies still a factor in clinical trial designs?
- Would the study of zonisamide for the treatment of seizures be feasible/practical?
- Are there other therapeutic gaps in the area of pediatric neurology that working group members would like to recommend for consideration under the BPCA program?

## Open Forum

Working group members recommended the following areas for consideration:

- Considering ADHD and related disorders as part of the neurology area
- Therapeutics for autism/autism spectrum disorder
- Acquiring data on the number and type of drugs prescribed for pediatric neurology disorders
- Identifying drugs that are most frequently prescribed for a particular disease or disorder and for which there are no or limited data to support their use
- Defining the general frequency of neurological conditions (for example, headache) in the pediatric population
- The ineffectiveness or even deleterious effects of long-term drug use in pediatric populations
- Developmental anesthesiology, including neonatal and perinatal anesthesiology, because of the drugs' potential for life-long effects

- Comparative studies (for example, efficacy and mechanisms of action) of drug use in pediatric versus adult populations
- Pharmacokinetic/pharmacodynamic studies
- Acute versus chronic medication use
- Therapeutics for traumatic brain injury
- Behavioral effects (for example, suicidality and drug abuse) of long-term medication use
- Use of appropriate animal models.

### **Next Steps:**

- Dr. Taylor-Zapata will attempt to provide a list of frequently used pediatric neurology drugs and what the drug are being used for.
- A second working group call will be scheduled in about 6–8 weeks.