

**Best Pharmaceuticals for Children Act (BPCA)  
Endocrine Therapeutics Working Group Conference Call/Webcast  
June 10, 2010  
10:00 a.m.–10:30 a.m. ET**

**Participants**

Audrey Austin, M.D.  
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Elizabeth Durmowicz, M.D.  
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**Purpose**

The purpose of this conference call/webcast was to:

- Review the BPCA priority list development process
- Review the role of therapeutic area working groups
- Describe the needs in pediatric endocrinology
- Provide an activities update
- Have an open forum
- Determine next steps.

**BPCA Priority List Development Process**

The goal of the BPCA program is to identify and prioritize the needs in pediatric therapeutics. The BPCA program develops a priority list of drugs, drug classes, and therapeutics. Each year, the National Institutes of Health identifies three new therapeutics areas and create working groups to focus on the areas. The 2010 areas are Neurology Disease Therapeutics, Gastroenterology Disease Therapeutics, and Endocrine Therapeutics.

**Role of Therapeutic Area Working Groups**

Therapeutic area working groups discuss the needs in their area of pediatric medicine and make recommendations of drugs or drug classes or other areas of research that impact therapeutics that need further study in pediatrics. The working groups meet two or three times in a calendar year, develop recommendations, and present the recommendations at the annual BPCA meeting. The outcome of these activities is consideration of future studies, workshops, and publications.

## Needs in Pediatric Endocrinology

At the November 2009 annual BPCA meeting, Dr. Rivkees presented the following pediatric endocrinology needs:

- Define incidence/prevalence of disorders
- Define treatment practices
- Define off-label treatment practices
- Define complications of therapy
- Postmarketing drug surveillance.

During the working group's April conference call/webcast, the following pediatric endocrinology needs were identified:

- Lack of convenient dosage forms for endocrine drugs for children (for example, thyroid hormones and hydrocortisone)
- Mining existing data to give insight into safety of drugs
- Shortages of pediatric endocrine therapeutics (for example, short supply of drugs such as diazoxide)
- Concern for aggressive off-label marketing by some pharmaceutical companies
- Working group as a forum to disseminate information to the scientific community about what is known and not known, what is approved, and so on
- Drug study considerations (for example, use of aromatase inhibitors, which are not approved to augment height, but are widely used).

## Activities Update

In response to the working group's recommendations to begin identifying major diagnoses of endocrine disorders and medications, the Ingenix United Health Group Analytic Platform Database was searched for use of endocrine-related drugs. This is a large claims database, which includes inpatient, pharmacy, and physician claims for about 20 million people—about 4.5 million of which are children (<18 years). For 4-year pharmacy coverage, there are 1.5 million children. The database is not nationally representative and does not include Medicaid patients.

The initial search used National Drug Codes—not ICD-9 codes—to determine which drug classes were being prescribed for pediatric endocrine disorders. The most prevalent were:

- Glucocorticoids
- Contraceptive hormones
- Insulin
- Glucagon
- Contraceptive implants/injections/systems
- Vasopressin derivatives
- Thyroid hormones and analogues.

There is a BPCA initiative to partner with pharmaceutical companies to develop new pediatric drug formulations. The initiatives for baclofen and hydroxyurea were not as successful as expected. The NIH is still exploring ways to identify partners to help develop new formulations.

Two current partners are the European Union and the Food and Drug Administration (FDA). Activities with these partners are progressing.

Dr. Durmowicz provided information on an FDA initiative for aggressive off-label marketing. The initiative is called the Bad Ad Program. The program's purpose is to help health care professionals recognize misleading prescription drug promotion and provide them with an easy way to report this activity to the FDA.

The next activity in developing the working group's recommendations is to identify specific pediatric endocrine diagnoses and their prevalence. Additional searches of the Ingenix database can be conducted. In addition, literature on drug classes, therapeutics, and diagnoses can be summarized.

## **Open Forum**

The working group discussed the following issues:

- Inclusion of Medicaid data on pediatric endocrine drugs and endocrine diagnoses (for example, the differences between Medicaid and commercial prescribing)
- Developing a list of the top 10–20 pediatric endocrine diagnoses using ICD-9 codes and determining the frequency of diagnoses
- Availability of information on age, demographics, socioeconomic status, and race/ethnicity
- Determining associations between drugs and diagnoses
- Concerns about the use of information on off-label drug use (for example, by insurance companies).

## **Next Steps**

- Drs. Rivkees and Geller will develop a list of the top 10–20 pediatric endocrine diagnoses.
- The list will be submitted to the contractor that will search the Ingenix database.
- Ideas for gathering additional information (for example, literature summaries) should be sent to Dr. Taylor-Zapata.