Best Pharmaceuticals for Children Act (BPCA)
Renal Disease Working Group Conference Call and Webcast
December 2, 2011
2:30 p.m.–3:15 p.m. ET

Participants

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Peter Belamarich, M.D.
Joseph Flynn, M.D.
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Michael Reed, Pharm.D., F.C.C.P., F.C.P.
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Bradley Warady, M.D.

Discussion

Recommendation templates and summaries of the working group's themes were distributed to participants before the call. The presentations for the BPCA Annual Meeting have not been finalized. Originally, each theme was limited to 10–15 minutes; the themes should be shortened to less than 10 minutes to allow time for discussion.

The group discussed the goals of the BPCA Annual Meeting:

- Dr. Kaskel said that the presentations should include a brief discussion of the problem, gaps in knowledge, and areas for future study.
- The audience will be pediatricians, clinicians, and researchers who are familiar with the BPCA, many of whom have attended the BPCA Annual Meeting in the past.
- No decisions are made at the meeting. The meeting is an opportunity for people to discuss concerns and potential areas for future research.
- The audience will include stakeholders from other subspecialties, industry, and the federal government, including the BPCA Program and the *Eunice Kennedy Shriver* National Institute of Child Health and Human Development (NICHD). Anyone may attend the meeting, and more than 100 people attended the first day of the 2010 meeting.
- The meeting provides a brief snapshot of gaps in knowledge in priority areas.
- There may be funding for research for priority areas.
- The BPCA Program and the NICHD provide input into research conducted by the Pediatric Trials Network (PTN).
- The working groups should provide recommendations for the BPCA Program. The National Institutes of Health and the U.S. Food and Drug Administration (FDA) will decide on the key gaps and work with the PTN to address those gaps.

Dr. Kaskel explained that in December 2010, the group was encouraged to combine acute kidney injury in continuous renal replacement therapy and anemia in a single write up for prioritization that year. In the spring and summer of 2011, the group added dyslipidemia, anticoagulation, and hypertension and agreed not to address vitamin D. On November 18, the FDA sent a letter to the American Society of Pediatric Nephrology regarding interest in trials of erythropoiesis-stimulating agent in pediatrics. The FDA also recently held a meeting on anticoagulation in pediatric diseases. While the working group is recommending some drugs that the BPCA Program has discussed before, the drugs have not been discussed in the context of renal disease.

The group discussed revisions to the presentations:

- The purpose of the presentations is to highlight the research needs.
- The audience needs to hear that good outcome data are lacking. A unifying theme is that the health of children with renal disease is not being addressed appropriately.
- Safety and efficacy should be highlighted. The BPCA Program may also emphasize areas that are underfunded or where there are gaps in knowledge.
- The mandate of the BPCA Program is to generate studies of off-patent drugs and to add labeling information for children.
- The presentations should focus on gaps in knowledge, how the drug is used, and how data can be obtained to allow nonexperts to use the drug safely and effectively in children.
- Presentations should address the types of studies and duration of studies that are needed, focusing on short-term outcomes.
- The BPCA Program focuses on drugs and devices.
- For blood pressure, lipids, and other measures, the FDA commonly accepts surrogate endpoints. The surrogate endpoints may not be the same in adults and children. Each presentation should include a slide that clearly defines the endpoints.
- Each presentation should identify the problem, the condition, the gaps in knowledge, the studies needed, and endpoints that can be measured in the short term.
- The BPCA Program may favor studies that can be completed in 1–2 years. If a longer study is needed, the group will need to justify the importance of the study. The presentations should emphasize short-term efficacy and safety issues. Long-term follow-up could be proposed if data suggest chronic effects.
- Short-term studies can be building blocks for long-term studies.
- Presentations should emphasize that studies are needed to understand safety issues.
- Presentations should mention existing research networks that studies can utilize.

Action Items:

- Dr. Warady will send Dr. Kaskel the number of patients in the North American Pediatric Renal Trials and Collaborative Studies network.
- Dr. Kaskel will send the group notes about the presentations by December 3.
- Dr. Kaskel will survey the group about arrival times for the BPCA Annual Meeting. The group may meet for dinner on December 7 and for breakfast on December 8.
- Presentations should be submitted to Ayesha Navagamuwa of Circle Solutions, Inc., by the morning of December 6.