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EDITORIAL

Best Pharmaceuticals for Children Act and Pediatric Research Equity Act: Time for Permanent Status

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The Best Pharmaceuticals for Children Act (BPCA) and Pediatric Research Equity Act (PREA) are companion legislation acts that together have resulted in a significant increase in new knowledge about the safe and effective use of medicines leading to a significant increase in the number of drugs that have pediatric labeling.[1] One estimate is that more drugs have been studied in children in the last decade than in the preceding 5 decades.[2] Together, these two laws have been invaluable in adding information to the drug label concerning the safe and effective use of more than 400 drugs in neonates, infants, children, and adolescents.

BPCA and PREA are set to expire October 1, 2012, and must be reauthorized to continue to promote safety and efficacy studies in children. In March 2012, the BPCA and PREA Reauthorization Act of 2012 was introduced; this Bill would 1) make the Acts permanent, 2) mandate expedited planning and completion for pediatric studies, 3) increase data collection for neonatal studies, and 4) increase transparency for the status of pediatric studies. The Pediatric Pharmacy Advocacy Group has joined the American Academy of Pediatrics and the Elizabeth Glaser Pediatric AIDS Foundation in supporting this important legislation.

In February 2012, the Institute of Medicine (IOM) released a report entitled *Safe and Effective Medicines for Children*,[3] which advocates making the BPCA and PREA laws permanent. The requirement that the bills be reauthorized every 5 years has occurred over the past 15 years and has served to create uncertainty for pharmaceutical manufacturers and the US Food and Drug Administration (FDA). Drug development is a prolonged process, taking 10–12 years from discovery to approval, and uncertainty about the requirement for conducting pediatric studies makes it difficult to plan and conduct the necessary studies.

In the early 1990s, the IOM focused concern on the lack of drug testing in children. In response to the IOM report, Congress and the FDA created legislation and policies to encourage greater incentives for the pharmaceutical industry to complete studies in children by providing an additional 6 months of exclusivity in the 1997 FDA Modernization Act.[4] Reauthorization of BPCA in 2002 created the Office of Pediatric Therapeutics within the FDA and created a Foundation at the National Institutes of Health to study "off-patent" drugs. PREA, first enacted in 2003, gives the FDA the authority to require drug manufacturers to complete studies in children for the same adult indications when the drugs are expected to be used in a substantial number of children. BPCA and PREA were both reauthorized by Congress in 2007.

Oncology drugs present a unique problem in children because children often have different cancers than those found in adults.[2] PREA currently requires that drugs be studied in children only for the same disease for which they are approved in adults. Drug companies cannot be required to study oncology drugs in children for different indications. A proposed solution would be to require that when a new cancer drug is approved that targets a specific disease pathway or gene mutation in an adult tumor, that drug would be required to be studied in pediatric tumors that share the same pathway or gene mutation.

It is time to make BPCA and PREA legislation permanent to assure continued investment in the study of safe and effective use of new medicines in infants and children.

ABBREVIATIONS BPCA, Best Pharmaceuticals for Children Act; FDA, Food and Drug Administration; IOM, Institute of Medicine; PREA, Pediatric Research Equity Act

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REFERENCES

1. Spielberg SP. The dawn of pediatric personalized therapeutics. Yaffe Award Lecture. *J Pediatr Pharmacol Ther*. 2010;15(1):10–16.
2. McCaul M, Van Hollen C for the Congressional Childhood Cancer Caucus. BPCA and PREA Reauthorization. 2011. http://childhoodcancer-mccaul.house.gov/issue/bpca-and-prea-reauthorization. Accessed July 12, 2012
3. National Research Council. *Safe and Effective Medicines for Children: Pediatric Studies Conducted Under the Best Pharmaceuticals for Children Act and the Pediatric Research Equity Act*. Washington, DC: National Academies Press, 2012.
4. Spielberg SP. Pediatric therapy for the new millennium. *J Pediatr Pharmacol Ther*. 2001; 6(1):6–9.