

Safe and Effective Drugs for Children

Best Pharmaceuticals for Children Act Pediatric Research Equity Act

BCPA and PREA: An Overview

Children are not just small adults. Drugs work differently in children than in adults and must be studied specifically for their use.

The **Best Pharmaceuticals for Children Act (BCPA)** and the **Pediatric Research Equity Act (PREA)** are two laws that encourage and require the study of drugs in children. Data resulting from BCPA and PREA studies are added to drug labels to give parents and providers essential information on the safety and efficacy of drugs used in children.

PREA requires drug companies to study adult drug indications in children when the product is likely to be used in a significant number of children or represents a meaningful therapeutic benefit over existing therapies. While PREA is a requirement, it cannot delay access to the adult version of the product.

BCPA is an incentive for drug companies to conduct FDA-requested pediatric studies—especially for off-label drug uses—in return for an additional six months of marketing exclusivity.

A Track Record of Success

BCPA and PREA have changed pediatric practice because all studies result in labeling changes that provide valuable new pediatric information. These studies have resulted in new dosing information, new indications of use, new safety information, and new data on effectiveness. Drugs studied under BCPA and PREA treat a wide range of diseases in children, including cancer, HIV/AIDS, diabetes, allergy and asthma.

426 drug labels have been revised with important pediatric information (181 under PREA, 147 under BCPA, 50 under both BCPA and PREA, and 48 under precursor regulations).

Before BCPA and PREA, the vast majority of drugs—more than 80%—used in children were used off-label, without data on their safety or efficacy. Today that number has been reduced to approximately 50%. While there has been significant success, more progress is needed.

Priorities for Reauthorization

Reauthorize these important laws. BCPA and PREA expire on October 1, 2012. These laws must be reauthorized to continue these important advances for children. PREA should be made permanent law—basic safety and efficacy provisions for adults do not expire and they should not for children. In addition, the program that funds the study of older off-patent drugs at the National Institutes of Health (NIH) should also be reauthorized.

Plan pediatric studies earlier. The precursor to PREA, the Pediatric Rule, required that drug companies discuss and plan for pediatric studies no later than the end of phase 2. Since PREA replaced the Pediatric Rule, the pediatric plan submission under PREA cannot occur until the time a company submits its drug application. This can lead to insufficient and inappropriate study plans and delays of important pediatric data. *Drug companies should begin discussions with FDA at the end of phase 2 of the drug development process, similar to the Pediatric Rule.*

Improve accountability. PREA studies can be waived or deferred to a later time post-market. Deadlines for deferred studies, however, are missed a vast majority of the time. Of all deferred PREA studies due after September 27, 2007, an alarming 78% are currently late or were submitted late. *The FDA should distinguish between reasonable and unreasonable delays and have enforcement tools comparable to those for post-market requirements in adults to ensure that pediatric data is gathered as soon as possible.*

Promote studies in younger age groups. Studying drugs in neonates—the smallest children—is very difficult. The GAO found that FDA lacks neonatal expertise. *Increase neonatal expertise at FDA and encourage additional discussion of neonates and younger age groups.*

Increase transparency. Pediatric researchers cannot access information on what drugs are currently being studied under BCPA. BCPA study requests declined by drug companies—evidence of important gaps in pediatric data—are never made public. Some pediatric data submitted prior to 2007 has also not been made public. *Improve transparency provisions to increase access to important pediatric information.*

SUPPORTING ORGANIZATIONS

Academic Pediatric Association
AIDS Alliance for Children, Youth, and Families
American Academy of Child and Adolescent Psychiatry
American Academy of Pediatrics
American Pediatric Society
American Psychiatric Association
American Society of Pediatric Nephrology
American Thoracic Society
Arthritis Foundation
Association of Medical School Pediatric Department Chairs

Children's Hospital Association
Council of Pediatric Subspecialties
Elizabeth Glaser Pediatric AIDS Foundation
March of Dimes
National Association of Pediatric Nurse Practitioners
National Organization for Rare Disorders
Pediatric Pharmacy Advocacy Group
Society for Pediatric Research
Society for Adolescent Health and Medicine

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