

BPCA 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 (BPCA)** and the **Pediatric Research Equity Act (PREA)** are two laws that encourage and require the study of drugs in children. Data resulting from BPCA 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 required, FDA does not delay the approval of the adult indication while studies are pending.

BPCA is a voluntary 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 added to all forms of the drug and at the end of other exclusivities.

A Track Record of Success

BPCA 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 information on dosing, indications of use, safety, and effectiveness. Drugs studied under BPCA and PREA treat a wide range of diseases in children, including cancer, HIV/AIDS, mental health disorders, allergy and asthma. To date, more than 664 drug labels have been revised with important pediatric information as a result of BPCA and PREA.

Before BPCA 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.

BPCA/PREA Priorities for User Fee Reauthorization

Remove the PREA Orphan Drug Exemption. Orphan drugs are currently exempt from PREA's pediatric study requirements. In recent years, roughly 40% of all drugs approved by FDA annually were designated as orphan drugs, meaning FDA cannot require these drugs to be studied in children under PREA despite that fact that 50-75% of orphan diseases occur in children. Removing the rare disease exemption in PREA would ensure that FDA can require the study of drugs for orphan diseases, where appropriate.

Removing the orphan exemption would also eliminate a concerning loophole. Currently, companies can receive orphan designation for the pediatric population affected by a disease even if it is not an orphan disease in adults. Some companies, however, have received this pediatric orphan designation and never actually conduct the pediatric studies—and FDA is unable to require these studies to be completed under PREA.

Allow PREA to apply to targeted therapies. Currently under PREA, FDA can only require a company to conduct pediatric studies for a new drug or a new use of a drug if the indication is the same in adults as in children. To increase children's access to modern therapeutics, Congress should allow FDA to use PREA to require

pediatric studies for a drug when it affects specific molecular targets or mechanisms that are shared between the adult and pediatric disease.

Improve accountability. PREA studies are typically deferred until after the drug is approved for adults and companies can receive a deferral extension to allow even more time. However, the deadlines to complete studies are too often missed. Congress should give FDA additional enforcement tools to ensure that critical pediatric studies are completed in a timely manner.

Plan pediatric studies earlier. Under current law, sponsors are required to submit an initial PREA pediatric study plan no later than the end of phase II in the drug development process for adults. FDA cannot require planning for pediatric studies at the end of phase I, even though companies are subject to that requirement in Europe. Congress should ensure that planning for pediatric studies under BPCA and PREA occurs in a timely manner, especially for the small subset of drugs to treat serious and life-threatening conditions.

Increase transparency. Details on the studies being conducted under BPCA are not made public until after all the studies are completed, which can be about 5-10 years after the FDA requested the studies. FDA cannot share the specifics of the BPCA study request with their counterparts in other countries, which hinders their ability to inform pediatric studies in those countries. The public is also not informed when companies decline BPCA study requests. Congress should provide greater transparency to improve collaboration and coordination between industry, researchers, and patients.

Reauthorize and make permanent the BPCA NIH Program. The BPCA NIH program funds the study of older off-patent drugs, which are some of the most commonly used drugs in children. Congress should make this program permanent just like BPCA and PREA and enable NIH to grow it over time.

Promote studies in neonates. Congress should build on the progress made at FDA by making permanent its neonatology expertise and issuing guidance regarding the development of studies in neonates.

Supporting Organizations

Academic Pediatric Association	Children's Hospital Association
AIDS Alliance for Women, Infants, Children, Youth & Families	Elizabeth Glaser Pediatric AIDS Foundation
American Academy of Pediatrics	March of Dimes
American Association of Child & Adolescent Psychiatry	National Association of Pediatric Nurse Practitioners
American Pediatric Society	North American Society for Pediatric Gastroenterology, Hepatology and Nutrition.
American Society of Clinical Oncology	Pediatric Infectious Diseases Society
American Society of Pediatric Hematology/Oncology	Pediatric Pharmacy Advocacy Group
American Thoracic Society	Pediatric Policy Council
Association of Medical School Pediatric Department Chairs	Society for Adolescent Health and Medicine
Association of Pediatric Hematology/Oncology Nurses	St. Baldrick's Foundation
Child Neurology Society	Society for Pediatric Research
Children's Cause for Cancer Advocacy	