

BEST PHARMACEUTICALS FOR CHILDREN ACT AND THE PEDIATRIC RESEARCH EQUITY ACT

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BACKGROUND

Children are not just small adults. Their bodies react differently to medications and their size can cause children to experience side-effects not seen in adults. Without clinical data, children may be given the wrong dosage or may receive treatment that is ineffective for their age and size. The results can be dire: toxicity, drug resistance, longer illnesses, needless pain and suffering, and higher costs to the health care system.

Congress first recognized the need to ensure that drugs were being studied in children in 1997 when it passed the Better Pharmaceuticals for Children Act (BPCA), a bill to incentivize the study of off-label uses in pediatric populations by offering companies an additional six months of patent life on their product. In 2003, Congress passed the Pediatric Research Equity Act (PREA) to study on-label indications in children, when safe and appropriate.

In the past five years alone, at least 130 products (80 under PREA and 50 under BPCA) have been studied for use in children. While it is clear that these programs are working to expand the number of treatment options for children, a lack of parity exists between the number of drugs studied for adults and the number studied for children.

IMPROVEMENTS TO PEDIATRIC DRUG BILLS IN 2012

The bipartisan reauthorization of BPCA and PREA makes significant improvements to the laws to increase the number of appropriately studied medications available for children. The legislation:

- Provides FDA the necessary enforcement tools to ensure that companies complete their required pediatric studies under PREA on time. If a company fails to complete the required studies and has not received an extension, FDA will issue a public notification of the company's failure to meet their obligations under PREA.
- Increases transparency on the status of pediatric clinical trials required under PREA. The new "Deferral Extension Pathway" established in the legislation requires the FDA to report publicly on the status of these studies and whether drug companies have received an extension.
- Ensures the timely submission of a company's "Pediatric Study Plan", a blueprint for how a company plans to study their drug in children. The company must submit this plan earlier in the drug approval process to give FDA and the company enough time to evaluate the proposal and ensure it is well thought-out.
- Ensures that neonatologists are involved in the process of reviewing and planning pediatric clinical trials. Newborn children are among the most difficult group to study because of their size, low blood supply, and host of competing health problems.