

PREA and BPCA: Spurring Pediatric Drug Development

Pediatric Drug Development: Historic Challenges

The Food and Drug Administration Modernization Act was enacted in 1997 and marked the first legislation that incentivized pediatric drug development. Prior to this legislation, there were few incentives to study U.S. Food and Drug Administration (FDA)-approved medicines for use in children. As a result, information on dosing, safety and efficacy in pediatric patients was inadequate or unavailable. The lack of pediatric drug research stemmed from the widespread concerns about exposing children to unproven medicines as well as the high costs and unique scientific, ethical and practical challenges of pediatric clinical trials.

It is well established that medicines may work differently in children and adults. The need for pediatric-specific information in drug labeling prompted action by policy makers.

The Solution: A Complimentary Approach to Pediatric Drug Development

The Best Pharmaceuticals for Children Act (BPCA) and Pediatric Research Equity Act (PREA), permanently reauthorized in 2012, work together to foster pediatric drug development. This balanced approach not only generates important safety and efficacy information on the use of medicines in children, but also enables biopharmaceutical companies to continue to make significant investments in pediatric drug research.

- **BPCA:** complements PREA and provides incentives (six months of added marketing exclusivity) to encourage manufacturers to conduct pediatric studies of medicines with the potential for use in children.
- **PREA:** authorizes the FDA to require pediatric research for indications approved or seeking approval in adults and product formulations appropriate for children.

Sources:

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Spurring Research for Pediatric Patients

Both PREA and BPCA are widely regarded as a success for patients, driving significant increases in pediatric research, product approvals and approved labeling for pediatric populations. Before BPCA and PREA became law, more than 80% of the medicines approved for adult use were being used in children, even though the safety and effectiveness had not yet been established in children. By 2009, that number had been reduced to about 50%.ⁱ By 2016 there had been more studies conducted in children in the last five years than in the previous 30 years combined.ⁱⁱ Continued commitment from the biopharmaceutical industry has resulted in great strides against pediatric diseases, including HIV/AIDS, asthma, rare diseases and many forms of pediatric cancer (particularly blood cancers).

By The Numbers



Since 1998, there have been over **960 labeling changes** reflecting pediatric information.ⁱⁱⁱ



Since the first temporary reauthorization of BPCA and PREA in 2007, there have been **more than 680 pediatric studies completed** under BPCA and PREA.^{iv}



Over 275 drugs have been granted pediatric exclusivity under BPCA.^v



There are currently **more than 2,500 industry sponsored pediatric clinical trials underway worldwide**, involving **more than 1.4 million pediatric patients** across a variety of therapeutic areas, including diseases where there is significant unmet need, such as infectious diseases, neurological conditions, genetic disorders, and several forms of cancer.^{vi}

Programs to Support Pediatric Drug Development

