

# PREA AND BPCA: SPURRING PEDIATRIC DRUG DEVELOPMENT

## HISTORIC CHALLENGE:

Prior to 1997, which is when the first pediatric drug development incentive legislation was enacted as part of the Food and Drug Administration Modernization Act (FDAMA), there were concerns about using FDA-approved medications in this population as children could be exposed to medicines that were unproven in children, resulting in inadequate or unavailable information on dosing, safety, efficacy and side effects. Additionally, the high costs and unique scientific, ethical and practical challenges of pediatric clinical trials discouraged clinical testing in children.

Medicines may work differently in children and adults. Despite the known importance of studying medicines for children, the need for pediatric specific information in drug labeling prompted action by policymakers.

## THE SOLUTION: A COMPLEMENTARY APPROACH TO 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, creating a balanced approach that generates important safety and efficacy information on use of medicines in children and enables biopharmaceutical companies to continue to make significant investments in pediatric drug research.

- **BPCA (2002):** complements PREA and provides incentives (6 months of added marketing exclusivity) to encourage manufacturers to conduct pediatric studies of medicines with the potential for use in children.
- **PREA (2003):** “authorizes FDA to require pediatric research for indications approved or seeking approval in adults and produce formulations appropriate for children.

## PREA AND BPCA: SPURRING RESEARCH FOR PATIENTS

PREA and BPCA are widely regarded as a success for patients, driving significant increases in pediatric research, product approvals, and approved labeling for the pediatric population. Before BPCA and PREA became law, more than 80% of the drugs approved for adult use were being used in children, even though the safety and effectiveness had not been established in children. By 2012, that number had been reduced to about 50%.<sup>1</sup> 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 750 labeling changes** reflecting pediatric information.<sup>2</sup>



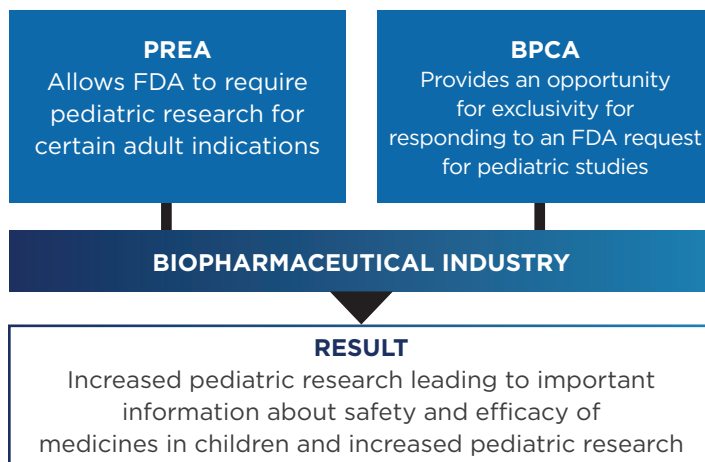
Since the reauthorization of BPCA and PREA in 2007, there have been **more than 680 pediatric studies have been completed** under BPCA and PREA.<sup>3,4</sup>



**Over 250 drugs** have been granted pediatric exclusivity under BPCA.<sup>5</sup>



There are currently **more than 2100 industry sponsored pediatric clinical trials are underway**, involving more than 1.2 million pediatric patients across a variety of therapeutic areas, including diseases where there is significant unmet need, such as infectious diseases, neurologic conditions, genetic disorders, and several forms of cancer.<sup>6</sup>



1 Karesh A; Food and Drug Administration (FDA). Pediatric drug development: regulatory expectations basic. <https://www.fda.gov/media/91673/download>. Published Fall 2015. Accessed May 2019.

2 Food and Drug Administration (FDA). New Pediatric Labeling Information Database. <https://www.accessdata.fda.gov/scripts/sda/sdnavigation.cfm?filter=&sortColumn=1a&sd=labelingdatabase&page=1>. Updated June 11, 2019. Accessed May 9, 2019.

3 Food and Drug Administration (FDA). Pediatric Studies Characteristics. <https://www.accessdata.fda.gov/scripts/SDA/sdNavigation.fdaaadescriptorstablewebdatabase&displayAll=false&page=8>. Updated Jan 9, 2012. Accessed May 9, 2019.

4. Food and Drug Administration (FDA). Number of pediatric studies completed under both PREA and BPCA. <https://www.accessdata.fda.gov/scripts/fdatrack/view/track.cfm?program=cder&status=public&id=CDER-RRDS-Number-of-pediatric-studies-completed&fy=All>. Updated December 21, 2017. Accessed May 9, 2019.

5 FDA, <https://www.fda.gov/drugs/development-resources/pediatric-sivity-5> granted. Accessed 12 June 2019.

6 Clinicaltrials.gov with the search terms “recruiting, not yet recruiting, active, not recruiting” - interventional studies - child - industry.” Accessed June 7, 2019.