



Improvements Made in the 2007 Reauthorization of The Best Pharmaceuticals for Children Act (BPCA) and the The Pediatric Research Equity Act (PREA)

EXTENDS BPCA AND PREA

- Extends BPCA incentive and PREA authority until October 1, 2012.

INCREASES AUTHORITY TO REFER AND REQUIRE STUDIES UNDER PREA

- Enhances the criteria for applying PREA to already marketed drugs. New language allows the Food and Drug Administration (FDA) to use a “benefit” standard as opposed to a “risk” standard to require studies.
- Eliminates burdensome “exhaustion” provision in favor of an expedited 30-day review of private funding before referral of declined written requests to PREA.
- If a study is declined by a drug company, requires FDA to determine whether the drug should be studied under PREA. If applicable, FDA will report why it did not use PREA.

INCREASES AUTHORITY AND EFFECTIVENESS OF BPCA

- Allows FDA to issue one study request for more than one use of a drug and to capture both on- and off-label uses.
- Allows FDA to ask for preclinical studies as part of a written request.
- Lengthens the period of time FDA has to review submitted studies from 90 to 180 days.
- Requires that pediatric studies under BPCA be submitted and exclusivity awarded nine months before expiration of patent to provide more market predictability.

INCREASES TRANSPARENCY OF PROGRAMS AND DISSEMINATION OF PEDIATRIC INFORMATION

- Requires FDA to make BPCA written requests public after the drug has been granted exclusivity.
- Requires FDA to track the number and type of studies completed, as well as labeling changes and other data resulting from BPCA and PREA.
- Requires Secretary to make publicly available the actual medical, statistical, and clinical pharmacology reviews for BPCA and PREA, not summaries.
- Increases knowledge of barriers to development of pediatric formulations by requiring that drug companies submit to FDA the reasons why a particular formulation cannot be developed.
- Requires prominent public disclosure when a manufacturer creates a pediatric formulation and fails to market it.
- Requires an annual, publicly available review from all drug companies who have received deferrals under PREA.
- Requires drug companies under BPCA and PREA to provide physicians and other health care providers with new pediatric labeling information annually.

INCREASES THE SPEED AND ACCURACY OF DRUG LABELING

- Gives FDA explicit authority to indicate on a label when a product has been studied in children.
- Removes the current dispute resolution provision requiring labeling to be the only remaining open issue before referral for resolution.
- Applies the dispute resolution process to all drugs under BPCA and PREA, not just those granted exclusivity.
- Sets a time certain for referral to Pediatric Advisory Committee if sponsor does not make requested label change.

STRENGTHENS THE REVIEW AND OVERSIGHT OF BPCA AND PREA

- New language establishes an internal FDA review committee for BPCA and PREA.
- Requires new internal committee to review BPCA written requests prior to issuance.
- Provides committee authority to review studies submitted in response to a BPCA written request, as needed.
- Requires internal committee to review PREA study plans and assessments prior to drug approval. Also requires review of deferrals and waivers.
- Asks the Institute of Medicine to review past study requests issued by FDA under both BPCA and PREA, make recommendations to FDA for future requests, and make recommendations for incentives to encourage the study of biologics in children.
- Requires the Government Accountability Office to produce a report on the results of BPCA and PREA with recommendations for improving the programs.
- Extends Pediatric Advisory Committee through October 1, 2012.

STRENGTHENS ADVERSE EVENT SURVEILLANCE

- Requires manufacturers to submit all post-market adverse events as part of the request for exclusivity under BPCA.
- Continues the requirement that all adverse events be reviewed by the Pediatric Advisory Committee for one year following the awarding of exclusivity. Provides for additional reporting after year one, as needed.
- Requires drugs studied under PREA to report adverse events and provides for one-year review by the Pediatric Advisory Committee.

ENHANCES ROLE OF NIH IN MEETING NEEDS IN PEDIATRIC THERAPEUTICS AND PHARMACOLOGY

- Extends and expands authority for NIH to examine needs in pediatric therapeutics, including drugs. Reauthorizes \$200 million for a pediatric research fund.
- Streamlines process by which NIH studies drugs for labeling.
- Includes pediatric pharmacologists in existing NIH career development and loan repayment programs.