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FDA Update, News Articles, Pharmacology

Update on success of law that encourages studies of off-patent drugs in children

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In 2002, Congress passed the Best Pharmaceuticals for Children Act (BPCA), which includes provisions for the National Institutes of Health (NIH) to study and improve the labeling of off-patent therapeutics for use in children.

Since then, over 8,000 children have been enrolled in 40 clinical trials at over 200 pediatric sites, resulting in 11 label changes (see table).

Pediatric labeling changes for products studied under BPCA

Date	Drug/device	Labeling change
June 2009	propylthiouracil	Safety update: liver injury
September 2010	pralidoxime	Pediatric dosing for organophosphate poisoning
December 2013	sodium nitroprusside	Dosing, pharmacokinetics (PK) and safety for controlled reduction of blood pressure
December 2014	meropenem	Dosing for neonates and infants younger than 90 days with complicated intra-abdominal infections
May 2015	Mercy TAPE	Body weight estimation for patients 2 months-16 years
April 2016	lisinopril	Hypertension in renal transplants
June 2016	lorazepam	Safety data added for status epilepticus, limitations in efficacy data
February 2018	ampicillin	Dosing for neonatal meningitis and septicemia based on gestation and postnatal age; safety information updated
October 2018	lithium	Indication for bipolar I disorder in patients 7 years-17 years; dosing, PK, safety, medication guide updates
January 2019	acyclovir	Dosing and PK information for neonatal herpes simplex infection
October 2019	Mercy BabyTAPE	Body weight estimation for patients younger than 90 days

The trials were sponsored by the Eunice Kennedy Shriver National Institute of Child Health and Human Development (NICHD), which established the BPCA Clinical Program to respond to the congressional mandate. The program's objectives are to

- identify and prioritize off-patent drugs to be studied (see resources);
- sponsor clinical trials;
- submit data to the Food and Drug Administration (FDA) in response to a Written Request (if issued); and
- make the study data publicly available on the NIH Data and Specimen Hub.

The FDA has 180 days to evaluate the study data submitted by the NIH and negotiate the appropriate labeling

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changes with drug companies. Under BPCA, the FDA can require drug companies to update their labeling based on the NICHD-sponsored trials.

To ensure the program's success, the NICHD also supports an infrastructure that includes a logistics support contract, a training program in pediatric pharmacology, research centers in developmental pharmacology, the BPCA Data Coordinating Center and the Pediatric Trials Network.

Resources

- Best Pharmaceuticals for Children Act (BPCA) Priority List of Needs in Pediatric Therapeutics for 2018-'19
- BPCA accomplishments
- National Institutes of Health Data and Specimen Hub
- Pediatric Trials Network
- Additional FDA Update columns