



Reauthorization of Better Pharmaceuticals for Children Act (BPCA) And Pediatric Research Equity Act (PREA)

Background

It is well known that children's bodies react to medications much differently than adults and that children can experience unique side effects not seen in adults. Despite this fact, up to 75% of drugs used by children have not been tested. In some tragic cases, children have died or suffered serious injury as a result of taking medicines deemed safe for adults. More often, children are overdosed, underdosed or receive treatments that are 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 healthcare system.

Both the Better Pharmaceuticals for Children Act (BPCA) and the Pediatric Research Equity Act (PREA) will expire on October 1, 2007 unless reauthorized by Congress. NORD strongly supports the renewal of these critical pieces of legislation, with the following improvements:

- Improve transparency and accountability by requiring that all study protocols and results be made public and by requiring the FDA to track the impact of both BPCA and PREA on studies generated and labeling changes made.
- Improve the accuracy and speed of labeling changes by providing authority to FDA to require labeling changes instead of negotiating with manufacturers for agreements..
- Improve postmarket surveillance of pediatric products by applying the adverse events reporting requirements to all products studied under BPCA and PREA.
- Give FDA permanent authority to require pediatric studies on new drugs and biologics.
- Enhance the role of the National Institutes of Health to conduct pediatric studies when important gaps exist in treatments of children's diseases (e.g., studies of drugs after their patents have expired, etc.).
- Ensure that BPCA continues to yield more and better drug studies in children.

Examples of Labeling Changes for Orphan Drugs Under BPCA and PREA

Orphan Drug	Orphan Indication
Sotalol/Betapace	Treatment of life-threatening ventricular tachyarrhythmias
Busulfan/Busulfex	As preparation therapy in the treatment of malignancies with bone marrow transplantation
Temozolomide/Temodar	Treatment of malignant glioma
Fludarabine/Fludara	Treatment of chronic lymphocytic leukemia
Anagrelide/Agrylin	Treatment of essential thrombocythemia
Meloxicam/Mobic	Treatment of juvenile rheumatoid arthritis
Atovaquone/Proguanil/Malarone	Prevention of Pneumocystis carinii pneumonia (PCP) in high-risk, HIV-infected patients defined by a history of one or more episodes of PCP and/or a peripheral CD4+ (T4 helper/inducer) lymphocyte count less than or equal to 200/mm ³
Octreotide/Sandostatin	Treatment of severe diarrhea and flushing associated with malignant carcinoid tumors
Imatinib mesylate/Gleevec	Treatment of chronic myelogenous leukemia
Ribavirin/IntronA/Rebetron	Treatment of chronic hepatitis C in pediatric patients.
Clofarabine/Clorar	Treatment of acute lymphoblastic leukemia

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