PEDIATRIC DRUG RESEARCH

The Study and Labeling of Drugs for Pediatric Use under the Best Pharmaceuticals for Children Act

What GAO Found

Drug sponsors have initiated pediatric drug studies for most of the on-patent drugs for which FDA has requested such studies under BPCA, but no drugs were studied when sponsors declined these requests. Sponsors agreed to 173 of the 214 written requests for pediatric studies of on-patent drugs. In cases where drug sponsors decline to study the drugs, BPCA provides for FDA to refer the study of these drugs to the Foundation for the National Institutes of Health (FNIH), a nonprofit corporation. FNIH had not funded studies for any of the nine drugs that FDA referred as of December 2005.

Few off-patent drugs identified by the National Institutes of Health (NIH) that need to be studied for pediatric use have been studied. BPCA provides for NIH to fund studies when drug sponsors decline written requests for off-patent drugs. While 40 such off-patent drugs were identified by 2005, FDA had issued written requests for 16. One written request was accepted by the drug sponsor. Of the remaining 15, NIH funded studies for 7 through December 2005.

Most drugs granted pediatric exclusivity under BPCA (about 87 percent) had labeling changes—often because the pediatric drug studies found that children may have been exposed to ineffective drugs, ineffective dosing, overdosing, or previously unknown side effects. However, the process for approving labeling changes was often lengthy. For 18 drugs that required labeling changes (about 40 percent), it took from 238 to 1,055 days for information to be reviewed and labeling changes to be approved.


![Diagram](https://via.placeholder.com/150)

Source: GAO.