May 22, 2007

PEDIATRIC DRUG RESEARCH

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

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Highlights

Highlights of GAO-07-898T, a testimony before the Subcommittee on Health, Committee on Energy and Commerce, House of Representatives

Why GAO Did This Study

About two-thirds of drugs that are prescribed for children have not been studied and labeled for pediatric use, placing children at risk of being exposed to ineffective treatment or incorrect dosing. The Best Pharmaceuticals for Children Act (BPCA), enacted in 2002, encourages the manufacturers, or sponsors, of drugs that still have marketing exclusivity—that is, are on-patent—to conduct pediatric drug studies, as requested by the Food and Drug Administration (FDA). If they do so, FDA may extend for 6 months the period during which no equivalent generic drugs can be marketed. This is referred to as pediatric exclusivity. BPCA also provides for the study of off-patent drugs.

GAO was asked to testify on the study and labeling of drugs for pediatric use under BPCA. This testimony is based on *Pediatric Drug Research: Studies Conducted under Best Pharmaceuticals for Children Act*, GAO-07-557 (Mar. 22, 2007). GAO assessed (1) the extent to which pediatric drug studies were being conducted under BPCA for on-patent drugs, (2) the extent to which pediatric drug studies were being conducted under BPCA for off-patent drugs, and (3) the impact of BPCA on the labeling of drugs for pediatric use and the process by which the labeling was changed. GAO examined data about the drugs for which FDA requested studies under BPCA from 2002 through 2005 and interviewed relevant federal officials.

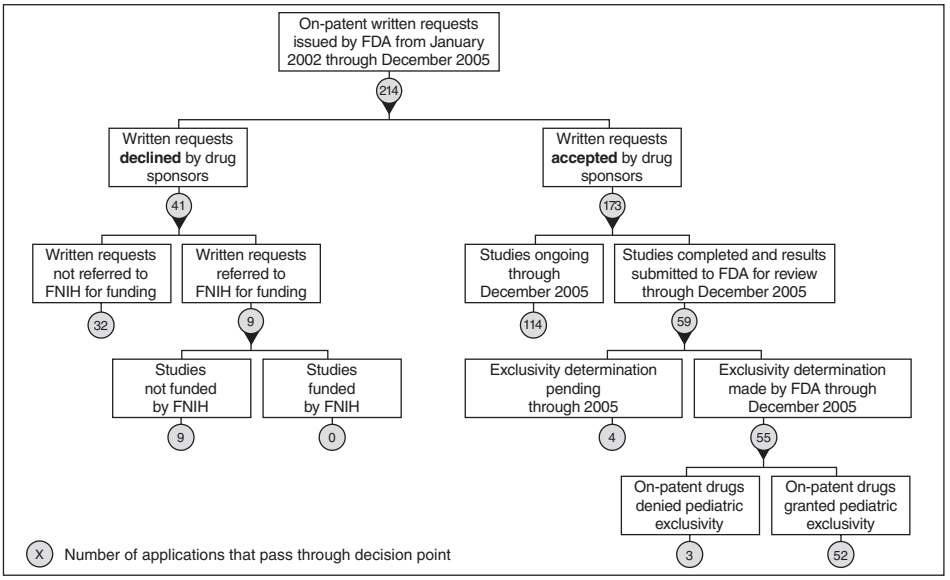
www.gao.gov/cgi-bin/getrpt?GAO-07-898T.

To view the full product, including the scope and methodology, click on the link above. For more information, contact Marcia Crosse at (202) 512-7119 or crossem@gao.gov.

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.

Written Requests Issued under BPCA for the Study of On-Patent Drugs (2002-2005)



Source: GAO.

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.