



Highlights of [GAO-07-557](#), a report to congressional committees

PEDIATRIC DRUG RESEARCH

Studies Conducted under Best Pharmaceuticals for Children Act

Why GAO Did This Study

About two-thirds of drugs that are prescribed for children have not been studied and labeled for pediatric use, which places 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 required that GAO assess the effect of BPCA on pediatric drug studies and labeling. As discussed with the committees of jurisdiction, GAO (1) assessed the extent to which pediatric drug studies were being conducted under BPCA for on-patent drugs, including when drug sponsors declined to conduct the studies; (2) evaluated the impact of BPCA on labeling drugs for pediatric use and the process by which the labeling was changed; and (3) illustrated the range of diseases treated by the drugs studied under BPCA. GAO examined data about the drugs for which FDA requested studies under BPCA from 2002 through 2005. GAO also interviewed officials from relevant federal agencies, pharmaceutical industry representatives, and health advocates.

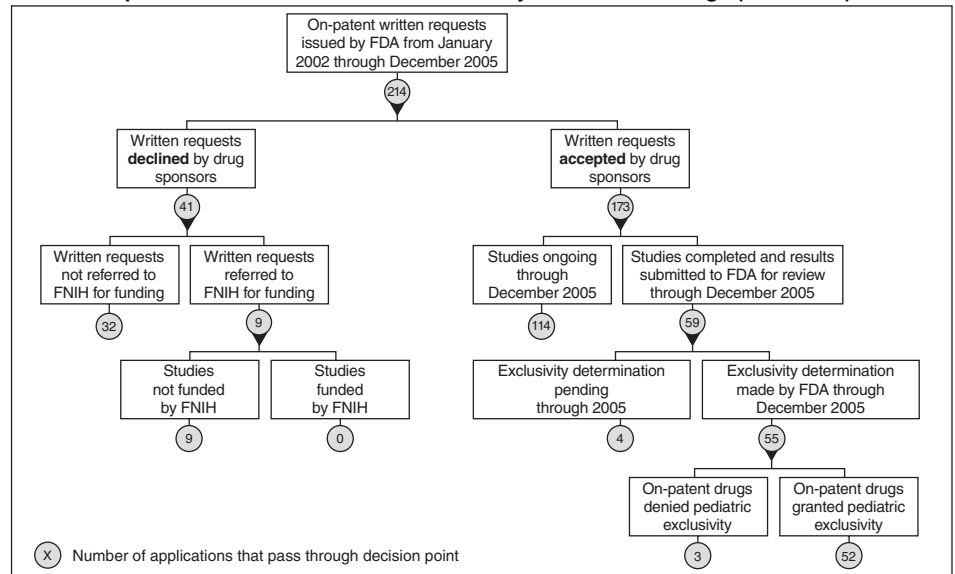
www.gao.gov/cgi-bin/getrpt?GAO-07-557.

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 studies, but no drugs were being studied when drug 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.

Most drugs (about 87 percent) granted pediatric exclusivity under BPCA 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. It took from 238 to 1,055 days for information to be reviewed and labeling changes to be approved for 18 drugs (about 40 percent), and 7 of those took more than 1 year. Drugs were studied under BPCA for the treatment of a wide range of diseases, including those that are common, serious, or life threatening to children. These drugs represented more than 17 broad categories of disease, such as cancer.

The Department of Health and Human Services stated that the report provides a significant amount of data and analysis and generally explains the BPCA process, but expressed concern that it did not sufficiently acknowledge the success of BPCA or clearly describe some elements of FDA’s process. GAO incorporated comments as appropriate.