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PEDIATRIC RESEARCH

Products Studied under Two Related Laws, but Improved Tracking Needed by FDA

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Highlights

Highlights of GAO-11-457, a report to congressional committees

Why GAO Did This Study

In 2007, Congress reauthorized two laws, the Pediatric Research Equity Act (PREA) and the Best Pharmaceuticals for Children Act (BPCA). PREA requires that sponsors conduct pediatric studies for certain products unless the Department of Health and Human Services' (HHS) Food and Drug Administration (FDA) grants a waiver or deferral. Sponsors submit studies to FDA in applications for review. BPCA is voluntary for sponsors. The FDA Amendments Act of 2007 required that GAO describe the effect of these laws since the 2007 reauthorization. GAO (1) examined how many and what types of products have been studied; (2) described the number and type of labeling changes and FDA's review periods; and (3) described challenges identified by stakeholders to conducting studies. GAO examined data on the studies from the 2007 reauthorization through June 2010, reviewed statutory requirements, and interviewed stakeholders and agency officials.

What GAO Recommends

GAO recommends that the Commissioner of FDA track applications during its review process and maintain aggregate data on applications subject to PREA. HHS agreed that better tracking of information is needed but disagreed with GAO's finding that it does not track applications. While FDA is able to identify the status of individual applications during its review, it has not maintained data that would allow it to better manage its review process.

View GAO-11-457 or key components.
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What GAO Found

At least 130 products—80 products under PREA and 50 under BPCA—have been studied for use in children since the 2007 reauthorization. However, FDA cannot be certain how many additional products may have been studied because FDA does not track and aggregate data about applications submitted under PREA that would allow it to manage the review process. FDA was unable to provide information about some applications that had been submitted to the agency that were subject to PREA. Recent improvements to FDA's data system might assist the agency in tracking future applications. Under PREA, FDA has granted most of the study waivers and deferrals requested by sponsors since the 2007 reauthorization. Under BPCA, FDA granted pediatric exclusivity—an additional 6 months of market exclusivity, which generally delays marketing of generic forms of the product—to the sponsors of 44 of the 50 drugs in exchange for conducting pediatric studies. Because BPCA is voluntary, sponsors may decline FDA's request for pediatric studies. Although BPCA includes provisions to encourage the study of drugs when sponsors have declined FDA's request, few drugs have been studied under these provisions.

Since the 2007 reauthorization, all of the 130 products with pediatric studies completed and applications reviewed under PREA and BPCA had labeling changes that included important pediatric information. The most commonly implemented labeling change expanded the pediatric age groups for which a product was indicated. The next most common type of labeling change indicated that safety and effectiveness had not been established in pediatric populations and provided a description of the study conducted. Additional labeling changes were recommended for products as a result of FDA's monitoring of adverse events associated with products after they had been approved for marketing. FDA officials said they need to complete their review of the application, including all studies, before they can reach agreement with the sponsor on labeling changes.

Stakeholders, including sponsors, pediatricians, and health advocacy organizations, described challenges faced by sponsors that could limit the success of PREA and BPCA. Those challenges included confusion about how to comply with PREA and BPCA due to a lack of guidance from FDA for changes to the laws from the 2007 reauthorization of PREA or BPCA. FDA officials explained that they mitigate this lack of guidance by discussing questions or concerns that sponsors have regarding their pediatric studies with sponsors throughout the process. An additional challenge sponsors described was a lack of economic incentives to study products with no remaining market exclusivity.