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PEDIATRIC DRUG RESEARCH

Food and Drug Administration Should More Efficiently Monitor Inclusion of Minority Children

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Highlights

Highlights of GAO-03-950, a report to the Committee on Health, Education, Labor and Pensions, U.S. Senate, and the Committee on Energy and Commerce, House of Representatives

Why GAO Did This Study

Drug effectiveness and adverse events can vary between children and adults and among racial and ethnic groups. The Food and Drug Administration (FDA) is authorized under the pediatric exclusivity provision to grant drug sponsors 6 months of additional exclusive marketing rights for conducting clinical drug studies in children. The Best Pharmaceuticals for Children Act of 2002 (BPCA) expanded this provision to require FDA to take into account the adequacy of minority representation in pediatric exclusivity studies. BPCA also directed GAO to evaluate the representation of minorities in such studies. GAO examined the extent to which minority children are represented, whether drugs that treat diseases disproportionately affecting minority groups are studied under the provision, and FDA's monitoring of the representation of minority children in the studies. GAO reviewed related FDA documents, FDA requests for pediatric studies and final study results, and interviewed FDA officials and other experts.

What GAO Recommends

GAO recommends that FDA specify that drug sponsors use the standard racial and ethnic group definitions described in FDA's January 2003 draft guidance to identify study participants. FDA concurred with the recommendation.

www.gao.gov/cgi-bin/getrpt?GAO-03-950.

To view the full product, including the scope and methodology, click on the link above. For more information, contact Janet Heinrich at (202) 512-7119.

What GAO Found

Compared with the proportions of children from racial and ethnic minority groups in the U.S. population, smaller proportions of children from minority groups were included in the pediatric clinical drug studies requested by FDA before the enactment of BPCA that GAO reviewed. However, FDA required, and drug sponsors included, larger proportions of African American children in clinical studies for hypertension drugs because there is evidence that hypertension is more prevalent and more severe among African Americans. Furthermore, FDA has requested that forthcoming studies for certain drugs include larger proportions of minority children.

Studies of some drugs that may be used to treat diseases or conditions that disproportionately affect minorities have been completed and additional such studies have been requested by FDA. From January 4, 2002, through March 6, 2003, FDA granted additional exclusive marketing rights to four drugs that may be used to treat conditions such as hypertension, type II diabetes, and sickle cell anemia—conditions or diseases that disproportionately affect minority children. During that time, FDA also issued written requests for studies of six drugs for these conditions.

FDA does not have a system in place to serve as a single source of data to allow the agency to efficiently determine the extent of participation of children by racial and ethnic group under the pediatric exclusivity provision. GAO found that some study reports submitted to FDA from drug sponsors did not specify the race and ethnicity of all study participants. Across all the studies for drugs granted additional exclusive marketing rights that GAO reviewed, 86 percent of study participants were identifiable by race or ethnicity, but the race or ethnicity of 14 percent of study participants was unknown. In January 2003, FDA issued draft guidance recommending that drug sponsors use standard definitions for race and ethnicity in drug studies. However, drug sponsors are not required to use these definitions. FDA has also begun to develop an agencywide system to monitor demographic characteristics of study participants, such as age, sex, and race.

FDA agreed with the GAO recommendation to specify the categories that sponsors should use to report minority representation as well as GAO's findings regarding the efficiency of its data collection systems. FDA expressed concerns about the GAO comparison of the proportion of minorities in drug studies to their proportion in the U.S. population. However, FDA had previously used the methodology GAO employed in its analyses of adult study participants.