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PRESCRIPTION DRUGS

FDA Has Met Most Performance Goals for Reviewing Applications

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Highlights

Highlights of GAO-12-500, a report to congressional requesters

Why GAO Did This Study

The Food and Drug Administration (FDA) within the Department of Health and Human Services (HHS) is responsible for overseeing the safety and efficacy of drugs and biologics sold in the United States. New drugs and biologics must be reviewed by FDA before they can be marketed, and the Prescription Drug User Fee Act (PDUFA) authorizes FDA to collect user fees from the pharmaceutical industry to support its review of prescription drug applications, including new drug applications (NDA), biologic license applications (BLA), and efficacy supplements that propose changes to the way approved drugs and biologics are marketed or used. Under each authorization of PDUFA since 1992, FDA committed to performance goals for its drug and biologic reviews.

In preparation for the next PDUFA reauthorization, GAO was asked to examine FDA's drug and biologic review processes. In this report, we (1) examine trends in FDA's NDA and BLA review performance for fiscal years (FY) 2000 through 2010, (2) examine trends in FDA's efficacy supplement review performance for FYs 2000 through 2010, and (3) describe issues stakeholders have raised about the drug and biologic review processes and steps FDA is taking that may address these issues. To do this work, GAO examined FDA drug and biologic review data, reviewed FDA user fee data, interviewed FDA officials, and interviewed two industry groups and five consumer advocacy groups. All of the stakeholder groups participated in at least half of the meetings held by FDA to discuss the reauthorization of the prescription drug user fee program.

View GAO-12-500. For more information, contact Marcia Crosse at (202) 512-7114 or crossem@gao.gov.

What GAO Found

FDA met most performance goals for priority and standard NDAs and BLAs received from FY 2000 through FY 2010. FDA meets its performance goals by completing its review and issuing an action letter—such as an approval or a response detailing deficiencies that are preventing the application from being approved—for a specified percentage of applications within a designated period of time. FDA designates NDAs and BLAs as either priority—if the product would provide significant therapeutic benefits when compared to available drugs—or standard. FDA met the performance goals for both priority and standard NDAs and BLAs for 10 of the 11 fiscal years GAO examined; FDA did not meet either of the goals for FY 2008. Although FDA had not yet issued an action letter for all of the applications it received in FY 2011 and results are therefore preliminary, FDA was meeting the goals for both priority and standard NDAs and BLAs on which it had taken action. Meanwhile, FDA review time for NDAs and BLAs—the time elapsed between FDA's receipt of an application and issuance of an action letter—increased slightly from FY 2000 through FY 2010. In addition, the percentage of NDAs and BLAs receiving an approval letter at the end of the first review cycle generally increased, although that percentage has decreased for priority NDAs and BLAs since FY 2007.

FDA met most of its performance goals for efficacy supplements from FY 2000 through FY 2010. Specifically, FDA met the performance goals for both priority and standard efficacy supplements for 10 of the 11 fiscal years GAO examined. FDA review time generally increased during the analysis period for both priority and standard efficacy supplements. The percentage of priority efficacy supplements receiving an approval letter at the end of the first review cycle fluctuated from FY 2000 through FY 2010, ranging between 47 percent and 80 percent during this time. The results for standard efficacy supplements showed a steadier increase with the percentage of first-cycle approval letters rising from 43 percent for FY 2000 applications to 69 percent for FY 2010 applications.

The industry groups and consumer advocacy groups we interviewed noted a number of perceived issues related to FDA's review of drug and biologic applications. The most commonly mentioned issues raised by industry and consumer advocacy stakeholder groups were actions or requirements that can increase review times (such as taking more than one cycle to approve applications) and insufficient communication between FDA and stakeholders throughout the review process. Industry stakeholders also noted a perceived lack of predictability and consistency in reviews. Consumer advocacy group stakeholders noted issues related to inadequate assurance of the safety and effectiveness of approved drugs. FDA is taking steps that may address many of these issues, including issuing new guidance, establishing new communication-related performance goals, training staff, and enhancing scientific decision making.

In commenting on a draft of this report, HHS generally agreed with GAO's findings and noted that they reflect what the agency reported for the same time period. HHS also called attention to activities FDA has undertaken to improve the prescription drug review process.