

# GAO Highlights

Highlights of [GAO-16-192](#), a report to the Ranking Member, Subcommittee on Labor, Health and Human Services, Education, and Related Agencies, Committee on Appropriations, House of Representatives

## Why GAO Did This Study

FDA oversees the safety and effectiveness of drugs sold on the U.S. market. When there is an unmet need for the treatment of a serious condition, FDA may use one or more of its expedited programs, such as fast track and breakthrough therapy designation, which are intended to bring drugs to market more quickly. FDA is also responsible for monitoring the safety of drugs and reporting on those efforts.

GAO was asked to provide information about FDA's expedited programs and its postmarket monitoring of expedited and nonexpedited drugs. This report examines (1) the number and types of requests for fast track or breakthrough therapy designation, (2) the number and types of FDA-approved drug applications that used an expedited program, and (3) the extent to which FDA's data on tracked safety issues and postmarket studies allowed the agency to meet its reporting and oversight responsibilities. GAO analyzed FDA data on requests for fast track or breakthrough therapy designation and approved drug applications that used an expedited program from October 1, 2006, to December 31, 2014 (the most recent available). GAO reviewed FDA information on tracked safety issues and postmarket studies, including FDA internal evaluations and guidance, and interviewed FDA officials.

## What GAO Recommends

FDA should develop plans to correct problems with its postmarket safety data and ensure that these data can be easily used for oversight. HHS agreed with GAO's recommendations and provided additional information on FDA's postmarket safety efforts.

View [GAO-16-192](#). For more information, contact Marcia Crosse at (202) 512-7114 or [crossem@gao.gov](mailto:crossem@gao.gov).

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## DRUG SAFETY

### FDA Expedites Many Applications, But Data for Postapproval Oversight Need Improvement

#### What GAO Found

From October 1, 2006, to December 31, 2014, the Food and Drug Administration's (FDA) Center for Drug Evaluation and Research (CDER) received about 1,000 requests for fast track designation and breakthrough therapy designation—two of the agency's four expedited programs to facilitate and expedite the development and review of new drugs. Drug sponsors are required to submit formal requests to use these two programs; for the other two expedited programs (accelerated approval and priority review) sponsors are not required to submit formal requests. Regardless of whether sponsors submit a request for an expedited program, they are required to submit a marketing application prior to offering a drug for sale in the United States; using an expedited program does not ensure FDA approval of the marketing application. Sponsors submitted more than 770 requests for fast track designation since fiscal year 2007, and FDA granted about two-thirds of these requests. Sponsors submitted more than 220 requests for breakthrough therapy designation since it was established in July 2012, and the agency denied more than half of these requests.

About a quarter of the drug applications CDER approved for the U.S. market from October 1, 2006, to December 31, 2014, used at least one expedited program, according to FDA data. Included among these applications were new drug applications, biologic license applications, and efficacy supplements, which allow for revisions to the original application, such as changes in the drug's indicated use. Although most of these applications used one program, some applications used two or more, including two oncology drug applications that used all four expedited programs (accelerated approval, breakthrough therapy designation, fast track designation, and priority review). The most common product area among these applications was oncology (19 percent).

FDA lacks reliable, readily accessible data on tracked safety issues and postmarket studies needed to meet certain postmarket safety reporting responsibilities and to conduct systematic oversight. Tracked safety issues are potential safety issues that FDA determines are significant and that it tracks using an internal database. Internal control standards for federal agencies specify that information should be recorded in a form and within a time frame that enables staff to carry out their responsibilities and that relevant, reliable, and timely information should be available for external reporting purposes. However, evaluations conducted by CDER of data in its database revealed problems with the completeness, timeliness, and accuracy of the data. These problems, as well as problems with the way data are recorded that impair their accessibility, have prevented FDA from publishing statutorily required reports on certain potential safety issues and postmarket studies in a timely manner, and have restricted the agency's ability to perform systematic oversight of postmarket drug safety. Although FDA has taken some steps to address the problems with its data, the agency lacks plans that comprehensively outline its efforts and establish related goals and time frames. Additionally, FDA does not have plans to use these data to inform its oversight of its expedited programs, such as determining if drugs that used an expedited program were subsequently associated with tracked safety issues at rates or of types that differed from drugs that used FDA's standard process.