November 2024

RARE DISEASE DRUGS

FDA Has Steps Underway to Strengthen Coordination of Activities Supporting Drug Development

GAO Highlights

Highlights of GAO-25-106774, a report to congressional committees

Why GAO Did This Study

Nearly one in 10 Americans have a rare disease, which is typically defined as any condition affecting fewer than 200,000 people in the United States. There are up to 10,000 rare diseases, however, only 5 percent of rare diseases have FDA-approved treatments.

The Consolidated Appropriations Act, 2023 includes a provision for GAO to examine FDA activities related to rare disease drug development and approval. This report, among other objectives, describes FDA's strategies to help ensure reviewers have the necessary expertise and use appropriate flexibilities in their reviews, describes FDA's programs to support rare disease drug development, and examines FDA's efforts to coordinate its rare disease efforts.

GAO reviewed relevant statutes, regulations, and FDA documentation. GAO also interviewed FDA officials, drug sponsors of approved rare disease drugs, and patient advocacy groups representing individuals with rare diseases.

View GAO-25-106774. For more information, contact John E. Dicken at (202) 512-7114 or dickenj@gao.gov.

What GAO Found

The Food and Drug Administration (FDA) is responsible for determining whether drugs are safe and effective. The agency also works with drug sponsors and others to support the development of drugs to treat rare diseases, recognizing the unique challenges involved, such as assessing drug effectiveness in small patient populations. Rare disease drugs must meet the same standards as other drugs to be marketed, but FDA reviewers may apply flexibility in determining the evidence needed to meet the standards. Within FDA, staff in two of the agency's centers—the Center for Drug Evaluation and Research (CDER) and the Center for Biologics Evaluation and Research (CBER)—review drug applications.

According to FDA officials, training and engagement with patient groups are among the important strategies to help ensure the agency's staff have the expertise needed to review rare disease drug applications. Agency officials also said that a multi-layer review process helps ensure consistency among reviewers in regulatory decision-making. FDA officials said they are committed to using available flexibilities where appropriate to ensure safe and effective rare disease drugs are approved for marketing.

GAO found that FDA has 18 rare disease-specific programs—most initiated since 2019—intended to help address complexities common to rare disease drug development. Each of these programs aimed to do at least one of the following: advance understanding of the diseases, expand stakeholder engagement, or support drug development efforts.

FDA Rare Disease-Specific Program Aims

Advancing the science underlying rare disease drug development	Expanding stakeholder education and outreach	Advising rare disease drug development programs
Strengthen understanding of rare diseases and science of drug development, such as through clinical trial design.	Strengthen engagement with patient advocacy groups and drug sponsors; better inform stakeholders of requirements for approval and the Food and Drug Administration's (FDA) role.	Provide drug sponsors opportunity to work more closely, more often, and earlier with FDA during drug development process.

Source: GAO analysis of FDA information (text); Iconic Prototype/stock.adobe.com (icons). | GAO-25-106774

FDA is the process of implementing a new Rare Disease Innovation Hub intended to enhance coordination between CDER and CBER and help expedite development and approval of safe and effective drugs for rare diseases. To do so, FDA will develop a cross-center strategic agenda with public input to help shape priorities and initiatives. According to agency officials, this new initiative will include the development of agencywide goals for the agency's rare disease activities. While it is still early, this initiative holds promise for guiding the agency's rare disease activities in a strategic and coordinated fashion. According to FDA, the new initiative will leverage each center's rare disease-focused activities and enhance existing cross-center collaborations. Before the agency announced its new initiative, the two centers focused on developing center-specific goals for their respective rare disease activities. FDA officials said both centers plan to align their goals with agencywide goals once they are defined.