

Why GAO Did This Study

When investigational drugs show promise for treating serious or life-threatening diseases, patients are often interested in obtaining access to them. Congress included a provision in the FDA Reauthorization Act of 2017 for GAO to review actions taken to facilitate access to these drugs.

This report describes (1) actions FDA and drug manufacturers have taken to broaden eligibility criteria for clinical trials, (2) actions FDA has taken to facilitate access to investigational drugs outside of clinical trials, and (3) information drug manufacturers have communicated to patients and physicians about access to investigational drugs outside of clinical trials.

GAO reviewed laws, regulations, FDA documents, and manufacturer policies and interviewed FDA officials and a non-generalizable selection of 10 manufacturers and 14 other stakeholders (including patient advocacy and physician organizations). The manufacturers were developing drugs to treat serious or life-threatening diseases, and were selected for variation in company size. GAO also reviewed information that a non-generalizable selection of 29 manufacturers communicated through their websites about access to investigational drugs outside of clinical trials. GAO selected manufacturers for variation in the type of serious diseases their investigational drugs were intended to treat, company size, and other factors.

HHS provided technical comments on a draft of this report, which GAO incorporated as appropriate.

View [GAO-19-630](#). For more information, contact John E. Dicken at (202) 512-7114 or dickenj@gao.gov.

INVESTIGATIONAL DRUGS

FDA and Drug Manufacturers Have Ongoing Efforts to Facilitate Access for Some Patients

What GAO Found

Individuals may access investigational drugs—those not yet approved for marketing in the United States by the Food and Drug Administration (FDA)—by participating in clinical trials conducted by drug manufacturers to test drug effectiveness and safety. FDA has ongoing efforts to help manufacturers identify the circumstances under which they could broaden clinical trial eligibility criteria to include patients who are commonly excluded, such as pediatric patients and patients with impaired liver and kidney function, without compromising study results.

- FDA issued guidance in March 2019 with recommendations on ways manufacturers could broaden eligibility criteria for cancer clinical trials, when clinically appropriate. In June 2019, FDA issued related guidance that applies to a wider range of clinical trials beyond cancer trials.
- One of the 10 manufacturers GAO interviewed reported broadening its eligibility criteria to include more patients, such as those with HIV. Another manufacturer has begun reviewing its eligibility criteria and expects to include adolescents, as appropriate, in future studies—a population that has generally been excluded from trials. However, these and two other manufacturers cited challenges in these efforts. One stated that expanding participation to patients who use other medications, for example, could adversely affect a study's ability to identify the effects of the studied drug.

Outside of clinical trials, patients with certain medical conditions, who are unable to enroll in a clinical trial, and have no other comparable medical options, may request to obtain access to investigational drugs. This can occur under FDA's expanded access program, or through a 2018 federal law known as "Right to Try." Under either pathway, a patient can only access the investigational drug if its manufacturer agrees to the request. FDA has taken steps to facilitate access to investigational drugs outside of clinical trials, and most manufacturers in GAO's review communicated information to patients and physicians through their websites about how to access their investigational drugs outside of clinical trials. For example:

- Since 2017, FDA took steps to simplify its expanded access program to make it easier to participate. In addition, to address concerns raised by manufacturers, FDA clarified guidance on how it would review data resulting from the program. Seven of the 10 manufacturers GAO interviewed viewed the guidance as an improvement.
- GAO's review of information communicated by 29 manufacturers on their websites found that 23 had policies about accessing investigational drugs outside of clinical trials. At the time of GAO's review, 19 of the 23 stated they would consider individual requests for access, while the other four stated they would not. More than half of the manufacturers stated that if they approve a request, they require additional steps, such as FDA review of the request.