HHS Office of Inspector General

REPORT HIGHLIGHTS

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How FDA Used Its Accelerated Approval Pathway Raised Concerns in 3 of 24 Drugs Reviewed

Why OIG Did This Review

- FDA can use the accelerated approval pathway to speed development and review of new drugs to treat serious and life-threatening conditions.
- Accelerated approval does not require that a drug demonstrate a clinical benefit prior to approval.
- As a result, there is risk that an accelerated approval drug will not ultimately provide a clinical benefit for patients, necessitating safeguards and transparency in FDA decision making.
- FDA's 2021 accelerated approval of aducanumab, a drug to treat Alzheimer's disease, raised concerns in Congress and the medical and research communities about FDA's judgment in approving the drug and the accelerated approval pathway in general.
- This review examines a sample of 24 drugs, including aducanumab, approved through the accelerated approval pathway for similar concerns.

What OIG Found

Our review identified concerns about FDA's use of the accelerated approval pathway in 3 of the 24 drugs we reviewed:

- For two of the three concerning approvals, FDA evaluated analyses not included in the sponsor's (i.e., pharmaceutical company's) original analysis plans, deviating from recommended practices.
- FDA approved these three drugs despite concerns from its own reviewers and/or advisory committees.
- For one drug, some meetings with the sponsor appeared to be missing from the administrative file and other meetings are not fully documented.

Additionally, two of the three drugs that raised concerns are now off the market, and completion of the confirmatory trial for the third drug is delayed.

What OIG Recommends

OIG recommends that FDA strengthen guardrails in certain circumstances to ensure appropriate and consistent use of the accelerated approval pathway. FDA should:

1. Define specific factors that would require FDA's accelerated approval council to advise on certain drug applications.
2. Take steps to ensure that appropriate documentation of meetings with sponsors is included in drug approval administrative files.

FDA concurred with the second recommendation but did not concur with the first recommendation.