JUNE 2025

WHEN SPEED UNDERMINES EVIDENCE:

The FDA's Flawed Accelerated Approval Pathway

The FDA's Accelerated Approval Pathway has critical flaws. Prioritizing speed-to-market risks exposing patients to ineffective—or even unsafe— treatments and erodes public trust in the agency. When CMS is required to cover drugs with unproven benefit, patients and taxpayers ultimately bear the cost.

Since the pathway's 1992 inception, nearly 42 percent of post-market trials—designed to determine the effectiveness of newly approved drugs—had not started within a year of receiving accelerated approval[1]. Post-market—or confirmatory— trials are essential to verify the effectiveness and safety of therapies granted accelerated approval.

These drugs often reach the market based only on proxy metrics for clinical benefit such as lab values or imaging results. But these surrogate endpoints fall short of actual clinical outcomes or measurable improvements in patient health. Patients are given false hope and public dollars are wasted when confirmatory trials are not completed, or when a failed drug remains approved.

To understand the limitations of accelerated approval, consider the case of Aduhelm — granted accelerated approval to treat Alzheimer's disease despite surrogate endpoints lacking proven benefit. The decision raised concerns about the FDA's judgment in approving the drug and about the accelerated approval pathway overall. Fortunately, CMS approved Medicare coverage only in clinical trials for further study and indeed, within 10 months the $56,000 drug was pulled from the market. Were it not for CMS' caution, thousands of patients would have spent millions on the unproven, risky drug.



ACHP urges policymakers to:

1. Allow health plans to retain the authority to assess clinical benefit of accelerated approval drugs based on interim and final results from confirmatory trials. Medicare should not require coverage of drugs approved through the accelerated approval pathway until confirmatory trials are completed.

2. Require automatic withdrawal if a drug fails to demonstrate clinical benefit in confirmatory trials or interim benchmarks.

Policymakers must act to restore accountability to the FDA's Accelerated Approval Pathway, so effective therapies can reach patients facing serious, life-threatening diseases. Requiring timely confirmatory trials and aligning Medicare coverage with demonstrated clinical benefit will protect patients, preserve trust and ensure taxpayer dollars are spent on treatments that truly work.

1. McKenzie, Heather. "Evolving FDA Accelerated Approval Pathway Suffers Spate of Withdrawals." BioSpace, 28 Oct. 2024. https://achp.pub/BioSpace_AAP

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