Many cancer drugs still unproven 5 years after accelerated approval

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New research questions the effectiveness of the U.S. Food and Drug

Administration's accelerated drug approval program after finding that many cancer drugs remain unproven five years later.

The study, published Sunday in the *Journal of the American Medical Association* and presented simultaneously at the American Association of Cancer Research's annual meeting in San Diego, found that 46 cancer drugs were granted accelerated approval between 2013 and 2017.

Of those, 41% showed no benefit after five years of follow-up. And of the 63% that were converted to regular approval, less than half (43%) demonstrated any clinical benefit in confirmatory trials.

"Five years after the initial accelerated approval, you should have a definitive answer," Dr. Ezekiel Emanuel, a cancer specialist and bioethicist at the University of Pennsylvania who was not involved in the study, told the Associated Press. "Thousands of people are getting those drugs. That seems a mistake if we don't know whether they work or not."

First created in 1992 to get new HIV drugs to desperate patients as quickly as possible, more than 80% of the program's accelerated approvals now go to cancer drugs, researchers found.

The program allows the FDA to grant early approval to drugs that show promising results for treating debilitating or fatal diseases. In exchange, drug companies are expected to do rigorous testing and produce better evidence before gaining full approval.

When it comes to withdrawing disappointing drugs from the market, the FDA or the drug company makes that call. Sometimes, the FDA has decided that less definitive evidence is good enough for a full approval, the *AP* reported.

Meanwhile, it's not clear how much cancer patients understand about

drugs with accelerated approval, study co-author Dr. Edward Scheffer Cliff, of Harvard Medical School, told the AP.

"We raise the question: Is that uncertainty being conveyed to patients?" Cliff said.

Still, drugs that got accelerated approval may be the only option for patients with rare or advanced cancers, Dr. Jennifer Litton, of MD Anderson Cancer Center in Houston, told the AP.

It's important for doctors to carefully explain the evidence, Litton said.

"It might be shrinking of tumor. It might be how long the tumor stays stable," Litton told the AP. "You can provide the data you have, but you shouldn't overpromise."

The accelerated approval program recently got some updates from Congress that gave the FDA more authority and streamlined the process for withdrawing drugs when companies don't meet their commitments, the AP reported.

Those changes allow the agency "to withdraw approval for a drug approved under accelerated approval, when appropriate, more quickly," FDA spokesperson Cherie Duvall-Jones told the AP.

More information: Ian T. T. Liu et al, Clinical Benefit and Regulatory Outcomes of Cancer Drugs Receiving Accelerated Approval, *JAMA* (2024). DOI: 10.1001/jama.2024.2396

The FDA has more on the its accelerated approval program.