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How Much Do Physicians Really Know About FDA Drug and Device Regulation?

— More education is clearly needed

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Medications, from blood pressure control to chemotherapy, and medical devices, from hip replacements to heart valves, represent the backbone of healthcare. Ubiquitous in everyday clinical medicine, drugs and devices are only available for use if they have received the FDA's stamp of approval. Most people -- including physicians -- expect that FDA approval means assurance of effectiveness and sufficient safety.

However, in the modern era, novel approaches to regulation around certain drugs and devices means that the level of evidence supporting different new products can vary quite substantially. Are physicians aware of these changes in regulation?

FDA Regulatory Standards Continue to Evolve

Recent legislation and changes at the FDA have affected the strength of evidenceopens in a new tab or window traditionally collected on investigational drugs and devices before they receive FDA approval. The FDA itself publicizesopens in a new tab or window the fact that more drugs and devices are coming to market through expeditedopens in a new tab or window regulatory pathways that condense the time of premarket testing. This impacts the quality of data available for many new therapies, as drugs and devices get approved based on fewer clinical trialsopens in a new tab or window with fewer patients that are more likely to use surrogate measures (like results from blood tests or imaging scans) instead of clinical endpoints.

When lower-quality evidence supports new drug or device approvals, there is greater uncertainty about the benefits and risks and how they may compare to alternatives. Accordingly, many new drugs and devices are approved with requirementsopens in a new tab or window that manufacturers conduct further clinical trials to generate evidence that can assure safety and efficacy.

Post-Approval Evidence Generation

Unfortunately, however, such post-approval studies can be delayed. Approximately 8 to 10 years after approval of the highest risk medical devices, only one-thirdopens in a new tab or window of post-approval studies were completed and publicly reported. For accelerated approval drugs -- a pathway reserved for drugs showing changes to surrogate measures only reasonably likely to predict clinical outcomes -- fewer than halfopens in a new tab or window are completed in agreed-upon timeframes.

The HHS Office of Inspector General found that some post-approval trials were delayed 5 to 12 yearsopens in a new tab or window past their original planned completion dates. A combination of limited preapproval evidence and delayed post-approval evidence means that patients can receive FDA-approved drugs and devices with important efficacy and safety questions that remain unanswered for many years.

When post-approval studies are completed, they sometimes show that the drug or device does not work. In the last few years, about two dozenopens in a new tab or window accelerated approval drug indications have been withdrawn from the U.S. market after negative or incomplete post-approval studies. This is expected: with shaky and more uncertain pre-approval evidence, some drugs and devices won't end up having confirmed benefits that outweigh their harms.

How Familiar Are Physicians With These Changes in FDA Regulation?

In the context of these modern-era regulatory challenges, we surveyedopens in a new tab or window a nationally representative sample of 509 internists, cardiologists, and medical oncologists certified by the American Board of Internal Medicine. We asked about their familiarity with FDA approval, evidence standards, and what regulatory actions they expect from the FDA.

Our first key finding was that only 41% of physicians said they have at least a moderate familiarity with FDA's drug approval process. And only 17% said the same for medical devices. This gap is understandable, as regulatory standards are not part of medical education. This needs to be remedied so that physicians can better advise patients about the drugs and devices they recommend.

Second, physicians thought that evidence from rigorous preapproval testing was very important to FDA approval. They wanted to see strong evidence across the board: use of randomization, blinding, and sufficient follow-up, and indication that the primary endpoints were met.

A final key finding was that physicians want the FDA to act when drugs or devices approved with remaining questions linger for too long without confirmatory results. For drugs and devices not meeting agreed-on timelines for post-approval clinical testing, 60% of survey respondents supported at least temporarily withdrawing approval of the product. And if post-approval evidence did not verify a clinical benefit, 89% wanted the FDA to withdraw approval.

How Can We Better Educate Physicians About FDA Approval?

Even though FDA approval of new products is fundamental to the practice of medicine, how products are approved is rarely covered at any level of medical education. There are several pathways to remedy this critical physician knowledge gap.

First, U.S. medical school accreditation standards should include didacticsopens in a new tab or window about the FDA drug and device approval processes. If students need to know about complicated pathways of pathophysiology, they should know about how the treatments they will eventually use for these diseases became available. Medical licensing exams should add test questions about FDA approval.

Second, subspecialty training should include tailored education relevant to physician practice. For example, radiologists should learn about FDA regulation of artificial intelligence, which is increasingly used in imaging, and oncologists should learn about the FDA's accelerated approval program, since 85% of drugsopens in a new tab or window approved through this pathway are for cancer.

Finally, for physicians who have already completed training and are in clinical practice, continuing medical education (CME) and clinical practice guidelines should include information about evidence required to support FDA approvals.

What Do Physicians Want, and What Should FDA Do?

Our survey findings might provide support for a re-examination of what have become routine regulatory practices related to the evidence accepted for new approvals and how post-approval commitments are managed. The move to permit more products on the market with less rigorous data has been driven by a perception that such trends align with the desires of patients, the medical community, and the public.

But our survey suggests that physicians, at least, may be concerned about new products approved based on limited testing, and seem to strongly support rapid follow-up of uncertainties and FDA action when products turn out to not work as expected. Striving for greater rigor in both premarket and postmarket testing will better meet the expectations of the majority of physicians who are charged with recommending these drugs and devices in clinical practice.

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