NATIONAL CENTER FOR HEALTH RESEARCH
The Voice For Prevention, Treatment And Policy

PDUFA: A Public Health Perspective

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Disclosures

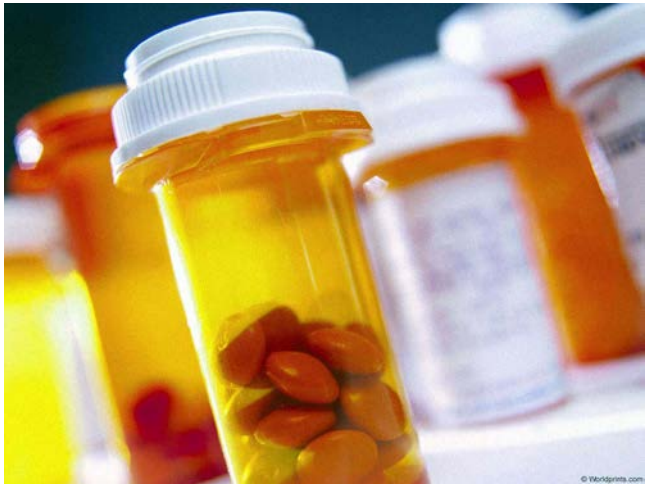
The National Center for Health Research is a nonprofit think tank that focuses on the safety and effectiveness of medical and consumer products and does not accept funding from companies that make medical products.

FDA Approval

FDA approval requires evidence that drugs are safe and effective, defined as having benefits that outweigh the risks for most patients. What is PDUFA doing premarket and postmarket to ensure those decisions are accurate?

FDA's Drug Approval Criteria

- → **Safe**
- → **Effective**
- → **Inspected**



Does it work as expected?

How sure can patients be that their medication works and is as safe as the label says?

Are labels understandable? How consistent are they with premarket data and the most recent data from Postmarket surveillance?

Is PDUFA Patient-Centered?

- → **Performance data are currently based on speed**
- → **Performance data should also be based on patient-centered outcomes**
- → **PDUFA does NOT address many issues most important to patients**

Is PDUFA Patient-Centered?

- → **Biomarkers/surrogate endpoints aren't clinically meaningful. They can't tell patients if the drug's benefits outweigh the risks. (PFS vs. OS, for example)**
- → **Test tube analyses can't say if benefits outweigh risks for patients**
- → **Non-inferiority standards can result in approving inferior drugs**

How can PDUFA improve?

- → **How good are safety data for male and female patients of different ages and race/ethnicity?**

- → **Are DTC ads misleading? Other ads?**

- → **Patients want inspections to be thorough, especially foreign inspections. PDUFA should help support inspections.**

How can PDUFA improve (cont'd)?

- → PDUFA should support FDA staff to improve safeguards for off-label prescribing. FDA should develop patient materials to explain off label prescribing and target off-label uses that are known to be ineffective or unsafe.

- → Are detailing activities, DTC ads, or ads to doctors directly or indirectly promoting off label use?

How can PDUFA improve the info available to patients and providers?

- → **PDUFA should support more postmarket surveillance (other than Sentinel)**

- → **PDUFA should provide support for FDA staff to create Patient Booklets, Informed Consent Checklists, etc.**

- → **PDUFA should support FDA "Dear Doctor" letters and warnings to patients**

Post-market studies

- → **Enforce clinical trial requirements**
- → **Sentinel, adverse event reports, and other real world data can supplement <u>not replace</u> clinical trial data**

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