



THE NEED FOR FDA REFORM: FOUR MODELS

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The Food and Drug Administration, one of the oldest regulatory agencies, is showing unfortunate signs of age, particularly in its drug and device approval process. The approval process was established with the best of intentions—namely, to keep unsafe products off the market—but it has always come at a cost in terms of delaying life-enriching, and even lifesaving, drugs and devices.

The current cumbersome approval process generates both expense and uncertainty for inventors. The slow pace of approvals from the agency imposes avoidable suffering on patients, even as the FDA falls further behind technological progress. The agency's review process needs to undergo comprehensive reform to streamline the process so that it may keep pace with modern developments and the need for speedier drug and device approval. The most important reform is for policymakers to determine where the FDA has a comparative advantage and where the private sector can take on some of the duties that the FDA has been performing.

The consequences of failing to implement comprehensive reform will be profound for innovators and, ultimately, patients. This policy brief summarizes four models for reform proposed by scholars affiliated with the Mercatus Center at George Mason University and others which would change how medical products are brought to markets and removed from markets by creating a process to adapt to technological growth and consumer demand in the 21st century.

THE COSTS OF EXCESSIVE REGULATORY PRECAUTION

There are several **costs involved in the FDA's current regulatory approval process** for devices¹ and drugs.²

The process is expensive and creates great uncertainty for inventors. FDA approval can cost tens of millions of dollars to produce what often amounts to thousands of pages of documentation and information for the FDA. For drugs, the cost is much higher than for medical devices.

- Inventors and manufacturers looking to market a device lack certainty about the likelihood of a drug or device’s approval, and they do not necessarily know how much testing the FDA will require before an approval.
- There is also no certainty about what the total cost of producing information required by the FDA will be.

Approval of a medical device can take months or years. For high-risk products, the process takes place after inventors and manufacturers spend years conducting clinical trials. The costs involved in seeking approval create disincentives for inventors to create new products.

- Delays for those products that do manage to get through can be excessive, potentially causing patients to needlessly suffer or die.
- The approval is somewhat easier for a medical device approved under the 510(k) process (for low- and medium-risk devices). This means that manufacturers have an incentive to make modest, rather than revolutionary, changes in device design.
- The FDA also has an incentive to slow down the process for approval, or deny approval entirely, because of asymmetric incentives: if it releases a device that harms people, the agency faces harsh criticism; if it harms people by failing to release a device, there are no ramifications.

The FDA is unable to keep pace with the technological growth and change available in the information technology age. With many rapid advancements in medicine and health, the FDA’s 40-year-old enabling law does not allow the agency to keep up.

Regulatory overreach is impeding favorable patient health outcomes. The current regulatory approach by the FDA often leaves the agency essentially seeking to practice medicine rather than making safety and effectiveness determinations. This dampens the development of drugs for diseases that affect large populations of Americans, while also creating delays and raising prices.

The FDA has used its **regulatory power to overreach** in at least two significant ways:

“FDA approval can cost tens of millions of dollars to produce what often amounts to thousands of pages of documentation and information for the FDA.”

- The FDA currently is using the benefit-risk preferences of an average patient to inform its decisions to approve or not approve drugs and devices. Because the preferences of patients are appreciably different, a large segment of patients will suffer because their preferences are not satisfied. This becomes particularly acute for those patients with debilitating or deadly symptoms for which there is no current FDA-approved remedy.
- The FDA requires most drugs to be evaluated for clinical efficacy, rather than the drug’s intermediate impact on the body (such as effects on LDL cholesterol or blood pressure). This requirement means that drug testing takes much longer and is much more expensive.

MODEL 1: COMPETING APPROVAL BODIES

One potential reform model grants approval authority to multiple private certification bodies,³ allowing them to compete with the FDA and each other on the price, quality, and timeliness of approvals. This is similar to a model that already exists in the European Union, which has reciprocity among private “notified bodies” that assess devices for safety standards. This encourages speedier—but still safe—medical device approval. This system could also be employed for drug approvals.⁴

Combining the best of the current European medical device approval system and an older system for maritime safety, the proposed model would allow private approval bodies to compete with the FDA for the trust of consumers. This system would also allow the FDA to shift some of its resources to become more of an information and enforcement agency and—perhaps—focus its resources on the highest-risk products.

This new system would include the following features:

- *Competition for trust.* Private medical product approval bodies would compete with the FDA for the trust of hospitals, insurers, physicians, and patients. Manufacturers of devices could submit their devices to private bodies, which could grant approval based on the safety and effectiveness of the devices. Patients and doctors would drive choices between private approvers and the FDA in a lively marketplace. This may also give rise to third-party rating agencies.
- *The FDA serving in a new role.* The FDA would also serve a useful role in setting broad good manufacturing practices (GMPs) and reviewing standards that could be monitored by nongovernmental bodies for compliance. The FDA could also retain approval for the most risky devices, gather and publish information on postmarket issues, and focus on enforcement.
- *Increased information for patients and doctors.* The information revolution has empowered patients and consumers by providing greater access to information

about medical drugs and devices. As new drugs and devices are developed in the market, offering consumers greater choice, more information will also benefit consumers as they are better able to make decisions regarding their own health.

MODEL 2: GLOBAL REGULATORY RECIPROCITY

A second solution involves global regulatory reciprocity among national drug and device approval agencies.⁵ George Mason University economists Daniel Klein and Alexander Tabarok argue that this reform would end the FDA's monopoly on drug and device approval and let drug and device manufacturers market their products here in the United States once they had gained approval in another major market, such as the European Union or Japan. With international reciprocity agreements in place, the FDA would have to compete for the business of drug companies. In a limited sense, this has already happened with medical tourism. Global reciprocity would ensure that FDA would have to "shape up or lose out on the fees" that come with drug and device approval.

This system would accomplish two objectives:

- *It would create a reciprocity arrangement with countries that "have a proven record of approving safe drugs."* This includes most Western European countries, Canada, Japan, and Australia.
- *It would eliminate the FDA's monopoly on drug approval.* US drug companies could submit drugs and devices for approval to authorities in reciprocal countries and gain approval in the United States without ever having to go through the FDA.

MODEL 3: "FREE-TO-CHOOSE MEDICINE"

A third reform model can be found in the "Free To Choose Medicine" proposal, which "would establish a dual track system for new drug testing that preserves the existing FDA-controlled process, while offering physicians and patients the choice to use not-yet-approved drugs after preliminary safety and efficacy testing."⁶ Reforms along these lines were recently introduced in Japan.⁷

The FDA retains monopoly control over drug testing and the drug market, which denies patients the freedom to choose their own treatment. The FDA insists on meeting a very precautionary standard of safety and efficacy, but no drug is either 100 percent safe or effective for everyone. Different patients will have different genetic makeups, different environmental stresses, and different risk tolerances, and some very ill patients demand earlier access to drugs even with the uncertainty and risk of harm that could result.

The Free To Choose Medicine (FTCM) option would allow consumer choice and competition. Legislation enacting the proposal would establish a dual track system for new drug testing while preserving the FDA's current control process. This dual track system would offer

“physicians and patients the choice to use not-yet-approved drugs after preliminary safety and efficacy testing.”

- The results of this alternative system would be captured in a publicly accessible database, which would give patients, doctors, pharmaceutical manufacturers, medical researchers, and regulators up-to-date information about the experience of patients using Free To Choose–track drugs.
- As an option for consumers, the FTTCM track for testing new drugs would provide more consumer choice, “promote mechanisms for fast-paced, adaptive learning,” and could deliver medicines to patients earlier and at a lower cost.
- Patient demand for the FTTCM track drugs could better reflect the value that the public places on accessing innovative new drugs.

MODEL 4: A RETURN TO A TRADITIONAL UNDERSTANDING OF “SAFETY” AND “EFFICACY”

Another recent Mercatus research paper outlines a set of reforms to rein in the FDA’s overly broad discretion when it makes safety and effectiveness determinations. It explains that “in a sense, the FDA has restated its mission . . . from permitting new products that can advance health to demanding certainty that products will not cause any harm.”⁸ By crafting an excessively precautionary standard, the FDA has become more and more restrictive in its decision-making.

This shift has not only increased the cost and time of drug development, “it has also moved the FDA from its proper role in making public health decisions to become an improper force driving private health decisions.” At a minimum, Congress should reassert authority over food and drug regulation to make sure “safety” and “effectiveness” are properly defined. These requirements would limit the FDA’s further intrusions into what should be private health decisions:

- The FDA should be required to *explicitly define safety* with regard to the likelihood of causing death, debilitation, or severe harm.
- The FDA should be required to *explicitly define effectiveness* as having positive activity on the function related to the disease.

PROSPECTS FOR REFORM

Optimally, elements of all these reforms would be adopted, although that could prove to be politically challenging. Regardless, more flexible approaches such as these will be vital as the world of hyper-personalized medicine unfolds, because the “world of accelerating medical advancements is ushering in an age of personalized medicine in which patients’ unique genetic makeup and biomarkers will increasingly lead to customized therapies in which samples are

inherently small.”⁹ This means that the FDA’s standards for randomized clinical trials will actually stand in the way of treatments customized for patients, because individual information is lost in these trials.

But even without such reforms, we should expect that the public will gain increasing control over their medical treatment decisions because of the new technological realities described above. This is why reform of the FDA’s approval process must also be accompanied by expanded risk education efforts. Another recent Mercatus paper looks at the dramatic changes being ushered in by the Information Revolution, digital devices, and the rise of personalized medicine.¹⁰ These changes will require policymakers to adapt to a changing world.

With the growth of technology, it will be difficult for national regulators to control everything at the same time that technological innovation is decentralizing and democratizing drug and medical device experimentation. Continuing to impose costs on innovators and restrict the personal liberty of consumers is an unworkable approach. Congress and the FDA need to reorient their policy focus away from top-down control and toward improved risk education and health literacy. Their goal should be to help create a more fully informed citizenry that is empowered with more and better information about relative risk tradeoffs.

CONCLUSION

The FDA’s role in drug and device approval needs to change, and only Congress can bring drug and device marketing into the 21st century. The FDA should be reformed to create a market in which different patient and doctor preferences regarding risks and benefits can be better served. Allowing for a diversity of preferences will improve patient health outcomes.

Decentralizing decision-making must include trusting doctors and patients to make informed decisions by allowing a technologically competitive marketplace to help them make life- and health-altering decisions. The models proposed in recent FDA reform research are workable solutions that encourage policymakers to trust in people, technology, and competitive markets, not government regulators and an outdated regulatory system.

LINKS

1. <http://mercatus.org/publication/us-medical-devices-choices-and-consequences>
2. <http://mercatus.org/publication/proper-role-fda-21st-century>
3. <http://mercatus.org/publication/us-medical-devices-choices-and-consequences>
4. <http://mercatus.org/publication/health-options-foreclosed-how-fda-denies-americans-benefits-medical-research>
5. http://www.fdareview.org/09_reform.php
6. <http://www.fed-soc.org/publications/detail/free-to-choose-medicine>
7. <http://www.forbes.com/sites/realspin/2015/12/01/give-the-fda-some-competition-with-free-to-choose-medicine>
8. <http://mercatus.org/publication/proper-role-fda-21st-century>
9. <http://www.forbes.com/sites/realspin/2015/12/01/give-the-fda-some-competition-with-free-to-choose-medicine>
10. <http://mercatus.org/publication/right-to-try-and-fda-future-personalized-medicine>

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