

## THE PROPER ROLE OF THE FDA FOR THE 21st CENTURY

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The current medical marketplace is vastly different from the marketplace that existed when the basic elements of Food and Drug Administration law were passed. Despite rapidly advancing technology and patients' increasing desire to try new drugs and devices, the FDA has strayed significantly from the statutorily defined safety and effectiveness standards for drug approvals. The FDA now very often demands proof of clinical utility, including survival and disease outcomes, as a requirement for premarket approval. But hard proof of clinical utility is elusive, even for drugs that are clearly shown to be safe and effective as labeled. Rather than blocking safe and effective drugs on such grounds, we should allow physicians and patients to make outcome-oriented decisions, and rely on the medical marketplace to drive physician adoption of safe and effective drugs that prove to have great clinical utility in real-world settings.

A new study for the Mercatus Center at George Mason University evaluates current FDA policy toward new medical products and concludes that the FDA must return to its role as gatekeeper of safe and effective drugs and devices. The FDA has made significant incursions into private health decisions by exercising its public health mandate in a manner that was not intended by Congress. Rather than allowing doctors and their patients to determine how best to use a drug and ensuring that drugs placed in the market are safe and effective, the FDA has increasingly become concerned with controlling the outcomes of future judgments by physicians and patients regarding benefits and risks. The new medical marketplace should refocus the FDA on safety and effectiveness, leaving patients and doctors to evaluate and determine benefits and risks based on their experiences with the drugs.

To read the study and learn more about its authors, Joseph V. Gulfo, MD, economist Jason Briggeman, and graduate student Ethan C. Roberts, see [“The Proper Role of the FDA for the 21st Century.”](#)

### THE FDA'S REGULATORY OVERREACH

The FDA is rightly supposed to demand proof of the safety and effectiveness of drugs and medical devices. This is its mission under the law. The FDA's job is to approve safe and effective new drugs,

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biologics, and devices for use in the medical marketplace, making them available to doctors as they care for patients.

Unfortunately, regulatory overreach by the FDA often leaves the agency nearly purporting to practice medicine rather than making safety and effectiveness determinations. This dampens the development of drugs for diseases that affect large populations of Americans, and also creates delays and raises prices.

- *Average patient assessments.* Current law arguably does not allow the FDA to refuse an application for drug approval on the basis of the FDA's predictions about how "benefit-risk" assessments will be made by an "average patient" and the patient's physician. Yet the FDA routinely denies drug applications on this basis, even though there are patients who may have a different benefit-risk assessment than the average patient.
- *Off-label uses.* The FDA has also strayed outside of the law by considering off-label uses of medical products. Current law requires the FDA to consider the impact of a proposed drug based on its proposed label, yet the FDA uses other measures, such as social and behavioral science, to anticipate and predict how patients may use drugs in ways not specified by their labels.

#### FDA FEAR LEADS TO DEMANDS FOR UNREASONABLE PROOF

The FDA acts as if it is motivated by the fear of being blamed for the failures of approved products, and as a result it frequently demands proof that new drugs and devices will deliver only positive outcomes for patients. Sometimes studies prove that a drug or device will safely do what it promises on its label, but the FDA is not satisfied without proof of what patients' ultimate health outcomes will be. This can lead to the delay or even rejection of new drug applications.

- For example, it might be easy to prove that a device can help doctors identify malignant lesions, but very hard to prove that use of the device will save a statistically significant number of lives. The FDA will require additional evidence and hold up approval while patient health is left to inferior technology.
- Determining whether products will save lives is very important, but trying to anticipate that result before product approval is extremely difficult—researchers are even susceptible to getting the answer wrong. The fact that predicting this outcome is difficult does not mean the FDA should block products that are safe and effective.

#### THE MEDICAL MARKETPLACE AS IT SHOULD BE

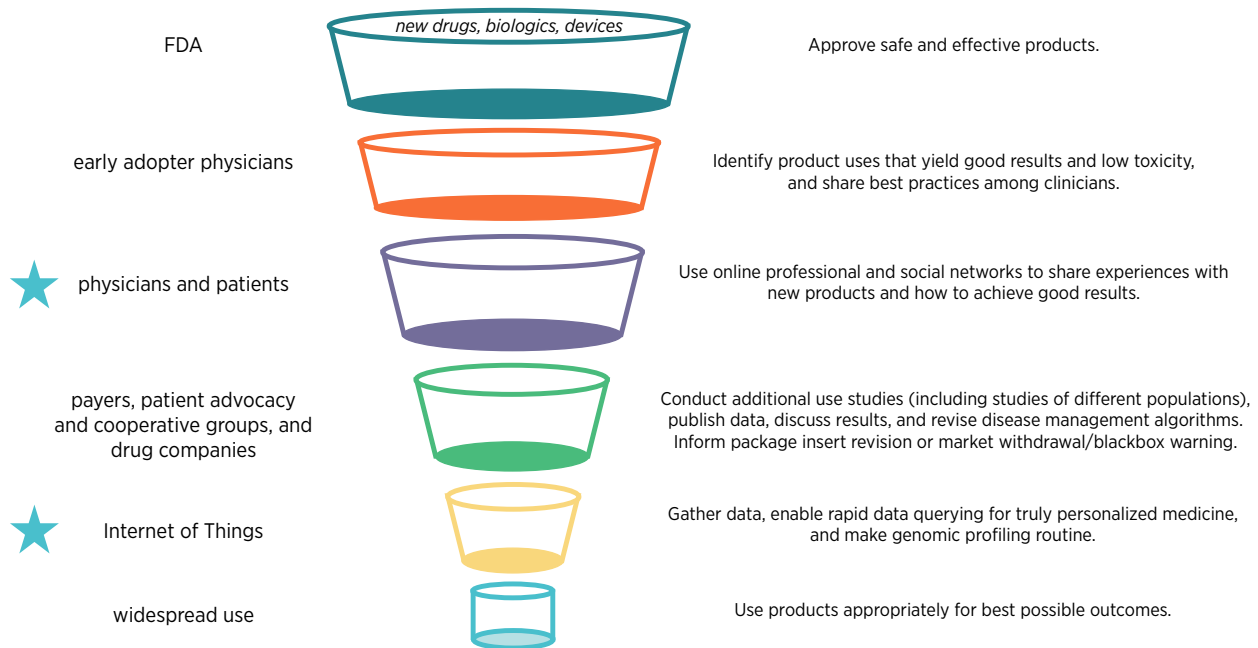
The job of the sophisticated medical marketplace (which includes early-adopter physicians, patients, payers, clinical trial consortiums, and physician education networks) is to select from among the safe and effective products approved by the FDA those that, in the real world, provide

the most clinical utility and patient benefit, with particular emphasis on individual patients through the practice of personalized medicine.

The FDA has endeavored to put itself at the bottom of this “drug-selection funnel” rather than in its proper role, at the top.

- By doing so, it has all but placed itself in the position of practicing medicine. This deprives doctors and patients of the opportunity to make the choices that best suit their circumstances.
- The FDA’s rightful place is at the top of the funnel, to screen out drugs and devices that should not be used by anyone.

### THE MEDICAL MARKETPLACE AS IT SHOULD BE TODAY



The figure shows the FDA at the top of the proposed new and more dynamic medical marketplace, in its proper role: permitting safe and effective products onto the market. It also shows how the ability to rapidly share and process knowledge helps doctors to improve patient outcomes at a rate and scope previously not possible. The new marketplace will better ensure that appropriate treatments are provided to patients.

### POLICY RECOMMENDATIONS

The 2017 reauthorization legislation of the Prescription Drug User Fee Act and Medical Device User Fee Act will spark significant debate. Policymakers need to understand the FDA’s role in the

medical marketplace and use the opportunity presented by these two acts' reauthorization to direct the FDA to perform tasks appropriate to its statutorily defined mission to promote health, limiting its incursions into private health decisions. There are several steps Congress can take to put the FDA back into its proper role:

- *Prohibit consideration of off-label uses.* Limit the FDA to consider only the safety of intended uses of a medical product, according to its label, rather than speculating about the safety of off-label uses or for off-label populations.
- *Explicitly define safety.* Define safety with regard to the likelihood of causing death, debilitation, or severe harm.
- *Explicitly define effectiveness.* Define effectiveness as having positive activity on the disease.
- *Use surrogate endpoints.* Require the FDA to expand its use of surrogate endpoints (including biomarkers) in trials and reviews.