

Department of Energy and Commerce CURES Initiative Roundtable
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Over the last fifty years patients in the United States have benefited from an era of unprecedented medical progress, which produced new and effective therapies for a host of previously untreatable diseases. However, the burden of federal regulatory complexity and associated costs of successfully navigating a new drug to final approval for marketing in the U.S. has increased to the point that many drugs are now approved by the European Medicine Evaluation Agency (EMA) of the European Union or other national regulatory bodies and become available to patients in other countries years before approval in the U.S., and some are never approved here. The FDA has, in recent years, initiated Fast Track Programs to speed the review of new drugs for serious diseases not having adequate alternative therapies but, despite these efforts, many promising drugs still meet with a lengthy and expensive review process and carry a significant risk of denial.

The time and expense of drug development is causing many pharma companies to look overseas for testing and marketing new drugs and to abandon research into many major health problems in the U.S. A number of steps could be taken to increase the efficiency of the FDA review and approval process. First, clear and open communication and expectations should be provided to companies approaching the agency with a proposed application, in a transparent process in collaboration with patients and academia, as well as industry. Continuous feedback should be given throughout, to avoid surprises on either side, as the process moves forward. Within the agency, some Divisions (e.g. the Rare Disease Division, the Office of Hematology and Oncology Drug Products) are already embracing this approach with a track record of success, but others are not. In addition, the agency should be more open to outside scientific advice from recognized impartial authorities, including those on its own review panels.

As clinical research paradigms evolve, the FDA should be open to exploring and accepting new methodologies for proving drug efficacy beyond the standard double blind placebo controlled trial, as well as wider consideration of the evidence provided by clinically relevant secondary endpoints. The FDA is fulfilling its watchdog function well given its resources, but consumer protection needs to be balanced against the future damage done by not have a potentially effective drug available to patients without other options. In the age of personalized medicine, the FDA should also be encouraged and permitted to tailor fit a given approval process to the circumstances of an individual drug and disease within broad parameters, without excessive burden from a standardized approach that is too proscriptive.

The FDA has become excessively risk averse in recent years because it is repeatedly taken to task by the media and the federal government when it approves a drug later found unsafe, even though the drug passed rigorous safety and efficacy testing and the agency should not be held accountable for approving drugs with rare and unforeseeable side effects appearing once in every 10,000 or 100,000 patients. Instead, post marketing surveillance programs using digital tools should be further developed to track these rare side effects, with the expectation that some will appear.

Finally, in the most serious of currently untreatable diseases, drugs having some benefit but also carrying serious risks which would otherwise preclude their approval should be given special consideration and, perhaps a special expedited pathway should be created and added to the Fast Track system for these conditions.