

## The “PATIENTS’ FDA” Act

*The “Promoting Accountability, Transparency, Innovation, Efficiency, and Timeliness at FDA” Act  
Senators Richard Burr and Tom Coburn, M.D*

The U.S. Food and Drug Administration (FDA) is responsible for assuring the safety, efficacy and security of human and veterinary drugs, biological products, medical devices, our nation’s food supply, cosmetics, and products that emit radiation. One element of the FDA’s statutory mission is to promote the public health, and the FDA accomplishes this mission in part by approving life-saving, life-enhancing innovations that make medicines safer, more effective, and more affordable. Because of the FDA’s broad regulatory authority, the FDA oversees products that amount to approximately 25 cents of every consumer dollar spent in the U.S. Therefore, by default, the FDA’s review and decision processes can have a substantial impact on significant sectors of our nation’s economy. As consumers and patients, the American people have a serious interest in ensuring that FDA is accountable, transparent, efficient, and making sound decisions in as timely a manner as possible.

Congressional appropriations for FDA’s work are complemented by user fees negotiated between the device and drug industries and the FDA. However, reporting only on the performance goals agreed to by industry and the FDA does not provide a complete picture of how well the FDA is working in fulfilling its mission on behalf of patients. The FDA is supported by both user fees *and taxpayer dollars*, so Congress has a critical oversight role in ensuring the FDA is meeting its requirements under the law. Moreover, as elected representatives of the American people, Congress institutionally has a duty to ensure the FDA is broadly fulfilling its statutory mission in promoting the public health through its review and regulation on a range of medical products.

The “PATIENTS’ FDA” Act is designed to complement the proposed agreements negotiated between the FDA and the drug and device industries. The “PATIENTS’ FDA” Act accomplishes this by requiring transparency in the review and decision progress against the initiatives and metrics agreed to in the prescription drug, generic drug, biosimilar, and medical device user fee agreements. This enhanced transparency and accountability can help improve regulatory certainty and ensure FDA is prepared to regulate cutting-edge 21<sup>st</sup> century medical products.

The reauthorization of the drug and device user fee agreements is an important opportunity for Congress to ensure that the FDA is fulfilling its mission on behalf of patients. Meaningful Congressional oversight can help highlight the processes that are working well at FDA, as well as reveal areas where the FDA needs to make improvements to ensure timely decisions on behalf of America’s patients.

### **TITLE I: ENSURING GREATER TRANSPARENCY AND ACCOUNTABILITY IN FDA DECISION-MAKING**

**Section 101: Advancing regulatory science to promote public health and innovation**—The user fee agreements acknowledge the FDA’s underlying commitment to advancing regulatory science – the approach by which FDA ensures application of current scientific knowledge and methods in its review and regulation of medical products. The focus on regulatory science is essential to ensure the FDA institutionally keeps pace with the increasing demands of a global economy and innovation in the 21<sup>st</sup> century.

- FDA will be required to develop a strategy and implementation plan for advancing regulatory science for medical products to promote the public health and advance innovation in regulatory decision-making.

- FDA will identify regulatory science priorities related to medical product decision-making, as well as regulatory and scientific gaps that impede the timely development and review of medical products.
- FDA will be required to track and annually report on their progress against their identified priorities, including the regulatory science goals outlined in the user fee agreements, and the integration and adoption of regulatory science advances.
- The Government Accountability Office (GAO) will provide an independent assessment of FDA's progress on regulatory science and provide recommendations on how to strengthen regulatory science initiatives.

**Sections 102-104: Detailed Reporting Requirements** — Compared with other industry user fee agreements, the medical device user fee agreement requires more granular, specific reporting requirements, which will provide important transparency about the FDA's review processes. The prescription drug, generic, and biosimilar user fee agreements should also include similarly detailed reporting requirements.

- The FDA is required to report on a deeper level of detail with respect to the performance goals agreed to in the prescription drug, generic drug, and biosimilar user fee agreements, and additional metrics (similar to the level of reporting FDA has agreed to in the medical device user fee agreement). For example, FDA will be required to report on the specific action counted against the performance goals (whether an application has been approved, not approved, withdrawn, etc.) and the total time it is taking for FDA to make final decisions about products.

**Section 105: Documentation of Regulatory Decisions**—One of the challenges inherent in FDA's review work is assessing the relative risks and benefits of a particular application pending for review. However, FDA reviewers already have certain tools to mitigate potential risk associated with the use of a certain product. Despite this, some sponsors (companies applying for approval of a medical product) have noted that some FDA reviewers request reams of additional information about a drug or device that is beyond the scope of data needed to meet the FDA's approval standard. FDA reviewers can improve their work with sponsors and applicants by increasing the transparency of the review process.

- FDA will be required to document the scientific and regulatory rationale for any significant drug and device decisions to the sponsor or applicant that submitted the application or report. Such documentation will provide important transparency into the FDA's regulatory decision-making. Documentation will also help to address concerns about evolving goalposts, and increase regulatory predictability, consistency, and accountability.

**Section 106: Review of Regulations and Guidance**

- Within one year of enactment, FDA will be required to review all human medical product regulations and guidance to ensure consistency with the Food, Drug, and Cosmetic Act and the regulatory principles of benefits of regulations and guidance justifying the costs and the adoption of the least burdensome approaches to regulation as outlined in President Obama's Executive Order 13563 to improve regulation and regulatory review.

**Section 107: Leveraging Information Technology to Fulfill FDA's Public Health Mission** - GAO has well documented FDA's challenge to sufficiently and successfully utilize its information technology (IT) processes. Moreover, GAO has noted how these challenges undermine FDA's ability to use accurate and timely information to augment its regulatory mission. GAO reports in 2009 and 2012 found that FDA has made mixed progress in establishing IT management capabilities that are essential in helping ensure a successful IT

modernization effort.<sup>1</sup> A comprehensive IT strategic plan, including results-oriented goals and performance measures, is vital for guiding and helping coordinate the FDA's IT activities. The user fee agreements include specific IT goals and it will be important for FDA to regularly report on specific metrics related to IT.

- FDA will be required to report on a comprehensive IT strategic plan consistent with related GAO recommendations, within one year of enactment.
- GAO will report on the progress of FDA meeting the results-oriented goals and performance measures set out in such plan.

## **TITLE II: RECALIBRATING RISK-BENEFIT CONSIDERATIONS**

**Sections 201-202: Recalibrating Patient Risk-Benefit Considerations**—Today, many patients accept the risks of a product because of the potential benefit it may afford them. However, too often well-intended decisions at the FDA may result in review decisions that prevent innovative products from being available to certain patients willing to accept greater risks than others. The FDA review and approval process should be recalibrated to better ensure patients have the opportunity themselves to weigh possible risks against the probable benefit of a particular drug or device.

- The user fee agreements include language regarding the steps FDA will take to ensure that patients' views are taken into consideration in the medical product development process and regulatory decisions, including considering patient tolerance for risk. Section 202 codifies in statute FDA's commitment to improve on patient risk-benefit considerations (as included in the user fee agreements) to ensure accountability for fulfilling the commitment the FDA and pharmaceutical and device industries already agreed to in the user fee agreements.

## **TITLE III: REDUCING UNNECESSARY DELAYS AND REGULATORY BURDENS**

**Section 301: Optimizing global clinical trials**—Drug and device sponsors may submit data from foreign and domestic clinical trials to support marketing applications. As companies compete in an increasingly global marketplace, sponsors are more and more relying heavily on data from foreign clinical trials to support their marketing application for drugs and biologics. In fact, a recent analysis of the [www.ClinicalTrials.gov](http://www.ClinicalTrials.gov) website found that the 20 largest US-based pharmaceutical companies were conducting about one-third of their clinical trials exclusively at foreign sites. FDA should work with its regulatory counterparts in the international community to optimize global clinical trials. FDA's partnership with its counterparts in other countries would help eliminate duplicative trials and enhance the efficiency of medical product development.

- FDA will be required to work with other specific regulatory authorities of similar standing, medical research companies, and international organizations to foster and encourage uniform, scientifically-driven clinical trial standards around the world.
- FDA will also be required to enhance its commitment to provide the least burdensome, consistent parallel scientific advice to manufacturers seeking simultaneous global development of new medical products in order to minimize the need for duplication of clinical studies, preclinical studies or non-clinical studies. This not only prevents duplicative processes, it helps reduce development costs.

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<sup>1</sup> <http://www.gao.gov/new.items/d09523.pdf>, <http://www.gao.gov/products/GAO-12-346>

**Section 302: Advancing American Patients’ Timely Access to Innovative Devices**—Many innovative devices that have not been approved or cleared by the FDA have been approved by FDA’s counterparts in Westernized countries. As a result, in certain cases patients in other countries may have more choices of medical devices than American patients. Unfortunately, current review processes amount to unnecessary regulatory burdens that can impede the FDA’s timely review of medical products, or, in some cases, deter device manufacturers from seeking approval of certain innovative devices in the United States. For example, as part of the FDA review process, a company may currently be required to conduct clinical testing in animals, despite the fact that the same device has already been used by patients in European countries without any safety problems or concerns. While it is necessary for devices approved in the United States to meet the FDA’s standard for clearance or approval, American patients deserve a commonsense regulatory framework that ensures they have timely access to the same innovative devices that consumers from other countries may enjoy.

- FDA is granted the discretion to, in limited circumstances, exempt a device from certain duplicative stages of clinical testing if the device has been approved in certain Western countries and not raised any safety concerns. The device would still be required to meet the relevant approval and clearance standards, and FDA could still require certain clinical requirements necessary for considering approval or clearance of the device.

**Section 303: Ensure legal sufficiency and consistency of FDA enforcement policies**—In 2009, FDA reversed longstanding policy that required prior legal review of regulatory action letters (i.e. warning letters) by the Agency’s Office of Chief Counsel (OCC).<sup>2</sup> While this change was likely a well-intended effort designed to limit enforcement delays, this change may have had the unintended effect of reducing the FDA’s consistent application of its enforcement authorities. Enforcement actions have significant repercussions for sponsors, consumers, and patients, and can often result in unintended consequences, such as drug shortages. It is in the interest of patients, sponsors, and the FDA to have senior attorneys review enforcement actions to ensure consistent application of legal principles and sound enforcement actions.

- FDA will be required to ensure legal review of regulatory letters by the Agency’s Office of Chief Counsel occurs before such letters are distributed publicly. The previous policy requiring FDA to submit all warning letters and untitled letters to OCC prior to their issuance to ensure “legal sufficiency and consistency with Agency policy” was put in place by HHS in November 2001.

#### **TITLE IV: STRENGTHENING THE ADVISORY COMMITTEE PROCESS FOR PATIENTS**

**Section 401: Strengthening Advisory Committees for Patients**—Currently, the FDA uses 50 Advisory Committees (AdComms) and panels to obtain independent expert advice on scientific, technical, and policy matters. Accordingly, patients deserve for the FDA to ensure that AdComm positions are filled by the most qualified experts and relevant medical and scientific expertise for the issues they are asked to consider. In addition to repealing the overly stringent conflicts of interest requirements put in place under the FDA Amendments Act of 2007, which has made it difficult for FDA to fill key advisory committee positions, the AdComms should be strengthened and improved, including standardizing the AdComm processes. FDA should also work with stakeholders (consumer groups, patient groups, industry) to increase the number of experts (known as Special Government Employees, or SGEs) across medical and scientific specialties.

- FDA will be required to issue guidance to standardize AdComm processes, including a template for AdComm bylaws and curriculum vitas of members.

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<sup>2</sup> <http://www.fda.gov/ICECI/EnforcementActions/WarningLetters/default.htm#browse>

- FDA will be required to report annually on the management of the pool of SGEs, specifically outlining increases in the numbers of available SGEs and deficiencies in specific medical specialties.
- FDA will be required to publish online an annual report containing, at a minimum, a summary of the AdComm activities and recommendations made during the previous fiscal year.
- FDA may require members of AdComms to sign an affidavit saying they have read the majority of materials and are reasonably prepared.

## TITLE V: MEDICAL DEVICE REGULATORY IMPROVEMENTS

**Section 501: Strengthen Tracking and Review of Applications for Investigational Device Exemptions**— Some sponsors have expressed concerns that the Investigational Device Exemption (IDE) process has grown increasingly onerous. A few sponsors have unfortunately seen the IDE process take more and more time, needlessly increasing costs and uncertainty for companies. To help address this dynamic, as part of the medical device user fee agreement, the FDA agreed to continue its efforts to improve IT systems with the goal of eventually facilitating availability of real-time status information for submissions.

- To further advance the policy already agreed to by FDA and the medical device industry, the FDA will be required to add a tracking number to device submissions and require the reviewer assigned to handle submissions to have prior experience with that type of device or technology, or other relevant experience, which should help increase predictability, consistency, and accountability throughout the review process

**Section 502: Investigational Device Exemptions**—There may be any number of reasons a sponsor wants to conduct certain clinical studies that are not directly related to the classification or approval of medical devices by the FDA. However, some sponsors have noted the propensity of the FDA to effectively prejudice the approval of a medical device, by basing its decision related to a request to conduct clinical investigations of a device on whether or not the FDA believes that the clinical study will be adequate to support the ultimate classification or approval of a device. If the FDA approves the investigational use of a device only using the more narrow regulatory standards of device approval or classification, clinical research in the U.S. could be unduly restricted as innovators would be effectively encouraged to conduct clinical trials overseas.

- FDA will be prohibited from disapproving clinical research based on a determination that the device will not meet the standards for approval or clearance. This reinforces the importance of freedom for scientific investigators to design and conduct clinical research studies involving investigational medical devices in the U.S. consistent with the protection of public health, safety, and ethical standards.

**Section 503: Device Submission Acceptance Criteria**—It is important that device submissions be of the highest quality and that FDA is consistent in its decisions related to accepting such submission.

- FDA will be required to implement revised device submission acceptance criteria, as they already agreed to do under the proposed medical device user fee agreement.
- GAO will be required to issue a report regarding the FDA's performance related to device submission acceptance criteria.

**Section 504: Transparency in Clearance Process**

- FDA will be required to publish detailed review summaries for 510(k) clearance to increase predictability, consistency, and accountability.

**Section 505: Restoring Regulatory Certainty with Respect to 510(k) Reports Required for Certain Modifications**

—Section 510(k) of the Food, Drug and Cosmetic Act requires device manufacturers to notify FDA of their intent to market a medical device at least 90 days in advance. Medical device manufacturers are required to submit a pre-market notification if they intend to introduce a device into commercial distribution for the first time or reintroduce a device that will be significantly changed or modified to the extent that its safety or effectiveness could be affected. Such change or modification could relate to the design, material, chemical composition, energy source, manufacturing process, or intended use.

Sponsors have raised concerns about recent guidance issued by FDA that could significantly increase the regulatory burden related to 510(k) modifications without clear benefit to patients. Clarifying current FDA practice is needed to restore regulatory certainty to the 510(k) modification process by clarifying under which circumstances a device manufacturer would need to submit a 510(k) application based on a modification to their device.

- FDA practice will be clarified to ensure that a manufacturer would not need to submit a new 510(k) report if the modification to the device undergoing modification is validated by same method (or current equivalent method) applied to the original device, including all changes made to the original device since the last version reviewed by FDA in compliance with the Quality System Regulation (21 C.F.R. Part 820).
- FDA will be required to annually report on the number of 510(k) modification submissions.

**Section 506: Meeting the Device Needs of Individual Patients**—Concerns have been raised that the current statutory and regulatory approach to custom medical device exemptions does not allow health care providers enough flexibility to meet the needs of the specific group of patients for which the exemption was intended to serve, such as pediatric patients.

- FDA’s practice would be modified to increase the ability for doctors to meet the device needs of individual patients within the narrow exemption process, but with minimal regulatory burden.

**Sections 511 and 512: Reauthorize and Strengthen Third-Party Device Reviews**—FDA currently uses third parties to review device applications and conduct inspections, but FDA’s utilization of third parties could be improved and increased. Improving the third-party review program to foster better participation in the program would decrease premarket clearance times and conserve valuable FDA resources.

- Ensures third-party reviewers have access to the device information necessary to carryout recommendations and reviews while at the same time protecting sensitive, proprietary information.
- Requires FDA to provide training to third-party reviewers and strengthens FDA’s accreditation oversight by limiting the period accreditation applies and setting forth a process for reaccreditation of third-party reviewers.

**TITLE VI: STRENGTHENING MANAGEMENT TO SUPPORT THE FDA’S PUBLIC HEALTH MISSION**

**Section 601: Integrated Strategy and Management Plan**—GAO has well documented FDA’s management challenges.<sup>3</sup> A February 2010 GAO report found that FDA does not fully use established practices for effective strategic planning and management. FDA agreed with GAO’s recommendations to take several actions to

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<sup>3</sup> <http://www.gao.gov/assets/310/300950.pdf>

improve FDA's strategic planning and management, such as development of a strategic management plan, and working to make FDA's performance measures more results-oriented. Three years later, many of these recommendations have not been adopted.

- Based on the GAO recommendations, FDA will be required to submit an integrated management strategy to Congress. This integrated management plan must identify strategic goals and priorities for CDER, CBER, and CDRH and describe the actions FDA will take to recruit, retain, train, and continue to develop the workforce at these Centers to fulfill FDA's public health mission. The plan must also identify results-oriented, outcome-based measures FDA will use to track its progress of achieving the strategic goals and priorities identified.
- GAO will issue a report assessing the effectiveness of FDA's actions to recruit, retain, train, and develop its workforce and the measures used to gauge progress against the identified strategic goals and priorities.

**Section 602: Independent Management Review**— The medical device user fee agreement includes the requirement for an independent assessment of FDA's management. This is a common-sense requirement that will help inform FDA's leadership and Congress, however it does not apply to the drug review process. Congress, consumers, and patients deserve an independent and objective look at FDA's management of its mission and resources.

- Requires FDA to contract with an independent management company to conduct an assessment of all of the drug review and approval processes.