Bipartisan Policy Center, Top Medical Innovation Priorities

BIPARTISAN POLICY CENTER

FDA: Advancing Medical Innovation **is a Bipartisan Policy Center initiative led by former Senator Bill Frist, MD, former Congressman Bart Gordon, and BPC Health Innovation Initiative Director Janet Marchibroda, with input from an expert advisory committee, stakeholders including patients, providers, and manufacturers. Read about BPC's recommendations here.**

BPC Recommendations	PDUFA VI Draft Commitment	Senate HELP Innovation Package	21st Century Cures
	✓=fully included P=partially included		
1. IMPROVING THE MEDICAL PRODUCT DEVELOPMENT PROCESS			
1. Utilize real-world evidence for pre- and post-market activities. Using real-world evidence drawn from claims and clinical and patient-generated data sources is critical to improving the development and delivery of drugs and devices. **Recommendation:** BPC proposes a new FDA program to evaluate and prioritize data from both clinical and patient experience to support post-approval study requirements, approve new indications for existing treatments, and improve clinical trials used for regulatory review. BPC also recommends furthering a virtual infrastructure for real-world evidence, supported by public- and private-sector investment, to inform FDA efforts and the broader health system.	✓		✓
2. Expand use of drug development tools: biomarkers. A biomarker is a physiological characteristic evaluated as an indicator of normal biologic processes, pathologic processes, or biological responses to a therapeutic intervention. Measuring pulse or blood pressure are two such markers. **Recommendation:** BPC proposes that FDA increase the use of biomarkers in helping to develop medical products. We recommend further development of scientific methods, improved transparency in their use, collaboration with experts and stakeholders, and public guidance.	✓		✓
3. Expand use of drug development tools: patient-reported outcomes. Patient-reported outcomes (PROs) are reports on a patient's health status that come directly from the patient without interpretation by a clinician. PRO instruments measure treatment benefit or risk in medical product clinical trials. **Recommendation:** BPC proposes a collaborative public process at the FDA to improve and expand the use of patient-reported outcomes in helping to develop medical products.	✓	✓	✓
4. Integrate patient perspectives into benefit-risk framework. Patients' perspectives can add significant value to the drug development process, particularly on the risks of specific treatments. **Recommendation:** BPC calls upon policymakers to establish and implement an FDA process under which an entity may submit patient preference data to enhance a structured risk-benefit framework; publish guidance; and specify that exchange of such patient perspectives, and truthful, non-misleading information, between patients or their caregivers and the medical or scientific staff of a manufacturer should not be considered, and thus not violate legal restrictions on, promotion or commercialization of an investigational drug or biologic.	✓	✓	✓

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5. Allow manufacturers to share certain scientific information with doctors on "off-label" use of approved medical products. Prescribing and using drugs, devices, and biologics for purposes other than their FDA-approved uses is growing more common. Yet manufacturers are not allowed to promote their products for off-label use. **Recommendation:** BPC urges the FDA to delineate ways in which manufacturers can provide truthful, non-misleading, scientific information. Rules allowing manufacturers to share off-label safety and efficacy data with researchers, regulators, and insurers will help validate emerging uses for established therapies.			
6. Promote harmonization of international standards. Productive international regulatory cooperation can help regulators prioritize resources by learning from each other and leveraging the work of others. Some have suggested that closer regulatory cooperation would reduce costs in drug and device development and promote faster patient access. **Recommendation:** BPC proposes that FDA establish a clear process for recognizing standards for medical devices and require the FDA to publish guidance regarding such process. BPC recommends the FDA and the U.S. Trade Representative report on progress on international standards harmonization, and encourage FDA's commitment, including those related to manufacturing facilities and sharing of best practices. BPC also proposed that FDA explore reciprocity of approval among highly developed trading partners for well-understood drug or device classes or products for which there is high unmet medical need.			**P**
7. Improve interoperability of health information technology. Despite billions of dollars invested in EHRs, interoperability and sharing of information across settings and providers remains low **Recommendation:** BPC proposes that the federal government adopt voluntary consensus standards for health IT, with regular oversight and reports on federal agency compliance with such standards. The federal government would publish such standards, assure testing prior to adoption and identify standards for federal adoption through rulemaking, to assure adequate public input.	**P**	✓	✓
2. INCREASING REGULATORY CLARITY			
8. Improve regulatory clarity for health information technology. FDA has authority to regulate electronic health records and other clinical software under The Food, Drug and Cosmetic Act. The Office of the National Coordinator (ONC) has health IT oversight authority under the HITECH Act. Coordination is essential. **Recommendation:** BPC proposes that Congress and FDA clarify that regulation of electronic health records should fall outside of FDA's authority to regulated medical devices. HHS should authorize independent bodies to develop voluntary consensus standards, evaluate standards compliance, and facilitate voluntary patient safety reporting to support health IT safety.	**P**	**P**	**P**

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9. Clarify regulatory authority related to laboratory-developed tests (LDTs). Regulatory authority associated with laboratory-developed tests (LDTs) is unclear, uneven, and duplicative. LDTs are in vitro diagnostic tests that are developed, validated, and used for in-house pathology and diagnostic purposes. **Recommendation:** BPC proposes the development of a risk-based regulatory framework for the regulation of LDTs that promotes innovation, protects patient safety, and avoids regulatory duplication. BPC recommends a framework specifies a risk classification for LDTs, ensures clinical validity, assures that information on diagnostic errors stemming from LDTs is available to the public, and addresses areas of overlap and regulatory uncertainty as it relates to the role of FDA and CMS through its Clinical Laboratory Improvement Amendments (CLIA) authorities.			
10. Advance Precision Medicine. Precision medicine targets individuals' unique genetic variations, environment, and lifestyle. During 2015, Congress provided $200 million for developing the scientific evidence needed to move the concept of precision medicine into clinical practice. **Recommendation:** BPC's proposed inter-agency working group would build upon the Precision Medicine Initiative, developing regulatory approaches that protect patient safety, promote innovation, and accommodate rapid changes in science.		✓	✓
11. Improve combination product regulation. Innovative treatments are often hybrids, employing a combination of devices, drugs or biologics. FDA's Office of Combination Products designates a primary center for review; however, the distinct roles and responsibilities between the Center for Drug Evaluation and Research (CDER), Center for Devices and Radiological Health (CDRH), and Center for Biologics and Research (CBER) are not clearly delineated. This situation has resulted in conflicting feedback to sponsors, duplicative testing on patients, delays and inefficient use of resources. **Recommendation:** FDA should: address the lack of coordination, slow response times, and clarity on data requirements; publish a timely list of request decisions and rationales to ensure consistency across similar combination products; and track/report on attendance of each relevant center at milestone meetings and meeting review timelines and user fee performance goals of the coordinating center.	✓	✓	✓
12. Develop a regulatory framework for regenerative cellular therapy. Cell therapy—which involves the use of cells to restore healthy function in the human body—represents one of the most promising areas for the next generation of groundbreaking treatments, which are generally not accessible to patients. **Recommendation:** BPC recommends creation of a regulatory pathway that reflects the unique attributes of cell therapy and serves as a "middle" ground between Section 361 "practice of medicine" which does not require pre-market review and Section 351, which requires a full biologics licensing application (BLA), which ordinarily costs up to $1 billion and takes 10 to 12 years.		**P**	

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13. Improve FDA's process for creating guidances and regulations. The FDA's standards and interpretations—whether they are released via guidances or other means—should be transparent and predictable for all stakeholders. **Recommendation:** BPC recommends that FDA improve the process for creating guidances and regulations. The FDA should use formal rulemaking processes when making substantive policy changes. BPC proposes that the FDA be authorized and encouraged to use public-private partnerships to develop and draft guidance documents, while leaving final approval authority with FDA. BPC also recommends that the FDA explore and address administrative barriers to finalizing guidances.		✓	
3. STRENGTHENING THE FDA'S ABILITY TO CARRY OUT ITS MISSION			
14. Strengthen the U.S. Food and Drug Administration. FDA still lacks adequate capacity and scientific expertise to keep pace with medical innovation. Congress increased the agency's annual funding for FY 2016 by $133 million. **Recommendation:** BPC urges that FDA be allowed to direct-hire staff, increase the number of exceptions to salary caps, and permit greater staff attendance of scientific meetings. BPC also recommends that the Office of Management and Budget (OMB) Paperwork Reduction Act be waived, knowledge/workflow management improvements, and expansion of the internal IT infrastructure.	✓	✓	✓
15. Encourage the effective use of public-private partnerships at the FDA. Public-private partnerships and advisory committees, when used appropriately, can expand the resources available to FDA and address gaps in scientific expertise and capacity issues. **Recommendation:** BPC proposes to reconfirm and encourage FDA to use its existing authority to use partners and trusted intermediaries to augment FDA's internal resources, particularly for novel or complex technologies. BPC recommends FDA to monitor, evaluate, and report on the outcomes and effectiveness of existing public private partnerships.	✓		
16. Improve the FDA's internal review processes. Ensuring the implementation of best practices across FDA centers would contribute to reducing the cost and time to discover, develop, and approve drugs, biologics, devices, and diagnostics, enabling more timely access to drugs and biologics, and ensure safety. **Recommendation:** BPC proposes that FDA implement strategies in response to an organizational study that evaluates review times, identifies root causes of delays, identifies best practices, and recommends measurable goals and actions to support faster turnaround times. BPC recommends that FDA to develop an inter-agency training program to implement best practices across centers and divisions and a monitoring system to track and report progress against implementation goals and impact.	✓	✓	

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4. INCREASING INVESTMENT IN MEDICAL PRODUCTS TO ADDRESS UNMET AND PUBLIC HEALTH NEEDS			
17. Accelerate the development and approval of antibiotics. One of the most urgent public health needs in the United States is new treatments for antibiotic resistant infections. **Recommendation:** BPC proposes to accelerate the development and approval of antibiotics by establishing a FDA program to expedite the approval of certain antibacterial and antifungal drugs for use in limited populations of patients at the request of the sponsor. This program should require prominent labeling that indicates it is for a limited and specific population and require the sponsor to submit promotional materials to FDA for approval; and allow FDA to remove labeling and promotion restrictions if the drug is approved for broader use. BPC recommends that FDA publish guidance describing criteria, process, and other considerations for demonstrating the safety and effectiveness of antibacterial and antifungal drugs approved for use in limited populations. BPC also proposes that FDA publish an assessment of the program, hold a public meeting, and consider expansion of the limited use pathway and program beyond antibacterial and antifungal drugs.		✓	✓
18. Improve processes for early patient access to medical products. For those patients with the most pressing unmet needs, the FDA has a process for expanded access, also called "compassionate use," that provides a pathway for patients to gain access to unapproved investigational drugs, biologics, and medical devices. **Recommendation:** BPC proposes improved processes for early patient access to medical products by requiring sponsors to make their policies on expanded access during clinical trials publicly available, including procedures for requests, qualification criteria, and a single point of contact. BPC also recommends that FDA finalize guidance regarding how it interprets and uses adverse drug event data resulting from drug use under expanded access programs.			✓
19. Increase incentives for the development of medical products with unmet medical needs. To continue searching for the next generation of treatments, drug-makers need sufficient protections for marketed drugs to recover their investments in drug discovery, including the large majority of compounds that fail to make it through the research and clinical-trial phases. **Recommendation:** BPC proposes increased incentives for the development of medical products with unmet medical needs by creating a new regulatory pathway for dormant therapies at the FDA, by giving FDA the authority to designate a new treatment as a dormant therapy if intended to treat an unmet medical need.			