10 ways to:
Advance Medical Innovation

BIPARTISAN POLICY CENTER

FDA: Advancing Medical Innovation is a Bipartisan Policy Center initiative led by former Senator Bill Frist, MD, former Representative Bart Gordon, and BPC Health Innovation Initiative Director Janet Marchibroda, with input from expert advisers, federal agency and private sector stakeholders including patients, providers, and manufacturers.

1

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 and urges implementation of organizational reviews, knowledge/workflow management improvements, and expansion of the internal IT infrastructure.

2

Advance Precision Medicine. Precision medicine targets individuals' unique genetic variations, environment, and lifestyle. During 2015, Congress provided $2.4 billion 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.

3

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.

4

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.

5

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. Despite the promise, cell therapies are generally not accessible to patients in the U.S. because of the outdated approach toward regulation within the FDA.

Recommendation: BPC recommends an accelerated pathway for cell therapies, whereby patients are treated with adult cells that do not cause a significant unintended immune response, after preliminary clinical evidence of safety and reasonable expectation of effectiveness have been demonstrated, and without initiation of phase III investigations. These therapies would then be granted temporary, conditional approval. Treatments that do not fall into the new category would be treated as they are today – under "practice of medicine" (no FDA regulation), or through FDA's full Biologics Licensing Application (BLA) process.

10 ways to:
Advance Medical Innovation

BIPARTISAN POLICY CENTER

FDA: Advancing Medical Innovation is a Bipartisan Policy Center initiative led by former Senator Bill Frist, MD, former Representative Bart Gordon, and BPC Health Innovation Initiative Director Janet Marchibroda, with input from expert advisers, federal agency and private sector stakeholders including patients, providers, and manufacturers.

6

Improve regulatory clarity for health information technology. FDA has some authority to regulate electronic health records and other clinical software under The Food, Drug and Cosmetic Act. The Office of the National Coordinator for Health IT (ONC) has health IT oversight authority under the HITECH Act. Coordination among agencies is essential.

Recommendation: Under BPC's proposed risk-based regulatory framework, the HHS Secretary would authorize independent bodies to develop voluntary consensus standards, evaluate standards compliance, and facilitate voluntary patient safety reporting. Such a model would serve to continually improve the development, implementation, and use of health IT. It would also ensure health IT is not regulated as a medical device by FDA unless determined that the product poses a significant risk to patient safety.

7

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 developing a national virtual infrastructure for real-world evidence, supported by public- and private-sector investment, to inform FDA efforts and the broader health system.

8

Expand use of drug development tools: biomarkers and patient-reported outcomes. A biomarker is a physiological characteristic that normal biologic processes, pathologic processes, or biological responses to a therapeutic intervention. Measuring pulse or blood pressure are two such markers. Patient-reported outcomes (PROs) are reports on a patient's health status that come directly from the patient without interpretation by a clinician. Both tools can inform the drug development process.

Recommendation: BPC proposes that FDA increase for 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. BPC also proposed a collaborative public process to improve and expand the use of patient-reported outcomes.

9

Improve interoperability of health information technology. Despite the tens of billions of dollars invested in electronic health records, interoperability –sharing information across settings and providers – still remains low. Barriers include the lack of a business case and technical infrastructure, costs associated with information exchange, and the need for standards.

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.

10

Integrate patient perspectives into benefit-risk framework. Patients' perspectives can add significant value to the drug development process, particularly on the risks and 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 regarding these submissions, methodologies, standards, and potential experimental designs; 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.