BIPARTISAN POLICY CENTER

*FDA: Advancing Medical Innovation*

# Recommendations for better data and development tools at FDA

*Evidence and drug development tools gathered outside of carefully controlled trials could significantly strengthen the evidence base for approval, safety, and efficacy. Examples include data from clinical and patient experience, biomarkers, and patient perspectives. BPC has recommended actions Congress and the FDA can take to improve the use of these types of data and tools to inform approval processes.*

## Recommendations:

### Accelerate the generation and use of more relevant evidence:

1. Require FDA to develop a program to evaluate and prioritize the use of real-world evidence—including data from both clinical and patient experience—to support post-approval study requirements, approval of new indications for existing medical products, and ultimately improved clinical trials used for regulatory review. To support this work, require FDA to develop a detailed plan within 18 months, begin implementation of a program within 24 months, and issue final guidance within 48 months.
2. Direct FDA to develop a framework for modernizing the traditional, randomized, large-scale (Phase III) clinical trials model of evidence development for regulatory review, engaging experts and stakeholders through a collaborative public process. The framework should provide an approach and regulatory requirements for incorporating data from pragmatic, randomized studies of broader populations where care is provided under more typical settings, in addition to data from randomized clinical trials.
3. Promote public- and private-sector investment in the development of a broad-based, nationwide virtual infrastructure to obtain more robust real-world evidence on both the safety and effectiveness of drugs and devices.

### Improve and expand the use of drug development tools, including biomarkers and patient-reported outcomes:

1. Require FDA to establish a framework and process for the submission, review, and qualification of drug development tools.
2. Authorize and strongly encourage FDA to engage with experts and stakeholders through consortia, to support the review of qualification submissions.
3. Improve transparency of drug development tool-related activities by requiring FDA to make information regarding the number of qualification-related requests and plans submitted and drug development tools qualified, publicly available.
4. Require FDA to develop—through a collaborative public process—guidance on biomarkers which contains:
    a. A conceptual framework describing appropriate standards and scientific approaches to support development;
    b. Recommendations for demonstrating the predictability of surrogate endpoints for purposes of supporting accelerated approval; and
    c. Description of the requirements for entities seeking qualification, reasonable timelines for FDA review, and processes by which both entities and FDA may consult with biomedical research consortia or others with expert knowledge and insights.

### Assure the incorporation of patient perspectives into benefit-risk assessment:

1. Require FDA to establish and implement a process under which an entity may submit patient preference data to enhance a structured risk-benefit framework.
2. Require FDA to publish guidance for the submission of patient preference data; methodological considerations and approaches for both collection and assessment of such data for benefit-risk; and methodologies, standards, and potential experimental designs for patient-reported outcomes.
3. Specify that the exchange of truthful and non-misleading information among patients, patient caregivers, or patient advocates and medical or scientific staff of a manufacturer, the purpose of which is to discover and understand patient or caregiver perspectives related to the specific disease from which a patient suffers, shall not be considered promotion or commercialization of the investigational drug or biologic, or a violation of the Federal Drug and Cosmetic Act.

For a full list of BPC recommendations on advancing medical innovation, see http://bipartisanpolicy.org/library/advancing-medical-innovation-for-a-healthier-america/