Developing an innovative medicine is a lengthy and complex process, taking an average of 10 or more years. The clinical trial component alone takes roughly six to seven years. With just 12 percent of drugs that enter clinical trials resulting in an approved medicine, the average research and development cost for each successful drug is estimated at $2.6 billion (including the cost of failures). Against this backdrop, pro-patient, pro-science, pro-market reforms at the Food and Drug Administration (FDA) would enhance the competitive market for biopharmaceuticals, drive efficiency in drug development and discovery and help hold down costs.

**Encourage Use of 21st Century Tools for Drug Evaluation, Review and Approval**

Scientific advances are re-shaping our understanding of the causes of disease, creating new avenues of research, exploration and discovery. New and powerful tools emphasize individual patient characteristics and include innovative clinical trial design, advanced statistical methods and use of real-world evidence.

**Example #1:** Biomarkers hold promise for improving and accelerating drug development. A biomarker is an objective measure of normal biologic processes, pathologic processes or biological responses to a therapeutic intervention. Broader use of biomarkers may speed and improve the quality of evidence development to support medical product evaluation and may accelerate product development timelines. For example, idiopathic pulmonary fibrosis (IPF) is a serious and life-threatening disease of the lung that involves progressive scarring leading to loss of lung function and ultimately death. Its cause is unknown. In late 2015, two new drugs were approved for treatment of IPF based on a biomarker-based measurement of delay in decline in lung function. Relying on the biomarker instead of mortality-based assessment allowed much earlier consideration of the drugs’ efficacy by the FDA, leading to speedier approvals and access for patients with this difficult disease. In addition to biomarkers, patient-reported outcomes could help enhance FDA decision-making by integrating the patient’s perspective into benefit-risk evaluations.

**Example #2:** New approaches to clinical trial design and statistical methods that leverage scientific advances could lead to greater efficiencies in drug development that could reduce the cost and time to bring a new medicine to market. The randomized, controlled clinical trial design needed for FDA approval has become increasingly complex over time, and regulatory acceptance of innovative approaches has not kept pace with advances in the field. The use of adaptive designs and advanced statistical methods, for example, would increase clinical trial efficiency. Appropriate use of real-world evidence also would allow information other than that derived from traditional studies to aid regulatory decision-making.

**Solution:** We need to modernize the FDA to keep pace with scientific discovery, including catalyzing the agency’s acceptance of innovative drug development tools and real-world evidence to drive greater efficiency. This will yield a more competitive, innovative and sustainable biopharmaceutical ecosystem that better reflects patient experience and perspective.
Ensure FDA Drug Approval Is Scientifically Sound and Efficient

Medical and basic science is advancing at a breathtaking rate. New developments – including those in immunologic and cell therapies, personalized medicine and regenerative medicine – hold the promise of treating debilitating diseases such as Alzheimer’s, cancer, diabetes and many rare disorders.

Example #1: For the FDA to keep up with the rapid pace of scientific advances, it must be able to deploy the most modern technologies and access the brightest minds to review cutting-edge scientific developments. Ensuring that FDA’s drug review staff infrastructure is strong will avoid needless delays in drug review and approval that lead to longer development times, missed opportunities, higher drug development costs and delays in treatments reaching patients.

Solution: FDA must be allowed to add internal expertise by granting the agency sufficient and efficient hiring authority to attract and retain a strong scientific workforce, including biostatisticians, pharmacologists and geneticists. FDA should also be allowed to pilot new ways to access external expertise through collaborative partnerships with academia and the National Institutes of Health.

Reduce the Generic Backlog and Incentivize Competition Where Needed

A generic drug enters the market at the end of an innovative medication’s lifecycle. When it does, the FDA allows the manufacturer to submit an Abbreviated New Drug Application (ANDA), which does not require repetition of time consuming and costly clinical trials the innovative biopharmaceutical company conducted. As a result, generics can enter the market at a fraction of the price of an innovator medicine. With nearly 90 percent of all U.S. retail prescriptions filled with generics, their timely approval is critical to patient access and the long-term sustainability of our health care system.

Example #1: Due to a significant backlog of ANDAs at the FDA, on average it currently takes over four years for the FDA to act on a single application.

Solution: To speed access to generic drugs, the next reauthorization of the Generic Drug User Fee Act (GDUFA) should consider additional steps to improve ANDA review efficiency. In this way, the current backlog of pending applications and the average time required to review generic drug applications can be reduced.

Example #2: For serious diseases or conditions in small patient populations, lack of availability of effective medicines with no remaining patent life or regulatory exclusivity, coupled with no or limited brand or generic competition, may constitute an important public health risk.

Solutions: We need to explore opportunities to encourage competition and catalyze generic entry when the market demonstrates a need. One approach might be to incentivize new sources of older, off-patent medicines for unmet medical needs with appropriate safeguards to prevent abuse. Financial incentives could encourage generic entry, such as an expanded tax credit for the development and manufacturing of generic drugs or a targeted grant program to support generic manufacturing investments and maintain production for eligible products. Another solution may be to provide regulatory incentives so more than one generic drug manufacturer enters the market for treating serious conditions.