FDA’s Efforts to Enhance Patient Perspective in Drug Development and Review

FDA will work in close partnership with patients to incorporate their experience into benefit-risk assessments.

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Statement from FDA Commissioner Scott Gottlieb, M.D., on FDA’s efforts to enhance the patient perspective and experience in drug development and review

Benefit-risk assessment is at the heart of what we do to ensure that Americans have access to medical products that are safe, effective and meet their needs.

But we’re also deeply aware that serious chronic illnesses aren’t monolithic. Patient perception of the benefits and risks of different treatment options can vary based on the stage of the disease, the age of onset, alternative therapies available to treat the disease (if any) and whether a novel therapy improves a patient’s ability to function normally, slows the rate of disease progression or impacts other aspects of a patient’s quality of life.

A 45-year-old father of two who is diagnosed with aggressive prostate cancer may have very different goals than an 80-year-old man diagnosed with the same disease.

To address these realities, we’ll continue working in close partnership with patients to incorporate their experience into our benefit-risk assessments. We know that first-hand knowledge of living with a serious illness – communicated in science-based terms that patients value and understand – is integral to facilitating the successful development of safe and effective products that can deliver meaningful benefits in each disease, or disease state.

Today we have many more tools to measure these patient benefits – including wearable devices, medical apps and even machine-learning programs. These tools can bring us a better understanding of how patients experience their illness, including how it affects their day-to-day feeling or functioning and how a given treatment may impact the course of that illness.

Tools for capturing the patient experience may be quantitative or qualitative, but they are transforming nearly every aspect of medical product development. Patients are teaching us about the benefits that matter most to them and the risks that they are most concerned about. Patients

are, rightly so, becoming the driving force of the medical research enterprise.

Improving the science of medical product development – what we call translational science – is integral to improving the efficiency of medical research. Routinely reviewing and updating the tools we use to make benefit-risk assessments is one of the most important parts of that process.

Structured and Transparent Benefit-Risk Assessments

Today, I'd like to focus on one part of our efforts in this area: the FDA's ongoing work to enhance our benefit-risk assessment and communication in the human drug review process.

This work began in 2013 as part of the Prescription Drug User Fee Act (PDUFA) V. Our priority to enhance benefit-risk assessment has continued with new efforts we began undertaking in 2017, as part of PDUFA VI and further expanded under 21st Century Cures.

Our work implementing these key pieces of legislation is improving clarity and consistency in communicating the reasoning behind the FDA's drug regulatory decisions. It's also helping integrate the patient's perspective into drug development and regulatory decision-making.

I'm pleased to announce that today we're issuing an update to our implementation plan, titled "Benefit-Risk Assessment in Drug Regulatory Decision-Making." This document provides an overview of the steps the FDA has taken since 2013 to enhance benefit-risk assessment in human drug review, which included implementation of the FDA's Benefit-Risk Framework into our drug regulatory review processes and documentation.

This document also provides a roadmap for enhancing the Benefit-Risk Framework, working toward a goal of providing guidance by June 2020 that articulates the FDA's decision-making context and framework for benefit-risk assessment. This forthcoming guidance will also outline how patient experience data and related information can be used to inform benefit-risk assessment.

So, what exactly is the Benefit-Risk Framework?

In order for a drug or biologic product to be approved, the FDA conducts a comprehensive analysis of all available data to determine if the drug is effective and that its expected benefits outweigh its potential risks. This assessment is fundamental to our regulatory process. The goal of the FDA's Benefit-Risk Framework is to improve the clarity and consistency in communicating the reasoning behind drug regulatory decisions, and ensure that the FDA reviewers' detailed assessments can be readily understood in the broader context of patient care and public health.

The structured framework also helps drug sponsors and other external stakeholders better understand the factors that contribute to the FDA's decision-making process when evaluating new drugs, including drugs under development. A standard Benefit-Risk Framework will also better ensure that the patient community can continue to engage effectively with the agency, and help us improve how we evaluate benefits and risks from the patient's perspective.

The Benefit-Risk Framework has been applied in our reviews of novel drugs and biologics over the past few years, and we're now using it more broadly. We recently had an independent third party conduct an evaluation of the FDA's implementation of the drug Benefit-Risk Framework. It found that FDA review staff, industry, healthcare providers, and patients and caregivers find the framework to be useful and effective in communicating the reasoning behind the FDA's decisions.

Incorporating Patient Voice into Benefit-Risk Assessments

The Benefit-Risk Framework recognizes that when FDA reviewers conduct a benefit-risk assessment, they consider not only the submitted evidence related to the benefit and risk and effects reported in clinical studies, but also, importantly, the "clinical context" of the disease. This context encompasses two major considerations: 1) an analysis of the disease condition, including the severity of the condition; and 2) the degree of unmet medical need. As part of this work, the FDA recognizes a need to learn about the clinical context more comprehensively and directly from the perspective of the patients who live with the disease and their caregivers.

After conducting patient-focused drug development meetings in over 20 disease areas, the FDA concluded that patient input can: 1) inform the clinical context and provide insights to frame the assessment of benefits and risk; and 2) provide a direct source of evidence regarding the benefits and risks, if methodologically-sound data collection tools could be developed and used within clinical studies of an investigational therapy. The FDA is now developing guidance to enable more widespread development of such patient experience data to inform regulatory decision-making, as part of our implementation of PDUFA VI and 21st Century Cures. Other efforts to more systematically incorporate patients' experiences and perspectives include:

- Hosting patient-focused drug development public meetings to advance a more systematic way of gathering patients' perspectives on their conditions and available treatments;
- Encouraging patient stakeholders and others to conduct their own externally-led, patient-focused drug development meetings;
- Providing patients, caregivers, advocates and others with more channels to provide meaningful input into drug development and regulatory decision-making, and to more easily access information provided by others; and
- Launching pilot programs – and advancing policies, in collaboration with the medical community – that help foster the design of clinical trials that place less burden on patients.

The benefit-risk implementation plan issued today is part of the FDA's ongoing commitment to advancing our mission of protecting and promoting public health. It marks another important step forward in increasing transparency of FDA decisions as well as streamlining the process by which we obtain input from the patient and stakeholder communities. We know that, in the battle against disease, engaged and informed patients are our best allies and our greatest resource.

The FDA, an agency within the U.S. Department of Health and Human Services, protects the public health by assuring the safety, effectiveness, and security of human and veterinary drugs, vaccines and other biological products for human use, and medical devices. The agency also is responsible for the safety and security of our nation’s food supply, cosmetics, dietary supplements, products that give off electronic radiation, and for regulating tobacco products.

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