New Agency Efforts to Advance the Patient Voice in Medical Product Development

We learn through scientific advances, but also by listening to patients.

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Statement fromFDA Commissioner ScottGottlieb, M.D., on new agency efforts to advance the patient voice in medical product development andFDA regulatory decision-making

Over the past decade, advances in our understanding of the basic biology of serious and life-threatening diseases has led to the development and FDA approval of targeted treatments for patients with a specific molecular sub-type. These more targeted treatments often address unmet medical needs. They can represent significant improvements over the prior standard of care.

This medical progress is the result of scientific advancement. But it's also a consequence of a bipartisan consensus that has supported investments in medical research and a modern regulatory process to advance safe and effective therapies.

For instance, Congress codified the FDA's early and intensive engagement with sponsors through the Breakthrough Therapy designation. This pathway helps advance treatments that demonstrate significant clinical benefits over the current standard of care. This designation was a recognition that as the science for drug development advanced and enabled more targeted approaches to the treatment of unmet medical needs, the FDA needed the ability to work more closely with sponsors to ensure that trial designs and endpoints were appropriate for the type of therapy and the disease state under consideration.

The benefits of these more efficient approaches can be seen in the treatment of diseases like cancer; where the FDA has modernized the trial designs and endpoints we accept to demonstrate efficacy for some subtypes of tumors after numerous consultations with stakeholders. This includes the support of the FDA's Oncologic Drugs Advisory Committee, as well input from a diverse group of experts, researchers and especially the feedback we receive from patients and their families.

We heard the message that modernized approval endpoints in oncology were necessary because many of the older approaches to clinical trial recruitment and design dated back to an era of

combination cytotoxic chemotherapies, when patients were exposed to significant treatment toxicities. These therapies sometimes offered only modest advances in efficacy, and they came with serious side effects. So the endpoints we assessed in trials were designed to offset the significant risks these drugs posed. New and more targeted therapies, however, operate differently. There are more opportunities to demonstrate significantly higher rates of benefit in carefully selected patients—and often show reduced toxicity—in smaller cohorts of patients who might express a marker that predicts clinical response. In these cases, surrogate endpoints—or measures of intermediate clinical benefit—can be both clinically relevant and highly valued by patients. These measures might include objective response rate or progression-free survival for solid tumors, or cytogenic response rate and minimal residual disease for hematological cancers.

We learn through scientific advances, but also by listening to patients. Our work demands that we must continue to reflect on how we can make the science of drug development and review more modern and more patient-centered, so that approved products impact the metrics that real-world patients and families value most. This requires ongoing engagement with the patient community. Done well, the result is more and more of our review of new medical products benefits from a better understanding of the patient's experience, providing our reviewers with the critically-important context of a disease, and helping them to understand what's most important to patients related to treatment benefits, risks and disease burden.

To that end, the FDA has made it a priority to work with companies and other stakeholders on gathering information from patients about their views and needs, and on building the tools that are needed to capture patient input in a way that provides meaningful data. To date, the FDA has held Patient Focused Drug Development (PFDD) meetings in more than 20 disease areas where we've heard directly from those impacted by diseases, including opioid use disorder, autism, HIV, Parkinson's disease and various conditions involving pain. These meetings have given the FDA's professional staff a deeper understanding of patient and caregiver experiences. We have gained from this experience. Also important to this effort is educating companies about rigorous approaches to obtaining and incorporating this important and unique input into product development. We must also explain how the FDA will incorporate this information into our regulatory decision-making.

Today we're issuing a new draft guidance to inform patients and product developers on these principles. This guidance is the first of four guidances we'll issue. This set of guidances describes how patient experience data and other relevant information from patients and caregivers can be collected and used for medical product development and regulatory decision-making.

The FDA recognizes the need to engage the wider stakeholder community and provide guidance on approaches to bridge early-stage efforts, such as the PFDD meetings we hosted, to more systematic, methodologically-sound approaches to collect patient input so that it becomes data that can further inform regulatory decision-making. The draft guidance issued today, Patient-Focused Drug Development: Collecting Comprehensive and Representative Input, addresses sampling methods for collecting representative information on patient experience to inform the development and evaluation of medical products throughout the medical product lifecycle.

This draft guidance also discusses methods on how to operationalize and standardize the collection, analysis and dissemination of patient experience data. It includes a glossary of terms that the FDA intends to use in one or more of the four draft guidance documents. It is an important step in describing how patient experience data can be collected and used for medical product development and regulatory decision-making.

Taken together, the four guidances are part of the FDA's PFDD efforts in accordance with the 21st Century Cures Act and The Food and Drug Administration Reauthorization Act of 2017 Title I. Through the PFDD initiative, started in the Prescription Drug User Fee Act V, the FDA has been addressing the need to better enable patients to provide meaningful input into drug and biologic development.

We'll continue to build on these efforts at the FDA. As the nature of drug development becomes more targeted and as more of the new treatments address specific aspects of disease, our approach to development and regulation must also become more patient focused. Through the input we receive from the patient community we can bridge this critical opportunity between the science and needs of patients. The new guidance document sets forth a framework for collecting this information. The goal is that the resulting treatments offer more of the benefits that matter most to patients.

The agency looks forward to receiving public comment on the draft guidance.

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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