FDA Takes Steps to Modernize, Improve and Promote Development of Targeted Therapies

This is part of the mission to modernize clinical trials and advance the development of safe and effective drugs.

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Statement by FDA Commissioner Scott Gottlieb, MD, on FDA's new steps to modernize drug development, improve efficiency and promote innovation of targeted therapies

The FDA continues to advance new policies, modernize our programs and advance opportunities for developing more targeted therapies. Using new technology platforms such as cell and gene therapies and small molecule drugs that target the genomic basis of disease, there are more opportunities to intervene in the underlying mechanisms that cause a disease, and potentially arrest and even reverse its progress. Our efforts are aimed at making sure that our regulatory framework is adapted to these challenges and opportunities, allows for the efficient development of these innovations and the robust demonstration of their safety and efficacy. Our comprehensive efforts are aimed at improving every stage of drug development. We're focused on making the process of generating pre-clinical and clinical evidence required for making risk-based regulatory decisions more modern, more scientifically rigorous, and more efficient.

The scientific opportunities we're seeing today demand that we make sure our policies are as sophisticated as the treatments that are being developed. As the nature of drug discovery and development has become more focused on basic mechanisms of disease, targeted at specific genetic or molecular dysfunctions, science is bringing forward more novel opportunities to meaningfully address human disease.

In response, we're developing technology- and disease-specific regulatory frameworks for innovations that may not have previously had a clear development pathway. We also want to ensure that the development processes are efficient enough to support multiple therapeutic options, not just first-in-class innovations. We need to facilitate second-and-third-to-market innovation as a way to promote more competition within drug classes. This competition can offer important therapeutic differentiation along with opportunities for price competition that can lower costs and broaden patient access.

Thus far this year, our Center for Drug Evaluation and Research has already approved 45-novel drugs and biologics, close to our total of 46 in 2017, which was the most approved in more than 20 years. This year we believe we are on pace to have another very strong year. Newly approved products treat a wide range of patients suffering from many different medical conditions—from rare disorders to common diseases. These new treatments are offering new hope for improved quality of life, and in some cases, improved chances of surviving life-threatening illnesses. Among them are, for example, three novel treatments for migraine; innovative treatments for rare diseases such as Dravet syndrome and Fabry disease; as well as the first-of-its-kind targeted RNA-based therapy to treat polyneuropathy caused by hereditary transthyretin-mediated amyloidosis. To continue this kind of novel innovation, we're focused on advancing new efforts to modernize the drug development process.

This impressive field of new therapies reflects the fact that more diseases are being redefined based on their molecular underpinning, creating more opportunities to intervene in more diseases and at different points in the disease process. As researchers unlock the molecular basis of disease, they are developing new and more highly effective targets for intervention. At the same time, these new treatments are, in more cases, showing a robust treatment effect—allowing scientists to more quickly bring these products through the development process and demonstrate efficacy and safety in more efficient trials. Today, we're announcing two new policy documents aimed at seizing these challenges and opportunities.

Two areas we're continuing to focus on are modernizing our approach to the design of clinical trials and making the overall drug development process more efficient and less costly, while maintaining regulatory standards. Using more sophisticated approaches to learn about the safety and efficacy of treatments, we can reduce the barriers to bringing new science forward and make sure more patients can benefit sooner from improved treatments.

One of the most promising ways to make drug development more efficient—while enabling providers and patients to get better information about how a new medicine works—is by developing the science around innovative approaches to the design of clinical trials. These are approaches that can show more quickly and efficiently how a treatment will impact a specific patient population. We're at a pivotal point in medicine, in which researchers are exploring how to tailor treatments that target unique characteristics of an individual's disease, such as the genetic profile of a person's tumor or a biomarker common in a specific disease state. We recently announced a new pilot program designed to encourage the use of complex innovative trial designs, particularly in areas with small patient populations or unmet need, and ensure greater collaboration when it comes to designing more novel and efficient clinical trials. And we've provided greater clarity in our thinking related to novel clinical trial designs, with two recent guidances, one on master protocols in oncology trials and one on the use of adaptive designs for clinical trials.

Today we're issuing two guidance documents to provide drug developers greater clarity and direction as they pursue the next generation of therapies and treatments for patients.

The first is the draft guidance, Hematologic Malignancies: Regulatory Considerations for Use of Minimal Residual Disease in Development of Drug and Biological Products for Treatment, which the FDA is developing to assist sponsors planning to use minimal residual disease (MRD) as a biomarker in clinical trials of drugs or biologics to treat specific blood cancers. MRD is a general measure of tumor burden. As a result of important workshops where we've heard from stakeholders, and an analysis of marketing applications showing inconsistent quality of MRD data, the FDA identified a need to provide sponsors with guidance on the use of MRD as a biomarker in regulatory submissions. MRD as a general measure of tumor burden has multiple potential regulatory and clinical uses as a biomarker to help with more informed drug development. Depending upon the clinical setting, MRD may reflect a patient's response to treatment or it may be used as a prognostic tool to assess the risk of future relapse. As such, it may be considered for use to enrich clinical trial populations, or to guide allocation into specific treatment arms in clinical trials and could be developed as a potential surrogate endpoint. With the advent of newer technologies, such as the recently authorized ClonoSEQ assay, a next generation sequencing-based test that allows for the detection of MRD at lower levels of disease, MRD assessments may be useful in clinical trials and have the potential to expedite product development.

Additionally, the agency today issued the final guidance, Developing Targeted Therapies in Low-Frequency Molecular Subsets of a Disease, which was issued as draft guidance in December 2017. This final guidance addresses the topic of finding treatments that address the underlying molecular changes (e.g., genetic variants) that often cause or contribute to diseases, including uncommon, or rare molecular changes that are present in a small subset of patients. By providing clarity on the regulatory and scientific frameworks for product developers, safe and effective targeted treatments can be identified with scientifically valid tests and ultimately, made available to patients more efficiently. The guidance provides medical product developers with greater clarity on the FDA's recommendations for researching and developing the next generation of individualized therapies. The guidance discusses an approach for drug developers to enroll patients based on the identification of rare variants into clinical trials for targeted therapies when reasonable scientific evidence suggests the drug could be effective in patients with these genomic findings. The guidance also discusses the evidence needed to demonstrate effectiveness for a variety of molecular subsets within a particular disease. This approach could lead to more consistent development and approval of targeted therapies for patients who are likely to benefit from them.

Understanding the molecular basis of many diseases has paved the way for the development of targeted therapies. For instance, the FDA has already approved a cancer therapy based upon whether the patient's tumor shows a specific biomarker, rather than based upon the tissue of origin of the tumor—a so-called "tissue agnostic" indication for the drug. We think innovative approaches have the potential to increase the likelihood of finding viable treatment options for those with less common mutations. This is especially true when paired with innovative clinical trial approaches, such as those using a master protocol, where the efficacy and safety of a number of drugs can be studied at the same time, each targeting a different genetic subset of a type of cancer.

Today’s guidance documents are part of the FDA’s science-based mission to modernize clinical trials and advance the development of safe and effective drugs and biologics for the American public.

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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