Examining Patient Access to Investigational Drugs

October 3, 2017 By Food and Drug Administration (FDA)

Statement of Scott Gottlieb, MD, Commissioner of Food And Drugs Before the Subcommittee on Health, Committee on Energy and Commerce, U.S. House of Representatives

INTRODUCTION

Mr. Chairman and Members of the Subcommittee, I am Dr. Scott Gottlieb, Commissioner of Food and Drugs at the Food and Drug Administration (FDA or the Agency), which is part of the Department of Health and Human Services (HHS). Thank you for the opportunity to testify today on efforts to expand access to investigational drugs and biologics for terminally ill patients.

Access to investigational products for patients facing serious or immediately life-threatening diseases is not an abstract issue to me. As a cancer survivor who used a commercially-available combination therapy in an off-label manner, I understand, on a very personal level, that patients who are fighting serious or life-threatening diseases want the flexibility to try new therapeutic approaches, including access to investigational medical products, particularly when there is no other FDA-approved treatment option.

As a physician and the Commissioner of Food and Drugs, I also take very seriously the responsibility entrusted to FDA by Congress to ensure the safety and efficacy of the medicines on which our Nation's patients and their doctors depend. FDA works closely with industry sponsors to promote rigorous clinical trials for testing investigational products and accelerating access to safe and effective new treatments. The Agency has also long recognized that investigational products may serve as the only lifeline to patients with serious or immediately life-threatening diseases or conditions, who are unable to participate in clinical trials – and has sought to assist these vulnerable patients and their caregivers. Therefore, I am pleased to be here today to talk about our work in this critical area and new steps that we are undertaking that I sincerely believe will better serve patients, physicians, and their caregivers in their fight against terminal diseases.

FDA's Expanded Access Program

FDA has a long history of supporting patient access to investigational new treatments. This includes working with drug companies through the clinical trial process that may lead to FDA approval of treatment, and through our expanded access program.

For over two decades, the Agency has had in place this expanded access pathway to help many patients gain access to investigational products. Since the late 1980s, FDA has facilitated access to investigational medical products for the treatment of certain seriously ill patients. In 2009, following amendments to the Federal Food, Drug, and Cosmetic Act (FD&C Act), FDA revised its regulations to consolidate and expand the various provisions regarding the use of investigational drugs and biological products for expanded access use.

In recent years, FDA has received over 1,000 applications annually for expanded access to treat patients with investigational drugs and biologics. FDA authorizes 99 percent of these requests. Emergency requests for individual patients are usually granted immediately over the phone and non-emergency requests are generally processed within a few days.

FDA believes that difficult decisions about individual treatment are best made by patients with the support and guidance of their treating physicians – and the expanded access program is predicated on this belief. To qualify for the program, the patient's treating physician has to determine that the probable risk to the person from the investigational drug is not greater than the probable risk from the patient's disease or condition. Once the physician makes this determination and together the patient and physician decide that it is appropriate to pursue this treatment option, the physician approaches the pharmaceutical company to obtain agreement from the sponsor/company that it will provide the drug being sought. If the company agrees, the physician then submits the request to FDA. Key protections are included for patients receiving experimental treatments through the expanded access program. These protections include specific labeling requirements, prohibitions on promoting or commercializing investigational drugs by sponsors and investigators, and limits on the costs charged to patients for investigational drugs.

While the Agency permits almost all expanded access applications to proceed, it makes meaningful changes in approximately 10 percent of these cases to enhance patient safety. For example, modifications may be made to adjust dosing amounts, increase safety monitoring, and bolster informed consent. The changes are based on the scientific and medical expertise of our staff, and informed by confidential information provided to FDA by product sponsors during the course of development. This information is often unavailable to the treating physician – and the larger medical community – and becomes available only after a drug is approved.

It is important to note that access to investigational products requires the active cooperation of the treating physician, industry, and FDA in order to be successful. The most common obstacle to access to the investigational product is the willingness or ability of companies to provide it. Neither FDA nor physicians or patients can compel a company to make a product available – and companies may decline requests for a variety of reasons. For example, they may have produced only a limited quantity of the product (companies ramp up manufacturing after marketing approval), have minimal resources to administer expanded access requests, or have concerns that granting requests for expanded access may exacerbate the challenge of recruiting clinical trial participants – and delay product development.

Once FDA permits treatment to proceed, the treating physician is responsible for obtaining informed consent from the patient and approval from an ethics committee, known as an Institutional Review Board (IRB), before administering the drug.

Recent Efforts to Streamline Expanded Access and Increase Transparency

Since its inception, FDA has continually worked to improve the expanded access program to better serve patients and physicians. Many of these changes have been made in response to feedback the Agency received from stakeholders. The Agency has a dedicated staff to assist physicians and patients to navigate the expanded access process and expedited telephone process for daytime and after-hours emergency requests. We are committed to helping patients and physicians.

Last year, FDA implemented significant changes to streamline the process for requesting expanded access for individual patients and promote greater transparency. We took a comprehensive and thorough look at the information requested by the Agency -- and slashed the number of required fields and attachments to streamline this process. It now takes about 45 minutes to complete a single patient application form and requires just one attachment (compared with up to eight attachments previously required). The new form is accompanied by step-by-step instructions on how to complete it.

We also released guidance that explains what expanded access is, when and how to submit a request, and what type of information should be included in requests. We clarified when and how patients may be charged for investigational drugs, notably that the sponsor may generally recover only its direct costs of making the drug available to the patient. Simultaneously, FDA revamped its expanded access website and produced Fact Sheets for physicians, patients, and industry.

FDA and the National Institutes of Health/National Library of Medicine (NIH), working with patient advocacy groups, industry and others, have taken a number of steps to provide more information about the availability of clinical trials and how to obtain investigational products through expanded access. A final HHS rule, administered by NIH and FDA, which became effective earlier this year, expands the legal requirements for submission of information to ClinicalTrials.gov about the availability of expanded access for drugs being studied in applicable drug clinical trials and how to obtain information about such access for persons who do not qualify to participate in the clinical trial. Clinical trial sponsors who manufacture the drug product and are required to submit information to ClinicalTrials.gov must indicate in the registration record whether expanded access is available. If available, those sponsors must submit specific information to ClinicalTrials.gov that enables patients and healthcare providers to obtain further information about access to the product. Information on the availability of expanded access must be updated when changes occur. FDA and NIH continue to provide information to assist clinical trial sponsors and investigators to help them understand their responsibilities under the final rule.

In July, we collaborated with the Reagan-Udall Foundation, patient advocacy groups, the pharmaceutical industry, and other federal agencies to launch a new online tool called the Expanded Access Navigator (Navigator). The Navigator was created to serve as a comprehensive

online resource for information on obtaining investigational therapies to treat patients with serious or life-threatening illnesses who lack therapeutic alternatives. It collects for the first time, in one centralized online location, links to drug manufacturers' expanded access policies, procedures, and points of contact. The Navigator also includes additional information to help guide patients and physicians through the steps to obtain such treatments.

The Navigator offers a valuable resource for information on available investigational therapies including clinical trials. The patient and caregiver section provides links to resources on clinical trial participation, their physician's role in helping them obtain an investigational drug, and contact information for FDA's Office of Health and Constituent Affairs' Expanded Access Team. Physicians can use the tool to identify investigational treatment options, learn about important factors to discuss with patients when considering expanded access, and get contact information for FDA's Division of Drug Information for assistance with their expanded access application.

Today, FDA announced additional improvements including changes required by the recently enacted FDA Reauthorization Act of 2017 (FDARA) and the 21st Century Cures Act. Current regulations require physicians to obtain approval for expanded access requests by a full IRB in order to treat a patient with an investigational drug. IRB review is an important step to protect the rights, safety, and well-being of human subjects in clinical research – but practical delays in convening the full review board can impede prompt decision-making and vital treatment. As required by FDARA and as part of a plan to simplify the process for single patient expanded access, we just announced FDA's plan to streamline IRB review for expanded access protocols for individual patients. At the request of the physician, just one IRB member – the chair or another appropriate person – can concur with the treatment use. I believe a simplified IRB process will facilitate access while still protecting patients.

The Agency also recognizes companies' concerns identified in the Government Accountability Office (GAO)'s recent report regarding expanded access-related adverse events data – and is taking action to address them. Patients receiving treatment through expanded access may have more advanced disease than clinical trial participants, have multiple diseases, and/or be receiving other drugs at the same time. These factors make it more difficult to be certain about the cause of an adverse event. FDA is updating its guidance entitled, "Expanded Access to Investigational Drugs for Treatment Use: Questions and Answers" today to explain the reason for FDA's review, to better explain our expectations with respect to adverse event reporting, and to clarify the context in which FDA reviews this information for industry.

I am confident these changes will help to address recent issues raised; however, FDA acknowledges that we can seek to continually evaluate our expanded access program for areas of improvement. We recently awarded a five-year Blanket Purchase Agreement (BPA) to a strategic consulting firm to assist FDA with modernizing regulatory science and advancing medical products development. An evaluation of the Expanded Access program will be the first Task Order awarded under this BPA. We welcome suggestions for additional improvements.

S. 204, Right to Try Act

FDA appreciates the strong desire on the part of patients and families facing life-threatening illnesses to access experimental treatments, and how right to try policies could be limited but important avenues for these patients in some situations. Supporting patients facing such difficult situations by helping to facilitate their access to investigational therapies is a high priority for the administration. FDA appreciates the opportunity to work with Congress on right to try legislation. Right to try policies are rooted in a strong desire to help patients facing desperate situations access experimental therapies with the hope that they will help them. As noted above, this administration has supported expanding access to experimental therapies — FDA has continued to refine our Expanded Access program, authorizing over 99% of all such requests. The administration looks forward to working with Congress to help patients and their families explore available treatment options in a responsible and ethical manner, including through right to try legislation.

HHS and FDA provided technical assistance to the Senate sponsors of S. 204, the "Trickett Wendler, Frank Mongiello, Jordan McLinn, and Matthew Bellina Right to Try Act of 2017," on earlier versions of the legislation passed by the Senate. We appreciate the changes included in the bill so far and look forward to continuing to work with Congress and this Committee to further refine this legislation as it advances.

Technical Suggestions

d

The President and Vice President generally support policies that would broaden access to unapproved products to patients facing terminal illness. In order to provide greater consistency with the intent of most supporters of such policies, we recommend narrowing the eligibility in S. 204 from patients who face a "life-threatening disease or condition" to "terminal illness." Many chronic conditions are life-threatening, but medical and behavioral interventions make them manageable without recourse to unapproved therapies.

In addition, the term used in the bill for "terminal illness" would benefit from a clear definition. We recommend defining it as "a stage of disease in which there is a reasonable likelihood that death will occur within a matter of months."

Aligning Accountability Measures

The FD&C Act and FDA's implementing regulations include several key protections for patients, such as provisions that ensure that unapproved products are not marketed to patients as if they are approved, and that companies are restricted in what they can charge for investigational products. These include requirements to label investigational products as such, restrictions on promoting and commercializing investigational drugs, and limits on the amount a patient may be charged. The exemption language included in S. 204 appears to bind only sponsors and investigators to these requirements, which are found in FDA's regulations at 21 CFR sections 312.6, 312.7, and 312.8. However, it does not appear to bind others covered by the bill, which

includes “any person who manufactures, distributes, prescribes, dispenses, introduces or delivers for introduction into interstate commerce, or provides to an eligible patient an eligible investigational drug,” to these requirements. We recommend revisions clarifying that the applicable regulatory requirements would, in fact, apply to all persons listed in the bill.

Similarly, we believe that the liability effect of S. 204 may be different from what Congress intended, and suggest adjustments. If enacted without changes, sponsors and other persons providing eligible investigational drugs to eligible patients under the specified conditions of the bill would not be subject to a number of sections of the FD&C Act and FDA regulations related to clinical trials, premarket approval, and labeling. The current language in S. 204 may therefore preclude FDA from taking enforcement actions based on those provisions. However, we believe that the Senate intended FDA to retain authority to address violations of other sections of the FD&C Act, for example, those pertaining to good manufacturing practices, intentional adulteration, and truthful and not misleading labeling, and suggest edits to clarify this. We also suggest language to clarify that the Agency would not be liable under the Federal Tort Claims Act (FTCA) for actions or omissions related to the provision of eligible investigational drugs to eligible patients, as we do not believe that the Senate intended to create a cause of action under the FTCA.

CONCLUSION

Patients are at the forefront of FDA’s mission to protect and promote the public health and we look forward to continuing to engage with Congress as the Agency fulfills its public health mission on behalf of patients.

The clinical trial process is crucial to the development of innovative new medical products that can improve or save patients’ lives. Adequate policies and processes must be in place to appropriately balance individual patients’ needs for access to investigational therapies while recognizing the importance of maintaining a rigorous clinical trial paradigm for testing investigational products to demonstrate safety and efficacy.

FDA is proud of its efforts to serve patients through its expanded access program who are unable to participate in trials and are in the difficult, heart-wrenching position of having no other therapeutic options. We look forward to making additional changes to enhance our efforts and working with you on these important issues.

I am happy to answer any questions you may have.

This statement was originally published on the Food and Drug Administration website on October 3, 2017.

© 2026 Smart + Strong All Rights Reserved.