New Efforts to Strengthen FDA's Expanded Access Program

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Statement from FDA Commissioner Scott Gottlieb, MD, on new efforts to strengthen FDA's expanded access program

Since the 1970s, the FDA has helped to facilitate access to promising investigational medical products for patients with serious or immediately life-threatening diseases who are unable to access products through clinical trials. As a cancer survivor, 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 investigational medical products. This is especially relevant when there's no other FDA-approved treatment option available to a patient.

FDA is deeply committed to facilitating this access, while also protecting patients and helping them to be able to make informed decisions with their physicians. We also take steps to help make sure that such access doesn't interfere or jeopardize investigational trials that could support a medical product's development or timely approval for the treatment indication.

This is the mission of our Expanded Access (EA) program. EA provides a pathway for patients to gain access to investigational drugs, biologics and medical devices for serious diseases and immediately life-threatening conditions outside of clinical trials when no comparable or satisfactory approved alternative therapy options are available. We're taking new steps to improve this framework.

Over the last five years, FDA has authorized more than 9,000 applications across drugs, biologics and devices through the agency's expanded access program. Furthermore, we've authorized approximately 99% of all the requests we have received, across all application types.

FDA staff is deeply committed to this program and ensuring that it works quickly and effectively for patients and their physicians. Emergency requests for individual patients are usually granted immediately by phone. Non-emergency requests are generally processed within a few days.

Despite the success of the EA program, we recognize that there are opportunities for improvement. We've taken steps to expand and update the program over the last year. Many of

these changes were made in response to feedback the agency received from stakeholders, as well as input from Congress, on how we can make the program more effective.

One improvement FDA made was streamlining the required supporting documentation for expanded access requests submitted by a physician for access to a drug or biological for the treatment of an individual patient. These changes reduced the administrative burden for these physicians. Following these changes, we estimate that it takes about 45 minutes, on average, to complete a patient application form. That form typically requires just one attachment.

We also simplified the process for Institutional Review Board (IRB) review. For single patient EA, we've modified the IRB review process to permit just one IRB member – the chair or another appropriate person – to concur with the treatment use rather than the entire Board.

We also clarified in guidance how we use safety data generated from using an investigational drug or biologic through the EA pathway, recognizing and addressing companies' concerns that EA-related adverse event data could be used in ways that complicate the review process.

As part of FDA's commitment to continuous operational improvement of the expanded access program, we announced last year that we commissioned an independent assessment of the program. The goals were to better understand the current EA program's performance and identify ways to improve it. It considered stakeholder perspectives from across the health care ecosystem, including patients and their advocates/caregivers, healthcare providers and the health systems that support health care providers, payers, IRBs, manufacturers, and FDA staff.

I'd like to highlight some of the key findings from this assessment, and how we are addressing the opportunities that we've identified as ways to strengthen the expanded access program.

The assessment found overall support for FDA's program, but as we hoped in pursuing this analysis, also identified new steps we can take to improve upon our efforts.

For example, the assessment found that external stakeholders' overall perceptions of FDA's expanded access program—and FDA's role in administering it—are very positive. Stakeholders across the health care system highlighted FDA's commitment to expediting the review of EA applications, the FDA's collaborative nature, and our focus on continuous improvement.

Physicians with direct experience submitting an EA application to the FDA reported positive impressions of the program. Similarly, patients and their advocates described the program as a crucial route to access investigational therapies when other alternatives have been exhausted.

Manufacturers, patient advocates, IRB representatives, and physicians all noted that FDA has taken key steps to reduce the administrative burden associated with submitting requests and recognized our commitment to facilitate medically appropriate access via the EA program.

Stakeholders also reported some challenges across the physician and patient journey through the program, that, if properly addressed, could meaningfully enhance the program. This feedback is crucial in our continued efforts to improve the program.

We're already acting on these findings.

For one thing, the assessment found that confusion with program navigation, multi-stakeholder coordination, and administrative burden were the most frequently-cited challenges. The assessment recommendations include improving FDA's public website content and investing in resources to support patient/physician program navigation. These are areas where we've already taken steps to improve the program, or where we are working on improvements.

For instance, based on the feedback from the assessment, today we announced that FDA's EA webpages will be updated to improve usability through streamlining of content and a more user-friendly organization. This includes a reduction in duplication, as well as the addition of new pages with commonly requested information, such as forms and keywords.

These updates will begin rolling out today and will continue in the future as we identify new opportunities to improve the usability of information on our EA webpages.

As part of our recently announced proposed reorganization of the Office of the Commissioner, we intend to formally establish an agency-wide Patient Affairs Staff and Health Care Provider Affairs Program, under the oversight of the Office of Clinical Policy and Programs. This will enhance our engagement with these important external stakeholder groups. The Patient Affairs Staff is already in place and charged with serving as the "home base" and primary point of entry for patients and physicians starting the EA process and navigating them through the steps.

Additionally, we've established an agency-wide Expanded Access Coordinating Committee, which facilitates cross-Center communication and promotes discussion to rapidly address cross-cutting issues related to expanded access to promote consistency and best practices.

FDA is deeply committed to our Expanded Access program and facilitating access to medical products outside of clinical trials when no alternative therapy options are available to patients. And we are deeply committed to continuing to enhance this program going forward.

It's important to note that while expanded access represents FDA's primary avenue for facilitating access for certain patients to unapproved, investigational treatments, the "Trickett Wendler, Frank Mongiello, Jordan McLinn, and Matthew Bellina Right to Try Act of 2017," recently signed into law by the President, represents a separate and distinct pathway. The FDA has established a work group to consider what steps may be required to implement this legislation in a way that advances Congress' intent to promote access and protect patients. Any work we undertake will build on our long-standing commitments to help patients facing life-threatening diseases or conditions access investigational medicines. As a first step, today we are launching a Right to Try webpage that will assist patients in understanding this alternative pathway.

We're dedicated to making sure that patients facing serious conditions have access promising investigational medicines. We will continue to take new steps to advance these goals.

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