An Overview of FDA’s Expanded Access Guidances

Summary

Listen as our expert, Jay Jackson, discusses recent final guidance issued by the FDA on compassionate use.

Transcript

Jay: Compassionate use, otherwise known as expanded access or preapproval access, is a mechanism through which patients can access investigational products outside of clinical trials. FDA’s program, formally called Expanded Access, oversees compassionate use of investigational products, and the agency approves over 99% of requests that it receives.

The finalized guidance issued by the FDA clarify and streamline the expanded access process through which physicians apply for access on their patients’ behalf. In particular, one final guidance formally establishes the streamlined Investigational New Drug (IND) form for expanded access, called Form 3926. The FDA estimates that the shortened Form 3926 should take only about 45 minutes to complete, as it only includes those sections required to request individual expanded access when an investigational product already has an IND. The shorter form is intended to be less burdensome and clarify which parts of the previous, longer form a physician needs to fill out. The Form 3926 guidance also gives some clarifying instructions and background for physicians, such as approval timelines, the concept behind the risk–benefit determination made for expanded access, and the need to obtain a letter of authorization from the drug’s sponsor to reference the full IND. The guidance also explains that by submitting the abbreviated IND form and receiving approval, the requesting physician becomes the sponsor-investigator.
with all the regulatory requirements associated with that designation, such as safety monitoring.

The FDA finalized two other sets of guidance regarding expanded access at the same time. The Questions & Answers guidance addresses common questions about expanded access. These include details on the three expanded access categories: individual patient, intermediate-size patient populations, and large groups or treatment use. The FDA also outlined the different types of expanded access regulatory submissions, noted when each submission should be used, and provided a table detailing which form should be used for each submission. The guidance also provides reasons why the FDA may deny an expanded access request and makes clear that FDA cannot compel sponsors to provide access to investigational products.

In the third and final guidance, the FDA addresses its methods for authorizing drug manufacturers to charge for investigational products. Sponsors must both demonstrate that charging will not interfere with drug development and provide cost recovery calculations meeting the same statutory standards as in clinical trials. The guidance explains that sponsors can generally only charge for direct costs, such as manufacturing, shipping, and handling. The guidance also notes that the FDA has no control over who may be charged for investigational drugs, whether it be the patient, insurer, or other entity.

Overall, while these final sets of guidance do not necessarily establish any surprising new policy from the FDA, they should be a welcome clarification for patients and physicians who want to gain access to investigational products.