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FDA Issues Guidance on Expanded Access

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The Agency improves the process for access to investigational drugs and biologics for patients who lack other options.

On June 2, the US Food and Drug Administration (FDA or the Agency) announced that it finalized three guidance documents intended to facilitate expanded access programs (EAPs). Through these guidance documents, FDA reaffirmed its commitment to assisting patients' access to potentially life-saving treatments and is attempting to reduce the time required for physicians to prepare expanded access requests.

Regulatory Requirements Unchanged

FDA has not changed the regulations on EAPs, which outline three program categories: individual patient, including emergency use; intermediate size; and larger scale treatment.[1] Rather, through these guidance documents, FDA has signaled the importance of facilitating expanded access for patients with serious or immediately life-threatening diseases or conditions who have no comparable or alternative therapy, as well as for such patients' physicians.[2] FDA recognized that the regulatory process can be burdensome, especially for individual patient use, and is attempting to clarify commonly asked questions and streamline the individual patient process.

Individual Patient Expanded Access Applications

FDA's first guidance document, *Individual Patient Expanded Access Applications: Form FDA 3926* (Individual Patient Guidance), provides "a streamlined alternative" for submitting an individual patient Investigational New Drug (IND) application and certain follow-up submissions, which is intended to shorten the application time.[3] The form calls for, among other items, information on the patient to be treated, the investigational drug and treatment plan, and the physician.[4]

Clarification of Existing EAP Requirements

The second and third guidance documents, *Expanded Access to Investigational Drugs for Treatment Use—Questions and Answers* (Treatment Use Guidance) and *Charging for Investigational Drugs*

Under an IND—Questions and Answers (Charging Guidance), clarify EAP requirements and processes.[5] The Treatment Use Guidance addresses questions regarding EAPs, the contexts in which each program is appropriate, submission requirements, and other general questions concerning FDA's approach.[6] The Charging Guidance addresses questions regarding authorization to charge for an investigational drug, FDA's review of such requests, charging requirements, when authorization is required, and permitted cost recoveries.[7]

Key Takeaways

Although FDA has aimed to facilitate EAPs through these guidance documents, its regulations pertaining to EAPs remain unchanged. Accordingly, there are still a number of requirements that EAP sponsors and investigators must meet. There are also a number of areas for EAP and full IND sponsors to consider.

- **Drug Supply and Letters of Authorization (LOAs)**—For EAPs sponsored by persons other than the full IND sponsor, the EAP sponsor will need to secure a supply of the drug and should obtain an LOA to reference the full IND.[8] If the EAP sponsor cannot obtain an LOA, the Agency should be contacted to determine the correct path forward.[9]

- **Regulatory Compliance**—For all EAPs, sponsors and investigators need to abide by FDA's clinical trial requirements to avoid potential enforcement action.[10] FDA has, however, expressed a willingness to work with individual patient program sponsors to find alternatives for potentially burdensome requirements, such as full institutional review board review.[11]

- **Cost Recovery**—An EAP's costs do not necessarily need to be prohibitive, but FDA authorization is needed to pass certain permitted costs to patients.[12] FDA authorization is not needed, however, to charge for certain costs incurred at the clinical trial site.[13]

- **Interaction with a Sponsor's IND**—EAP and full IND sponsors should keep in mind that EAPs may interact with INDs for product approval. Although rare, EAP results could influence FDA review of a marketing application.[14] For example, FDA may consider safety information collected during the course of an EAP.[15] Similarly, nothing prohibits the collection of efficacy data during an EAP.[16]

- **Study Design**—Finally, sponsors of IND studies should be mindful regarding the design of open-label safety studies because FDA will assess whether, based on the study design, such trials are actually open-label safety studies or EAP studies.[17] If deemed an EAP study, the trial would not support product approval.

Conclusion

Through these three final guidance documents, FDA has paved a clearer path for potential EAP sponsors. At the same time, however, sponsors and investigators should be mindful of their responsibilities and requirements, which have not changed, so that they may maintain regulatory compliance and ensure that patients are ethically treated.

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[1] 21 C.F.R. §§ 312.300-312.320.

[2] 21 C.F.R. § 312.305(a)(1).

[3] FDA Guidance for Industry, Individual Patient Expanded Access Applications: Form FDA 3926 at 1, 4-5, 7 (June 2016), http://www.fda.gov/downloads/Drugs/GuidanceComplianceRegulatoryInformation/Guidances/UCM432717.pdf.

[4] FDA Form 3926 Individual Patient Expanded Access Investigational New Drug Application (IND) (expir. Apr. 30, 2019) here.

[5] FDA Guidance for Industry, Expanded Access to Investigational Drugs for Treatment Use—Questions and Answers (June 2016),here. FDA Guidance for Industry, Charging for investigational Drugs Under an IND—Questions and Answers (June 2016) here.

[6] Treatment Use Guidance.

[7] Charging Guidance.

[8] Individual Patient Guidance at 5; Treatment Use Guidance at 5, 11.

[9] *Id.*

[10] 21 C.F.R. Part 312.

[11] Treatment Use Guidance at 6.

[12] Charging Guidance at 7–8.

[13] Charging Guidance at 8.

[14] Treatment Use Guidance at 18.

[15] *Id.*

[16] *Id.*

[17] *Id.*

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