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CLIENT ALERT

FDA SET TO SIMPLIFY THE COMPASSIONATE USE PROCESS

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On February 4, 2015, the U.S. Food and Drug Administration ("FDA") issued draft guidance titled *Individual Patient Expanded Access Applications: Form FDA 3926* (the "Draft Guidance"). The Draft Guidance provides a streamlined alternative procedure for submitting expanded access (or "compassionate use") requests to the FDA to use investigational new drugs outside of a clinical trial setting. Once finalized, the Draft Guidance is intended to simplify the investigational new drug application ("IND") process for physicians seeking to use investigational new drugs for an individual patient who has a serious or immediately life-threatening disease or condition and who has no viable treatment alternative.

The Draft Guidance states that the FDA may permit expanded access to investigational new drugs for individual patients who meet the following criteria, as set forth in 21 C.F.R. § 312.305(a) and 21 C.F.R. § 312.310(a):

- The patient to be treated has a serious or immediately life-threatening disease or condition, and there is no comparable or satisfactory alternative therapy to diagnose, monitor, or treat the disease or condition;
- The potential patient benefit justifies the potential risks of the treatment used and those potential risks are not unreasonable in the context of the disease or condition to be treated;
- Providing the investigational new drug for the requested use will not interfere with the initiation, conduct or completion of clinical investigations that could support marketing approval of the expanded access use or otherwise compromise the potential development of the expanded access use;
- The physician must determine that the probable risk to the patient from the investigational new drug is not greater than the probable risk from the disease or condition; and
- The FDA must determine the patient cannot obtain the investigational new drug under another IND or protocol.

The Draft Guidance also introduces Form FDA 3926 ("Form 3926"), which will assist physicians in the process of requesting expanded access for individual patients. The FDA expressed its concern "that physicians requesting expanded access for an individual patient may have encountered difficulty in completing Form FDA 1571 (currently used by sponsors for all types of IND submissions) and the associated documents, because it is not tailored to requests for individual patient expanded access." According to the FDA, the existing process can take physicians as long as 100 hours to complete and requires 26 separate types of information and 7 attachments; whereas it is estimated that the new Form 3926 will only take physicians 45 minutes to complete. *See* Peter Lurie, M.D, *A Big Step to Help the Patients Most in Need*, FDA Voice, Feb. 4, 2015, http://blogs.fda.gov/fdavoice/index.php/2015/02/a-big-step-to-help-the-patients-most-in-need/.

This new IND process will now require that a physician, prior to submission of the Form 3926, ensures that the drug manufacturer is willing to provide the investigational new drug and additionally obtains a letter of authorization from the drug manufacturer stating that it will supply the investigational new drug to the physician for the patient requesting compassionate use. Notably, when requesting expanded access for an individual patient, the physician is considered a sponsor-investigator. As such, the physician is responsible for complying with the regulatory obligations for both sponsors and investigators, including submitting investigational new drug safety reports and maintaining adequate drug disposition records. Additionally, the physician remains responsible for obtaining an Institutional Review Board's approval for the compassionate use protocol and the patient's informed consent before initiating treatment (even in the case of emergency use).

The Draft Guidance also provides for access to investigational new drugs in emergency situations when a physician does not have time to complete and submit a Form 3926. In such instances, the physician may direct requests to use the investigational new drug to the appropriate FDA review division (review divisions are organized generally by therapeutic class) by telephone or any other rapid means of communication. However, the physician would still be required to meet the above criteria to permit expanded access to use the investigational new drug and complete the Form 3926 within 15 working days of the FDA's emergency authorization of the expanded access use.

The goal of the Draft Guidance and new Form 3926 is to make the submission process easier on physicians requesting access for individual patients suffering from critical illnesses. Once finalized, it is anticipated that this new process will significantly reduce the amount of time and effort it will take physicians to request compassionate use for their patients and help streamline expanded access to investigational new drugs. The FDA has also unveiled a new website to further assist physicians and patients seeking access to compassionate use and investigational treatment options.

The Draft Guidance is not yet finalized and the FDA is accepting public comments, which should be submitted no later than April 13, 2015, to ensure consideration in the final guidance. Instructions for submitting comments can be viewed on the *Federal Register*'s website.

For more information on the Draft Guidance or related issues, please feel free to contact any member of the firm's Health Care Law practice group for a further discussion.

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