

New Rules for Investigational Drugs Effective October 13, 2009

The Food and Drug Administration (FDA) released two final rules regarding (i) patient access to investigational drugs for treatment purposes and (ii) a sponsor's ability to charge for investigational drugs. For purposes of this Alert we will refer to the first rule as the "Final Access Rule" and the second as the "Final Charging Rule". Both of these rules become effective October 13, 2009.

The Final Access Rule

The Final Access Rule changes the current law by clarifying the criteria for expanded access to investigational drugs, requirements for submissions to the FDA in order to request approval to engage such expanded access, reporting to safeguard patient safety and when such expanded treatment may begin.

The Final Access Rule allows individuals who meet certain criteria and intermediate-size patient populations with expanded access to investigational drugs. The Final Access Rule also describes allowable access to larger populations under a treatment protocol or as part of a clinical trial conducted pursuant to an IND application.

In all instances, the following criteria must exist to obtain FDA approval for expanded access:

- The patient or patients to be treated have 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 use and those potential risks are not unreasonable in the context of the disease or condition to be treated; and
- Providing the investigational 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 Final Charging Rule

As a general rule, a sponsor cannot charge participants in a clinical trial for drugs administered in that trial, unless the charge is first approved by the FDA. The Final Charging Rule describes the criteria that must be satisfied in order for a sponsor to charge patients for its own investigational or approved drug in a clinical trial conducted under an IND application or under an expanded access program approved by the FDA (as discussed above).

The Final Charging Rule amends the previous charging regulation to clarify the situations in which charging for a drug in a clinical trial is appropriate. The Final Charging Rule describes the criteria a drug manufacturer must demonstrate in order to charge patients for (i) its investigational or approved drug in a clinical trial and (ii) an investigational drug being made available for expanded access. Moreover, the Final Charging Rule clarifies what costs can be recovered by the sponsor when charging for an investigational drug. It should be noted that the sponsor does not have to obtain FDA approval prior to

charging a patient for an approved drug obtained from an unaffiliated third party manufacturer. Regular insurance reimbursement mechanisms would be utilized in this instance.

Requirements for a Sponsor to Charge Patients for its Own Drug (e.g. investigational or approved used in a clinical trial under an IND)

The FDA noted that the cost of an investigational drug used in a clinical trial is an anticipated cost of drug development and should ordinarily be borne by the sponsor. Therefore, the Final Charging Rule permits a sponsor to charge a study subject for an investigational drug in a clinical trial only if the sponsor:

- Provides evidence that the drug has a potential clinical benefit that, if demonstrated in the clinical trial(s), would provide a significant advantage over available products in the diagnosis, treatment, mitigation, or prevention of a disease or condition;
- Shows that the data to be obtained from the clinical trial would be necessary to obtaining initial approval of a drug, or would support a significant change in the labeling of an approved drug; and
- Demonstrates that the clinical trial could not be conducted without charging because the cost of the drug is extraordinary to the sponsor.

According to the regulations, a cost may be considered “extraordinary” due to manufacturing complexity, scarcity of natural resources, the large quantity of drugs needed or some combination of these or other extraordinary circumstances.

Charging for Expanded Access

As stated above, the FDA published the Final Charging Rule and the Final Access Rule concurrently. Generally, a sponsor who is conducting a clinical trial under an IND may not charge patients for expanded access to its investigational drug that is used for treatment unless it has first received FDA approval. The Final Charging Rule describes the circumstances under which a sponsor may charge for expanded access to an investigational drug used for treatment.

The Final Access Rule creates two new categories for expanded access: individual patients and intermediate- size patient populations. The Final Charging Rule allows sponsors to charge such individual patients and intermediate-size patient populations for investigational drugs used for treatment purposes. These two categories of expanded access were not in the previous charging rule. The Final Charging Rule provides the needed clarification to charge for investigational drugs in these categories.

The FDA’s primary concern is that charging for expanded access to investigational drugs used for treatment purposes may interfere with drug development. Therefore, the Final Charging Rule requires sponsors to provide reasonable assurances that charging will not interfere with developing the drug for marketing approval and sets forth specific information that sponsors must provide to meet this requirement.

Recoverable Costs

The Final Charging Rule describes what costs are recoverable when a sponsor charges for an investigational drug in a clinical trial and for expanded access for treatment use. Where the FDA permits a sponsor to charge for its investigational drug, the sponsor can only recover its direct costs (i.e., those that can be specifically and exclusively attributed to provision of the drug for investigational use). Indirect costs (e.g., research, and development, administrative and labor costs) cannot be recovered. In the case of an expanded access use for treatment the sponsor can recover costs of monitoring the expanded access IND or protocol and the cost of other compliance obligations directly associated with the expanded access IND.

Sponsors should re-evaluate their charging policies and procedures to ensure they incorporate the requirements of these new final rules.

If you have questions regarding this Alert, please contact [Linda A. Malek](mailto:lmalek@mosessinger.com), Chair of Moses & Singer's [Healthcare](#) practice, at 212.554.7814 or lmalek@mosessinger.com.

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