Expanded Access to Unapproved Drugs

Background

Expanded access, or "compassionate use," is the use of investigational medical product outside of a clinical trial.

Use of an investigational medical product by a patient within a clinical trial is preferred as they generate data that may lead to the approval of products and, consequently, to wider availability.

Expanded access allows for patient enrollment in a clinical trial in cases where it would not otherwise be possible:
- A patient is not eligible for any ongoing clinical trials.
- There are no ongoing clinical trials.
Background

21 CFR part 312 subpart I covers expanded access, including:

- General requirements for access
- Criteria that must be met to authorize expanded access
- Requirements for expanded access submissions
- Safeguards that will protect patients and preserve the ability to develop meaningful data about the use of the investigational product

Background

Under current FDA regulations for investigational drugs, there are three categories of expanded access:

- Expanded access for individual patients, including for emergency use
- Expanded access for intermediate-size patient populations
- Expanded access for widespread use

All forms of expanded access use protocols require IRB approval prior to implementation, unless the use meets emergency use criteria.
**Expanded Access Requirements for Individual Patient Access to Investigational Drugs**

The patient and his or her licensed physician must both be willing to participate.

The patient must:
- Have a serious or immediately life-threatening disease or condition;
- Have no alternative therapy to diagnose, monitor, or treat the disease or condition; and
- Be unable to obtain the investigational drug under another IND or to participate in a clinical trial.

**Expanded Access Categories for Drugs**

For each of the three expanded access categories, there are two types of regulatory submissions that can be used:
- a new investigational new drug application (IND)
- a protocol (treatment plan) submitted as a protocol amendment to an existing IND
**Individual Patient Expanded Access IND (Single patient IND)**

Access to an investigational drug for use by a single patient submitted as a protocol under a new IND

- The investigational product may or may not be under development.
- The protocol must be received by FDA and have IRB approval before treatment.
- Unless FDA notifies the sponsor earlier, there is a 30-day waiting period before drug treatment may begin.

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**Individual Patient Expanded Access Protocol (Single patient protocol)**

Access to an investigational drug for use by a single patient submitted as a new protocol to an existing IND by the sponsor of the existing IND

- Several patients may follow the same protocol.
- Investigational product may or may not be under development.
- No 30-day waiting period before treatment begins
- The protocol must be received by FDA and have IRB approval before treatment.
**Individual Patient Access in an Emergency (Emergency expanded access use and emergency access)**

Emergency IND or Individual Patient Access IND for Emergency use:
Access to an investigational drug by a single patient in an emergency situation before written submission to the FDA can be made - submitted as a protocol under a new IND

- Treatment is initially requested and authorized by telephone or other rapid means of electronic communication.
- Treatment may start immediately upon FDA authorization.
- A protocol must be sent to FDA within 15 business days of FDA approval.

**Individual Patient Access in an Emergency (also known as emergency expanded access use and emergency access)**

Individual Patient Expanded Access Protocol for Emergency Use:
Access to an investigational drug for use by a single patient in an emergency situation before a written submission can be made to the FDA - submitted as a new protocol to an existing IND by the sponsor of the existing IND.

- Treatment is initially requested and authorized by telephone or other rapid means of communication
- Treatment may start immediately upon FDA authorization.
- A protocol must be sent to FDA within 15 business days of FDA approval.

In an emergency situation where there is not sufficient time to obtain IRB review prior to beginning treatment, the emergency use of the investigational drug must be reported to the IRB within five working days, as required under 21 CFR 56.104(c).
Intermediate-size Patient Population
Expanded Access IND

Access to an investigational drug for use by more than one patient, but fewer than are treated under typical treatment IND or protocol, submitted as a protocol under a new IND

- The investigational product may or may not be under development for marketing.
- Unless FDA notifies the sponsor earlier, there is a 30-day waiting period before drug treatment may begin.

Intermediate-size Patient Population
Expanded Access Protocol

Access to an investigational drug for use by more than one patient, but fewer than are treated under typical treatment IND or protocol, submitted as a protocol to an existing IND by the sponsor of the existing IND

- Investigational product may or may not be under development for marketing.
- No 30-day waiting period before drug treatment begins
- The protocol must be received by FDA and have IRB approval before treatment may begin.
Expanded Access for Widespread Use
Treatment IND

Access to an investigational drug (including a biologic) for treatment use by a large (widespread) population, submitted as a protocol under a new IND

- The investigational product must be under active development for marketing.
- Unless FDA notifies the sponsor earlier, there is a 30-day waiting period before drug treatment may begin.

Expanded Access for Widespread Use
Treatment Protocol

Access to an investigational drug for treatment use by a large (widespread) population, submitted as a protocol to an existing IND by the sponsor of the existing IND

- The investigational product must be under development for marketing.
- Unless FDA notifies the sponsor earlier, there is a 30-day waiting period before drug treatment may begin.
Sources:
http://www.fda.gov/RegulatoryInformation/Guidances/ucm389154.htm

FDA Expanded Access - Compassionate Use
http://www.fda.gov/NewsEvents/PublicHealthFocus/ExpandedAccessCompassionateUse/ucm2008039.htm#Expanded_Access_Requirements

Questions?
Thank you