EXPANDED ACCESS USE OF AN INVESTIGATIONAL DRUG OR BIOLOGIC

disease or condition rather than to obtain data about the drug usually derived from clinical trials. This term is used broadly by the FDA. It can cover treatment use and emergency use. The terms expanded access, access, and treatment use are all used interchangeably.

Clinical Trial: A research project in which one or more human subjects are prospectively assigned to one or more interventions (which may include placebo or other control) to evaluate the effects of the interventions on biomedical or behavioral health-related outcomes.

Immediately Life-Threatening Disease: A stage of a disease in which there is a reasonable likelihood that death will occur within a matter of months or in which premature death is likely without early treatment.

Serious Disease or Condition: A disease or condition associated with morbidity that has substantial impact on day-to-day functioning. Short-lived and self-limiting morbidity will usually not be sufficient, but the morbidity need not be irreversible, provided it is persistent or recurrent. Whether a disease or condition is serious is a matter of clinical judgment, based on its impact on such factors as survival, day-to-day functioning, or the likelihood that the disease, if left untreated, will progress from a less severe condition to a more serious one

Individual Patient Expanded Access [21 CFR 312.310]: In addition to FDA required criteria above, the treating physician must determine that the probably risk to the person from the investigational drug is not greater than the probable risk from the disease or condition and must determine that the patient cannot obtain the drug under another IND or protocol.

Intermediate-size Patient Population Expanded Access [21 CFR 312.315]: In addition to FDA required criteria above, FDA must determine there is enough evidence that the drug is safe at the dose and duration proposed for expanded access use to justify a clinical trial of the drug in the approximate number of patients expected to receive the drug under expanded access and there is at least preliminary evidence of effectiveness of the drug, or a plausible pharmacological effect of the drug in the anticipated patient population.

There are a number of reasons an intermediate-size expanded access program may be needed: 1) A drug is not being developed because the disease or condition is so rare that the sponsor is unable to recruit patients for a clinical trial. 2) The drug is being studies in a clinical trial, but patients requesting the drug for expanded access are unable to participate in the trial (such as due to a different disease state, enrollment is closed, or geographical restrictions). 3) A drug is an approved drug that is no longer marketed for safety reasons.

Treatment IND or Treatment Protocol [21 CFR 312.320]: In addition to FDA criteria required above, FDA must determine: 1) The drug is being investigated in a clinical trial under an IND designed to support a marketing application for the expended access use or all clinical trials of the drug have been completed and 2) The sponsor is actively pursuing marketing approval of the drug for the expanded access use with due diligence

PROCEDURE:

1. The treating physician should consult with the IRB office regarding the proposed treatment.

2. The treating physician should contact the Sponsor to determine if the Sponsor is willing to provide the investigational drug for expanded access, outside the context of a clinical trial, and then ask the Sponsor to submit or file for a Treatment Use IND with the FDA.
    a. The treating physician may need to contact FDA for a new expanded access IND if the Sponsor declines to be the sponsor of the expanded access use (typically seen with single patient expanded access). In this case, the

4. Following the treatment use of an investigational drug/biologic the patient shall be monitored to detect any possible problems arising from the use of the investigational drug/biologic. If treatment is approved by FDA for a specific duration of treatment or chronic therapy, the treating physician must submit reports to FDA in accordance with 21 CFR 312.64, including safety reports and annual progress reports. At conclusion of the treatment, the treating physician must provide FDA with a written summary of the results of the expanded access, including adverse events.

5. MCW IRB requires follow-up reports to be submitted at the end of the treatment period or no later than 12 months after the initial approval was granted. The follow up must be submitted via eBridge CPR submission.

6. If any problems occur as a result of using the investigational drug/biologic these should be reported promptly to the IRB (via a Reportable Event), the Sponsor and/or FDA.

REFERENCES:
21 CFR 312 subpart I
FDA website and guidance documents

SUPPORTING DOCUMENTS:
IRB SOP: Submitting New Projects
IRB SOP: Continuing Progress Reports
IRB SOP: Emergency Use of Investigational Drugs or Biologics

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