



REPORTING REQUIREMENTS FOR SINGLE-PATIENT EA

If single-patient expanded access (EA) is permitted for a patient, the sponsoring physician is required to comply with FDA reporting requirements during and after the treatment. Clinical trial outcomes are primary criteria on which FDA bases its review of a new drug. While the primary intent of expanded access is to treat a patient and not to collect data on the investigational treatment, reporting for EA can contribute to broader knowledge about the safety and effectiveness of a treatment, particularly if the patient represents a population not included in the clinical trials.

Review the reporting requirements outlined in [21 CFR 312](#), including responsibilities for a physician treating a patient with an investigational treatment:

IND SAFETY REPORTS

Physicians must immediately report (within 7 or 15 days, depending on the reaction) to the pharmaceutical company and the FDA if any serious and unexpected adverse events occur during the treatment, whether or not the physician considers it to be drug-related. Review the FDA IND Safety Report [here](#). A serious adverse event is defined as an undesirable experience associated with the use of the treatment that results in one of the following outcomes:

- Death
- Life-threatening adverse event
- In-patient hospitalization or prolongation of existing hospitalization
- Persistent or significant incapacity or substantial disruption of the ability to conduct normal life functions
- Congenital anomaly or birth defect

Other adverse events also must be reported, but the reports need not be immediate and can be included in the final and/or annual report. Adverse events can include, but are not limited to, examples listed in the physician's treatment protocol or in the pharmaceutical company's investigational treatment brochure. In addition to reporting the adverse event, physicians must provide an assessment of whether there is a reasonable possibility that the investigational treatment caused the event.

Physicians are also required to report to the institutional review board (IRB) all unanticipated problems that could put the patient's safety or life at risk. In most cases, adverse events deemed necessary for an IND safety report also qualify as an unanticipated problem that must be reported to the IRB. Other unanticipated problems that do not qualify as adverse events, like informed consent or privacy issues, should also be reported to the IRB.

CHANGES

Any changes to the treatment plan or protocol must be communicated to the FDA. Submit [Form FDA 3926](#) with the amendment: check box 3.b. for Follow-up Submission, check "Change in Treatment Plan", "General Correspondence", or "Response to FDA Request for Information" in field 9, and complete fields 8-11.



ANNUAL REPORTS

For treatments that last longer than a year, the physician must submit annual reports to the FDA on the progress of the treatment. The physician also should submit all reports to the pharmaceutical company.

FINAL REPORT

A physician must provide the pharmaceutical company and the FDA with a written report shortly after the completion of the treatment. Final reports should include a summary of the results of the treatment, the patient's reaction, serious and non-serious adverse events, and any other information outlined in the IRB approval letter. Submit Form FDA 3926 with the written summary: check box 3.b. for Follow-up Submission, check "Summary of Expanded Access Use (treatment completed)" in field 9, and complete fields 8-11.

Additionally, physicians may be asked to provide the following information to the FDA or the drug developer upon request:

- Records of the disposition of an investigational treatment
- Case histories that record all observations and other data pertaining to the treatment and supporting documents like signed informed consent forms and medical records

QUESTIONS ABOUT REPORTING TO FDA

If you have questions, you may contact FDA's **Division of Drug Information** at 855-543-3784 or **druginfo@fda.hhs.gov** or the **FDA Office of Health and Constituent Affairs** at 301-796-8460, or **PatientNetwork@fda.hhs.gov**.