

## Guidance for the IRB Submission of an Expanded Access (Treatment) IND of an Unapproved Drug

The guidance below is for use of an unapproved drug to treat a patient or patients, outside of a clinical trial. The FDA must authorize any type of expanded access use in advance, even Emergency use for individuals, via an IND (Investigational New Drug).

The Emory IRB also must approve in advance any Expanded Access use, **except** for emergency use situations in which it is not possible to obtain prospective IRB review/IRB chair concurrence.

In addition, FDA may approve a waiver of full IRB review in response to a request for alternative IRB review when submitting [Form FDA 3926](#) to the FDA. If this process is approved, you will only need IRB chair concurrence before treatment begins. This could be possible for both, emergency and non-emergency single, expanded access uses. If the FDA does not approve the process or you do not request the full IRB review waiver during the [Form FDA 3926](#) submission, you will be required to submit a new study in eIRB. We could assist you with that process by giving you a link to a submission with pre-populated information.

For information about what would qualify as treatment use, follow this [link](#). For instructions on how to submit an Expanded Access use request to the FDA, please review the [IRB P&Ps](#), or this [FDA webpage](#). The FDA also has a Q&A at this [link](#) and specific guidance about requests using Form FDA 3926 at this [link](#).

**If you need assistance with the expanded access submission to the FDA, please contact the Office of Compliance at [compliance@emory.edu](mailto:compliance@emory.edu) or at (404) 727-2398.**

Please follow the relevant instructions below, based on the type of Expanded Access: whether the use is considered an emergency use or non-emergency “compassionate use”.

### **Emergency use of an unapproved drug**

The treatment use in an emergency situation does not need IRB approval. FDA needs to authorize the use before treatment occurs. The IRB should receive a report within 5 business days of the expanded access emergency use. This could be done via email to an [IRB Education and QA team member](#) or via the eIRB submission if one is required.

### **Submission process if alternative waiver of full IRB review was selected**

If you submitted [Form FDA 3926](#) to the FDA and selected the box under the Field 10.b (request for authorization to use alternative IRB review procedures), you will need to contact a member of the [IRB Education and QA team](#) to help you with obtaining IRB Chair concurrence. In order to secure IRB chair concurrence, please email us the following information:

- A copy of all information submitted (or that you need to submit) to the FDA in connection with the Expanded Access use request, including Form FDA 3926.
- Informed consent form used (or to be used) or information demonstrating qualification for Emergency Use exception from informed consent if consent was not secured before use. See the IRB P&P entitled: Waiver or Alteration of Informed consent for Research, subsection entitled Emergency Medical Care Exception –

Exception to the Requirement to Obtain Informed Consent for the Use of a FDA-Regulated Item in Emergency Medical Care Situations. Use the IRB [consent document for expanded access use](#) when creating the consent document.

- Documentation of FDA authorization for the Expanded Access Use request. This could be an email communication.

Our staff will email our IRB chair this information and let you know if there are any changes required before the IRB concurrence letter can be issued. The IRB staff will create a submission shell with this information for reference.

**If alternative waiver of full IRB review was not requested or not approved by FDA**

For assistance with the eIRB submission process, you may want to review this [instructional video](#) or contact the [IRB Education and QA team](#). We can provide you will a pre-populated submission template to use. You will find that several sections do not apply to your situation. Please fill in these fields with “N/A” and the IRB will alert you if additional information is required.

The eIRB submission should include the following:

In the “Informed Consent Process” section:

- A copy of the informed consent that was used, if applicable. Please use our template, which can be found [here](#).
- If consent was not obtained before use, please let us know why, and upload in the informed consent process section instead the informed consent form. The physician and a licensed physician who is not participating in the medical care protocol must certify in writing that:
  - The patient was under a life-threatening situation.
  - There was an inability to communicate with or obtain legally effective informed consent from the patient.
  - There was not sufficient time to obtain informed consent from the subject or legally authorized representative.
  - There was no available alternative method of FDA-approved or generally recognized therapy that provided an equal or greater likelihood of saving the patient’s life.

In the “Research Design” section:

A copy of all information submitted to the FDA in connection with the Expanded Access use request, under the protocol section, especially:

- Reason for intended treatment use of the Investigational New Drug.
- List of available therapeutic options that would usually be tried before using the Investigational New Drug OR an explanation of why the Investigational New Drug is preferable to other available therapies.
- Criteria for patient selection for Expanded Access if the use is for more than one individual OR a description of patient’s disease/condition, medical history and previous treatment for Expanded Access for an individual patient.
- Dose and method of administration for the Investigational New Drug and duration of therapy.
- Description of the clinical procedures, laboratory test or other monitoring necessary to evaluate the effects for the drug and minimize its risks.

In the “Drugs” section:

- Drug information under the “Drugs” section. This is triggered by selecting “Investigational drug or

- investigational use of an approved drug” in the “Biomedical Research” section of the smartform.
- Documentation of FDA authorization for the Expanded Access Use request.

**NOTE:** If you are not collecting information for research, the initial HIPAA related questions in the smartform may be answered “no,” allowing you to skip further HIPAA questions.

### **Non-Emergency Treatment or “Compassionate” use of an unapproved drug**

For non-emergency treatment/compassionate use, you are required to secure both IRB approval and FDA authorization before use.

#### **Submission process if waiver of full IRB review was selected**

If you submitted [Form FDA 3926](#) to the FDA and selected the box under the Field 10.b (request for authorization to use alternative IRB review procedures), you will need to contact a member of the [IRB Education and QA team](#) to help you with obtaining IRB Chair concurrence **before the use**. In order to secure IRB chair concurrence, please email us the following information:

- A copy of all information submitted (or that you need to submit) to the FDA in connection with the Expanded Access use request, including Form FDA 3926.
- A clean copy of the informed consent form on the required [template](#). Please, do not tamper with the header, but please do add a version date at the bottom of the form.
- Documentation of FDA authorization for the Expanded Access Use request

Our staff will email our IRB chair this information and let you know if there are any changes required before the IRB concurrence letter can be issued. The IRB staff will create a submission shell with this information for reference.

After the use, you are required to notify us within 5 days of use about the status of the patient. The FDA will need a similar report.

#### **If the alternative IRB review was not requested or FDA has requested IRB review**

You will need to create a new submission in eIRB. For assistance with the eIRB submission process,, you may want to review this [instructional video](#) or contact the [IRB Education and QA team](#) for assistance. You will find that several sections do not apply to your situation. Please fill in these fields with “N/A” and the IRB will alert you if additional information is required.

The eIRB submission should include the following:

##### In the “Informed Consent Process” section:

- A copy of the informed consent that was used, if applicable. Please use our template, which can be found [here](#).

##### In the “Research Design” section:

A copy of all information submitted to the FDA in connection with the Expanded Access use request, under the protocol section, especially:

- Reason for intended treatment use of the Investigational New Drug.

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- List of available therapeutic options that would usually be tried before using the Investigational New Drug OR an explanation of why the Investigational New Drug is preferable to other available therapies.
- Criteria for patient selection for Expanded Access if the use is for more than one individual OR a description of patient's disease/condition, medical history and previous treatment for Expanded Access for an individual patient.
- Dose and method of administration for the Investigational New Drug and duration of therapy.
- Description of the clinical procedures, laboratory test or other monitoring necessary to evaluate the effects for the drug and minimize its risks.

### In the "Drugs" section:

- Drug information under the "Drugs" section. This is triggered by selecting "Investigational drug or investigational use of an approved drug" in the "Biomedical Research" section of the smartform.
- Documentation of FDA authorization for the Expanded Access Use request.

**NOTE:** If you are not collecting information for research, the initial HIPAA related questions in the smartform may be answered "no," allowing you to skip further HIPAA questions.

### **REFERENCES**

- 21 CFR Parts 312
- Emory IRB policies and procedures
- FDA webpage: Expanded Access (Compassionate Use) at [https://www.fda.gov/NewsEvents/PublicHealthFocus/ExpandedAccessCompassionateUse/default.htm#Investigational\\_Drugs](https://www.fda.gov/NewsEvents/PublicHealthFocus/ExpandedAccessCompassionateUse/default.htm#Investigational_Drugs)
- FDA Guidance: Expanded Access to Investigational Drugs for Treatment Use — Questions and Answers at <https://www.fda.gov/downloads/drugs/guidances/ucm351261.pdf>