**Appli Application Form for Expanded Access Use – Intermediate-size Patient Population**

1. *Name of requesting physician (sponsor-investigator):*
2. *Identity of the investigational drug or REMS-restricted, approved drug for which expanded access use is being sought:*
3. *Describe, below, the patient population to be treated.*
4. *Address, below, the rationale for the intended use of the drug in this patient population, including a list of available therapeutic options that would ordinarily be tried before resorting to this drug or an explanation of why the use of this drug is preferable to the use of available therapeutic options.*
5. *Attach, to this application form, an Expanded Access Protocol that addresses each of the following:*

* *The criteria for patient selection/exclusion*
* *The method of administration of the drug, dose and duration of therapy*
* *A description of the clinical procedures, laboratory tests, or other monitoring necessary to evaluate the effects of the drug and minimize its risks*

1. *Identity of the facility where the drug will be manufactured:*
2. *Address, below, chemistry, manufacturing, and control information adequate to ensure that the drug meets appropriate standards of identity, strength, quality and purity. Alternately, incorporate the statement, “Refer to manufacturer information (see attached cross-reference letter)” and attach a letter from the drug manufacturer that permits the FDA to access the manufacturer’s IND or Drug Master File for this CMC information.*
3. *Provide, below, pharmacology and toxicology information adequate to conclude that the drug is reasonably safe at the dose and duration proposed for the expanded access use. Alternately, incorporate the statement “Refer to attached manufacturer information” and attach a copy of the current version of the manufacturer’s Investigator Brochure for the drug; and/or incorporate the statement “Refer to manufacturer information (see attached cross reference letter)” and attach a letter from the drug manufacturer that permits the FDA to access the manufacturer’s IND or NDA for this pharmacology and toxicology information.*
4. *Indicate, below, if the drug is being developed or is not being developed (i.e.,*

*whether or not the drug is currently being evaluated in a clinical trial submitted*

*under a separate IND application, or is a REMS-restricted, approved drug).*

* *If the drug is not being actively developed, explain why the drug cannot be*

*developed for the expanded access use and under what circumstances*

*the drug could be developed. (Refer to subscripts 1 and 2)*

* *If the drug is currently being developed and studied as part of a clinical*

*trial, explain why the patients to be treated under this expanded access*

*cannot be enrolled in the clinical trial and under what circumstances a*

*clinical trial for the expanded access use could be conducted in these*

*patients. (Refer to subscript 3)*

1E.g.; the drug is not being developed because the sponsor is an individual physician (i.e., sponsor-investigator), not a commercial pharmaceutical company; and/or the drug is not being developed because the expanded access disease or condition is so rare that it would be difficult to recruit subjects for a traditional clinical trial.

2E.g., the drug is an approved drug product that is no longer marketed for safety reasons or is unavailable through marketing due to failure to meet the conditions of the approved application; or the drug contains the same active moiety as an approved drug product that is unavailable through marketing due to failure to meet the conditions of the approved application or a drug shortage.

3E.g., the patients may not be able to participate in the clinical trial because they have a different disease or stage of disease than that being studied or otherwise do not meet the enrollment criteria; because enrollment into the clinical trial is closed; or because the clinical trial site(s) is (are) not geographically accessible.

1. *Additional Items to accompany the submission:*

* *Form FDA 1572,* [*http://www.IIS.pitt.edu/fda-forms*](http://www.IIS.pitt.edu/fda-forms)
* *CV of Investigator*
* *Informed Consent Document*

**Submission Requirements: – Expanded Use IND – Intermediate –size Patient Populations:**

1. General requirements:

**a. The Expanded Access IND submission to the FDA must include completed FDA Form 1571** [**http://www.IIS.pitt.edu/fda-forms**](http://www.IIS.pitt.edu/fda-forms)**. Please complete all applicable boxes.**

* Box 1: Incorporate the name of the requesting physician (i.e., sponsor-investigator)
* Box 3: Incorporate the address of IND and IDE Support (IIS) as the address of the sponsor-investigator; i.e.:

*Academic department of requesting physician*

University of Pittsburgh

Hieber Building, Suite 401

3500 Fifth Avenue

Pittsburgh, PA 15213

* Box 6B: Check “Research”
* Box 10: Serial 0000, for an Initial Application
* Box 11: Check the box corresponding to “Other” and specify
* “Expanded Access Use”
* Box 13: Check the box corresponding to “Intermediate Size Patient Population, 21 CFR 312.315
* Box 16 Specify the name of the requesting physician (i.e., sponsor-investigator)
* Box 17: Specify the name of the requesting physician (i.e., sponsor-investigator)
* Box 20: Enter IND and IDE Support (IIS) fax number (412-648-4010)
* Box 21: Incorporate the address of IND and IDE Support (IIS) as the address of the sponsor-investigator (see above)

**b. Expanded Access IND submissions and all subsequent related**

**correspondence must be submitted to the FDA through IND and IDE**

**Support (IIS).**

* + Provide the IIS with a single PDF of the Expanded Access IND submission.
  + The IIS shall promptly forward the Expanded Access IND submission and all subsequent correspondence to, as applicable, the FDA or the requesting physician (i.e., sponsor-investigator).

**c. The Expanded Access IND submission to the FDA should include a(n):**

* + Cover letter requesting review/approval of the expanded access use of the (identified) investigational drug or REMS-restricted, approved drug. The cover letter should be plainly marked “EXPANDED ACCESS SUBMISSION – INTERMEDIATE-SIZE PATIENT POPULATION”
  + The cover letter to the FDA should be addressed to:
* For investigational or REMS-restricted, approved biological drug products regulated by the Center for Biologics Evaluation and Research (CBER):

Food and Drug Administration

Center for Biologics Evaluation and Research

Document Control Center

10903 New Hampshire Avenue

Building 71, Room G112

Silver Spring, MD 20993-0002

**Attn.: Expanded Access Submission**

* For all other investigational or REMS-restricted, approved drugs:

Food and Drug Administration

Center for Drug Evaluation and Research

5901-B Ammendale Road

Beltsville, MD 20705-1266

**Attn.: Expanded Access Submission**