Expanded Access *"Compassionate Use"* Individual patient IND:

Written request to FDA for an individual patient use IND for an unapproved investigational drug

ICTR Navigators

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1.0 Table of Contents

2.0 Abbreviations

IND	Investigational New Drug
CFR	Code of Federal Regulations
FDA	U.S. Food and Drug Administration
CDER	Center for Drug Evaluation and Research
CBER	Center for Biologics Evaluation and Research

3.0 FDA Websites & Guidances

A list of several FDA websites and guidances containing useful information for investigators applying for a Compassionate Use IND is provided below:

FDA Forms website
http://www.fda.gov/aboutfda/reportsmanualsforms/forms/default.htm

Physician Request for an Individual Patient IND under Expanded Access for Non-emergency or Emergency Use
http://www.fda.gov/Drugs/DevelopmentApprovalProcess/HowDrugsareDevelopedandApproved/ApprovalApplications/InvestigationalNewDrugINDApplication/ucm107434.htm

FDA Access to Investigational Drugs Outside of a Clinical Trial (Expanded Access)
http://www.fda.gov/ForConsumers/ByAudience/ForPatientAdvocates/AccesstoInvestigationalDrugs/ucm176098.htm

FDA Title 21 Section 312.2 INVESTIGATIONAL NEW DRUG APPLICATION (Applicability)
http://www.accessdata.fda.gov/scripts/cdrh/cfdocs/cfCFR/CFRSearch.cfm?fr=312.2

FDA Title 21 Regulations Search Engine (e.g., IND regulations 21CRF312) website
http://www.accessdata.fda.gov/scripts/cdrh/cfdocs/cfCFR/CFRSearch.cfm

4.0 Introduction

The enclosed information is intended to provide a process overview and a template for a non-emergent request for compassionate use of an investigational drug (i.e., drugs not currently approved by the FDA for commercial marketing) for an individual patient.

This guidance is based on FDA regulations governing the emergency use of test articles. While the phrase "compassionate use" is commonly used to describe some of the ways of making unapproved products available to patients, there is no FDA regulation or policy defining a "compassionate use." FDA refers to compassionate use requests for individual patients as a "single patient IND" study, wider use would usually take place under a "treatment IND" or "treatment protocol" under an existing commercial IND. In contrast to treatment INDs and protocols, compassionate use single patient INDs do not require any determination by the FDA that the investigational drug is reasonably safe or effective.

4.1 Regulatory References Specific to this Guidance Document

Physician Request for an Individual Patient IND under Expanded Access for Non-emergency or Emergency Use

Subpart I—Expanded Access to Investigational Drugs for Treatment Use

Sec. 312.305 Requirements for all expanded access uses

The criteria, submission requirements, safeguards, and beginning treatment information set out in this section apply to all expanded access uses described in this subpart. Additional criteria, submission requirements, and safeguards that apply to specific types of expanded access are described in 312.310 through 312.320.

(a)Criteria

FDA must determine that:

(1) The patient or patients to be treated have a serious or immediately life-threatening disease or condition, and there is no comparable or satisfactory alternative therapy to diagnose, monitor, or treat the disease or condition;

(2) The potential patient benefit justifies the potential risks of the treatment use and those potential risks are not unreasonable in the context of the disease or condition to be treated; and

(3) Providing the investigational drug for the requested use will not interfere with the initiation, conduct, or completion of clinical investigations that could support marketing approval of the expanded access use or otherwise compromise the potential development of the expanded access use.

(b)Submission

(1) An expanded access submission is required for each type of expanded access described in this subpart. The submission may be a new IND or a protocol amendment to an existing IND. Information required for a submission may be supplied by referring to pertinent information contained in an existing IND if the sponsor of the existing IND grants a right of reference to the IND.

(2) The expanded access submission must include:

(i) A cover sheet (Form FDA 1571) meeting the requirements of 312.23(a);

(ii) The rationale for the intended use of the drug, including a list of available therapeutic options that would ordinarily be tried before resorting to the investigational drug or an explanation of why the use of the investigational drug is preferable to the use of available therapeutic options;

(iii) The criteria for patient selection or, for an individual patient, a description of the patient's disease or condition, including recent medical history and previous treatments of the disease or condition;

(iv) The method of administration of the drug, dose, and duration of therapy

(v) A description of the facility where the drug will be manufactured;

(vi) Chemistry, manufacturing, and controls information adequate to ensure the proper identification, quality, purity, and strength of the investigational drug;

(vii) Pharmacology and toxicology information adequate to conclude that the drug is reasonably safe at the dose and duration proposed for expanded access use (ordinarily, information that would be adequate to permit clinical testing of the drug in a population of the size expected to be treated); and

(viii) A description of clinical procedures, laboratory tests, or other monitoring necessary to evaluate the effects of the drug and minimize its risks.

(3) The expanded access submission and its mailing cover must be plainly marked "EXPANDED ACCESS SUBMISSION." If the expanded access submission is for a treatment IND/treatment protocol, the applicable box on Form 1571 must be checked.

(c)Safeguards

The responsibilities of sponsors and investigators set forth in subpart D of this part are applicable to expanded access use under this subpart as described in this paragraph.

(1) A licensed physician under whose immediate direction an investigational drug is administered or dispensed for an expanded access use under this subpart is considered an investigator , for purposes of this part, and must comply with the responsibilities for investigators set forth in subpart D of this part to the extent they are applicable to the expanded access use.

(2) An individual or entity that submits an expanded access IND or protocol under this subpart is considered a sponsor , for purposes of this part, and must comply with the responsibilities for sponsors set forth in subpart D of this part to the extent they are applicable to the expanded access use.

(3) A licensed physician under whose immediate direction an investigational drug is administered or dispensed, and who submits an IND for expanded access use under this subpart is considered a sponsor-investigator, for purposes of this part, and must comply with the responsibilities for sponsors and investigators set forth in subpart D of this part to the extent they are applicable to the expanded access use.

(4) Investigators

In all cases of expanded access, investigators are responsible for reporting adverse drug events to the sponsor, ensuring that the informed consent requirements of part 50 of this chapter are met, ensuring that IRB review of the expanded access use is obtained in a manner consistent with the requirements of part 56 of this chapter, and maintaining accurate case histories and drug disposition records and retaining records in a manner consistent with the requirements of 312.62. Depending on the type of expanded access, other investigator responsibilities under subpart D may also apply.

(5) Sponsors

In all cases of expanded access, sponsors are responsible for submitting IND safety reports and annual reports (when the IND or protocol continues for 1 year or longer) to FDA as required by 312.32 and 312.33, ensuring that licensed physicians are qualified to administer the investigational drug for the expanded access use, providing licensed physicians with the information needed to minimize the risk and maximize the potential benefits of the investigational drug (the investigator's brochure must be provided if one exists for the drug), maintaining an effective IND for the expanded access use, and maintaining adequate drug disposition records and retaining records in a manner consistent with the requirements of 312.57. Depending on the type of expanded access, other sponsor responsibilities under subpart D may also apply.

(d)Beginning treatment

(1)INDs

An expanded access IND goes into effect 30 days after FDA receives the IND or on earlier notification by FDA that the expanded access use may begin.

(2)Protocols

With the following exceptions, expanded access use under a protocol submitted under an existing IND may begin as described in 312.30(a).

(i) Expanded access use under the emergency procedures described in 312.310(d) may begin when the use is authorized by the FDA reviewing official.

(ii) Expanded access use under 312.320 may begin 30 days after FDA receives the protocol or upon earlier notification by FDA that use may begin.

(3)Clinical holds

FDA may place any expanded access IND on clinical hold as described in 312.42.

Subpart I—Expanded Access to Investigational Drugs for Treatment Use

Sec. 312.310 Individual patients, including for emergency use

Under this section, FDA may permit an investigational drug to be used for the treatment of an individual patient by a licensed physician.

(a)Criteria

The criteria in 312.305(a) must be met; and the following determinations must be made:

(1) The physician must determine that the probable risk to the person from the investigational drug is not greater than the probable risk from the disease or condition; and

(2) FDA must determine that the patient cannot obtain the drug under another IND or protocol.

(b)Submission

The expanded access submission must include information adequate to demonstrate that the criteria in 312.305(a) and paragraph (a) of this section have been met. The expanded access submission must meet the requirements of 312.305(b).

(1) If the drug is the subject of an existing IND, the expanded access submission may be made by the sponsor or by a licensed physician.

(2) A sponsor may satisfy the submission requirements by amending its existing IND to include a protocol for individual patient expanded access.

(3) A licensed physician may satisfy the submission requirements by obtaining from the sponsor permission for FDA to refer to any information in the IND that would be needed to support the expanded access request (right of reference) and by providing any other required information not contained in the IND (usually only the information specific to the individual patient).

(c)Safeguards

(1) Treatment is generally limited to a single course of therapy for a specified duration unless FDA expressly authorizes multiple courses or chronic therapy.

(2) At the conclusion of treatment, the licensed physician or sponsor must provide FDA with a written summary of the results of the expanded access use, including adverse effects.

(3) FDA may require sponsors to monitor an individual patient expanded access use if the use is for an extended duration.

(4) When a significant number of similar individual patient expanded access requests have been submitted, FDA may ask the sponsor to submit an IND or protocol for the use under 312.315 or 312.320.

(d)Emergency procedures

If there is an emergency that requires the patient to be treated before a written submission can be made, FDA may authorize the expanded access use to begin without a written submission. The FDA reviewing official may authorize the emergency use by telephone.

(1) Emergency expanded access use may be requested by telephone, facsimile, or other means of electronic communications. For investigational biological drug products regulated by the Center for Biologics Evaluation and Research, the request should be directed to the Office of Communication, Outreach and Development, Center for Biologics Evaluation and Research, 301-827-1800 or 1-800-835-4709, e-mail:ocod@fda.hhs.gov For all other investigational drugs, the request for authorization should be directed to the Division of Drug Information, Center for Drug Evaluation and Research, 301-796-3400, e-mail:druginfo@fda.hhs.gov After normal working hours (8 a.m. to 4:30 p.m.), the request should be directed to the FDA Emergency Call Center, 866-300-4374, e-mail:emergency.operations@fda.hhs.gov

(2) The licensed physician or sponsor must explain how the expanded access use will meet the requirements of 312.305 and 312.310 and must agree to submit an expanded access submission within 15 working days of FDA's authorization of the use.

5.0 Submission Binding and Labeling

All Expanded Access IND submissions must be in triplicate, one original and two copies.

Submissions received loose or otherwise inadequately bound may be returned to the sponsor-investigator for proper binding and resubmission, which can significantly delay the FDA review process. If a submission cannot be held together securely by a staple, approximately 15 pages, then it is highly recommended that the sponsor-investigator use the below recommendation.

If the Sponsor-investigator chooses to use a different type of binder than suggested, the binders should be labeled as suggested in the CDER and CBER binding recommendations. If another method is chosen, CDER and CBER request uniformity in the manner in which the information is bound.

5.1 FDA CDER Submission Binding Recommendations

General guidelines for binding a paper IND application being submitted to CDER are provided below. This information can be found at the FDA's website in a guidance document entitled, *"FDA IND, NDA, ANDA, or Drug Master File Binders*".

FDA CDER Binding Recommendations:

a. Original/Archive Copy: Red POLYETHYLENE BINDER (or ACCO Executive Red Stock Number 25079 or Smead Red Stock Number 81752 or similar type*)

i. Front (flat size) - 248 x 292mm (9-3/4 x 11-1/2")
ii. Back (flat size) - 248 x 305mm (9-3/4 x 12") (size includes 13 mm (1/2") lip at top)

1. Must be high impact linear plastic (matte finish or similar) - Must be able to withstand temperatures up to 150 degrees - Material should have a surface smooth enough to allow printing with a complete bonding of ink to the surface after a minimum of one hour drying time - Should be free from streaks, blisters, scratches and mottling . Binder weight MUST be .023-.025 gauge - Ink color MUST be BLACK

b. Duplicate/First Review Copy: Green PAPER BINDER (or ACCO Green Stock Number 25076 or similar type *)

i. Front (flat size) - 267 x 292mm (10-1/2 x 11-1/2")
ii. Back (flat size) - 267 x 305mm (10-1/2 x 12") (size includes 13mm (1/2") lip at top)

1. Binder MUST be of 11-point plate rope stock of extra heavy weight - Ink color MUST be BLACK

c. Duplicate/Second Review Copy: Orange PAPER BINDER (or ACCO Tangerine Stock Number 25977 or Smead Orange Stock Number 81652 or similar type or any color except Green or Red.*)

i. Same specifications as the Duplicate/First Review Copy

Identification of Binders:

a. Required on front folder in a clear, sharp, permanent-type print in BLACK ink – Permanent adhesive labels may be used in a clear, sharp print - Printing must withstand a "Scotch Tape Test" which consists of pressing a strip of "Scotch" tape firmly on the printed area and removing - There should be NO transfer of the printed area on the tape.

b. Binders are to be identified with the following label:

EXPANDED ACCESS IND SUBMISSION **INDIVIDUAL PATIENT**
IND. NO.: _____
SPONSOR NAME: _____
NAME OF DRUG: _____

NOTE: *Leave "IND NO." line blank for initial submission labels.*

Information about ACCO Folders:

Note: *Vendor information below is provided as an example of where these items may be purchased and is not intended as an endorsement of the vendor or a mandatory vendor to be used to purchase the folders.*

a. Red (ACCO USA, Executive Red, #25079) – used for archive (original copy)

i. Example Vendor
1. Office Depot:
http://www.officedepot.com/a/products/193664/Acco-Presstex-60percent-Recycled-Binder-Side/

b. Green (ACCO USA, Green, #25076) – used for 1st review copy

i. Example Vendor
1. Office Depot:
http://www.officedepot.com/a/products/193623/Acco-Presstex-60percent-Recycled-Binder-Side/

c. Light Blue (ACCO USA, #25072) – used for 2nd review copy **NOTE:** The FDA requests "Tangerine or Orange" binders, but these can be very difficult to find. In the Navigators' experience, the FDA will accept these light blue binders in place of the tangerine/orange requested.

i. Example Vendor
   1. Office Depot: http://www.officedepot.com/a/products/102905/Acco-Presstex-60percent-Recycled-Binder-Side/

FDA-CDER Mailing Address:

Food and Drug Administration
Center for Drug Evaluation and Research
Central Document Room
5901-B Ammendale Rd.
Beltsville, MD 20705-1266
Attn.: Expanded Access IND Submission

5.2 FDA CBER Submission Binding Recommendations:

As there are no specific regulations, guidance documents, or requirements provided by the **FDA CBER** for binding of IND related paper regulatory submissions to CDER, but they do request uniformity in the manner in which the information is bound. The following binding guidance information presented is from CBER and may be found at the FDA's website in a document entitled ***"SOPP 8007: DCC Binding Procedures for Regulatory Documents"***.

Suggested Binders:

1. FDA Binding Recommendations:

   a. Archive - ACCO Gray Stock Number 25974 or Smead Stock Number 81552 or Oxford Stock Number ESS-129005 or similar type

   b. Duplicate (or First Review Copy) - ACCO Executive Red Stock Number 25079 or Smead Red Stock Number 81752 or similar type

   c. Second and additional review copies - Any color pressboard report binder except Gray or Red.

Identification of Binders:

1. Binders should be identified with name of sponsor, name of product, IND number (after assignment), and date of submission.

2. Suggested Label Format:

EXPANDED ACCESS IND SUBMISSION INDIVIDUAL PATIENT
IND. NO.: _____
SPONSOR NAME: _____
NAME OF DRUG: _____

NOTE: *Leave "IND NO." line blank for initial submission labels*

Information about ACCO Folders:

Note: The vendor information listed below is provided as an **example** of where these items may be purchased and is not intended as an endorsement of the vendor or a mandatory vendor to be used for purchase.

1. Archive (original copy)- Gray (ACCO Gray Stock Number 25974 or Smead Stock Number 81552 or Oxford Stock Number ESS-129005 or similar type)
    a. Example Vendor
        1. GovGroup.com: http://www.govgroup.com/pressguard-binder-cover-smd81552-2183502-prd1.htm
2. Duplicate (or First Review Copy) – Red (ACCO Executive Red Stock Number 25079 or Smead Red Stock Number 81752 or similar type)
    a. Example Vendor
        1. Office Depot: http://www.officedepot.com/a/products/934380/ACCO-60percent-Recycled-Pressboard-Binder-With/
3. Second and additional review copies - Any color pressboard report binder except Gray or Red.
    a. Example Vendor
        1. Office Depot: http://www.officedepot.com/a/products/193664/ACCO-Presstex-60percent-Recycled-Binder-Side/

FDA-CBER Mailing Address:

Food and Drug Administration
Center for Biologics Evaluation and Research
HFM-99, Room 200N
1401 Rockville Pike
Rockville, MD 20852-1448
Attn.: Expanded Access IND Submission

6.0 Header and Footer

The following is suggested format of document headers and footers to be used with this submission. Note: Headers and footers are not included in the template and must be inserted manually and should be modified as appropriate.

Header:

[Left Hand Side]	*[Right Hand Side]*
Application Date: *[INSERT DATE]*	IND Number: *pending*
Expanded Access Use-Individual Pt.	Serial Number: *[1571 Serial #]*

Footer:

[Left Hand Side]	*[Right Hand Side]*
John Hopkins University	Page *[##]*
[INSERT: Sponsor-Investigator Name]	
Confidential and Proprietary	

7.0 Website Address Hyperlinks and Additional Information

All hyperlinks to websites included in this document are operational as of the date of this version. If any non-functional hyperlinks are identified in this document, please contact the ICTR Research Navigators via the contact information below so that the links may be updated.

For questions regarding any of the information presented or use of the template, please contact the ICTR Research Navigators at **ICTR_Navigators@jhmi.edu** or via telephone at 410-614-5383.

8.0 Expanded Access IND Guidance and Template

See next page.

[INSERT: Sponsor-Investigator letterhead or address]
[INSERT: DATE]

Food and Drug Administration
Center for Drug Evaluation and Research
Central Document Room
5901-B Ammendale Rd.
Beltsville, MD 20705-1266

OR

Food and Drug Administration
Center for Biologics Evaluation and Research
HFM-99, Room 200N
1401 Rockville Pike
Rockville, MD 20852-1448

RE: EXPANDED ACCESS IND-INDIVIDUAL PATIENT

Dear ***[INSERT: 'FDA Reviewers' OR other appropriate salutation]***:

The purpose of this submission is to request review and approval of the expanded access use of ***[INSERT: name of investigational agent]*** investigational drug for an individual patient who meets the criteria set forth in 21 CFR 312.305 and 21 CFR 312.310. All required materials are enclosed for your review.

Thank you in advance for your attention and consideration of this request.

Sincerely,

[INSERT: Sponsor-Investigator Name]
[INSERT: Title]
[INSERT: Affiliation]

Attachments:
FDA form 1571
Expanded Access Use Individual Patient IND

Cover Page

EXPANDED ACCESS IND-INDIVIDUAL PATIENT

DATE

Investigational Drug Product:	*Name*
IND Serial Number:	*Use 0000 for initial submission*
Sponsor-Investigator:	*Name* *Full Contact Information*

1.0 Form FDA 1571

***[Regulatory Reference: 21CFR312.23 (a) (1)** - Cover sheet (Form FDA-1571)*

An FDA Form 1571 is a required cover sheet for the IND application containing the following:

(i) The name, address, and telephone number of the sponsor, the date of the application, and the name of the investigational new drug.

(ii) Identification of the phase or phases of the clinical investigation to be conducted.

(iii) A commitment not to begin clinical investigations until an IND covering the investigations is in effect.

(iv) A commitment that an Institutional Review Board (IRB) that complies with the requirements set forth in part 56 will be responsible for the initial and continuing review and approval of each of the studies in the proposed clinical investigation and that the investigator will report to the IRB proposed changes in the research activity in accordance with the requirements of part 56.

(v) A commitment to conduct the investigation in accordance with all other applicable regulatory requirements.

(vi) The name and title of the person responsible for monitoring the conduct and progress of the clinical investigations.

(vii) The name(s) and title(s) of the person(s) responsible under 312.32 for review and evaluation of information relevant to the safety of the drug.

(viii) If a sponsor [Sponsor-investigator] has transferred any obligations for the conduct of any clinical study to a contract research organization a statement containing the name and address of the contract research organization, identification of the clinical study, and a listing of the obligations transferred. If all obligations governing the conduct of the study have been transferred, a general statement of this transfer--in lieu of a listing of the specific obligations transferred--may be submitted.

(ix) The signature of the sponsor [Sponsor-investigator] or the sponsor's [Sponsor-investigator's] authorized representative. If the person signing the application does not reside or have a place of business within the United States, the IND is required to contain the name and address of, and be countersigned by, an attorney, agent, or other authorized official who resides or maintains a place of business within the United States.

WEB ADDRESS TO FDA FORMS AND FORM INSTRUCTIONS

Fillable form FDA 1571:
http://www.fda.gov/downloads/AboutFDA/ReportsManualsForms/Forms/UCM083533.pdf

Full instructions for form FDA 1571 completion::
http://www.fda.gov/Drugs/DevelopmentApprovalProcess/HowDrugsareDevelopedandApproved/ApprovalApplications/InvestigationalNewDrugINDApplication/ucm071098.htm#form1571]

Special Instructions for Expanded Access Individual Patient IND submissions:

Box 1	Name of Sponsor-Investigator
Box 2-5:	Self-explanatory
Box 6	Name of investigational drug
Box 7	Specify “Expanded Access Use” followed by a brief description of the corresponding disease or condition that will be diagnosed, monitored or treated using the investigational drug
Box 8	Indicate “Other” and specify “Expanded Access Use”
Box 9	Include number of the IND for which a Right of Reference has been obtained for this submission
Box 10	As this is an initial IND, indicate “0000” in serial number box
Box 11	If this is a new IND application, check “Initial Investigational New Drug (IND) Application. If this is instead an expanded access protocol to an existing IND, check ‘New Protocol’ box. Finally, check the box corresponding to “Other” and specify “Expanded Access Use”.
Box 12	Check the box corresponding to “Form FDA 1571”
Box 13-15	Self-explanatory
Box 16-17	If the sponsor-investigator intends to designate an authorized representative with whom the FDA can communicate regarding the study, include name here. The name of the sponsor's authorizing representative would be entered and that individual must sign the form.
Box 18-19	These need not be provided if they duplicate Boxes 3 & 4
Box 20	The date here is the date the form is signed by the sponsor-investigator

2.0 Table of Contents

3.0 Sponsor-Investigator Identification

*[**INSTRUCTIONS**: Provide name, title, address, and contact information including telephone number and facsimile number of sponsor-investigator (requesting physician) Consider also designating here an authorized representative with whom the FDA can communicate regarding the study. The designated person must understand their role. (i.e. they may not respond to clinical questions.) Designation of a representative can save a great deal of time when the FDA is contacting the sponsor-investigator about administrative requirements that the investigator's authorized representative (e.g. regulatory specialist) can immediately address. If an authorized representative is designated here, be sure to include their information on the Form FDA 1571 Boxes 16-17 as well.]*

4.0 Brief Clinical History

*[**INSTRUCTIONS**: Provide a brief clinical history of the patient for which the drug is being requested. Include the diagnosis or condition of the patient, disease status, prior therapy, response to prior therapy, and recent medical history.]*

5.0 Investigational Drug and Rationale for Expanded Access Use request

*[**INSTRUCTIONS:** Provide FDA reviewers the identity and manufacturer of the investigational drug being requested for use under expanded access as well as the rationale for the proposed treatment. Include a list of available therapeutic options that would ordinarily be tried before the investigational drug or an explanation of why use of the investigational drug is preferable to use of available therapeutic options.]*

:

6.0 Proposed Treatment Plan

[INSTRUCTIONS: *Provide the planned route of administration of the investigational drug, dose, and duration of therapy. Include here description of the clinical procedures, laboratory tests, or other monitoring necessary to evaluate the effects of the drug and minimize its risks. Also discuss any planned modifications in the event of toxicity (e.g. dose reduction or treatment delays.) Reference published protocols or journal articles if appropriate.]*

7.0 Chemistry, Manufacturing, and Controls

*[**INSTRUCTIONS:** Provide here available chemistry, manufacturing, and controls information including description of the manufacturing facility. This information should ensure that the drug meets appropriate standards of identity, strength, quality and purity.*

The requirement for this information may be met by providing a Letter of Authorization (LOA), also called a Right of Reference, to refer to this information if it has been previously submitted to FDA (for example, to an existing IND or NDA). The treating physician should contact the sponsor of the previously submitted information for such authorization which permits the FDA to access the manufacturer's IND or Drug Master File for this CMC information. The letter of authorization should include relevant identifying information, such as the sponsor's relevant application (e.g., IND) number. If appropriate, incorporate the statement, "Refer to manufacturer information (see attached Letter of Authorization/Right of Reference)" and include letter in submission.]

8.0 Pharmacology and Toxicology Information

*[**INSTRUCTIONS:** Provide pharmacology and toxicology information adequate to conclude that the drug is reasonably safe at the dose and duration proposed for the expanded access use. You may instead incorporate the statement "Refer to manufacturer information (see attached Letter of Authorization)" 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.]*

9.0 Informed Consent Statement

*[**INSTRUCTIONS:** Provide here a statement that informed consent and approval of the use by an appropriate Institutional Review Board (IRB) will be obtained prior to initiating treatment.]*

10.0 Investigator Qualifications

*[**INSTRUCTIONS:** Provide here a statement that specifies the training, experience, and licensure of the treating physician. The first two pages of a Curriculum Vitae (CV) typically contain this information and are usually sufficient.]*

11.0 References

*[**INSTRUCTIONS:** Provide FDA reviewers a list of any references cited within the application. Note: The bibliographical information may instead be provided after each of the application sections. If the Sponsor-Investigator chooses to do this, delete this section header both here and from the Table of Contents.]*

12.0 Appendices

*[**INSTRUCTIONS:** Provide a list of appendices in the order in which they are referenced in the application. Before each new appendix, include a cover page describing the document(s) that will be found in within.]*

*[**COMMENT:** While not required, the Sponsor-Investigator can provide copies of any key papers referenced within the body of the application. This will make it easier for the FDA reviewers to access them should they want more information. As with the other appendices, include a cover page listing, in order of appearance, the citations for the papers being provided with the application.]*