

PURPOSE

This form is meant to guide treating physicians planning **non-emergency (compassionate) use of an unapproved device of a single patient.**

USE

This form is for use by treating physicians to-

- (1) Determine whether their plan to use an unapproved device under the U.S. Food and Drug Administration (FDA) Expanded Access Program (EAP) for **non-emergency** clinical treatment of a single patient (i.e., **compassionate use**) meets the FDA requirements for expanded access.
- (2) Assure compliance with FDA requirements and UAMS Institutional Review Board (IRB) policies.
- (3) Guide them through the procedures required by the FDA and -UAMS IRB.

NOTES

- EAPs involve the use of an investigational device to diagnose, monitor or treat a patient's disease or condition, **not to obtain information about the safety or effectiveness of the device.**
- Outcome information for single patient use is **not considered to be research data** and may not be used, presented, or published as research.
- Speak with someone in UAMS or Arkansas Children's (AC) research network, or your department/division, **as soon as possible** when planning use through an EAP. See **Step 3.**
- UAMS and AC may have differing requirements regarding the use of an investigational product.

INSTRUCTIONS

- Treating physician must ensure that all the qualifying criteria (see **Step 1**) are met prior to treatment.
- Treating physician must follow **Steps 1-7** outlined below.
- A full IRB submission (see **Step 7**) is required prior to use of the device, including IRB approval of the treatment plan and consent. All required attachments (see **Step 2**) must be submitted to UAMS IRB on a new submission form through [CLARA](#).
- Principal Investigator (i.e., treating physician) will serve as "Sponsor-Investigator" of **non-emergency** single patient expanded access uses by default; however, UAMS Office of Research Regulatory Affairs (ORRA) may serve as Sponsor in certain circumstances. If ORRA will serve as Sponsor, all required attachments (see **Step 2**) must be submitted to [ORRA](#) via email: ORRARegulatoryUnit@uams.edu.
- Many of the steps below can be done concurrently.

STEP 1 – EVALUATE WHETHER USE MEETS FDA CRITERIA FOR SINGLE PATIENT NON-EMERGENCY EXPANDED ACCESS USE

ALL the criteria listed below must be met (Final determination by treating physician [1-3]):

1. **The patient has a life-threatening or serious disease or condition;**
 - *Life-Threatening*: FDA considers “life-threatening condition” to include serious diseases or conditions such as sight-threatening and limb-threatening conditions as well as other situations involving risk of irreversible morbidity. ([Frequently Asked Questions About Medical Devices](#))
 - *Serious disease or condition*: Serious disease or condition means 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. ([Expanded Access Keywords, Definitions, and Resources](#))
2. **There is no comparable or satisfactory alternative therapy to diagnose, monitor, or treat the disease or condition;**
3. **Potential patient benefit justifies the potential risks of the investigational device; and**
4. **Investigational device is not being studied in a clinical investigation OR investigational device is being studied in a clinical investigation, however, patient does not meet requirements for inclusion but treating physician believes the device may provide a benefit in treating or diagnosing the patient’s disease or condition.**

STEP 2 - OBTAIN AUTHORIZATION FROM MANUFACTURER AND FDA

FDA cannot require a manufacturer to provide an investigational device for compassionate use. However, manufacturers are required to have expanded access policies in place and must post these policies in a publicly accessible location. **Treating physicians should check the manufacturer’s website for information, which may include details on how to submit a request.**

If the device manufacturer agrees to provide the device under compassionate use, there are two different processes to follow to obtain FDA approval, depending on whether there is an IDE for that device.

INSTRUCTIONS FOR FDA SUBMISSION IF THERE IS AN IDE FOR THE DEVICE

Neither the local treating physician nor UAMS/ORRA will serve as Sponsor; however, ORRA will be available for guidance.

The IDE sponsor who submitted the existing IDE to conduct the clinical study for the device should submit an [IDE supplement](#) requesting approval for a compassionate use to treat the patient.

When there is an IDE for the device, compassionate use request IDE supplements have the same statutory 30-day review cycle as other IDE submissions. However, the patient need is considered when reviewing these requests.

INSTRUCTIONS FOR FDA SUBMISSION IF THERE IS NO IDE FOR THE DEVICE

In cases where there is no active IDE, a compassionate use request for a single patient may be submitted by the physician or manufacturer.

Submission should include the following:

1. A description of the patient's condition and the circumstances necessitating treatment;
2. A discussion of why alternative therapies are unsatisfactory;
3. A discussion of why the probable risk of using the investigational device is no greater than the probable risk from the disease or condition;
4. An identification of any deviations in the approved clinical protocol or clinical treatment plan that may be needed to treat the patient;
5. The patient protection measures that will be followed:
 - a. A draft of the informed consent document that will be used;
 - b. Clearance from the institution as specified by their policies;
 - c. Concurrence of the IRB chairperson;
 - d. An independent assessment from an uninvolved physician; **and**
 - e. Authorization from the device manufacturer on the use of the device.
6. Description of the device provided by the manufacturer

These materials will also be reviewed by IRB (see [Step 7](#)).

For single patient non-emergency expanded access use at UAMS and AC, the treating physician may work with packet and address questions regarding submission process.

Submit to the following address:

Food and Drug Administration
Center for Devices and Radiological Health
10903 New Hampshire Ave
Document Control Center
WO66 Rm G-609
Silver Spring, MD 20993

Compassionate use request may also be submitted online through the [CDRH Customer Collaboration Portal](#).

Physicians and manufacturers can contact CDRHExpandedAccess@fda.hhs.gov for assistance.

The physician should not treat the patient identified in the request until the FDA approves use of the device under the proposed circumstances. In reviewing this type of request, the FDA will consider the above information as well as whether the preliminary evidence of safety and effectiveness justifies such use and whether such use would interfere with the conduct of a clinical trial to support marketing approval.

After a compassionate use request is received, the FDA will either approve, approve with conditions, or disapprove the request. The FDA typically reviews compassionate use requests within 15 days on average, and in as little as one day in some cases.

STEP 3 – COORDINATE WITH APPROPRIATE PERSONNEL

Contact the applicable staff where device will be used/dispensed (UAMS, AC) **as soon as possible** to inform them of the planned shipment and use of the device. **Do not wait until you have obtained authorization from the FDA and/or the manufacturer.**

Provide the appropriate group above with the following information.

- Name of the device.
- The source from which you are obtaining it (e.g., the device manufacturer's name).
- Any information regarding shipping, storage, administration, preparation instructions, and dispensing instructions.
- Estimated date and time of use.

You may need to arrange for shipping of the device to UAMS or AC. If the manufacturer requires a letter from the IRB before considering your request or shipping the item, contact [UAMS IRB](#).

It may also be necessary to contact Clinical Engineering ([UAMS](#) and AC Healthcare Technology Management [HTM](#)) to have the device tagged for maintenance, calibration, etc.

STEP 4 – NOTIFY OTHER APPLICABLE PARTIES AT UAMS/AC

If applicable, notify the following parties (including patient name, medical record number and proposed date(s) of treatment, along with FDA acknowledgement letter when received [if requested])-

- **Legal:** Dori Wong-Scoggins (Dori@uams.edu) or Joe Underwood (JUnderwood@uams.edu) (UAMS); Andrea Dixon (DixonAW@archildrens.org) (AC)
- **Budget:** Determine who will be responsible for paying for device and associated procedures, including monitoring, coordinating, follow-up
- **Conflict of Interest:** West Ashley (WLAshley@uams.edu) (if applicable)

STEP 5 – INFORMED CONSENT

FDA requires the **consent process for single patient non-emergency use to include all the standard elements of a research consent**. The UAMS IRB Consent Form - Expanded Access template meets this requirement for consent form content. **Informed consent MUST be obtained prior to use of an investigational device in a compassionate use situation.**

When administering an expanded access use device to a patient with a non-English language preference, the consent form should be translated into the patient's primary language (if time allows). [Short form written consent \(21 CFR 50.27\(b\)\(2\)\)](#) procedures can also be followed. See also [UAMS Policy 15.4 about short form consent processes](#) for information.

STEP 6 – SAFEGUARDS

As a treating physician, you are responsible for devising an appropriate schedule for monitoring the patient, taking into consideration the investigational nature of the device and the specific needs of the patient. The patient should be monitored to detect any possible problems arising from the use of the device.

Ensure you are familiar with these requirements and can maintain appropriate records and oversight.

Following the compassionate use of the device, a **follow-up report should be submitted to FDA** (ORRA can assist: ORRARegulatoryUnit@uams.edu) **within 45 days of device use** in which summary information regarding patient outcome is presented. **If any problems occurred because of device use, they should be discussed in the supplement and reported to the reviewing IRB as soon as possible.**

STEP 7 – IRB NOTIFICATION / ASSESSMENT

Non-emergency single patient IDEs require IRB review and approval before the patient is treated and must be submitted to the IRB on a CLARA new submission form.

If available, provide a copy of the FDA’s approval.

UAMS IRB Staff will review the completed submission in CLARA, including the required attachments (see **Step 2** [1-6]). The Consent form must contain all required elements. See the UAMS Expanded Access Consent form template for guidance. (see **Step 5**).

While the IRB may approve the compassionate use before FDA approval is finalized, the device may not be used to treat the patient until the FDA approves the compassionate use request.

REFERENCES

- [Expanded Access | Information for Physicians](#)
- [Expanded Access for Medical Devices](#)
- [UAMS Office of Research Regulatory Affairs \(ORRA\)](#)
- [UAMS Institutional Review Board \(IRB\)](#)
- [Reagan-Udall Foundation for the FDA: Understanding Expanded Access](#)
- [FDA Information Sheet Guidance For IRBs, Clinical Investigators, and Sponsor: Frequently Asked Questions About Medical Devices](#)
- [IDE Applications](#)