

**OFFICE OF HUMAN RESEARCH AFFAIRS (OHRA)**

**EXPANDED ACCESS GUIDANCE & PROCESS CHECKLIST**

Overview & Criteria

Expanded access, also referred to as “compassionate use”, is the use of investigational drugs, biologics or medical devices, not yet approved by the FDA, for the treatment of a patient with an immediate life-threatening condition or serious disease or condition. These are treatments used outside of clinical trials when there are no comparable or satisfactory alternative therapies.

To receive expanded access the [following criteria](https://www.fda.gov/news-events/public-health-focus/expanded-access) for the patient must be met:

* Patient has a serious disease or condition, or whose life is immediately threatened by their disease or condition
* There is no comparable or satisfactory alternative therapy to diagnose, monitor, or treat the disease or condition
* Patient enrollment in a clinical trial is not possible
* Potential patient benefit justifies the potential risks of treatment
* Providing the investigational medical product will not interfere with investigational trials that could support a medical product’s development or marketing approval for the treatment indication

Expanded access requires approval from all of the following:

* FDA: patient must meet criteria under law and FDA regulations
* Physician: the licensed physician must approve
* IRB: Institutional Review Board (IRB) needs to approve the expanded access is appropriate
* Company: the company producing the product needs to approve expanded access is appropriate

Approval Steps

Determining the type of expanded access, process on how to request expanded access and forms for request can be found on the [FDA website](https://www.fda.gov/news-events/expanded-access/expanded-access-how-submit-request-forms).

**Types of** [**Expanded Access**](https://www.fda.gov/news-events/expanded-access/expanded-access-categories-drugs-including-biologics) **include:**

1. Single Patient
	1. Emergency
	2. Non-Emergency Use
2. Intermediate size patient population
3. Large patient population

Licensed physicians, patients, IRBs, the company and FDA, must follow the appropriate steps to receive approval for expanded access.

1. Patients speak with licensed physician about all possible treatments (considerations: stage of the disease, type of illness, conditions experienced by the patient).
	* Patient information regarding what expanded access means can be found [here](https://www.fda.gov/news-events/expanded-access/expanded-access-information-patients)
2. Licensed physician reviews and agrees with the [roles and responsibilities](https://www.fda.gov/news-events/expanded-access/expanded-access-information-physicians):
	* Determine there are no available clinical trials for the patient
	* Confirm patient’s current disease or condition qualifies for expanded access
	* Identify the appropriate expanded access request type
	* Confirm industry will provide investigational medical product
	* Facilitate process
	* Manage the treatment
3. Physician contacts the company to request the investigational medical product for expanded access use.
4. For sponsor-investigator single patient expanded access requests, if the company is willing to provide the investigational medical product for treatment under expanded access, the expanded access request can be submitted to the FDA.
	* Before request to the FDA:
		+ Patient and physician agree with expanded access, and physician agrees to roles and responsibilities
		+ Company agrees to provide investigational product
		+ Paperwork is submitted to both the FDA and IRB
5. Once final agreement from everyone is approved, treatment can begin.

Note: there must be an informed consent form and the patient must be aware they may opt out of participation at any time (for assistance in creating an appropriate consent form, please contact Tracy Ziolek at either tracy\_ziolek@uhg.com or 215-868-3114).

**Cost**

Expanded access costs are determined by private insurance, Medicaid, or the company manufacturing the product. As a result, some products may not be covered and will include additional costs to the patient.

**FDA Note on Investigational Products**

Investigational drugs, biologics or medical devices have not yet been approved or cleared by FDA and FDA has not found these products to be safe and effective for their specific use. Furthermore, the investigational medical product may, or may not, be effective in the treatment of the condition, and use of the product may cause unexpected serious side effects.

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| [***FDA Contact Information***](https://www.fda.gov/news-events/public-health-focus/expanded-access)**1.** During **Normal Business Hours** (8 a.m. - 4:30 p.m. ET, weekdays)For specific questions during normal business hours:* Investigational drugs: 301-796-3400 or druginfo@fda.hhs.gov [CDER's Division of Drug Information], or contact the appropriate [review division](https://www.fda.gov/news-events/public-health-focus/expanded-access#reviewdiv), if known
* Investigational medical devices: 301-796-7100 or DICE@fda.hhs.gov [CDRH's Division of Industry and Consumer Education]
* Investigational biologics: 240-402-8020 or 800-835-4709 or industry.biologics@fda.hhs.gov [CBER's Office of Communication, Outreach and Development]

For **general questions**, or if you are unsure of who to contact, contact the Patient Affairs Staff at 301-796-8460 or patientaffairs@fda.hhs.gov.**2.** After 4:30 p.m. ET **weekdays and all day on weekends**For **emergency requests** for all medical products (drugs, biologics, and medical devices) contact **FDA's Emergency Call Center** at 866-300-4374. |

**The following is a checklist to assist physicians with the process of initiating an expanded access protocol**

**Expanded Access Licensed Physician Checklist**

**Step 1 - Please check applicable boxes below:**

[ ]  Patient has a serious disease or condition, or whose life is immediately threatened by their disease or condition

[ ]  There is no comparable or satisfactory alternative therapy to diagnose, monitor, or treat the disease or condition

[ ]  Patient enrollment in a clinical trial is not possible

[ ]  Potential patient benefit justifies the potential risks of treatment

[ ]  Providing the investigational medical product will not interfere with the investigational trials that cold support a medical product’s development or marketing approval for the treatment indication

*If all boxes are checked, please move to step 2. Unfortunately, if not all boxes are checked your patient does not apply for expanded access, please look for current treatments, therapies and clinical trials.*

**Step 2 - Please check applicable box below:**

Have the Patient and licensed physician discussed all possible treatments and considered the stage of the disease, type of illness and conditions/symptoms experienced by patient?

[ ]  Yes [ ]  No

*If “No” box is checked, please discuss all possible treatments with the patient and repeat Step 2.*

**Step 3 - Please check applicable boxes below:**

As the physician of this case, do you agree to complete the roles and responsibilities listed below (check box for “yes”, leave box blank for “no”):

[ ]  Determine there are no available clinical trials for the patient

[ ]  Confirm patient’s current disease or condition qualifies for expanded access

[ ]  Identify the appropriate expanded access request type

[ ]  Confirm industry will provide investigational medical product

[ ]  Facilitate process

[ ]  Manage treatment

*If all boxes are not checked, please reconsider your role in this case and determine if yourself or another licensed physician would better fill this role and care of this case.*

**Step 4 - Please check applicable box below:**

Has the physician contacted the company to request investigational medical product for expanded use?

[ ]  Yes [ ]  No

*If “No” box is checked, please request the investigational medical product from the company and repeat Step 4.*

**Step 5 - Please check applicable box below:**

Has the company agreed to provide their investigational product?

[ ]  Yes [ ]  No

*If “No” box is checked, please consider another treatment or therapy.*

**Step 6 – Determine both the type of expanded access AND appropriate request form for this case.** **Please check one box in each column:**

|  |  |
| --- | --- |
| ***Type of expanded access (Check one)*** | ***Type of request form (Check one)*** |
| [ ]  Single Patient Emergency Use | [ ]  Individual Patient IND  |
| [ ]  Single Patient Non-Emergency Use | [ ]  Emergency Use Individual Patient IND |
| [ ]  Intermediate size patient population | [ ]  Intermediate-Size Population IND |
| [ ]  Large patient population | [ ]  Treatment IND |
|  | [ ]  Individual Patient Protocol |
|  | [ ]  Emergency Use Individual Patient Protocol |
|  | [ ]  Intermediate-Size Population Protocol |
|  | [ ]  Treatment Protocol |

**Step 7 - Submit paperwork to the IRB** (including informed consent document)

For assistance in creating an appropriate consent form, please contact Tracy Ziolek at either tracy\_ziolek@uhg.com or 215-868-3114).

**Step 8 - Submit request and paperwork to the FDA.**

**Step 9** - **Please check applicable boxes below (a checked box indicates that step is complete and leaving a box blank indicates that step is incomplete):**

[ ]  The Patient approved expanded access

[ ]  The Licensed Physician approved expanded access

[ ]  The Company approved expanded access

[ ]  The IRB approved expanded access

[ ]  The FDA approved expanded access

If all boxes are checked, treatment can begin. Unfortunately, if all boxes are *not* checked, please review the steps and/or reconsider the treatment or therapy plan for your patient.