

Investigational Drug Application (IND) for Expanded Access in *Intermediate-Size Patient Populations* or Expanded Access for *Widespread Treatment Use*

All applicable regulations, VCU IRB and Health System policy and procedures must be followed regarding the use of investigational agents.

Note: For **emergency expanded access**, existing trials or an **individual patient IND or protocol** should be utilized. If needed for multiple patients, a separate request would be needed for each patient seeking expanded access, but the commercial sponsor can make multiple such requests under its existing IND.

This guidance document focuses on **non-emergent expanded access for intermediate-size patient populations** or **expanded access for widespread treatment use**; for information on **individual use applications (including emergency IND use)** please review our other guidance document.

Types of multi-patient expanded access investigational drug FDA submissions:

1. **Intermediate-size patient population** expanded access IND or *intermediate-size patient population expanded access protocol*
 - a. Investigational drug use is desired for *more than one* patient but generally fewer patients (although no numerical upper limit) than are treated under a typical treatment IND or protocol, submitted under a new IND.
 - b. **Potentially appropriate/needed if:**
 - i. Multiple single patient uses involving an investigational product that is not being developed or that cannot meet the conditions for approval
 - ii. Use is likely or predictable by a number of patients with a shared indication who may benefit from access
 - iii. If a REMS restricts use of an approved product outside of the approved indication but a different patient population might benefit from access
 - iv. Access to treatment with an approved drug that is on shortage or no longer marketed (if unapproved source is available); this would be the appropriate pathway, as treatment IND is only for drugs being actively developed for marketing approval.
 - c. FDA believes it is generally most efficient for a sponsor to **consolidate to a single intermediate-size IND or protocol** for patients with the same condition for a particular drug; HOWEVER regulations do not preclude the possibility of the need for multiple in certain infrequent situations.
 - d. Note: Unless otherwise communicated, for **intermediate IND** the **FDA requires a 30-day period** from the date FDA receives the **intermediate IND submission AND IRB approval** before treatment with the drug may begin.
 - e. Note: There is **no 30-day period for intermediate protocol**, as long as FDA and IRB approval treatment may begin.
2. **Treatment IND or treatment protocol** (also called **expanded access for widespread use**)
 - a. Investigational drug use desired for **large (widespread) population, submitted as new IND**; Unless otherwise communicated, **FDA requires a 30-day period** from the

date FDA receives the **new IND and IRB approval** before treatment with the drug may begin)

- b. **Typically initiated by a manufacturer/sponsor rather than individual physician – for this reason this guidance will focus on intermediate-size patient population IND or protocol**
- c. **Potentially appropriate if:**
 - i. Needed to bridge a gap between completion of a clinical trial and marketing (regardless of intended size of patient population) to prevent treatment interruption
- d. Note: FDA believes that expanded access INDs or protocols treating larger patient populations generally have the greatest potential to interfere with clinical trials/development due to their risk of interfering with patient recruitment

Please note: The term “Managed Access Programs” (MAPs) is sometimes used to cover various types of programs, including “Compassionate Use” (CU), “Expanded Access” (EAP), “Named Patient Program or Supply” (NPP or NPS), and others. FDA nomenclature is EAP, while EU nomenclature is typically CU/NPP, etc.

Recommended Steps

- A. **Intended for non-emergent treatment use only (see [FDA’s expanded access “how to”](#))**
 - 1. **Contact manufacturer** - Upon identification of potential need in multiple patients or repeated/subsequent patients, contact manufacturer/sponsor to see if they are willing to provide the investigational drug for expanded access use. If so, they should provide documentation of agreement via
 - Letter of Authorization** (LOA; [see FDA’s online template](#) if needed)
Note: In cases where it is not possible to obtain an LOA (e.g., the entity supplying the drug does not have an IND filed with FDA), physicians should contact the relevant FDA review division to determine what information is needed to support the expanded access submission
 - 2. **Obtain VCU [IRB approval](#)** (prior to FDA submission per VCU policy; see [IRB SOPs](#))
 - Pre-use: IRB submission
 - Pre-use: IRB approval received
 - 3. **[Contact FDA](#)** for authorization
[FDA needed items](#)
 - Brief cover letter** (what is being request and who they will contact)
 - FDA 1571** (IND form) and **FDA 1572** (statement of investigator form) should be used; see [FDA’s instructions on submission](#) (see [FDA’s instructions for 1571/1572](#); also [FDA detailed instructions for 1571](#))
 - Treating physician assessment** (only if patients are known, if prospective application then omit) of patient condition and available treatment alternatives (include rationale for intended use, 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)
 - Informed Consent Statement** that will be used to consent patients
 - Letter of Authorization** from sponsor/manufacturer (within 15 calendar days)
 - 4. **Obtain informed consent** from patient or legally authorized representative prior to treating (Note: **DO NOT USE** the health system’s standard informed consent. A general consent template for informed consent to investigational drug treatment is [available on](#)

IRB website if a drug specific one is not supplied by the sponsor/manufacturer. Please review options to ensure you are picking the template that best fits your specific utilization scenario).

- Pre-use: Investigational drug informed consent obtained from patient or patient's LAR

B. **Notify VCU Health Investigational Drug Services (IDS)** – IDS will receive, store, and dispense the drug appropriately, without charging for our services.

- Contact the IDS at 804-828-7901 (FAX to 804-827-0181) to inform us of the impending shipment of an Expanded Access IND drug AND

- Please also send notification and copies of all documents/forms/tracking via email to investigational.drug@vcuhealth.org

- You may also reach out to the following resources for regulatory guidance and assistance – **to meet minimum necessary privacy standard, please reference patients by their initials and eIND number only (do not share PHI such as name, DOB, or MRN):**

[VCU Clinical Research Regulatory Affairs](#)

- Provide all necessary information (treatment protocol, investigator's drug brochure, accountability logs, drug order forms) as soon as possible so that the pharmacist can set up dispensing and drug preparation procedures prior to receiving the investigational agent(s).
- Arrange to have the investigational agent(s) **shipped to the following address:**
VCU Health MAIN IDS
Main Hospital, Basement, B-300
1250 E. Marshall Street
Richmond, Virginia 23298-5051
- Order to be placed via *custom investigational medication or infusion pathway* in Epic if needed prior to Epic custom build completion.

C. **Assess charging/cost recovery** – Check with manufacturer/sponsor to assess charging for investigational use in an intermediate-size patient population; **sponsor is permitted to charge** for direct drug costs as well as cost of monitoring, complying with IND reporting requirements, and other administrative costs; **see [FDA guidance on charging](#)**. **If the agent must be purchased**, be sure to:

- Pre-treatment - include **a request in the IND application to bill the patient** and let the IDS pharmacy know of that decision. If the FDA indicates that the agent may be billed to the patient, it is also necessary to include the fact that the patient will be billed in the informed consent. **Note: only the direct cost of making drug available** to patient may be charged.
- Pre-treatment – if patient is to be billed **get prior authorization** from the patient's insurance. If the provider of an investigational agent charges for their product, determine the cost and obtain approval from the Pharmacy Manager On-Call (via pager if on nights or weekends) before placing an order. The Pharmacy Manager On-Call can provide a Purchase Order number for the Health System, if required.

D. **Follow-up Documentation**

1. **[FDA items](#)**

- Safety reporting (if applicable)
- Any protocol or information amendments
- Summary of expanded access use upon treatment completion

- Annual report if treatment not complete; within 60 days of the anniversary date (not required if FDA notified of completion before one year passed)

Key contact information:

1. [FDA](#)
2. [VCU Health Investigational Drug Services \(Intranet\)](#)
3. [VCU Office of Research and Innovation](#)
4. [VCU IRB](#)

Helpful communications/guidance:

1. Search “Investigational” in Policy Manager for applicable VCU Health Policies & Procedures
2. [VCU IRB Written Policies & Procedures](#)
3. [FDA: Expanded Access Overview](#)
4. [FDA: Expanded Access: How to Submit a Request for Expanded Access](#)
5. [FDA: Charging for Investigational Drugs under IND](#)