Individual Patient **Expanded Access Investigational Drug Application (IND)**

****ALL applicable regulations, VCU IRB (see below for hyperlink to IRB SOPs) and Health System policy and procedures must be followed regarding the use of investigational agents. Emergent Treatment: when urgent afterhours treatment is needed, please page IDS Pharmacist On-Call at 9089 with all applicable known details as soon as you become aware of any potential eIND need.****

Types of single patient investigational drug usage FDA submissions: Both ***single patient IND*** and ***single patient protocol submissions*** can be emergency or non-emergency.

A. **Individual patient expanded access IND** (also sometimes informally called "compassionate use"; ***single patient IND***; preapproval access) – *pathway for most individual uses*
    1. No pending IND for drug OR manufacturer declines to amend existing protocol but grants use of drug (may prefer treating physician to take on role of sponsor-investigator)

B. **Individual patient expanded access protocol** (also called ***single patient protocol***) – *rare*
    1. If a company (IND sponsor) is concerned that the data from the single patient may reflect negatively on an on-going clinical trial, you may ask it to submit a new protocol or treatment plan under an existing IND. **Only the sponsor (a company *or* individual who holds an IND) may apply for a single patient protocol submission**. If the company is the sponsor of the existing IND, it can – but rarely does — submit an individual patient access protocol to its existing IND to allow your patient access to the drug.

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

IMPORTANT: The provider (treating physician) who acquires the medication and obtains authorization for use from the FDA is responsible for ALL follow up submissions and required documentation to the IRB and FDA.

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.

Required Steps for Individual Use

A. **If emergent treatment required:** Treating physician responsible for determining patient meets criteria outlined by the FDA for authorization of emergency use
    1. **Complete the initial steps below** AND also the applicable non-emergent steps below after use, within the specified timeframes (see FDA's emergency IND timeline)
    2. **Treatment may begin immediately following completion of the checklist below:**
        - ☐ Pre-use: Any previous use at VCUHS? (per FDA guidance - subsequent use of the test article is subject to prospective IRB review, however, FDA does not deny use requests based on lack of time to obtain subsequent use prospective IRB review)

- Pre-use: Any ongoing clinical trials/existing IND patient is eligible for? (see clinicaltrials.gov and manufacturer's website)
- Pre-use: Obtain sponsor/manufacturer authorization/approval (LOA); Also request consent form for the specific agent from sponsor/manufacturer if one is available (otherwise blank IRB consent template must completed)
- Pre-use: FDA authorization of use/shipment in advance of IND submission
- **Pre-use: Informed consent by patient or patient's legally authorized representative (LAR)**
- **Pre-use: Notify VCU Institutional Review Board (IRB) of intention to exercise the "Emergency Use Provision" whenever possible – may reach out via fax, email, or phone (communication should include NO PHI).**

B. **For non-emergent treatment** (see FDA's non-emergent guide) –

Note: Unless otherwise communicated, **FDA requires a 30-day period** from the date FDA receives the **single patient IND** and IRB approval is also attained before treatment with the drug may begin.

1. **Contact manufacturer** - Upon identification of potential need in a specific patient, 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)
2. **Contact FDA** for rapid authorization
   FDA items:
   - **Brief cover letter** (what is being request and who they will contact)
   - **FDA 3926** (expanded access IND form) - **if single patient IND**(see FDA guidance/use criteria); note - FDA expects expanded access INDs to reference existing IND for drug information if one is currently pending; **FDA 1571** (IND form) and **FDA 1572** (statement of investigator form) should be used instead for **single patient protocol, must be submitted within 15 calendar days of treatment**
   - **Treating physician assessment** 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)
   - **Consent form** that was or will be used to consent the patient
   - **Letter of Authorization** from sponsor/manufacturer (within 15 calendar days)
3. **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
4. **Obtain VCU IRB approval**

- **Important note for emergency use: treating physician must file IRB submission within a five working day time-frame (but still should notify IRB pre-use for Emergency Provision if possible)**
- IRB submission
- IRB approval received

C. **Notify VCU Health Investigational Drug Services (IDS)** – IDS will receive, store, and dispense the drug appropriately, without charging for our services.
   - **Emergent Treatment:** when urgent afterhours treatment is needed, **please page IDS Pharmacist On-Call at 9089** with all applicable details **as soon as you become aware of any potential eIND need.**
   - **Please also email investigational.drug@vcuhealth.org** to inform us of the potential use AND forward copies of all documents/forms/shipment tracking to investigational.drug@vcuhealth.org as well
   - 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 custom investigational infusion pathway* in the Epic.

D. **Assess charging/cost recovery** - although not typical, sometimes investigational agents must be purchased from the manufacturer/sponsor and may or may not be chargeable to the patient, **dependent upon sponsor meeting FDA criteria for charging authorization. If the agent must be purchased from the manufacturer (not provided at no cost)**, 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 – **get prior authorization;** if patient is to be billed **get prior authorization** from the patient's insurance.
   - Pre-treatment – obtain ordering approval; if the sponsor/manufacturer 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.

E. **Follow-up Documentation**

1. **FDA items**
    - ☐ Safety reporting (if applicable)
    - ☐ Any protocol or information amendments
    - ☐ On completion of treatment: resubmit FDA 3926 with summary of expanded access use marked for field 9 (treatment completed)
    - ☐ Annual report: if treatment *not* complete (ongoing), resubmit FDA 3926 within 60 days of the anniversary date (not required if FDA notified of completion before one year passed) use marked for field 9 (annual report)
2. **VCU IRB items**
    - ☐ Describe how the individual treated was in a life-threatening situation in which no standard acceptable treatment was available and in which there was not sufficient time to obtain VCU IRB approval (if applicable)
    - ☐ Document when contact was made to the IRB and what information was transmitted (if pre-use contact was possible)
    - ☐ Identify how the requirements for an IND were met (if applicable - see several options, above)
    - ☐ Identify the informed consent process used (see options, above)
    - ☐ Any subsequent use of the test article is subject to prospective VCU IRB review

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 System Policies & Procedures
2. VCU IRB Written Policies & Procedures
3. FDA: Expanded Access: How to Submit a Request (Forms)
4. FDA: Physicians How to Request Single Patient Expanded Access
5. FDA: Expanded Access Categories for Drugs (guidance on types of expanded access)