Expanded Access Oversight Committee (EAOC) Charter

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1. Background

When seriously ill patients face the dilemma of having exhausted all standard of care treatment options, the use of investigational agents may be warranted. While enrollment in a clinical trial is the standard approach for patients to gain access to investigational agents, not all patients qualify for clinical trials. In these instances, FDA's expanded access pathway, also known as compassionate use, may allow use of therapies that are not yet FDA approved outside of the traditional clinical trial space for the purpose of treating the patient. However, this is a complex and tightly regulated process whereby physicians and drug companies coordinate the allowable disposition of these treatments on a case-by-case basis through the Federal Drug Administration (FDA).

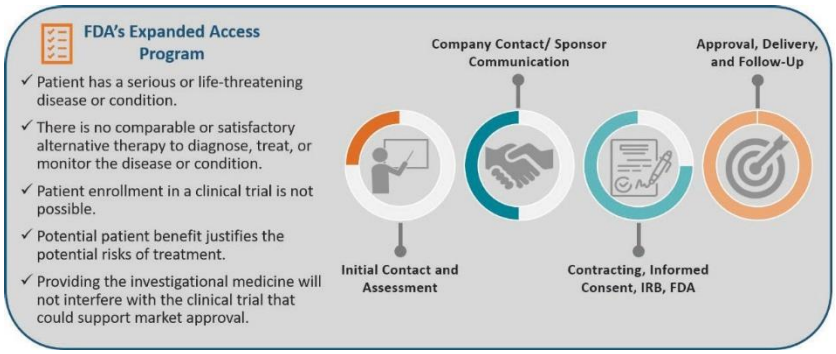

Figure 1. Overview of Expanded Access requirements and process.

To mitigate the burden of navigating a complex regulatory landscape and to facilitate the expedient acquisition of access to investigational treatments for VUMC patients across both pediatric and adult settings, VUMC has developed a robust institutional Expanded Access support pipeline that integrates key stakeholders throughout the Expanded Access process including the clinical teams, the IRB, the office of contracts management (OCM), clinical

pharmacies and the Access to Investigational Medicines (AIM) team, VICTR's Expanded Access support infrastructure.

Physicians who would like to request Expanded Access use of an investigational medicine for their patient do so by submitting the Expanded Access Medication request form which alerts key personnel of the incoming case, including pharmacy personnel and AIM team members, who work closely with the provider, the clinical team, and all key stakeholders to help shepherd each case through the required steps and regulatory approvals to enable patient treatment as quickly as possible **(Figure 1).**

2. Purpose and Scope

Institution-wide requests for Expanded Access have been increasing at VUMC in recent years for both emergency use (life-threatening situation in which no acceptable standard treatment is available and in which there is not sufficient time to obtain full IRB approval) and non-emergency use (non-emergency situation where all regulatory approvals are granted before patient treatment). For example, the number of cases supported by the AIM team doubled from 2022 (10 cases supported) to 2023 (23 cases supported) **(Figure 2)**, highlighting the high demand for physician and clinical team support in navigating the complex Expanded Access process.

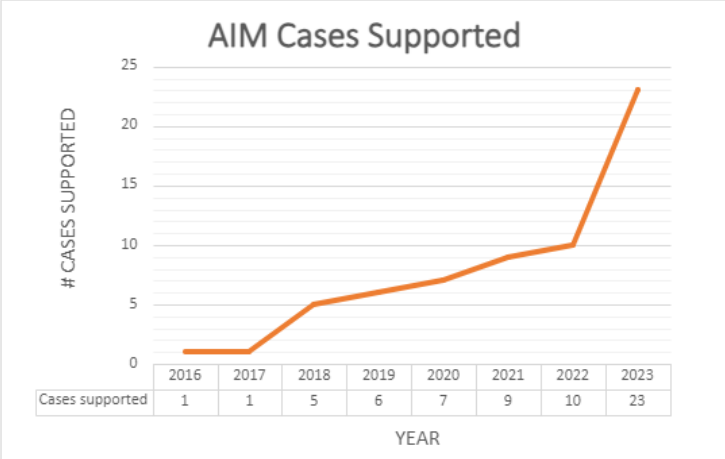

Figure 2. AIM cases supported by year.

Additionally, due to recent changes within the Centers for Medicare and Medicaid Services (CMS), Expanded Access cases are now assigned a billing code and thus the institution transitioned to managing cases through the adult and pediatric clinical pharmacies rather than the investigational drug services (IDS) pharmacy.

In response to this growth, the Expanded Access Oversight Committee (EAOC) was established in August 2024 to help promote transparency and streamline processes to meet the needs of providers and patients across VUMC. The EAOC will review all *non-emergency* institutional Expanded Access requests (received through the Expanded Access Medication Request Form that meet the minimum requirements for Expanded Access use). Committee members will evaluate each Expanded Access case to assess the appropriateness within the Expanded Access pathway and the risk-benefit ratio for the patient. The EAOC will also ensure clinical and pharmacy teams are well-positioned/sufficiently staffed and resourced to support the request. The EAOC will be comprised of standing members (representing key areas governing Expanded Access including pharmacy leadership, VICTR leadership, VICC leadership, VUMC Institutional Review Board [IRB]) and case-specific members including the requesting physician, the requesting physician's department head or division chair, and pharmacy representatives for the pharmacy areas for the submitted medication.

3. Committee Members

Participating members of the EAOC will vary depending on the personnel involved in each case. Standing members will review each case via the process described below and will vote to determine if the case should move forward. Case-specific members will be included for awareness and to provide clinical input as needed.

Standing (voting) members will include:

- VICTR Leadership (including Drs. Wes Self, Sean Collins, Evan Brittain, Todd Rice, peds expert TBD)
- VICC Leadership (Dr. Ben Park)
- VUMC IRB representative (Kristin Straznicky) – *non-voting member*
- VUMC Pharmacy Leadership
  - Ashley Houser and Jennifer Hale – Pediatric pharmacy
  - Bob Lobo – Adult pharmacy
  - Donna Torr – Investigational Drug Pharmacy (IDS), as needed

Case-specific members will include:

- Requesting physician
- Requesting physician's department chair or division chief
- Pharmacy director or manager who oversees the pharmacy area for the submitted medication (e.g., adult retail, pediatric retail, VICC, VUH, VMG, etc.)
- Clinical pharmacist for the pharmacy area for the submitted medication

4. Process for Case Review

a. Committee members will be alerted of new non-emergency Expanded Access cases via an email sent by the managing AIM team member within 24 hours of receiving the formal REDCap request.

b. Information provided to the committee members to aid in their review will include the link to the REDCap submission along with the following case summary (see template example in Appendix A).

   - Patient age, diagnosis, prognosis, and medical history including treatments previously tried or currently receiving
   - Does the patient qualify for any clinical trials? (if not, why)
   - Name of medication requested (and company)
   - Rationale for use of the medication given the patient's condition
   - Dose, dosage form, route of administration
   - Planned duration of treatment
   - Team/service and treatment setting
   - Planned/anticipated date of treatment
   - FDA approval for any indication? If yes, list indication

b. Committee members may make a request for additional information to the physician/clinical team or AIM team additional as needed.

5. Process for Decision Making and Voting

a. Standing committee members will provide any feedback within two business days via email reply all to the group. Feedback may include, but is not limited to, clinical concerns, logistical considerations, or potential issues/challenges for consideration by the group.
b. Discussions will occur via email and voting members will strive to reach consensus.
    - ***Note***: Committee members may request a virtual meeting (via Zoom or Teams) at any time throughout the process to discuss unique case requirements, considerations and circumstances requiring more in-depth discussion. A quorum (defined as at least four standing committee members) will be used for scheduling and will be required for meetings to proceed.
c. Within two business days, standing committee members will vote via REDCap (voting link will be provided by the AIM team case lead, who will facilitate the voting process).
    - ***Note***: If a virtual meeting is requested and held, voting may occur during that time.
d. Approval by a majority of the quorum of standing committee members will be required in order for the case to move forward. A quorum will be defined as at least four standing committee members.
e. If the standing committee members do not express concern or submit feedback within two business days, the case will move forward by default.

6. Roles and Responsibilities

Members of the EAOC will work together to:

a. Review new Expanded Access cases via the process described above and provide feedback if required within two business days to include logistic considerations, clinical concerns, or potential problems or issues to be aware of.
b. Meet via Zoom/Teams if more extensive discussion is required or requested.
c. Develop recommendations for continued process improvement yearly or at appropriate decision points (standing members).

AIM team members will:

a. Facilitate communication, discussion, and voting via the process described above.
b. Send a summary email to the Committee outlining the immediate outcome of the case (e.g., if treatment was initiated and if not, the reason for not initiating, any clinical updates, etc.).
c. At yearly meetings, the AIM team will present a review of longer-term case outcomes (e.g., if treatment has been completed, any safety events, etc.) to standing committee members.

Member	Standing or Case-Specific	Role
VICTR AIM Manager	Standing (non-voting)	Expanded Access case management and regulatory support
IRB Representative	Standing (non-voting)	IRB Point of Contact
VICTR Leadership	Standing	Clinical Review
VICC Leadership	Standing	Clinical Review
VUMC Pharmacy Leadership	Standing	Clinical/Pharmacy Review
Requesting Physician	Case-Specific (non-voting)	Clinical Care
Department Chair	Case-Specific (non-voting)	Clinical Review

Pharmacy Director or Manager	Case-Specific (non-voting)	Drug Logistics/Clinical Care
Clinical Pharmacist	Case-Specific (non-voting)	Drug Logistics/Clinical Care

Appendix A: Example of Email Committee Members Will Receive for Initial Review of Request

Dear Expanded Access Oversight Committee Members,

We received a new non-emergency Expanded Access request on [insert date and time here]. Key case details are below for your review (full case details submitted via REDCap can be found here):

Patient age, diagnosis, prognosis, and medical history including treatments previously tried or currently receiving
Is the patient a candidate for the relevant clinical trials for this condition (if not, why)?
Name of medication requested (and company)
Rationale for why the proposed Expanded Access medication may help this patient (e.g., mechanism of action related to the patient's condition)
Dose, dosage form, route of administration
Planned duration of treatment
Team/service and treatment setting
Planned/anticipated date of treatment
FDA approval for any indication? If yes, list indication
Other background or notes that would be helpful for committee review

Feel free to ask questions or request additional information and note that a virtual meeting may be requested if additional discussion is required to reach consensus.

Please reply all to this email with one of the following responses within 2 business days:

1. *No concerns or feedback*
2. *Provide your feedback for the group to consider (including but not limited to logistical considerations, and/or potential problems or issues)*
3. *Questions or clarifying information required (please provide your questions or request for additional information)*
4. *Additional discussion needed and virtual meeting requested*

After group discussion, the AIM team will facilitate formal voting by the standing committee members and the results will be conveyed to the group.

If no concerns are raised or no feedback is received, the case will move forward for processing through the Expanded Access pathway process.

Thank you for your time and we look forward to hearing from you.

The VICTR Access to Investigational Medicines (AIM) Team