

Streamlining Expanded Access: Implementation of a Centralized Management Model

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1. Background

Expanded Access (EA), also referred to as single-patient Investigational New Drug (IND) or Compassionate Use, is a regulatory pathway allowing patients to receive investigational therapies outside of a clinical trial when no comparable alternatives are available. EAs are authorized by the Food and Drug Administration (FDA) under 21 Code of Federal Regulations (CFR) 312.305 and 312.310 and are particularly relevant in oncology due to rapid therapeutic innovation, rare tumor indications, and the molecular stratification of tumor types. Furthermore, they address a critical need for patients facing trial ineligibility due to performance status or prior therapies.

EA pathways include: Single-patient EA (non-emergency) requests, which require FDA and Internal Review Board (IRB) approval prior to treatment; Single-patient EA (emergency) requests, in which treatment can begin after verbal authorization from the FDA but before all written approvals are obtained; and Multi-patient EA requests, which are often managed by the sponsor as opposed to the treating physician.

While critical for patients with limited options, EA implementation is administratively complex and resource intensive.

2. Goals

To support timely patient access to EA therapies while upholding strict ethical and regulatory standards, the Helen Diller Family Comprehensive Cancer Center (HDFCCC) will be launching a pilot initiative designed to streamline EA operations, improve budgeting accuracy, and strengthen utilization tracking. This pilot project will focus on single-patient non-emergency EAs for rare cancers.

3. Solutions and Methods

To centralize and standardize management of single-patient EA cases, a dedicated project manager oversees the full lifecycle of each request, from initial physician inquiry through to regulatory submission, treatment initiation, monitoring, and case closure. Coordinated implementation has been supported by cross-functional engagement across Contracts, Investigational Drug Services (IDS), Regulatory Affairs, and Study Operations. Formalized standard operating procedures (SOPs) and structured workflows will be developed to clarify roles, reduce process variability, and improve efficiency.

To ensure appropriate and ethical use of EA, a peer review committee has been established to evaluate clinical justifications and confirm alignment with regulatory and internal pilot project criteria. To measure the impact of the centralized EA model, key operational metrics will be tracked, including the number of EA requests, time from request to treatment initiation, duration of EA therapy, and total institutional cost per case.

4. Outcomes

Retrospective review of institutional data over the previous five years demonstrates that HDFCCC opened an average of five single-patient EA protocols annually, with most requests originating from

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programs in pediatric oncology, neurologic oncology, and hematologic malignancy. Current assessments reveal variability in operational practices across disease groups and departments. Recent increases in single patient EA requests highlight the need for robust and standardized EA workflows.

Centralized oversight and defined tracking of operational metrics will provide data to accurately characterize resource utilization, workflow efficiency, and financial impact of single-patient EA cases at our institution.

5. Lessons Learned and Future Directions

The pilot project will run through 2027, during which time operational and financial metrics will be collected and analyzed. Findings will inform an institutional decision on whether to continue or expand the centralized EA management model. Our long-term goal is to establish a sustainable framework for EA management that balances patient need, ethical oversight, regulatory compliance, and institutional stewardship of resources.