UCSF Helen Diller Family Comprehensive Cancer Center

Streamlining Expanded Access: Implementation of a Centralized Management Model

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Background

Expanded Access (EA), also referred to as single-patient 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. EA protocols are authorized by the FDA under 21 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 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.

Objectives

- Ensure patient access to EA therapies
- Streamline operational Processes
- Build institutional capacity
- Improve patient selection for EA protocols
- Understand barriers through retrospective analysis

Current State Analysis

92 — Expanded Access Submissions over 10 years

~73% — Resulted in Treatment
Demonstrates meaningful access and program feasibility

~27% — Did Not Proceed to Treatment
Highlights critical gap between intent and therapy delivery

Understanding why 1 /4 patients don't reach treatment is essential to improvement

- Rapid patient decline or disease progression
- Access and logistical barriers
- Variability across disease groups leading to inconsistent patient selection practices

Review of institutional data over the previous five years demonstrates that HDFCCC opened an average of 9 EA protocols annually, with ~5 of those being non-emergency single-patient. Most requests came from pediatric oncology, neurologic oncology, and hematologic malignancy programs. Current assessments reveal variability in operational practices across disease groups and departments.

Recent increases in single-patient EA requests have led to the need to operationalize the program for better standardization and scalability.

We expect this trend to continue particularly in the cell and gene therapy spaces where treatment is often patient specific.

Centralization and Standardization

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. The full lifecycle of an EA is considered from initial physician inquiry through to treatment initiation and case closure.

Areas of Focus

Dedicated project manager to oversee the full lifecycle of each request – one person for all EAs regardless of disease program.

Cross-functional engagement across Contracts, Investigational Drug Services, Regulatory Affairs, and Study Operations.

Formalized standard operating procedures (SOPs) and structured workflows to clarify roles, reduce process variability, and improve efficiency.

Peer review committee to evaluate clinical justifications for each patient and confirm alignment with regulatory and internal pilot project criteria.

Looking Forward

UCSF has a strong EA foundation on which to build and scale. Historical data demonstrate the feasibility of implementing the single patient EA pathway in a timely and compliant manner. However, these data also highlight opportunities to improve both the number of patients receiving treatment and the speed at which it becomes available. Through our centralized management model, we aim to achieve:

- Early identification of eligible candidates within clinical workflows across all disease programs
- Robust and rapid feasibility assessments
- A streamlined activation process supported by pre-negotiated frameworks
- Program-level oversight to ensure ongoing quality assurance and regulatory compliance

One Program.
Faster Access.
Innovative Care.

At the conclusion of the pilot, operational metrics, including time from request to treatment initiation and duration of EA therapy, and financial metrics will be collected and evaluated to assess the effectiveness of the centralized EA management model.

The findings will inform an institutional decision regarding continuation and potential expansion of the model. Our long-term objective is to establish a sustainable framework for EA management that balances patient needs, ethical oversight, regulatory compliance, and responsible stewardship of institutional resources.