

# **Expanded Access Programs for Drugs and Biologics**

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# Outline

- Expanded Access Programs (EAP)
- Other initiatives to improve access **WITHIN** clinical trials



# Expanded Access

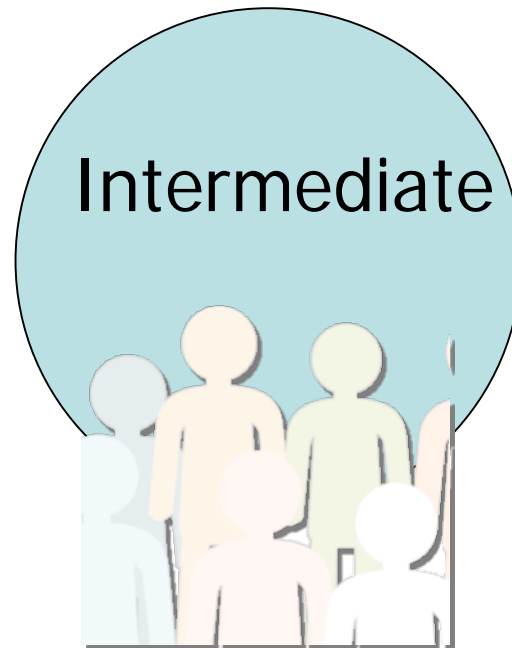
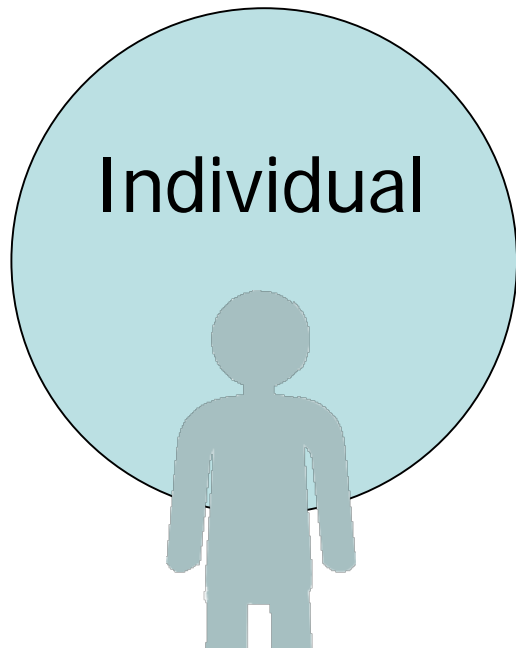
- “Compassionate” use
- You have a serious illness and you’ve tried everything else
- You and your doctor think an investigational drug (not FDA approved) might be a good option
- The drug may be studied in clinical trials, but you are not able participate in these trials



# Types of Expanded Access Programs



- 3 types of EAPs are defined in the code of federal regulations (CFR):



# Single Patient IND



- Generally patients with multiply relapsed or refractory cancer
- Reasons for requesting expanded access may include:
  - Promising evidence of activity with a drug in a disease with a similar molecular target or histology
  - Patient received benefit while participating on a previous clinical trial
  - Ineligible for clinical trial but reason to think potential benefit outweighs the risk
  - Clinical trial is closed to accrual
  - Drug is not currently being developed
  - Clinical trial site not accessible to patient (regional)

# How to Apply for Expanded Access?











# Stay tuned: FDA Call Center Pilot

- “Project Facilitate”
- Make the EA process even more efficient
- Telephone number for physicians
  - Resources: independent IRBs
  - Contact information for the drug company
- FDA/OCE to host a public meeting in mid-May



**BLIND  
CORNER  
PROCEED  
WITH  
CAUTION**

- 1. Risk has not been established for investigational drug**
- 2. Potential benefit is often overestimated**

## Pros

- Provides access to potentially lifesaving therapies to patients who have no other alternatives, & may be willing to accept greater risk
- Provides patients a measure of autonomy over their own health care decision
- Bridges gap between drug development and FDA approval
- May provide data to support development
- May offer hope for patients with no other available options



## Cons

- Risk has not been established
- May overestimate benefit and underestimate risk
- Drug availability
  - manufacturing
  - fear that adverse events on EAP may disrupt drug development (MYTH!)
- Paperwork! (improved, & ongoing initiatives to overcome)

# Could Expanded Access Be Made Obsolete?

- Expanded access programs are in place when no appropriate alternatives exist, but the **best access is an approved drug**
- **To be part of the road to approval, enrollment/treatment on clinical trials is critical**

- Considerations for decreasing the need for expanded access in oncology:
  - Expansion of eligibility criteria (broadly)
    - Age, CNS disease, organ dysfunction
  - Separate cohort within a clinical trial with broad eligibility criteria
  - Novel trial designs: Master protocols
    - May allow assessment of multiple diseases, treatments, or biomarkers in one protocol
    - Example: Pediatric MATCH
  - Initiatives in pediatrics: FDARA
  
- The future: novel surrogate endpoints, real-world data mining, personalized medicine

# Summary

- Expanded access programs provide access to unapproved, investigational therapies to patients who have no other alternatives
- The single patient IND is the type of expanded access oncologists would most likely encounter
- The single patient IND requires agreement from the patient and doctor, the drug company, the FDA, and the IRB
- Oncology stakeholders are considering options to try and improve access to unapproved drugs



# Resources for Single Patient INDs



- <https://www.fda.gov/Drugs/DevelopmentApprovalProcess/HowDrugsareDevelopedandApproved/ApprovalApplications/InvestigationalNewDrugINDApplication/ucm107434.htm>

## Drugs

Home > Drugs > Development & Approval Process (Drugs) > How Drugs are Developed and Approved > Types of Applications > Investigational New Drug (IND) Application

### Investigational New Drug (IND) Application

Emergency Investigational New Drug (EIND) Applications for Antiviral Products

IND Forms and Instructions

Investigator-Initiated Investigational New Drug (IND) Applications

Pre-IND Consultation Program

Regulatory Information for INDs

Resources for You

## For Physicians: How to Request Single Patient Expanded Access (“Compassionate Use”)

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When a physician wants to submit a Single Patient Expanded Access request to obtain an unapproved investigational drug for an individual patient, he or she must first ensure that the manufacturer is willing to provide the investigational drug for expanded access use. If the manufacturer agrees to provide the drug, the physician should follow the steps below to submit an Investigational New Drug Application (IND) to the FDA.

### Emergency Requests:

In an emergency situation, the request to use an unapproved investigational drug may be made via telephone or other rapid means of communication, and authorization to ship and use the drug may be given by the FDA official over the telephone. In these situations, known as emergency IND (eIND) requests, shipment of and treatment with the drug may begin prior to FDA’s receipt of the written IND submission that is to follow the initial request. An [emergency IND timeline](#) is available online to guide you through the process.



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