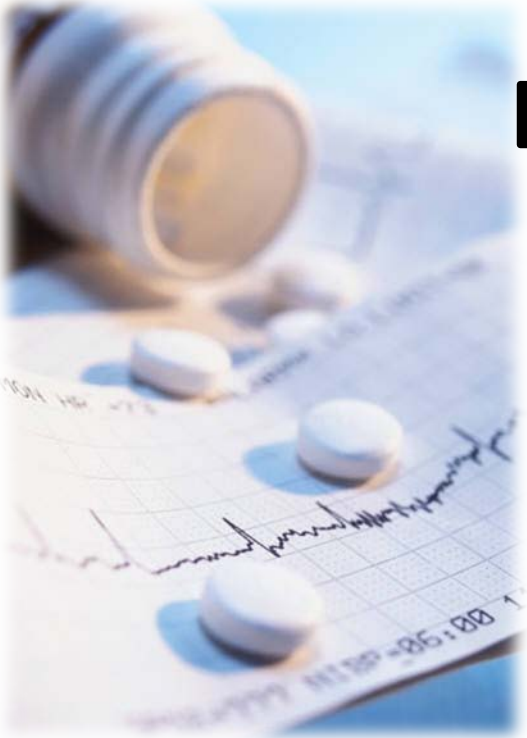
Drugs

Devices



Expanded Access

(including Emergency Use
&
Humanitarian Use Devices)

Bertha deLanda, CIP
IRB Training Specialist
Research Compliance Office
March 2012

What Is Expanded Access?

- **Permits** use of an investigational **drug** or **device**
  - for treatment use,
  - outside of a controlled clinical trial
  - specific criteria
  - reporting/monitoring requirements
- **1° purpose**: to diagnose, monitor, or treat a patient's disease/condition
- For all expanded access use, **prior IRB review and approval is needed** (with the exception of Emergency Use)

GUI-19m "Expanded Access to Investigational Drugs and Devices"

Drugs: Expanded Access

21CFR 312.300 (Subpart I)

- **Aim:**

  To facilitate the availability of investigational drugs to patients with serious diseases or conditions when there is no comparable or satisfactory alternative therapy to diagnose, monitor, or treat them

Immediately life-threatening disease or condition:
There is reasonable likelihood that death will occur within…months or premature death is likely without early treatment

Serious disease or condition:
A disease/condition associated with morbidity that has substantial impact on day-to-day functioning.

DRUGS: EAP Categories

Expanded Access Program

All 3 categories must meet basic criteria in **21 CFR 312.305(a)**

Submission to FDA
Prior approval from the FDA is required for every category of EAP
21 CFR 312.305(b)

Single (Individual) Patients

21 CFR 312.310

Intermediate-Size Patient Populations

21 CFR 312.315

Treatment IND or Treatment Protocol (widespread treatment use)

21 CFR 312.320

Includes EMERGENCY USE
21 CFR 56.102(d)

DRUGS: Expanded Access

Criteria and Reporting/Monitoring Requirements

(not a complete list)

Criteria	Reporting/Monitoring
Patient(s)…have a serious/immediately life-threatening disease or condition, & there is no comparable or satisfactory alternative	Submission to the FDA may be a **new IND**, or a protocol **amendment** to an existing IND **All** require scheduled reporting to the FDA on follow-ups, progress, and event notifications
Potential patient benefit justifies the potential risks, &…risks aren't unreasonable in relation to disease/condition	
This use won't interfere with any ongoing clinical investigations that could compromise the marketing approval or potential development of EA use	

See GUI- 19m for additional criteria and requirements

Devices : Categories

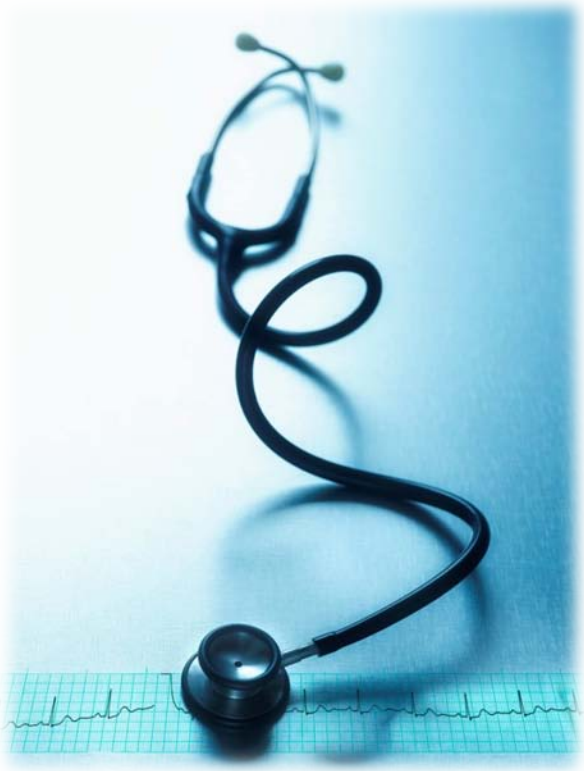
Compassionate Use

Single Patient/Small Group Access

Treatment Use

(Treatment IDE)

Larger Group/More Widespread Use



Continued Access

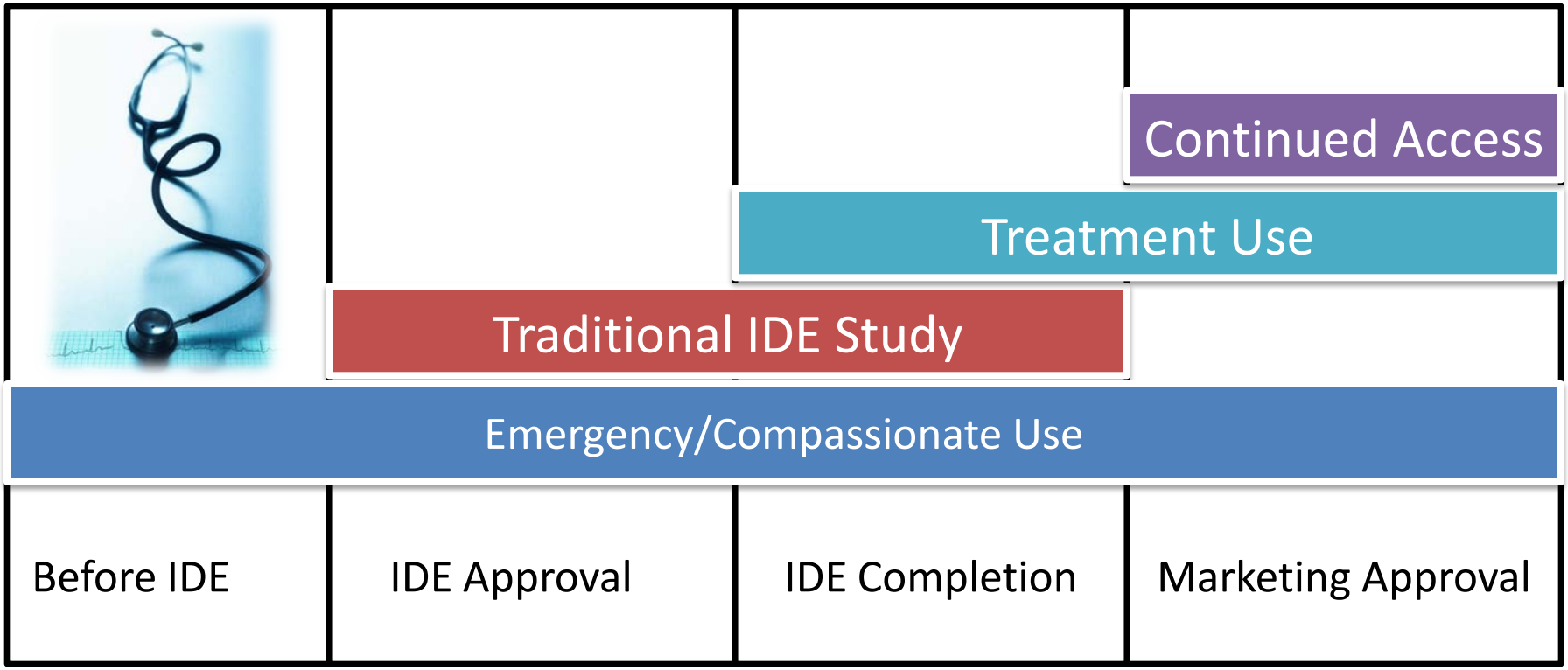
after the clinical trial under IDE is completed

and

while the marketing application is being prepared by the sponsor or reviewed by FDA

Can also be Emergency Use 21 CFR 56.102(d)

Timeline for **Device** Development



Device: Expanded Access

Criteria and Reporting/Monitoring Requirements

(not a complete list)

See GUI- 19m for additional criteria and requirements

	Criteria	Reporting/Monitoring
Compassionate Use	Existing concurrent clinical trial but patient does not meet inclusion/exclusion criteria	Follow-up report should be submitted to FDA as an IDE supplement
Treatment Use	No comparable or satisfactory alternative device/therapy to treat or diagnose that stage of the disease/condition	Semi-annual progress reports **21 CFR 812.150(b)(5)**
Continued Access	Public health need or preliminary evidence of effectiveness	Request for extension submitted as IDE supplement

All require scheduled reporting to the FDA on follow-ups, progress, and event notifications

Emergency Use
Drug or Device

Definition:

The use of a test article on a human subject in a life-threatening situation in which:

no standard acceptable treatment is available,

and in which there is not sufficient time to obtain IRB approval.

Emergency Use of a Test Article

GUI-6

Investigational **drug**, **device**, or **biologic**; Per 21 CFR 56.104(c)

- The PD must submit the following to the IRB within **5 working days** after use:
  - **APP-11m** (Sections A, B, C, and D if informed consent was not obtained)
  - Signed informed consents, if obtained

✓ **Drug**: PD/sponsor must submit IND/amendment to FDA within **15 working days (NEW REQUIREMENT)**

✓ **Device**: when no IDE, PD must report to the FDA within **5 working days**

Humanitarian Use Device
GUI-36m

- **Humanitarian Use Device (HUD):** A device intended to benefit patients by treating /diagnosing a disease/condition that affects fewer than 4,000 individuals in the United States per year.
- **Humanitarian Device Exemptions (HDE):** Issued by the FDA; an approved HDE authorizes marketing of the HUD.
- Application provides info for FDA to make certain determinations (e.g., risk/benefits).

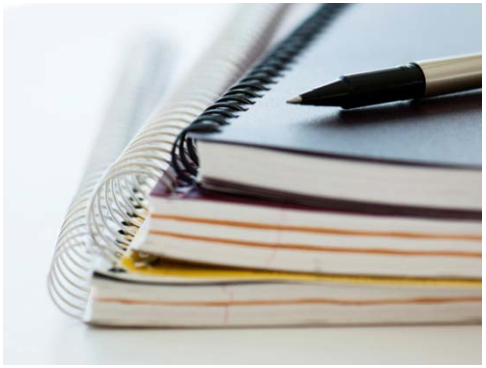
Humanitarian Use Device, cont.
GUI-36m

- **Requires IRB approval** prior to use - subject to full review.

- **Clinical Use**
  - ✓ Subject to continuing review/approval.
  - ✓ If applicable, the expedited procedure may be used at continuing review.
  - ✓ A consent form is not required.

- **Research Use** (if looking for a new indication) is considered a clinical investigation.

Resources



GUIDANCE/HRPP

GUI-6 – Emergency Use of a Test Article
GUI-19m – Expanded Access to Investigational Drugs/Devices
GUI-36m – Humanitarian Use Devices
FAQs on our website at www.humansubjects.stanford.edu
Chapter 5.8 HRPP, Expanded Access
Chapter 5.9 HRPP, Emergency Use of a Test Article

FDA Website

Devices - http://www.fda.gov/MedicalDevices/default.htm
Drugs - http://www.fda.gov/Drugs/default.htm

NEW Brochure – Expanded Access to Investigational (Test) Articles