Expanded Access
(including Emergency Use & Humanitarian Use Devices)

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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”

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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)

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DRUGS: Expanded Access

Criteria and Reporting/Monitoring Requirements
(not a complete list)

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

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**

- **Before IDE**
- **IDE Approval**
- **IDE Completion**
- **Marketing Approval**

- **Traditional IDE Study**
- **Emergency/Compassionate Use**
- **Treatment Use**
- **Continued Access**

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**Device: Expanded Access**

Criteria and Reporting/Monitoring Requirements

*(not a complete list)*

See GUI-19m for additional criteria and requirements

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<td><strong>Compassionate Use</strong></td>
<td></td>
</tr>
<tr>
<td>Existing concurrent clinical trial but patient does not meet inclusion/exclusion criteria</td>
<td>Follow-up report should be submitted to FDA as an IDE supplement</td>
</tr>
<tr>
<td><strong>Treatment Use</strong></td>
<td></td>
</tr>
<tr>
<td>No comparable or satisfactory alternative device/therapy to treat or diagnose that stage of the disease/condition</td>
<td>Semi-annual progress reports 21 CFR 812.150(b)(5)</td>
</tr>
<tr>
<td><strong>Continued Access</strong></td>
<td></td>
</tr>
<tr>
<td>Public health need or preliminary evidence of effectiveness</td>
<td>Request for extension submitted as IDE supplement</td>
</tr>
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*All require scheduled reporting to the FDA on follow-ups, progress, and event notifications*

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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.

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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**

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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