Special circumstances regarding expanded access at Stanford

In most circumstances, medical research using investigational drugs, biologics and devices requires IRB approval and must follow FDA regulations.

However, there are **expanded access** procedures for patients to obtain access to these investigational (test) articles in serious or life-threatening situations for treatment purposes.

Know the facts regarding
- emergency use,
- compassionate use,
- humanitarian use devices.

For more information contact:

Food and Drug Administration
1-866-1-866-300-4374
or 1-301-796-8240
www.fda.gov

Institutional Review Board (IRB)
Stanford University
http://humansubjects.stanford.edu
650-724-7141 (IRB Education) or
650-721-6399 (Main #)

Stanford IRB Expanded Access guidance

FDA: Expanded Access to Investigational Drugs (FAQ)

Expanded Access to Investigational (Test) Articles:

Emergency Use
Compassionate Use
Humanitarian Use

- ◊ **Drugs**
- ◊ **Biologics**
- ◊ **Devices**

Stanford University
Research Compliance Office

Expanded Access to Investigational (Test) Articles (Emergency, Compassionate Use) and Humanitarian Use of a Device

Emergency Use

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.

- **Contact manufacturer** for permission to use/ obtain test article (notify FDA if not possible)
- **Contact the FDA:** 1-866-300-4374 or 1-301-796-8240
- **NO PRIOR IRB APPROVAL** required. Report use to IRB within 5 working days

Criteria (all must be met):

- **Life-threatening/severely debilitating condition and no acceptable standard treatment is available**
- **No available IRB-approved protocol**
- **Potential benefits justify the potential risks to the patient**
- **Availability of investigational agent or device from sponsor**

Emergency Use of a Test Article is not research.

Consent Requirements Informed consent of the patient or their legally authorized representative is required, **unless both the investigator and a physician (not otherwise participating in the investigation)** can certify in writing:

- It's a life-threatening situation;
- Consent cannot be obtained from the patient (cannot communicate or is not competent to give consent), or from their legally authorized representative (unavailable or unknown); and
- No alternative approved treatment/therapy is available that provides an equal or greater likelihood of saving the patient's life.

Compassionate Use

Term used for provision of investigational products to patients outside of an ongoing clinical trial.

- **Contact the manufacturer** for permission to use/ obtain the test article
- **Contact FDA** to obtain an expanded access IND or IDE under treatment protocol
- **PRIOR IRB APPROVAL & CONSENT** required.

Examples:

- **Treatment IND**: Use of a drug being studied in a clinical investigation in patients not enrolled in the clinical trial.
- Single-Patient Treatment IND: Has the same requirements as a standard IND.
- Orphan Drugs: Drugs to treat rare diseases affecting less than 200,000 people in the US. See FDA Office of Orphan Products Development.
- Treatment IDE: Use of investigational devices on desperately ill patients when :

  - **Use is required to treat or diagnose a serious or immediately life-threatening disease or condition;**
  - **No comparable or satisfactory alternative device is available;**
  - **The device is under investigation in a controlled clinical trial for the same use under an approved IDE, or all clinical trials have been completed;**
  - **The sponsor of the controlled clinical trial is pursuing marketing approval/ clearance of the investigational device with due diligence.**

Humanitarian Use Device (HUD)

Intended to benefit patients by treating or diagnosing a disease or condition that affects fewer than 4,000 individuals in U.S. per year.

HUD regulations provide for Humanitarian Device Exemptions (HDE) by the FDA for marketing the HUD.

- **Contact FDA** if used off-label
- **Contact manufacturer** for HDE packet
- **PRIOR IRB APPROVAL** is required. *Research Consent* **is not required, but** *Clinical Consent* **is.**

The scope of the IRB approval is to confirm the planned use is consistent with the HDE's FDA-approved indication.

QUICK FACTS

EMERGENCY USE

>> Prior IRB approval is NOT REQUIRED <<

Written documentation of the emergency use must be submitted to the IRB **within 5 working days after** the use of the test article. Submit: *Emergency Use of a Test Article – Notification to the IRB , APP-11m* available at: http://humansubjects.stanford.edu

For information about **Emergency Use**

COMPASSIONATE OR HUMANITARIAN USE

>> Prior IRB approval IS REQUIRED <<

Submit an eProtocol application for IRB review.

Non-emergency information, contact IRB Education: 650-724-7141; IRBeducation@stanford.edu