Emergency Use
and Compassionate Use
of Unapproved Devices

Presented by Fabienne Santel, MD

Division of Bioresearch Monitoring
Office of Compliance
Center for Devices and Radiological Health
Food and Drug Administration
Learning objectives

- Understand when an emergency need to use an unapproved device may occur
- Describe the role and responsibilities of the physician who wants to use the device on a patient for emergency or compassionate use
- Identify the IRB’s responsibilities in the compassionate use of an unapproved device
Emergency use of an unapproved device

- What is an emergency situation
- Physician’s role and responsibilities before and after emergency use
- Informed consent
Compassionate use of an unapproved device

- Describe what is meant by compassionate use
- The physician responsibilities with compassionate use
- The sponsor and the IRB’s role in compassionate use
- Practice
Emergency use of an unapproved device

Emergency use of an unapproved test article is **not** research.
What is an emergency situation?

- All conditions must exist
  - Life-threatening disease or serious condition requiring immediate use
  - No generally accepted alternative for treating the condition is available
  - There is no time to use existing procedures to obtain FDA approval of an IDE.
When can a device be used in an emergency situation?

- There is no Investigational Device Exemption (IDE)
- Physician wants to use the device in a way not approved under an existing IDE
- A physician is not part of the IDE study
Physician’s role

- Determine whether the following conditions are met
  - Patient is in a life threatening situation
  - Immediate use of the device is needed
  - No alternative
  - No time for FDA approval of an IDE
Physician’s role

- Assess the potential for benefits from the unapproved use
- Have substantial reason to believe that benefits will exist
Physician’s responsibilities

- Physician should follow patient protection measures
  - Institutional clearance per institution policy
  - IRB chairperson concurrence
  - Authorization from the sponsor - if an IDE exists
Physician’s responsibilities

- Independent assessment by a physician who is not participating in the investigation
- Informed consent from patient or legally authorized representative
  - Does not have to follow the informed consent requirements at 21 CFR 50.25
What if there is no time to find an uninvolved physician? (21 CFR 50.23)

The physician makes the determinations
- Life threatening disease or condition
- Immediate need
- No alternative
- No time for FDA approval of an IDE
- Assessment of potential benefit
- Substantial reason to believe benefit will occur
Physician’s responsibilities

- The physician has his/her evaluation reviewed and evaluated in writing by an uninvolved physician.
- Submit that report to IRB within 5 working days after the use.
What if **No** informed consent can be obtained? (21 CFR 50.23)

The physician and a physician who is not participating in the clinical investigation must certify in writing ALL of the following:

– Life-threatening situation necessitating the use of the device
– No alternative therapy
Physician’s responsibilities after emergency use

- If an IDE exists, notify the sponsor
  - The sponsor must report to FDA
- If an IDE does not exist, notify FDA of the emergency use and provide FDA
  - a written summary of the emergency use,
  - patient protection measures, and
  - any scientific results
Physician’s responsibilities after emergency use

- Report to the IRB within five days and otherwise comply with IRB provisions
- Evaluate the likelihood of a similar need for the device
- If similar need is likely, obtain an IDE from FDA for the subsequent use and IRB approval
Compassionate use of an unapproved test article

- Single patient or small group use of an unapproved device
Compassionate use of an unapproved device

- Compassionate use is not research
- Unapproved device for serious disease or condition
- No alternative
- Patient does not meet inclusion criteria
Physician’s responsibilities

- A physician can use an unapproved device to treat, diagnose, or monitor a patient with a serious disease or condition.
- The probable risk to the patient is not greater than the probable risk from the disease.
Physician’s responsibilities

- Physician requests authorization from sponsor
- Sponsor may agree or disagree
- The physician should not treat the patient until FDA concurs with the use
Physician’s responsibilities

- Devise schedule for patient monitoring
- Address specific needs of the patient
- Detect possible problems
Physician’s responsibilities

- Obtain independent assessment from uninvolved physician
- Obtain IRB chair’s concurrence
- Clearance from institution, if appropriate
- Obtain consent from the patient
- Report any problems as a result of the device use to the IRB and sponsor
- Write a summary of the use and give to sponsor
Sponsor responsibilities

- If sponsor disagrees with the use
  - The physician cannot use the device

- If sponsor authorizes the use
  - An IDE supplement is submitted to FDA requesting approval for a protocol deviation [21 CFR 812.35(a)].
Sponsor responsibilities

- IDE Supplement
  - FDA’s concurrence is based on:
    information submitted; evidence that safety and effectiveness justifies the use; the use would not interfere with the conduct of a clinical trial to support marketing approval

- Prior FDA concurrence is required before compassionate use occurs
IRB and compassionate use

- IRB chair concurrence (documented)
- Ensure FDA concurrence
- Review consent document
- Receive reports after the use
- Receive reports of problems
Let’s put this into practice

- Interventional Cardiologist completed IDE study of a stent
- Continued to use the stent in his patients (>10) when he found coronary thrombosis. He declared this emergency use.

What would concern your IRB?
Let’s put this into practice

- On an FDA inspection, we found that a physician used an IDE device for compassionate use. There was no FDA or IRB concurrence.

How can an IRB help prevent this from happening?
Let’s put this into practice

- Approved biliary stent used in the carotid artery for carotid stenosis.
- Physician said he did this to treat his patient.

Is this emergency use or compassionate use or neither?
References for emergency use

- **Regulation**
  - 21 CFR 56.104(c)
  - 21 CFR 50.23
  - 21 CFR 812.35(a)(2)
  - 21 CFR 812.150(a)(4)

- **Guidance**
  - Federal Register/vol.50, No. 204/Tuesday, October 22, 1985
  - FDA Information Sheets
References for compassionate use

- Food, Drug, and Cosmetic Act
  - Section 561 Expanded Access to Unapproved Therapies and Diagnostics
- Regulation
  - 21 CFR 812.35(a)
- Guidance
  - www.fda.gov/cdrh/devadvice/
    type in “Expanded Access” and search
Key points

- Physicians can use unapproved devices in emergency situations
- IRBs must receive reports within five working days
- Subsequent emergency use needs IDE
- Compassionate use requires prior FDA and IRB concurrence
- In compassionate use, IRB’s should: document their concurrence, ensure FDA concurrence, receive and review reports