Expanded Access 102

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Reminder…What is Expanded Access?

- Pathway for patients with serious or immediately life-threatening condition
- Allows the use of an investigational medical product for treatment
  - Drug, biologic, or device
- Emergency Use & Compassionate Use are types of Expanded Access

Other reminders…

- How is this presentation different?
- Clarification of what is a treatment vs a clinical trial (Why does this sound so much like a clinical trial when it is *clinical investigation?*

Definitions… understanding Form 3926

An individual patient under a sponsor IND if they are using a treatment or drug for an extended length of time.

Time doesn’t dictate. Could be one time use, could be until the drug/biologic is approved.

Safety and Efficacy – The FDA perspective…

- Everyone should be in the clinical trial instead of only the expanded access pathway.

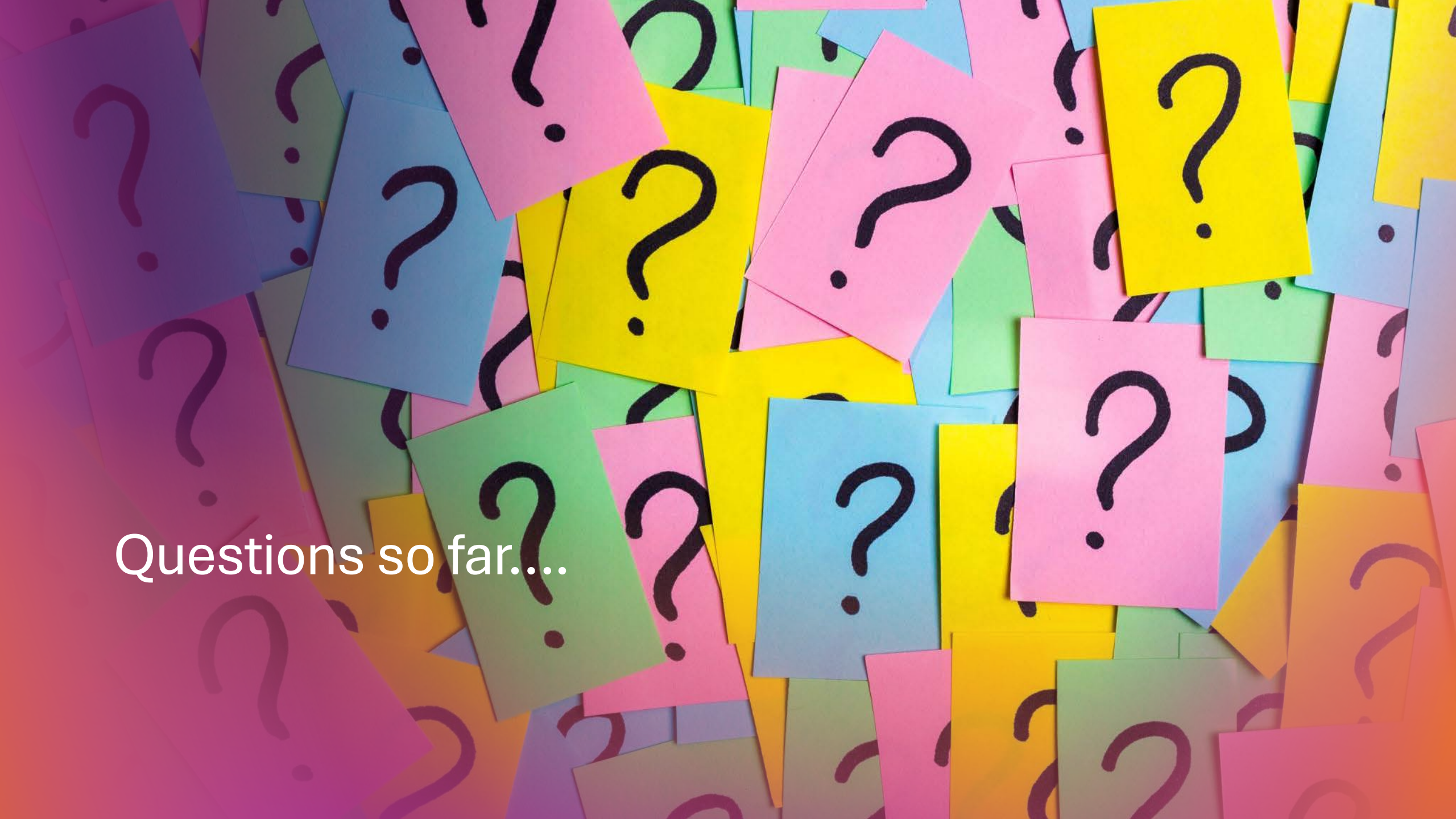
Where does the responsibility lie? …maybe both?

Physician

- Manufactures approval that will go to the IRB and FDA
- If an FDA Expanded access IDE is obtained, this can be given to the IRB
- Obtain instructions for use (IFU)
- Develop informed consent form
- Submit to IRB

IRB

- Qualification of the Physician (may require additional documentation depending on if the IRB is at the institution or not)
- Review of the IRB Application


Questions so far....


Case Study
Device (Knee
arthrodesis
implant device)

Case Study: Compassionate Use of a Device

- With limited treatment options, this 70 + year old with a history of knee replacement, hip fracture, and recurrent urinary tract infections, and experiences recurrent knee infections.
- Despite numerous joint procedures and antibiotics problems continued
- the knee replacement was removed and replaced with an antibiotic eluting cement spacer until a permanent treatment was identified.
  - (the spacer contains a drug that gives off a little antibiotic at a time)
- The treating physician elected to pursue the compassionate use of the MUTARS arthrodesis implant device.

Approvals Needed for Compassionate Use	What happens?	Who goes first?	Can there be a discussion ?
Manufacturer	Approve the use of the device for expanded access	Physician contacts manufacturer	Maybe – between physician and manufacturer
IRB	Concurrent approval	IRB will always wait for the FDA for final approval.	Physician and IRB may discuss treatment plan and other clinical care
FDA		FDA might want to see the information that affects the IRB submission (eligibility/ICF/Treatment plan)	Physicians may provide information to the FDA that is different than what the FDA recommends with appropriate justification. Things might not be regulatory (patient care – treatment decision and the FDA listens!)

Who submits to the FDA for the Compassionate Use of a Device?

- If existing IDE – IDE Sponsor submits to FDA
- If no IDE – Sponsor or Sponsor-Investigator submits to FDA:
    - Description of the device provided by manufacturer
    - Description of the patient's condition and circumstances that necessitate treatment
    - Discussion of why there argroup,lternatives
    - Discussion of why the risk is no greater that the probable risk from the disease or condition
    - Additional radiographic imaging
    - Patient protection measures
        - Independent physician assessment
        - Authorization for use from manufacturer
        - Institutional clearance
        - IRB approval/IRB Chair concurrence
        - Informed consent draft (should be expanded access specific)
        - Patient monitoring plan

******If a small group, you must have a protocol******

What's Submitted to the IRB for the Compassionate Use of Device?

- IRB Submission
  - Protocol/treatment plan
  - Informed consent document
  - Instructions for use (IFU)
  - Manufacturer authorization
  - IRB Intake form
- IRB approval may be conditioned on FDA approval
- Can use an IRB Chair concurrence if approved by FDA

When does Compassionate Use become a Treatment IDE?

Size

- Compassionate Use is 1 patient or a small group
  - FDA does not define “small group”
- Only guidance is that a “small group” is less than the size of a clinical trial
- Based on past experience, likely pretty small (under 50)
- Treatment IDE is more like a clinical trial

Clinical Trial Status

- Expanded Access is always for:
  - Patients when there is no clinical trial; or
  - Patients who don’t qualify for a clinical trial
- A Treatment IDE can only be approved by the FDA when clinical trials data suggest the device is effective

What's the Process for a treatment IDE for a Device?

1. Device must be under investigation in a controlled clinical trial for the same use under an approved IDE
    1. Alternatively, all trials may be completed
2. The sponsor of the trial(s) is pursuing marketing approval or clearance of the investigational device with due diligence
3. Sponsor or Sponsor-Investigator submits full IDE to FDA
4. IRB reviews like any other IDE study
    - Full board review required, no pathway for chair or designee review
    - Approval may be conditioned on FDA approval
    - Can rely upon another IRB

Questions about Expanded Access/Compassionate Use for Devices???

Resources

- The FDA Expanded Access webpage
  - Summarizes categories and initial requirements of Expanded Access process to include information for treating physicians and patients
- Expanded Access consent templates:
  - UC San Diego
  - UC Davis
- Emergency Use Worksheets:
  - UC San Diego
  - UC Davis