Monday Morning Practice Pearls #70

What is an Expanded Access Use IND/IDE (also called "Single Patient" or "Compassionate Use") ?

Per NIH Policy 502: Expanded Access, Including Emergency Use of Investigational Drugs, Biologics, and Medical Devices (Test Articles): A potential pathway for a patient with an immediately life-threatening condition or serious disease or condition to gain access to an investigational medical product (drug, biologic, or medical device) for treatment outside of clinical trials when no comparable or satisfactory alternative therapy options are available. Expanded Access Use must comply with FDA regulations pertaining to INDs/IDEs.

Per FDA website on Expanded Access, this use may be appropriate when all the following apply:

- Patient has a serious disease or condition, or whose life is immediately threatened by their disease or condition.
- There is no comparable or satisfactory alternative therapy to diagnose, monitor, or treat the disease or condition.
- Patient enrollment in a clinical trial is not possible.
- Potential patient benefit justifies the potential risks of treatment.
- Providing the investigational medical product will not interfere with investigational trials that could support a medical product's development or marketing approval for the treatment indication.

Requirements for using an investigational product for expanded access:

- Manufacturer/supplier approval for expanded access use of the investigational medical product – as needed, should be requested by treating physician
- Sponsor authorization for expanded access use
    - OSRO policy website
        - Policy 404: Manufacturer Expanded Access Policy
        - Policy 409: Single Patient Expanded Access Policy
    - CTEP Investigator's Handbook, Appendix VIII: National Cancer Institute Procedure of Investigational Agents Acquired for Special Exception Treatment of Individual Patients
- FDA approval for expanded access use – may be done by Sponsor
- Notify pharmacy that expanded access has been requested for investigational IND agent
    - General email to IDCU: CC-PHARIDCU@mail.nih.gov
    - Must provide treatment plan (same information that is provided to IRB)
    - Provide any other drug specific documents that may be available (e.g., Investigator's Brochure)
- Obtain IRB approval or IRB Chair concurrence for expanded access use
    - Submit plan for use of investigational agent, including a brief summary of patient status and need for expanded access use, treatment plan, duration of treatment, monitoring procedures, safety monitoring and reporting (these do not get posted in Protocol View)
    - Use Expanded Access Consent template on the NIH Consent Templates and Guidance website

    Note: PSO has templates/examples for each of the above.

    - Submit IND Sponsor authorization (if applicable) and FDA approval
- Once IRB approval is received in writing
    - Notify Sponsor or manufacturer/supplier (as needed)
    - Contact pharmacy for investigational agent
    - Document in CRIS that patient requires expanded access (has serious/life-threatening disease, has undergone standard treatments without success, is ineligible for other ongoing trials, there is evidence of a potential benefit)
    - Consent patient using paper consent form (these consents do not get uploaded in iMED). Also, these patients do not get registered in PRES.
    - Document in CRIS: consent process, on-treatment procedures, study drug administration, AEs, etc., as usual
    - There are no "order sets" in CRIS for expanded access protocols so the PI/designee must enter required CRIS orders
- Refer to IRB outcome letter for reporting requirements (e.g., follow up with IRB within 5 days of use of the investigational product)
- Track SAE/AEs separately from any other study database
    - Report SAE/AEs to Sponsor/manufacturer per agreement
    - Report events to NIH IRB in accordance with NIH Policy 801
- Notify the IRB when treatment is complete, or if treatment is longer than 12 months, ensure ongoing IRB review and approval for expanded access protocols.
- Update Sponsor/manufacturer per their requirements
- Update the FDA per their requirements – consult with Sponsor

Please note:

- Expanded access is not considered research. The results/outcome(s) for the patient do not get included in the publication of any other ongoing/future clinical trial.
- There is no posting to ClinicalTrials.gov for expanded access and no results reporting per FDAAA.

Note, per NIH Policy 502: "Emergency Use is 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 Institutional Review Board (IRB) approval.**"