Ethical and Regulatory Issues Regarding Expanded Access

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Background

So What is Expanded Access?

- An investigational drug program through the FDA
  - Allows patients to access unapproved drugs for treatment purposes outside clinical trials (while data is collected to support approval)
  - Requests are either approved or denied at 3 points by the FDA, drug company, and the IRB

Project Overview

Project Design

- A preliminary study looking at 10 years worth of Expanded Access requests to the Penn IRB.
  - Evaluating application material, consent forms, letters to the IRB, FDA, and the drug company

Aims

- Exploring access issues
  - Who is being advised/ offered these drugs?
  - Are there certain departments utilizing EA more than others

- Are there any areas of improvement for EA at Penn
- To see if the data generalizable
  - The forms used by the Penn IRB, are they looking for the same information as other institutions?

Significance of Study

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- There is a limited amount of information on Expanded Access, so this study seeks to add more information.
  - Any information is useful.
  - It could answer questions in regards to access to Expanded Access. However, do we even WANT everyone to be able to access Expanded Access?
  - It could reveal institutional barriers, such as illuminating whether some departments use it more than others.

Methods

Sources of Data

Penn HSERA

- Submission system for the IRB
- Contains documents in relation to expanded access requests submitted to the IRB

Penn ERA

- Main database for tracking the forms
- Documents similar in theme to Penn HSERA

Our task: organizing and collecting data from both sources.

Data Collection Methods

Creation of the codebook
Our mentors provided us with a list of Expanded Access requests from the past five years, excluding devices.

1

Forming a data consolidation method
Based on data from May 2020, we formed headings to an excel table.

2

Consolidating the Data
Using this excel table, we created a master document that included data from both databases.

3

Analysis
After getting through 5 years worth of data, we began analysis.

4

Findings & Limitations

Preliminary Findings

1. Probable correlation between PI experience and the amount of investigational drug requests
    - Most request came from Infectious Disease and Cancer/Oncology department

1. There were two main drugs being requested: Clofazimine and Sanguinate
    - Sanguinate, a blood substitute, was mainly used by people identifying as Jehovah's witnesses
    - Clofazimine was the most requested drug used to treat ***Mycobacteria***
        - There has been successful clinical experience using this drug

Gaps in Data/ Future Areas of Research

1. There was a lack of demographic information beyond gender and age
    - Unable to identify the populations using drugs through EA
2. Absence of material
    - Forms from FDA and IRB were either not added to the database or were not complete
    - Whether or not a drug had a trial occuring or not
        - What phase the drug was in for clinical trials
3. Working with more IRBs to make research more generalizable

Limitations

- The data was very messy.
  - No consistency
    - Penn HSERA and Penn ERA having no files, or one file.
  - Some files couldn't be opened at all.
- No set protocol until 2019
  - The current IRB submission form for EA is relatively new and older protocols did not include one.

Lessons Learned

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- Research takes time (and can be very tedious!).
- Research is also very messy.
- Before you know what the data looks like, you may have questions that you ultimately cannot answer with the data you have.

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Any questions?