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Workshop 1

How Can Registries Contribute to the Development and Evaluation of CNS Therapeutics?

Workshop Organizers

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Participants

- Mike Markowitz
 - Sofija Jovic
 - Steve Sainati
 - Gregory Rosas
 - Adam Haim
 - Pharmaceutical companies
 - Support services (eg ratings)
 - Government
 - Independent investigators
- Larry Adler
 - Bryce Kasuba
 - Diane Robertson
 - Michael Ropacki
 - Mike Weiser

The goals of the workshop

- To determine if registries can contribute to the development and evaluation of CNS therapeutics
 - ▣ under what circumstances?
 - ▣ for what goals?
- To determine whether registries can provide efficiencies with the drug development process and inform clinical trials
- To identify circumstances under which registries can augment or even replace clinical trials

IN ORDER TO ADDRESS THESE GOALS:

- ❑ Define what we mean by a “registry”
 - ❑ Hybrid
- ❑ Determine the characteristics of registries that may (or may not) make them useful to clinical trials

Define Registries

What are the different kinds of Registries?

- **Disease registry**

A cohort study that follows patients with a specific disease or condition longitudinally to identify risk factors, treatments, disease progression, mortality

- **Disease Surveillance**, including incidence estimation (e.g., tumor registries)

Identification of new cases to estimate incidence or prevalence

What are the different kinds of registries?

- **Exposure registry**

A cohort study that follows patients starting a specific treatment longitudinally for outcomes such as adverse events, quality of life, healthcare resource use

- **Risk Management program (e.g., clozapine , Sabril)**

Enrollment of all patients treated with a specific pharmaceutical/biologic product into a risk mitigation program to ensure safe use conditions. Additional data re: adverse events may be collected

What are the different kinds of registries?

- **Directory of Potential Clinical Trial Participants**

Identification of patients who may qualify for a clinical trial

- **Individual “databases”** generated by hospitals based on their catchment area or intake clinics

Case series of patients with specific conditions or characteristics

What are the different kinds of registries?

- **Data registries attached to biological specimen repositories**
Cross sectional or longitudinal data (generally anonymized) that are collected in relation to collection of biologic specimen collection
- **Claims databases and electronic health records (e.g., Kaiser healthcare network)**
Use for disease and exposure registries

Implications for Clinical Trials

Which kinds of registries (if any) make the most sense for efficiency and effectiveness of clinical trials to develop and evaluate CNS treatments?

Implications for Clinical Trials

Consider

- Are there data that are collected as part of a registry that can be used independently to streamline recruitment into a trial (e.g., screen subjects) or reduce amount of data collection once enrolled into a trial (e.g., demographics, systematic diagnostic information, medication and treatment course history).
- Can registry data be accessed to provide outcome information in lieu of a clinical trial?

Implications for Clinical Trials

Statistical Considerations

- What biases are inherent in a registry in terms of who the participants are?
- What criteria can be used to evaluate a registry?
- How can that bias be made known and thereby evaluated?
- What are the statistical implications in the sampling?

Implications for Clinical Trials

Detailed Statistical Considerations

- Can a registry be a representative sample of the particular population?
- How does the use of a registry affect generalizability?
- Is enrollment of registry participants into clinical trials equivalent to other approaches to recruitment of participants for enrollment in clinical trials?
- If registries are used to identify participants for enrollment into clinical trials, should this be the sole source of patients or just one of several resources for a trial? Should there be an upper limit on the proportion of entrants to a trial from a registry?
- Would the use of several small registries constitute “cluster randomization?”

Logistical Considerations pertaining to registry establishment and maintenance

- Management
- Maintenance
- Quality Control
- Standards and Standardization
- Legal
- Ethical
- Financial
- Access

Potential Goals

- Understand the value of registries and what they can contribute to clinical development and post marketing
- Identify opportunities for public/private partnerships
- Encourage companies to work together and with public agencies to develop standards and quality