

Day two-session B.2

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B.2. Clinical Research, Patient Registries, Patient Care and Disease Management

Session Overview

Clinical research in rare diseases is increasingly leading to development of novel drug and genetic therapies and advances in disease management. There are numerous types of research activities in which patients with rare diseases can participate, including patient registries. This session will include in-depth discussions of the current regulatory environment and clinical research in rare diseases, particularly focusing on various aspects of patient registries such as design, applications, value to patients and physicians, and the impact of registry data on improving patient care.

Goals of the Session

- Understand the impact that recent legislation and governmental initiatives have on clinical research.
- Understand the various types and value of clinical research studies/trials for which patients with rare diseases may be recruited to participate.
- Understand the various designs and applications of patient registries for rare diseases and how they are used for clinical research and patient care.
- Understand how data reported from patient registries to patients and physicians can contribute to improved patient care.

Questions and Topics to Discuss

- What potential impact will regulatory agencies and other governmental influences have on rare disease clinical research? Will there be an impact on participation in a standardized rare disease patient registry?
- What are the types of clinical research studies/trials, including patient registries, in which patients with rare diseases can participate? How do they advance the understanding and management of rare diseases?
- How can a standardized rare disease patient registry interface with and facilitate rare disease clinical research?
- What are the benefits for patients, parents, healthcare providers, investigators, and advocacy groups to participate in a standardized rare disease patient registry? What are the benefits to participation in a disease-specific patient registry? How will disease-specific patient registries interface with a standardized rare disease registry?

- What are some of the applications of patient registries that can contribute to improved patient care? Identify unmet patient needs?
- With some of the more rare diseases, will there be enough patients to provide adequate patient enrollment for the increasing numbers of pre-approval clinical trials and mandated post-approval safety and comparative effectiveness studies of drug treatments?



Uniting Rare Diseases



Breakout session B.2 Clinical trials/research studies and patient care management

Goals, scope, architecture for a united registry system

- Interactions of existing and future registries
- Application of registries, ex: locator
- Point of origination
 - Clinician entered vs patient entered
 - Flow and entry in existing systems (health care systems, etc)
 - Quality of data
- Models
 - Copy
 - Cloud
 - Search

Stakeholders Benefits

- Ongoing communication with registry participants (return of registry data & research findings)
 - Attrition & longitudinal data collection
- Economy of scale:
 - Research – patient participation with quality & quantity
 - Advocacy groups – best practice(s) and resource efficiency

Important points to consider

- Stakeholder incentives
 - Value of data sharing
- Ascertainment bias
- Registries are dynamic

United rare disease registry

Suggestions:

- **Incremental and stepwise development for registry**
 - List of registries
 - A registry of registries (united model)
 - Use of existing applications (IT, etc)
 - Evaluation of existing models – to model stages of registry design