Application of Bayesian Studies in Rare Disease Settings
Webinar

Rare Diseases Forum

January 22, 2020
Agenda

- Moderator:
  - Veronica Miller, PhD, Executive Director Forum for Collaborative Research

- Brief Introduction & Overview

- Presentations
  - Karen Price, PhD
  - Satrajit Roychoudhury, PhD
  - Kelley Kidwell, PhD

- Moderated Discussion

- Conclusion and Next Steps
The Forum for Collaborative Research
Disease Areas

- The HBV Forum
- The HIV Forum
- The TAVI Forum (Transplantation Associated Viruses)
- The Liver Forum
- The PSC Forum
- The Rare Diseases Forum
The Rare Diseases Forum

https://forumresearch.org/rare-diseases-forum
The Rare Diseases Forum

- Advance/facilitate drug development for diagnosis and treatment of rare diseases
  - Independent and neutral venue for ongoing stakeholder dialogue

Objectives:

- Build scientific knowledge and develop common understanding of issues and potential solutions
- Maximize efficiency through collaboration
- Make use of innovation and use of novel analytics and efficient/rigorous evaluation of novel biotechnology
The Concept

- Enhanced clarity
- Innovation
- Collaboration
- Redundancy
- Development time
- Risk

“Starting with HIV, the Forum consistently and effectively applies the multi-stakeholder model to advance the development path for new treatments in multiple disease areas.” Eric A. Hughes, MD, PhD, Development Unit Head, Novartis Pharma AG
Characteristics

- Non-competitive
- Safe environment
- Independence
- Neutrality
- Transparency
- Information democracy
- Synergy vs duplication
- Equal voice

The traditional process:
Single sponsor communicating independently with single regulatory agency

The Forum process:
All sponsors communicating at the same time with multiple regulatory agencies

“The Forum bring together stakeholders from around the globe with cross-Atlantic and -Pacific perspectives to reduce interagency discordance.”

Laurent Fischer MD, Senior VP, Allergan
The Rare Diseases Forum

Workstream I

- Science of Small Trials: Study Design and Treatment Outcome Assessment
  - **WG 1**: Innovative scientific approaches
    - A: Scientific approaches when rarity, the severity of the condition, and the degree of unmet need are prominent features.
    - B: Scientific considerations to use of Enrichment and Randomized Withdrawal and other innovative study designs and approaches.
  - **WG 2**: Innovation in assessing outcomes
    - A: Clinically meaningful outcomes that take advantage of the whole range of potential outcome.
    - B: Innovative tools/technologies and lessons from related disciplines to assess outcomes affecting multiple functional domains, such as video capture and wearable devices (Co-chair: Elin Haf Davies).
  - **WG 3**: “Encyclopedia” of innovation

Workstream II

- Innovation in Clinical Trial Design
  - **WG 1**: Data quality and consistency/Fit-for-Purpose data
  - **WG 2**: Master protocols for RD
  - **WG 3**: Bayesian Methods in RD
Workstream II – WG 3
Application of Bayesian Studies to RD
Today’s goals

- Introduce the key features of Bayesian Methods
- Provide some case studies to illustrate
- Discuss application to Rare Diseases
  - When appropriate?
  - When not appropriate?
- Plan for next steps
  - Ongoing working group discussions
Presenters
Dr. Karen Price
  - Lead, Statistical Innovation Center, Eli Lilly & Company

Dr. Satrajit Roychoudhury
  - Senior Director, Statistical Research & Data Science Center, Pfizer

Dr. Kelley Kidwell
  - Associate Professor, Dept. of Biostatistics, University of Michigan