THE FORUM
For Collaborative Research℠

Application of Bayesian Studies in Rare Disease Settings

Webinar

Rare Diseases Forum

January 22, 2020

Berkeley Public Health

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

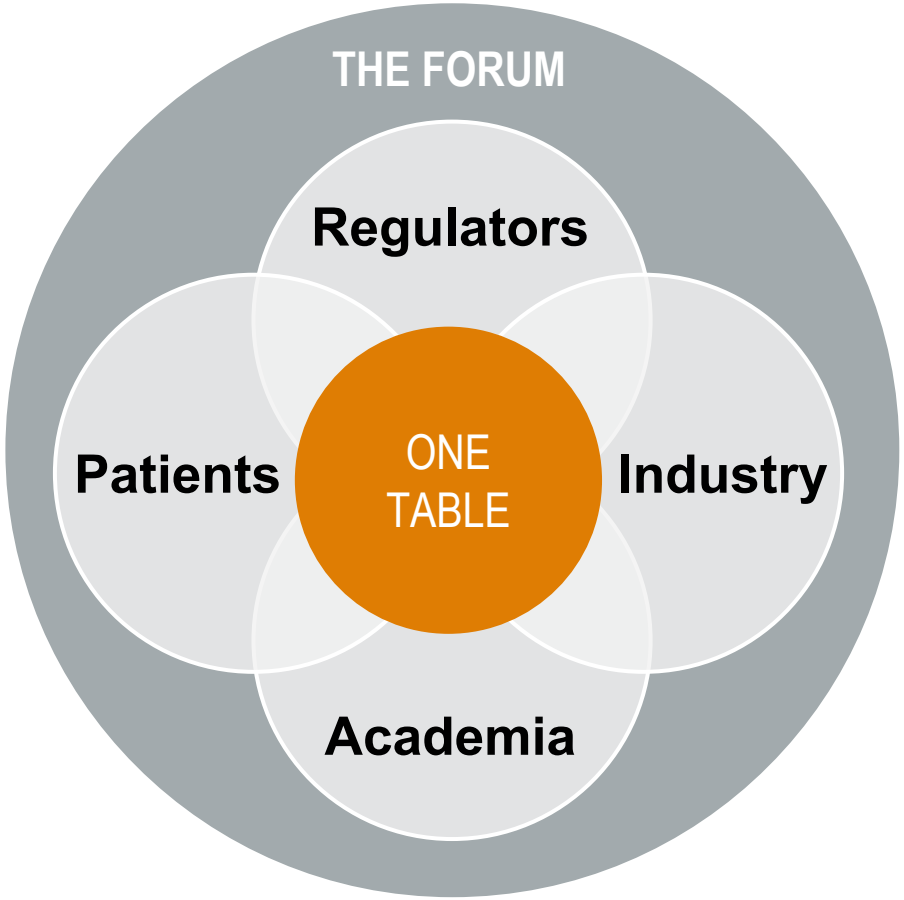
The Rare Diseases Forum

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 biotechnolgy

The Concept



Increase Efficiency

- Enhanced clarity
- Innovation
- Collaboration

Decrease Uncertainty

- 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 stake holders from around the globe with cross-Atlantic and -Pacific perspectives to reduce interagency discordance."

Laurent Fischer MD, Senior VP, Allergan

The Rare Diseases Forum

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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