Duke | MARGOLIS CENTER for Health Policy

Data Sharing to Accelerate Therapeutic Development for Rare Diseases

Virtual (Zoom)
August 18 & 19, 2020

Agenda

Day 1 | Sustaining a Robust Data Sharing Infrastructure for Rare Disease Drug Development

1:00 p.m. **Welcome and Overview**
Marta Wosińska, Duke-Margolis Center for Health Policy

1:10 p.m. **FDA Opening Remarks**
Theresa Mullin, U.S. Food & Drug Administration

1:20 p.m. **Session 1: Benefit of Shared Data in Rare Disease Characterization and Drug Development**

Objectives:

- Discuss the importance of shared data to support innovation and quality in the drug development pipeline for rare diseases.
- Identify and discuss the benefits of shared data in disease characterization and trial design as well as the impact of siloed, proprietary data on basic and translational research.
- Discuss current areas of unmet need in clinical research for rare diseases and how data sharing could supplement efforts to advance therapeutic development.

Presentations

- **Tiina K. Urv**, National Center for Translational Science, National Institutes of Health
- **Katie Donohue**, U.S. Food and Drug Administration

1:50 p.m. **Session 2: Leveraging Shared Data Sources for Rare Disease Drug Development**
Moderator: Marta Wosińska, Duke-Margolis Center for Health Policy

Objectives:

- Discuss landscape of potential shared patient-level data resources available to inform rare disease drug development.
- Discuss the utility of rare disease registry data in disease characterization and the development of targeted therapeutics.
- Discuss approaches to the integration of aggregate data sets in clinical trials, including considerations for primary data collection and database management.

Presentation

- **Karla Childers,** Johnson & Johnson

Reactant and Panel Discussion (50m)

- **Michelle Campbell**, U.S. Food & Drug Administration
- **Vanessa Boulanger,** National Organization of Rare Disorders
- **James M. Wilson,** University of Pennsylvania
- **Patroula Smpokou,** U.S. Food & Drug Administration
- **Petra Kauffman,** AveXis, Novartis

Open Discussion (20m)

3:15 p.m. **Break**

3:30 p.m. **Session 3: Ensuring Quality in Shared Data Resources— Data Interoperability, Protection, and Management**
Moderator: Marta Wosińska, Duke-Margolis Center for Health Policy

Objectives:

- Discuss common approaches to ensure high quality data collection for input into shared data resources, including data standardization and personal data management considerations as well as the utility of dynamic informed consent models.
- Discuss the role of regulatory agencies in strengthening rare disease data collection and shared data resources.

Presentation

- **Jeff Barrett,** Critical Path Institute

Reactant and Panel Discussion (50m)

- **Laurie Conklin,** ReveraGen
- **Sam Hume,** CDISC
- **Ron Bartek,** Friedreich's Ataxia Research Alliance
- **Shrujal Baxi,** Flatiron Health
- **Maria Matilde Kam,** U.S. Food & Drug Administration

Open Discussion (20m)

4:55 p.m. **Day One Closing Remarks and Adjournment**
Marta Wosińska, Duke-Margolis Center for Health Policy

Day 2 | Collaborative Research Approaches and Analytics Platforms to Support Drug Development and Address Challenges in Rare Disease Data Sharing

1:00 p.m. **Synopsis of Day One Discussion**
Mark McClellan, Duke-Margolis Center for Health Policy

1:10 p.m. **Session 4: Platform Analytics Tools to Support Rare Disease Drug Development**
Moderator: Mark McClellan, Duke-Margolis Center for Health Policy

Objectives:

- Provide an overview of the C-PATH Rare Disease Cures Accelerator-Data and Analytics Platform (RDCA-DAP) and discuss the utility of RDCA-DAP patient-level data and analytic tools in the development of disease models and other drug development tools to support innovative trial design for rare disease drug development.
- Discuss general challenges to dataset interoperability and ensuring the standardization of common data elements across trials for different rare diseases.
- Discuss general challenges securing multi-stakeholder collaboration and funding for the maintenance of shared data resources.

Presentation

- **Jane Larkindale**, Critical Path Institute

Reactant and Panel Discussion (45m)

- **Rebecca Li,** Vivli
- **Mike Feolo,** National Institutes of Health
- **Mads Dalsgaard,** Lundbeck
- **Atul Bhattaram**, U.S. Food & Drug Administration

Open Discussion (20m)

2:30 p.m. **Break**

2:45 p.m. **Session 5: Collaborative Research Networks to Support Rare Disease Drug Development**
Moderator: Mark McClellan, Duke-Margolis Center for Health Policy

Objectives:

- Provide an overview of the NIH Rare Diseases Clinical Research Network (RDCRN) and discuss the value of multi-center research collaborations to support data collection and data sharing in rare disease research.
- Discuss challenges with the use of shared data in clinical research, including any limitations associated with data collection practices and data anonymization.

Presentation

- **Eileen King,** RDCRN Data Management and Coordinating Center, Cincinnati Children's Hospital Medical Center

Reactant and Panel Discussion (45m)

- **Klaus Romero,** Critical Path Institute
- **Nicole Hamblett,** Seattle Children's Research Institute
- **Ian Winburn,** Pfizer
- **Dina Zand,** U.S. Food & Drug Administration

Open Discussion (20m)

4:05 p.m. **Session 6: Synthesis and Next Steps**

Moderator: Mark McClellan, Duke-Margolis Center for Health Policy

Objective:

- Discuss next steps and stakeholder roles in facilitating pre-competitive data sharing to support disease characterization and innovative clinical trial design in rare disease drug development.

Panel Discussion (45m)

- **Anne Pariser,** National Center for Translational Science, National Institutes of Health
- **Billy Dunn**, U.S. Food & Drug Administration
- **Vanessa Boulanger,** National Organization of Rare Disorders
- **Klaus Romero,** Critical Path Institute

4:50 p.m. **Day Two Closing Remarks and Adjournment**

Mark McClellan, Duke-Margolis Center for Health Policy

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