THE FORUM
For Collaborative Research℠

The Rare Diseases Forum Innovation in Trial Design Workshop

Veronica Miller, PhD

Forum for Collaborative Research

UC Berkeley School of Public Health

September 23, 2019

Washington, DC

Berkeley Public Health

Thanks & Acknowledgments

The Forum for Collaborative Research

Catalyzing Clinical Research to Improve Global Health

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Guiding Principle

Once new drug candidates and therapeutic strategies are identified, their efficient and safe development is in the best interest of all stakeholders, most of all, the patients.

"The Forum accelerates drug development by increasing efficiency through collaboration, not by lowering standards."

Veronica Miller PhD, Executive Director, The Forum for Collaborative Research

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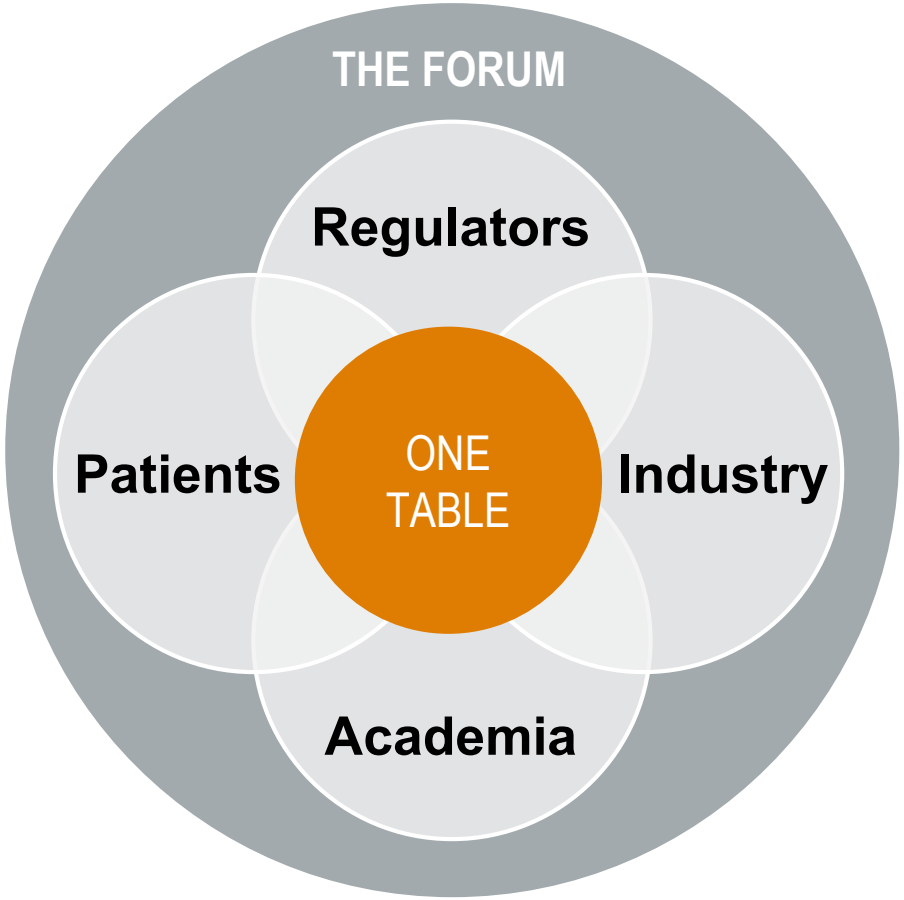
The Concept



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

Disease Areas

- In order of appearance
  - The HIV Forum 1997- present
  - The HCV Forum 2006-2016 (completed)
  - The Liver Forum 2014 - present
  - The CMV/transplantation Forum 2014- present
  - The HBV Forum 2016 - present
  - The PSC Forum 2017 - present
  - **The Rare Diseases Forum 2018 →**

Results

- Advance development of regulatory strategies
  - Evolving science and evolving consensus
- Generate evidence through collaboration
  - Efficient use of data
- Provide mechanism for patient-centered drug development
- Provide mechanism for innovation in data use and analytics

"The Forum addresses cutting edge regulatory science and policy issues with proven results."

George Hanna MD, VP Infectious Diseases Global Clinical Development, Merck & Co., Inc.

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Rare Diseases Forum Steering Committee

- **Co-Chairs**
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  - Industry: John F. Crowley Amicus Therapeutics
  - Forum: Veronica Miller PHD UC Berkeley

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    - Dina Zand MD CDER/DGIEP
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Rare Diseases Forum Workshop 1
Innovation in Trial Designs " Bringing Lessons from the Oncology Experience to Rare Diseases"
Planning Committee Members

Barry Byrne, MD, University of Florida

Chaohong Fan, MD, FDA/CBER

Dina Zand, MD, FDA/CDER

Ke Liu, MD, FDA/CBER

Keith Gregg, PhD, BioMarin

Laura Lee Johnson, PhD, FDA/CDER

Marie-Laure Nevoret, MD, Regenxbio

Nick Kenny, PhD, Syneos Health

Olivier Danos, PhD, Regenxbio

P.J Brooks, PhD, NIH/NCATS

Rachel Witten, MD, FDA/CBER

Sandi Lehrman, MD, Patient Advocate

Stephen Pakola, MD, Regenxbio

Tim Miller, PhD, Abeona Therapeutics

Vivian Fernandez, Regenxbio

Forum RD Team

- Luis Javier Hernandez
  - Rare Disease Forum Project Manager
- Leighla Dergham
  - U Maryland Intern
- Terry Daniels
  - Office Administrator
- Brenda Rodriguez
  - Development Director
- Prism Event Management
  - Paula Blay
  - Mairead O'Reilley

The Rare Diseases Forum
Goals & Objectives

Facilitate Development of New Therapies for Rare Diseases

- Innovation in trial design, including seamless/adaptive designs and master protocols
- Best practices and uses of natural history, registries and other sources of evidence
- Use of the totality of evidence and treatment assessment outcomes
- Biomarkers and disease intermediates
- Innovation in biostatistics

Rare Diseases Forum – Workstreams –Working Groups

I. Legal and scientific basis for establishing evidence – treatment assessment outcomes
   1. Legal underpinnings for use of evidence to establish patient benefit
   2. Assessing clinically meaningful treatment outcomes
      b. Use of digital technology in quantification of treatment outcomes
   3. Encyclopedia of innovation

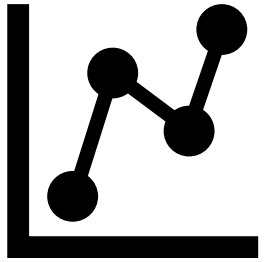
II. Innovation in clinical trial design

Goals for Today: Innovation in Trial Design

- Draw key lessons from oncology experience
- Consider application to rare diseases
- Establish concrete next steps for RD Forum

Perspectives

- Many different perspectives
  - “Master protocols are the way to go”
  - “Single arm studies w natural history comparators are the way to go”
  - “Bayesian approaches in RD is not possible bc we do not have enough priors”
  - “Basket approaches will not work if endpoints are not validated”



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One Drug
One Trial
One Disease

=

Collaboration
Cooperation
Communication
Community

Rules of the Game

- Open, constructive, dialogue and deliberation
- Bring your expertise
  - Leave your hat at the door
- What's said in the room, stays in the room
  - Reports and publications not for attribution

- Participants speak as individuals and express views that may not represent those of their organizations
- Social Media:
  - ✗ Quotes of speakers/panelists/audience
  - ✗ Detailing meeting proceedings
  - ✓ Photos during receptions
  - ✓ Referencing attendance
  - ✓ Interactions during breaks

Lessons from Oncology
Panel 1: Platform Trials

Lessons from Oncology
Panel 2: Basket Trials