The Rare Diseases Forum
Innovation in Trial Design Workshop

Veronica Miller, PhD
Forum for Collaborative Research
UC Berkeley School of Public Health
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Thanks & Acknowledgments
The Forum for Collaborative Research
Catalyzing Clinical Research to Improve Global Health
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
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
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.
Co-Chairs
- Academic: Marshall L. Summar MD NCMC/NORD
- Industry: John F. Crowley Amicus Therapeutics
- Forum: Veronica Miller PHD UC Berkeley

Members
- Regulatory –US FDA
  - Dragos Roman MD CDER/DGIEP
  - Dina Zand MD CDER/DGIEP
  - Rachel Witten MD CBER/OTAT
  - Christine Mueller MD OOPD
- Regulatory – EMA
  - Violeta Stoyanova MD COMP
- NIH
  - Anne Pariser MD NCATS
- Advocacy/Policy
  - Frank J. Sasinowski JD Everylife Foundation for Rare Diseases
  - Sandra Lehrman MD Advocate/Forum EC Co-Chair
  - Caroline Loewy KCNQ2 Cure, Global Genes Project
  - Peter L. Saltonstall NORD
  - Tara J Britt NC Rare Diseases Advisory Council
  - Susan Nichols Advocate, Falcon Therapeutics
- Industry
  - Jeffrey Sherman MD Horizon Pharma Inc
  - Timothy J. Miller PHD Abeona Therapeutics
- Academic
  - Steven Gray PHD UT SW Medical Center
  - Scott J. Steele PHD University of Rochester MC
Sponsors

- PTC Therapeutics
- RegenXBio
- Retrophin
- Solid Biosciences
Rare Diseases Forum Workshop 1
Innovation in Trial Designs “Bringing Lessons from the Oncology Experience to Rare Diseases”
Planning Committee Members

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- 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”
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