

Berkeley

 School of
Public Health



FORUM
for Collaborative
RESEARCH

Welcome and Introduction

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Forum for Collaborative Research

www.forumresearch.org



Forum Mission

- Public/private partnership including government agencies, industry, researchers and clinicians, insurers, foundations and patient advocacy community
- Mission: to facilitate and enhance healthcare research
 - Neutral & independent space
- Apply HIV collaborative model to other conditions
 - HCV (2007)
 - CMV in transplantation (drugs, vaccines, and other) (2014)
 - NASH and liver fibrosis/cirrhosis (2014)

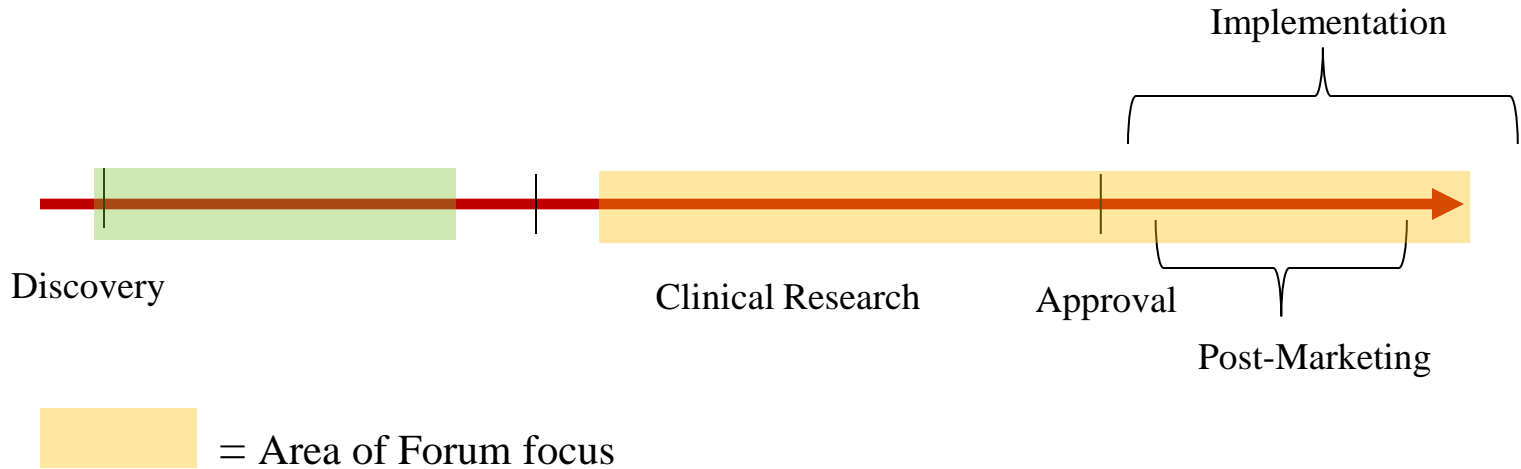


Forum's Operating Principle

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



Translational Medicine: From Development to Implementation



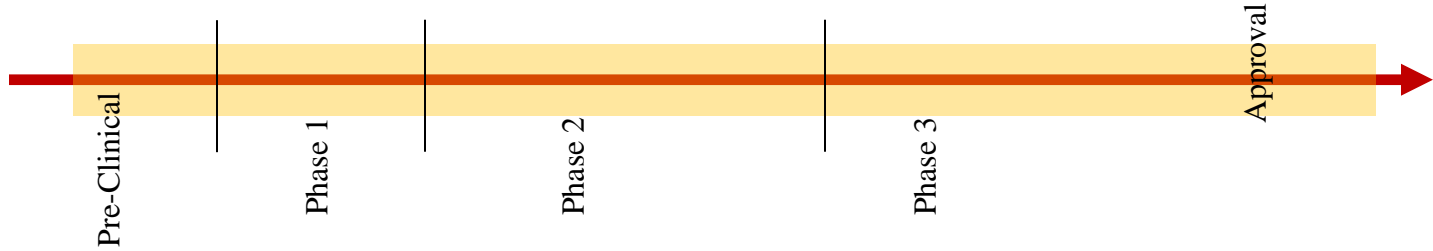


Accelerating Access to Better and Safer Drugs for All

- Forum Model:
 - Stakeholder collaborative model
 - Process of dialogue and deliberation
 - Government, industry, academia, community
 - HIV, HCV, CMV/ transplant recipients, HBV, Liver Forum
 - Identify gaps and barriers
 - Recommend consensus, solutions, fund/engage in new research path



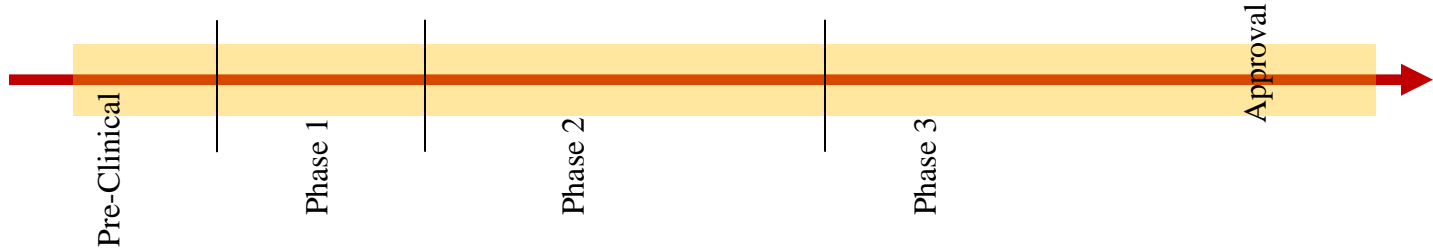
Clinical research & post-marketing commitments



- Expected costs and time to registration?
 - Uncertainty regarding acceptability of study designs
 - Interagency discordance on trial designs & acceptable endpoints
 - Post-marketing safety requirements
 - Inefficient investigator and patient recruitment



Regulatory Process



- Sponsor ↔ Agency
 - Single sponsor
 - Single agency
 - Confidential proceedings
- Advantages of Forum model
 - Involvement of *all* concerned stakeholders
 - Cross-Atlantic regulatory perspectives
 - Cross-Atlantic academic and patient/community perspective



Organizational Model

- Informal yet structured
 - Allow organic growth
 - Lack of bureaucracy
- “Non-player ecosystem orchestrator”
- “Multi-stakeholder partnerships that enables adaptive management”
- Experts participate as individual experts, not officially representing their respective organizations
- Not for attribution



Key Characteristics

- “Information Democracy”
- Non-competitive, safe environment
- Independent
- Co-ownership
- Synergy vs. duplication
- Transparency



Strategy

- Collaborative dialogue and deliberation on urgent gaps
- Build trust
- Allow consensus to evolve
 - Always science based
 - Don't force it if the science does not back it
 - Identify knowledge gaps and mechanisms to address these gaps
- Build more trust
- Tackle more challenging issues



Impact on Drug Development

- Increase
 - Clarity
 - Efficiency
 - Collaboration
 - Innovation
- Decrease
 - Uncertainty
 - Redundancy
 - Development time
 - Risk



PSC Forum

- Established based on work initiated at the March 3-4, 2016 *Trial Design and Endpoints for Clinical Trials in Adults and Children with PSC* meeting sponsored by the U.S. Food and Drug Administration (FDA) and the American Association for the Study of Liver Diseases (AASLD)
- Forum received requests from industry, academics and patient advocates to provide a venue for the continuation of work and to convene a PSC project group to address regulatory hurdles and advance drug development



PSC Forum: Process

- Steering Committee leads calls in key project areas identified through ongoing discussions with stakeholders with a focus on advancing the regulatory sciences
- Involves topic specific working groups to further focus the project agenda
- Flexible based on PSC Forum needs but involve conference calls and in-person meetings



PSC Agenda

- Project agenda set by the Steering Committee
 - Focus on accelerating drug development
 - Regulatory context
 - ◉ Identify gaps
 - ◉ Synergize ongoing efforts



PSC Forum Steering Committee

Category	Name	Affiliation
Regulatory	Lara Dimick-Santos	FDA/ CDER/ DGIEP
Regulatory	Elmer Schabel	EMA
Patient Advocate	Ricky Safer	PSC Partners Seeking a Cure
Patient Advocate	Martine Walmsley	PSC Support
Academic	Christopher Bowlus	UC Davis Medical Center
Academic	Gideon Hirschfield	University of Birmingham
Academic	Tom Karlsen	Oslo University Hospital Rikshospitalet
Academic	Kris Kowdley	Swedish Medical Center
Academic	Keith Lindor*	Arizona State University
Academic	Tamir Miloh	Baylor College of Medicine
Academic	Arun Sanyal	Virginia Commonwealth University
Industry	Rob Myers*	Gilead Sciences, Inc.
Industry	Stephen Rossi	NGM Biopharmaceuticals
Industry	David Shapiro	Intercept Pharmaceuticals
Forum	Veronica Miller	Forum for Collaborative Research



PSC Forum 1 Attendees

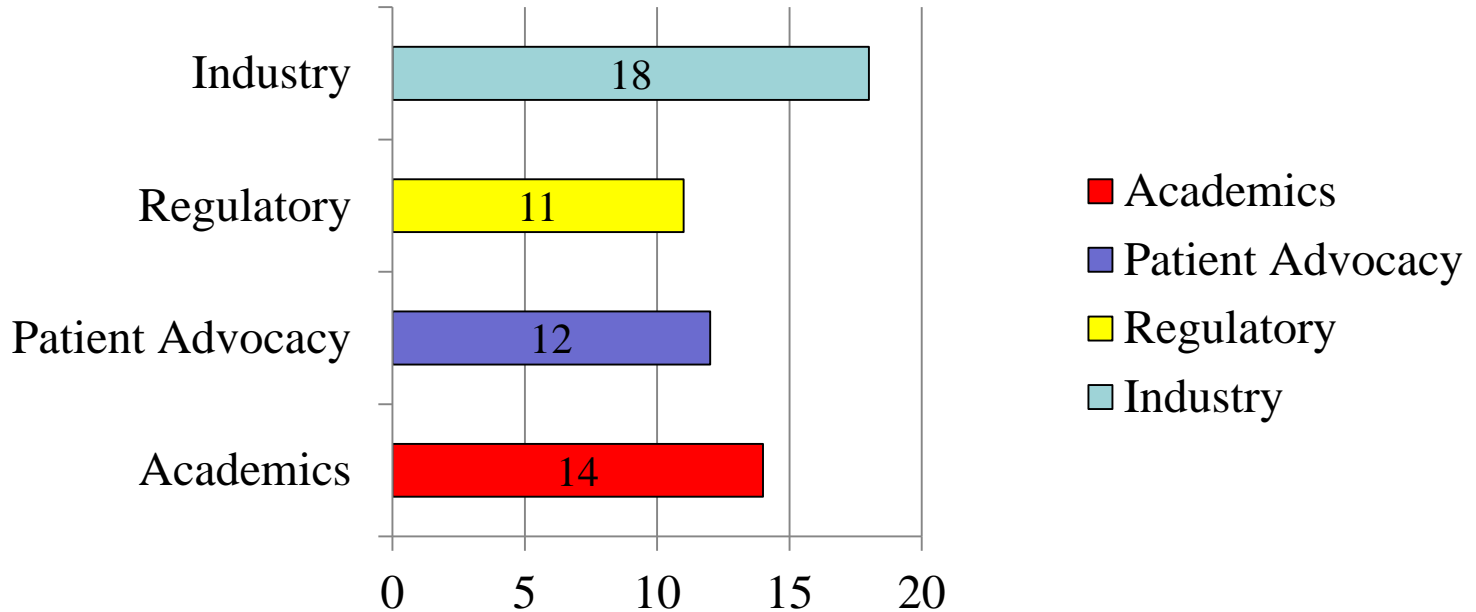
Registered
55

In-person: 52

Remotely: 3



PSC Forum 1 Attendees





PSC Forum Project Updates

- **PSC Patient Databases Working Group**
 - Launched, first conference call September
 - Co-Chairs:
 - ◉ Chris Bowlus- *UC Davis Medical Center*
 - ◉ Rachel Gomel- *PSC Partners Seeking a Cure*
- **PSC Inclusion/ Exclusion Criteria Working Group**
 - Launched, first conference call August
 - Co-Chairs:
 - ◉ Gideon Hirschfield- *University of Birmingham*
 - ◉ Kris Kowdley- *Swedish Medical Center*



Financial Sponsor





Many Thanks

- **Forum Staff**

- Malene Cobourne
- Terry Daniels
- Pedro Goicochea
- Katherine Greene
- Luis Javier Hernandez
- Victoria Mason
- Veronica Miller
- Brenda Rodriguez
- Ken Taymor
- Jessica Weber

- **Prism Event Management**

- Paula Blay
- Rachel Heitkamp
- Graham Hill
- Mairead O'Reilly
- Sarah Sanderson
- George Seaton



Thank You!