Forum for
Collaborative HIV Research

The Forum HIV Cure Project:
Focus on Regulatory Issues

A Collaborative Effort to Map the Regulatory Pathway for HIV Cure Strategies

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Acknowledgments

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Additional project support provided by:

Steering Committee

enhancing & facilitating HIV research

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- Daniel Kuritzkes, MD
- Veronica Miller, PhD

Community:

- David Evans
- Mark Harrington

Federal Government:

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- Sarah Read, MD MHS PhD
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- Jintanat Ananworanich, MD, PhD

Forum Staff

- Erik Lontok
- Nivedha Panneer
- Courtney Hutchison (Year 1)
- Sonia Navani (Year 1)

CLINICAL PHARMACOLOGY & THERAPEUTICS

nature publishing group

Open

STATE OF THE ART

Accelerated Access to Innovative Medicines for Patients in Need

LG Baird[1], R Banken[2], H-G Eichler[3], FB Kristensen[4], DK Lee[5], JCW Lim[6], R Lim[5], C Longson[7], E Pezalla[8], T Salmonson[9], D Samaha[2], S Tunis[10], J Woodcock[11] and G Hirsch[1]

- Current “siloed” system increases development cost, time and risk
- Inefficient use of accelerated access programs
- FDASIA Legislation (2012): apply principles more broadly
- Increased cross-jurisdictional and cross-functional interaction
- Increased engagement of all stakeholders

Forum "Process" in Drug Development

- HIV
  - Clinical trial design
  - Recognition of new toxicities
  - Long term safety monitoring
  - Immune based therapies
  - Introduction of new drug classes (CCR5)
- HCV
- CMV-transplantation
- Liver fibrosis/NASH

Context

- Benefit-risk is cornerstone of regulatory process
- Enthusiasm balanced by efforts to ensure safety, decrease/manage risk
- Requires:
  - Clarity of purpose (clear communication of goals)
  - Consensus + evolving consensus
  - Maximum research efficiency through collaboration
  - Each patient's data contribution as valid and informative as possible

Specific AIMS – Year 1 & 2

- Facilitate and advance HIV cure research by clarifying and resolving regulatory issues through multi-stakeholder dialogue
  - Provide an ongoing neutral and independent platform for targeted discussions with multi-stakeholder experts
  - Provide a productive mechanism for broader, public input on questions of acceptable risk, ethics, informed consent and appropriate populations
- **Forum Niche**
  - Regulatory Pathway: Facilitate FDA efforts for more systematic patient input on benefit-risk decision making (PDUFA V)

Year 1: Working Groups

- #1: Biomarkers & Endpoints
  - John Mellors & Michael Miller
- #2: Clinical Trials – Benefit – Risk
  - Jintanat Ananworanich & Joe Eron
- #3: Patient education, recruitment & informed consent
  - David Evans & Tim Henrich

Working Group Members

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Academia Community/Advocacy Federal Government Foreign Government Foundation Industry

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Charge to Working Groups

- Formulate, review, and discuss specific questions
- Establish baseline: where is the field now?
- Record consensus where possible
- Recommend path forward for future consensus

Motto: Need to start somewhere

Take small steps – build on success

Regulatory Path?

- Effect of strategy vs. “CURE”
  - Intermediate steps vs. end-goal
- Combination of strategies
  - What, when, how?
- Combination of experimental + approved
- Combination of experimental + experimental

Progress

- Document current status of field from a regulatory perspective
- Measurable definition of “cure”
- Risk mitigation strategies for clinical trials
- Components of appropriate informed consent
- Guidance on survey instruments to collect patient perception of benefit-risk

June 17: Public Input

- Opening by CBER director Karen Midthun
- Working groups reports and discussion
- Case studies
- 180+ in person /200+ webcast participants
- Post June 17
  - Project evaluation
  - Publication plan
  - Review/re-align

Specific Aims '14 – '15

- Clarify & resolve regulatory issues through multi-stakeholder dialogue
  - Independent/neutral platform for targeted discussions
  - Mechanism for broader public input of what is acceptable risk
- Community perception of HIV cure research & willingness to refer/participate

Specific Activities: WG Evolution

- WG 1: Animal model working group
- WG 2: Network for focused/targeted discussion on specific strategies
- WG 3:Interdisciplinary research interaction

Other Potential Activities

- Perceptions, beliefs and knowledge among patients, providers, communities and researchers
  - Influence on willingness to participate/refer
- Recruitment strategies for different patient populations
- Research interaction between social scientists, decision making science, behavioral economics, etc.

Advantages/Contribution

- Increased efficiency of development
  - All parties in the room vs. one-one
- Signal that field is structured/poised to move ahead
  - Recruitment of new partners and collaborators
- Support and synergize with other ongoing efforts
  - Annenberg group, IAS, etc.