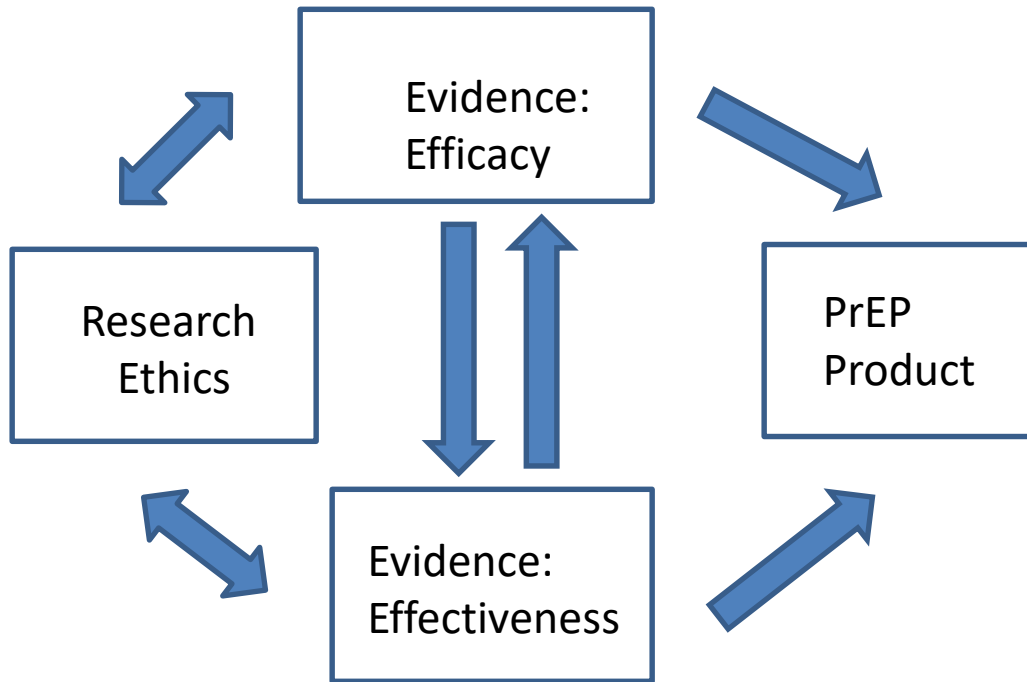


Regulatory Pathways & Clinical Trial Designs

Framing the Discussion

Veronica Miller, PhD

FCR-UCB SPH



Today's Regulatory Environment

- FDASIA 2012
- 21st Century Cures Act

Title III: Development

Subtitles

- A. Patient-Focused Drug Development
- B. Advancing New Drug Therapies
- C. Modern Trial Design and Evidence Development
- D. Patient Access to Therapies and Information
- E. Antimicrobial Innovation and Stewardship
- F. Medical Device Innovations
- G. Improving Scientific Expertise and Outreach at FDA
- H. Medical Countermeasures Innovation
- I. Vaccine Access, Certainty and Innovation
- J. Technical Corrections

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Ethical Framework

1. Value
 2. Scientific validity
 3. Fair subject selection
 4. Favorable benefit/risk
 5. Independent review
 6. Informed consent
 7. Respect
- Systematic framework
 - Universal
 - Necessary and sufficient
 - 1+2+3+4+5+6+7

Optimal Use of Limited Resources

TOTALITY OF EVIDENCE

ROLE OF REAL-WORLD EVIDENCE

HIV – CHANGING DRUG DEVELOPMENT PARADIGM

PREP Quandary

POTENTIAL APPROACHES

Regulatory Considerations for Approval: FDA perspective

Charu Mullick, MD
Medical Officer, Division of Antiviral Products
Center for Drug Evaluation and Research, FDA

9th IAS Conference on HIV Science, Paris
July 26, 2017

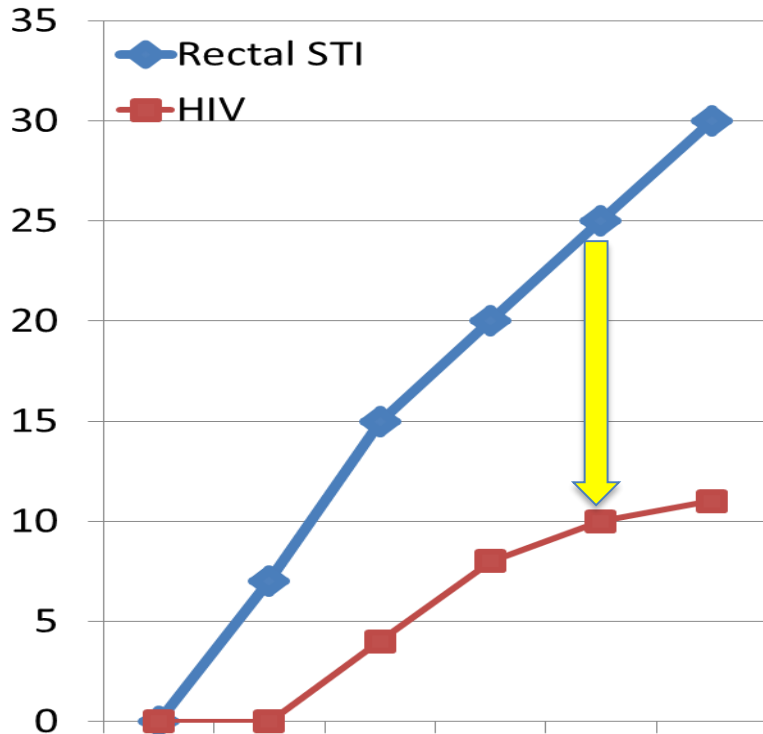
**The views in this presentation represent my opinion and not necessarily official policy of the
Food and Drug Administration**

PrEP trials

- In MSM, rectal STI is an indicator of unprotected RAI e.g., rectal gonorrhea, rectal chlamydia
- Unprotected RAI is considered the major route of HIV acquisition

Consideration for rectal STI incidence as a correlate or benchmark of HIV incidence in MSM

Rectal STI-HIV Association



- Quantify the association of rectal STI and HIV incidences
 - Use data from previous trials, databases where individuals were not receiving prophylaxis

- Association will *predict* the point estimate of HIV incidence *that is expected* for the observed rectal STI incidence in a future trial

HIV-STI Incidence WG

- FDA experts
- Investigators
 - Academic and industry
- Statisticians
 - Academic, industry and regulatory
- Data sources
- Data analyses plan(s)
- Analyses

From PoC to Confirmatory Trials

- Heterosexual transmission
 - Women
 - Men
- Adolescents