Forum for Collaborative HIV Research

Future of PrEP and Microbicide Research: Trial Design and Regulatory Issues

Washington, DC
January 7, 2013

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Why this Meeting Now?

- Current landscape of prevention research
- Complexities of designing/running prevention trials
  - Populations – no “one size fits all”
  - Resource requirements
  - Ethics
- Bring together expert stakeholders to review
  - What have we learned
  - How can we move forward
- Focus on regulatory issues

Session 1 – Setting the stage

- Truvada – lessons learned (being learned)
- Products in development

Session 2 - Statistical Challenges

- Given the statistical requirements, what is the feasibility of conducting the size trials needed to show clinical efficacy of an HIV prevention modality?
- Are there any methods for economizing sample sizes?
- From a statistical perspective, can tenofovir/emtricitabine serve as an active control?

Session 3

- Clinical Trial Design Options
  - Most clinically feasible trial options?
    - Superiority Studies
    - Non-inferiority
    - Dose-response
  - Role of Truvada
  - Adherence measures

Session 4 – Adherence vs. Efficacy and Proof-of-Concept

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•For proof-of-concept -- lack of signal due to potency vs adherence?

o How can adherence measurements be used to support efficacy claims for new drugs, new formulations, or dosing schemes

- Most reliable measures of adherence
- Systemic drug concentrations
- Alternative biological samples (e.g. PBMCs)
- Patient-reported adherence measurements
- Measuring adherence for topical products,
- Is there a role for enriching trial enrollment with subjects deemed likely to be adherent? What are the pitfalls of this approach?
- How should ongoing assessments and interventions to improve adherence in clinical trials be factored into the analysis of efficacy?

Session 5 - "How do we get to surrogacy?"

- • What kind of non clinical studies and clinical PK/PD relationships can be used for making potential correlations with clinical efficacy?
- • Is it feasible to evaluate PK/PD relationships at the targeted prevention site (e.g. rectum, vagina)?

SUMMING UP: Is the future any clearer?

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