Duke | Robert J. Margolis, MD Center for Health Policy

Public Workshop: Scientific and Regulatory Considerations for the Analytical Validation of Assays Used in the Qualification of Biomarkers in Biological Matrices

The Duke-Margolis Center for Health Policy • Washington, DC
June 14, 2017

9:00 a.m. **Welcome and Introductions**
Mark McClellan, Duke-Margolis Center for Health Policy
Gregory Daniel, Duke-Margolis Center for Health Policy

9:10 a.m. **Opening Remarks**
ShaAvhrée Buckman-Garner, U.S. Food and Drug Administration

9:30 a.m. **FDA Efforts to Support Biomarker Development and Qualification**
Presentation: *Christopher Leptak, U.S. Food and Drug Administration*

9:50 a.m. **Overview of the Analytical Validation White Paper Objectives and Key Terminology**
Presentation: *Steve Piccoli, Neoteric Consulting*
Q&A

10:30 a.m. **Session Ia: Review of the Draft Analytical Validation Framework**
Objective: Outline the key components of the whitepaper and draft framework, and elicit questions and feedback from attendees

Moderator: *Meena Subramanyam, Takeda*

Structure:

- **Presentation 1:** *Assay Design, Development, and Validation & Pre-analytical Considerations*
  - *Meena Subramanyam, Takeda*
- **Panelists**
  - *Shashi Amur, U.S. Food and Drug Administration*
  - *Robert Becker, U.S. Food and Drug Administration*
  - *Amanda Baker, Critical Path Institute*
  - *Jean Lee, BioQualQuan*
- **Moderated Q&A**

11:30 a.m. **Break**

11:45 a.m. **Session Ib: Review of the Draft Analytical Validation Framework, cont.**

Moderator: *Afshin Safavi, BioAgilytix Labs*

Structure:

- **Presentation 2:** *Assay Performance & Assay Validation Acceptance Criteria*
  - *Afshin Safavi, BioAgilytix Labs*
- **Panelists**
  - *Steve Gutman, Illumina Inc.*

        - *Robert Becker, U.S. Food and Drug Administration*
        - *Kylie Haskins, U.S. Food and Drug Administration*
        - *Jean Lee, BioQualQuan*
    - **Moderated Q&A**

12:45 p.m. **Lunch** (Lunch will not be provided)

1:45 p.m. **Session II: Applying the Framework in a Real-World Context: Biomarkers of Drug-induced Nephrotoxicity**

Objective: Review the development and qualification of the Predictive Safety Testing Consortium's (PSTC) nephrotoxicity biomarkers to provide real-world context for applying the analytical validation framework, with special emphasis on key criteria (specificity, selectivity, reproducibility, parallelism, quantification range, stability)

Moderator: *Steve Piccoli*, *Neoteric Consulting*

Structure:

- **Opening presentation**: *Steve Piccoli*, *Neoteric Consulting*
- **Panelists**:
    - *Aliza Thompson, U.S. Food and Drug Administration*
    - *Kellie Kelm, U.S. Food and Drug Administration*
    - *Frank Sistare, Merck*
    - *Meena Subramanyam, Takeda*
    - *Amanda Baker, Critical Path Institute*
- **Moderated Q&A**

3:30 p.m. **Break**

3:45 p.m. **Session III: Facilitated Discussion of the Framework**

Moderator: *John-Michael Sauer, Critical Path Institute*

Structure:

- **Panelists:**
    - *Robert Becker, U.S. Food and Drug Administration*
    - *Chris Leptak, U.S. Food and Drug Administration*
    - *Lisa McShane, National Cancer Institute*
    - *Elizabeth Mansfield, Grail Inc.*
    - *Afshin Safavi, BioAgilytix Labs*
    - *Steve Piccoli, Neoteric Consulting*
- **Moderated Q&A**

4:45 p.m. **Closing Remarks**

Gregory Daniel, Duke-Margolis Center for Health Policy

5:00 p.m. **Adjournment**

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June 15, 2017

9:00 a.m. **Welcome**
Mark McClellan, Duke-Margolis Center for Health Policy
Gregory Daniel, Duke-Margolis Center for Health Policy

9:15 a.m. **Opening Remarks**
Objective: Provide a brief recap from day one discussion and discuss goals for day two
Presenter: *John-Michael Sauer, Critical Path Institute*

9:30 a.m. **Session IV: Applying the Framework in a Real-World Context: Glutamate Dehydrogenase (GLDH) as a Biomarker of Hepatotoxicity**
Objective: Review the ongoing development and qualification of GLDH to provide additional real-world context for the application of the draft framework.

Moderator: *Jiri Aubrecht, Pfizer*

Structure:

- **Opening presentation**: *Jiri Aubrecht, Pfizer*
- **Panelists:**
  - *Juliane Lessard, U.S. Food and Drug Administration*
  - *Shelli Schomaker, Pfizer*
  - *John-Michael Sauer, Critical Path Institute*
- **Moderated Q&A**

11:15 a.m. **Break**

11:30 a.m. **Session V: Reflecting on the Framework: An Industry Perspective**
Objective: Discuss the framework in the context of other efforts to support biomarker assay performance characteristics, identify areas of consensus, and highlight any remaining gaps

Moderator: *Mark Arnold, Covance*

Structure:

- **Panelists**
  - *Lakshmi Amaravadi, Sanofi*
  - *Chad Ray, Zoetis*
  - *Stephen Lowes, Q ^{2} Solutions*
  - *Lauren Stevenson, Biogen*
- **Moderated Q&A**

12:30 p.m. **Lunch** (Lunch will not be provided)

1:30 p.m. **Session VI: Facilitating Dissemination and Implementation of the Draft Analytical Validation Framework**
Objective: Discuss strategies to ensure adequate dissemination and uptake of the framework, including the process for providing feedback and the development of a communications strategy to socialize the ideas to the broader scientific community

Moderator: *Gregory Daniel, Duke-Margolis Center for Health Policy*

Structure:

- **Panelists**
  - *Chris Leptak, U.S. Food and Drug Administration*
  - *Joseph Menetski, Foundation for the National Institutes of Health*
  - *Lisa McShane, National Cancer Institute*
  - *Martha Brumfield, Critical Path Institute*
- **Moderated Q&A**

2:30 p.m. **Session VII: Summary of Key Themes, Major Takeaways, and Next Steps**
Objective: Review the major themes of the workshop's discussion and identify and prioritize for action any outstanding items and questions. This will help to guide and inform the completion of the white paper as well as the communications strategy.

Moderators: *Mark McClellan, Duke-Margolis Center for Health Policy and ShaAvhrée Buckman-Garner, U.S. Food and Drug Administration*

Structure:

- **Panelists:**
  - *Shashi Amur, U.S. Food and Drug Administration*
  - *Sue-Jane Wang, U.S. Food and Drug Administration*
  - *John-Michael Sauer, Critical Path Institute*
  - *Steve Piccoli, Neoteric Consulting*
  - *Russell Grant, LabCorp*
  - *John Kadavil, U.S. Food and Drug Administration*
- **Moderated Q&A**

3:30 p.m. **Closing Remarks**
Mark McClellan, Duke-Margolis Center for Health Policy

3:45 p.m. **Adjournment**