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Characterizing FDA's Approach to Benefit-Risk Assessment throughout the Medical Product Life Cycle

Tommy Douglas Conference Center • Silver Spring, MD
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Following the sixth authorization of the Prescription Drug User Fee Act (PDUFA VI), the U.S. Food and Drug Administration (FDA) committed to improving the implementation and communication of its benefit-risk framework. In FY 2020, FDA plans to publish draft guidance on the benefit-risk assessment of new drugs and biologics. When finalized, the guidance will provide drug sponsors and other stakeholders with a clearer understanding of how considerations about a drug's benefits and risks factor into FDA's regulatory decisions throughout the drug development life cycle.

Stakeholders' perspectives and experiences regarding the benefit-risk framework are important to FDA's guidance development and to FDA's communication and implementation of the framework. Accordingly, this public meeting will cover: 1) FDA's planned benefit-risk assessment guidance; 2) activities that occur in pre-market development that best inform FDA's benefit-risk assessment; 3) effective communication of benefit-risk assessment information; and 4) the use of benefit-risk assessment to inform FDA and sponsor decision-making in the post-marketing setting.

8:30 a.m. **Morning Refreshments**

9:00 a.m. **Welcome and Overview**

- Mark McClellan, Duke-Margolis Center for Health Policy

9:05 a.m. **Opening Remarks**

- Theresa Mullin, U.S. Food & Drug Administration

9:15 a.m. **Introduction: FDA's approach to Benefit-Risk Assessment**

FDA will provide an overview of (a) the Agency's approach to benefit-risk assessment along the medical product lifecycle, and (b) key topics FDA plans to address in draft guidance on benefit-risk assessment.

Presentation:

- Sara Eggers, U.S. Food & Drug Administration
- Kerry Jo Lee, U.S. Food & Drug Administration

Audience Q&A

10:00 a.m. **Break**

10:15 a.m. **Session 1: Activities that Occur in Pre-Market Development that Best Inform FDA's Benefit-Risk Assessment**

Moderator: Mark McClellan

Presentation:

- James Smith, U.S. Food & Drug Administration

Reactants and Panel Discussion:

- Conny Berlin, Novartis International AG
- William Wang, Merck & Company, Inc.
- John Crowley, Amicus Therapeutics, Inc.
- Bray Patrick-Lake, Duke Clinical Research Institute
- Brett Hauber, RTI Health Solutions

Discussion Questions:

- Which decisions made in the course of drug development (e.g. regarding dose exploration, trial design, endpoint selection, risk mitigation) have the most significant impact on the benefit-risk assessment of a pre-marketing application?
- How does patient experience data, including patient input on disease burdens, meaningful outcomes, and potential benefit-risk tradeoffs, inform FDA's benefit-risk assessment? When should this information be collected?
- When may more formal or quantitative benefit-risk assessment methods add value to the body of evidence a sponsor has generated?
- When and how can FDA and sponsors most effectively engage in discussion on key benefit-risk considerations that can help inform the sponsor's drug development activities and FDA's benefit-risk assessment?

Audience Q&A

12:00 p.m. **Lunch**

1:00 p.m. **Session 2: Effectively Communicating Benefit-Risk Assessment Information**

Moderator: Mark McClellan

Presentation:

- Richard Forshee, U.S. Food & Drug Administration
- Ellis Unger, U.S. Food & Drug Administration

Reactants and Panel Discussion:

- Adora Ndu, BioMarin Pharmaceutical Inc.
- Rebecca Noel, Eli Lilly and Company
- Elaine Morrato, University of Colorado Denver
- Theresa Strong, Foundation for Prader-Willi Research
- John Wong, Tufts Medical Center

Discussion Questions:

- How can sponsors most effectively present information to support benefit-risk assessments in their marketing applications to FDA?
- How can information to support the benefit-risk assessment of a marketing application be most effectively presented at an advisory committee meeting?
- How can FDA enhance the use of its Benefit-Risk Framework as a tool to communicate the Agency's thinking on a product's benefit-risk assessment to sponsors and the public at the time of pre-market approval?

Audience Q&A

2:15 p.m. **Break**

2:30 p.m. **Session 3: Using Benefit-Risk Assessment to Inform FDA and Sponsor Decision-Making in the Post-Marketing Setting**
Moderator: Gregory Daniel, Duke-Margolis Center for Health Policy

Presentation:

- Judith Zander, U.S. Food & Drug Administration

Reactants and Panel Discussion:

- Laura Bloss, Amgen Inc.
- Juhaeri Juhaeri, Sanofi S.A.
- Veronique Kugener, Takeda Pharmaceutical Company Ltd.
- Robert Ratner, Georgetown University Medical School

Discussion Questions:

- When and how can sponsors and FDA most effectively engage in timely discussions of new information or activities relevant to key benefit-risk considerations in the postmarket setting?
- When and how can more formal or quantitative benefit-risk analyses add value to regulatory decision-making in the postmarket setting?
- When and how can patient input collected in the postmarket setting best inform the continued benefit-risk assessment for a marketed product?
- When and how can FDA's Benefit-Fisk Framework be effectively used as a tool to help communicate about benefit-risk assessments that occur in the postmarket setting?

Audience Q&A

3:45 p.m. **Session 4: Outlining Next Steps & Future Directions**
Moderator: Gregory Daniel

Reactants and Panel Discussion:

- Scott Evans, George Washington University
- Bray Patrick-Lake, Duke Clinical Research Institute
- Bennett Levitan, Janssen Pharmaceuticals, Inc.
- Richard Hermann, AstraZeneca PLC
- Peter Stein, U.S. Food & Drug Administration

Discussion Questions:

- What are the key takeaways from this meeting, or considerations for FDA to keep in mind as it develops its guidance on benefit-risk assessment?
- What are the most significant challenges to effectively advancing structured benefit-risk assessment for drug development and evaluation over the next five years? What next steps can FDA, sponsors, and other stakeholders take to address these challenges?

4:30 p.m. **Session 5: Open Public Comment**
Moderator: Gregory Daniel

4:55 p.m. **Closing Remarks and Adjournment**

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