

Characterizing Uncertainty: Day 1 Agenda Recap

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IOM Workshop on Characterizing and Communicating Uncertainty in
the Assessment of Benefits and Risks of Pharmaceutical Products

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Structured Approach to Benefit-Risk Assessment in Drug Regulatory Decision-Making

Draft PDUFA V Implementation Plan - February 2013
Fiscal Years 2013-2017

Background

Figure 1: FDA Benefit-Risk Framework

Decision Factor	Evidence and Uncertainties	Conclusions and Reasons
Analysis of Condition		
Current Treatment Options		
Benefit		
Risk		
Risk Management		
Benefit-Risk Summary Assessment		

FDA. (2013). *Structured approach to benefit-risk assessment for drug regulatory decision making*. Draft PDUFA V implementation plan (2/13). FY2013-2017.

Assumptions

- Use analysis to aid, not replace judgment
- Quantify individual benefits and risks to the greatest extent possible
- Do not seek common metric of benefits, risks, or both
- Separate scientific judgment from regulatory interpretation
- Acknowledge uncertainty

Two Key Sources of Uncertainty

Extrapolating clinical trial data to other
populations and conditions

Incorporating post-market data of varying
quality

IOM Role

Activity of Drug Policy Forum

Dedicated convening committee

Guided by Statement of Task

No conclusions or recommendations

Summary report on presentations and
discussions

Statement of Task

Challenges

Alternatives, in industry and elsewhere

Possible steps to development of methods
suited to regulatory decision making

Possible approaches to communicating
uncertainty to stakeholders, including
public

Day 1 Agenda

Importance of uncertainty – Janet Woodcock
I: Approaches to evaluating uncertainty and maximizing value of clinical trials

Tarek Hammad

Deborah Zarin

Michaela Kiernan

Sebastian Schneeweiss

II. Case studies

Patrick Frey (FDA)

Robert Temple (Tysabri)

Susan Limb (Anoro Ellipta)

Day 1 Agenda

III. Methods for characterizing uncertainty

Granger Morgan

David Mandel

John Ioannidis

Joel Greenhouse

IV. Uncertainty in regulatory decision making

Charles Manski

Timothy McDaniels

Joseph Arvai

V. Implementation (panel)

WORKSHOP IN BRIEF  APRIL 2014

For more information, visit www.iom.edu/characterizinguncertainty

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<http://www.iom.edu/Activities/Research/DrugForum/2014-FEB-13.aspx>