

# Characterizing & Understanding Uncertainties

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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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# Decision-making Approaches

- Expert elicitation
  - formal, scientific basis for “using judgment to supplement standard statistical tests”
  - how to incorporate informal evidence that informs an expert’s judgment alongside formal evidence
- Bayesian statistical methods
  - use of sensitivity analysis to test how assumptions about the prior distribution affect the posterior inference



# Regulatory Decision-making Applying Analytic Techniques

- Scientific basis for eliciting and incorporating stakeholder preferences
  - forecasting consequences, uncertainties, thresholds, “tipping points”
- Sensitivity analysis
  - constructed preferences around different levels of uncertainty
  - for point estimates
  - across a range of attributes



# Need for Regulatory Science

- Innovative Medicines Initiative (IMI) PROTECT\*
  - Public-private consortium coordinated by the European Medicines Agency (EMA)
    - commitment to methods development
    - 47 studies, 13 pilot programs
  - Metric indicies
  - Estimation techniques
  - Utility survey techniques
- To date, little broad interest in US

\* Pharmacoepidemiological Research on Outcomes of Therapeutics by a European Consortium - [www.imi-protect.eu](http://www.imi-protect.eu)

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# Segment Two: Characterizing and Understanding Uncertainties

## Discussion Questions

- Discuss analytical methods that hold promise for characterizing and incorporating uncertainty in the assessment of benefit and risk.
- What steps should the regulatory and research communities take to advance the methods and applicability of these tools?

