Session IV
Concluding Discussion: Lessons Learned and Potential Strategies for a Way Forward

Discussion Questions

INSTITUTE OF MEDICINE
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Segment One: Identifying and Mitigating Uncertainty Through Maximizing the Value of Evidence

Discussion Questions

- Given the inherent presence and range of uncertainties in the drug development process, discuss opportunities to maximize the value of information both pre- and post- market within the context of the evaluation of a drug.
- Consider challenges and opportunities to the benefit and risk assessment of a pharmaceutical product based on a particular stakeholder's risk tolerance.
  - How will identifying and addressing uncertainty and information gaps make a difference to stakeholders? In regulatory decisions? In prescribing patterns? In patient choices?
  - What types of information could be used to mitigate such uncertainties?

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?

Segment Three: Eliciting Values From Stakeholders, Particularly Patients

Discussion Questions

- Under what circumstances is it most important to elicit patient input on uncertainty, and why? Given the range of contexts in which patients need to make difficult decisions, how can uncertainty be effectively elicited throughout the drug development process?
- FDA committed, in PDUFA, to conducting patient-focused meetings.
  - Have we maximized the value of a public meeting as a vehicle to gather patient input, within its constraints? For example, reaching a broad population, asking the most appropriate questions, generating useful input, enabling patients to feel that their voice has been heard.
  - Where do they fit in the broader scope of ensuring patient input is incorporated into drug development, evaluation and regulatory decision making?
- Suggest next steps to more actively involve patients in efforts to characterize and communicate benefits, risks and uncertainties of pharmaceutical products.

Segment Four: Communicating Uncertainty About Benefit and Risk Assessments of Pharmaceutical Products

Discussion Questions

- Reflect on the information needs of different stakeholder groups (patients, doctors, research & development community, the media)
  - What communication mechanisms or networks will best enable these different stakeholders to access information about what is known, what is unknown, and the processes/steps FDA is taking to reduce any uncertainties about knowns and unknowns?
  - Is it possible to have a single standardized approaches when it comes to communicating uncertainty, or should particular communication strategies & networks be activated on a case-by-case basis given (a) the range of sources and magnitudes of uncertainty in drug risks and benefits; and (b) the range of risks involved (i.e. is it uncertainty about whether a drug causes death or a wide spread, but less adverse, side effect)?

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Segment Four: Communicating Uncertainty About Benefit and Risk Assessments of Pharmaceutical Products

Discussion Questions Continued

- Given the media's role in communicating uncertainty to lay audiences, what could be put into place to help both inform and educate journalists about drug risk/benefit uncertainties and the processes FDA is taking to reduce any uncertainty; and how can FDA work with their public information offices to facilitate this process?