Panel 3
Business Implications of Comparative Effectiveness

Research with stratified populations

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Key Decisions in the Lifecycle of a Pharmaceutical Product

Discovery

- **Ph 1**
- **Ph 2**
- **Ph 3**

**Marketing Approval**

- **Launch**

- **Patent Expiration**
- **Generic**

**Investment Decision**
- Decision to advance from Phase 2 to Phase 3
- Stakeholder = product developer

**Regulatory Decision**
- Decision to approve a product for marketing
- Stakeholder = regulatory agency

**Adoption/ Diffusion Decision**
- Decision to adopt and use a product in a population
- Stakeholder = payer or their intermediary

**Treatment Decision**
- Decision to prescribe a product for an individual patient
- Stakeholder = patient and their physician
Investment Decisions

• Phase 2 – Phase 3 investment decisions are informed by financial analyses (eNPV, real options, etc.)
  • Decisions based on opportunity costs for the portfolio
  • eNPV calculations historically based mostly on PTRS*
  • Best guess estimates of the probability of adoption and treatment use
• Increasing emphasis on more granular input for prediction of adoption/diffusion and treatment decisions
  • Simulation modeling to estimate the impact of policies such as CED on adoption/diffusion and eNPV (example follows)
• Need to minimize the risk (under uncertainty) of a:
  • False Positive: Developing something we can’t sell
  • False Negative: Stopping development of a beneficial treatment

Bottom Line: Need More Accurate Estimates of eNPV

* Probability of Technical and Regulatory Success
Simple Example of a Hypothetical “Asset” in the Investment Portfolio

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* $ values in millions
** Values hypothetical, made up by me
Brief Illustration: The Impact of CED on eNPV

• Estimated Base Case eNPV* and the impact of Coverage with Evidence Development in years 9 - 11
  • Scenario 0 = Base case + probability of trial success of 0.5
  • Scenario 1 = Base case + probability of trial success of 0.7
  • Scenario 2 = Base case + probability of trial success of 0.9
• Winners get 5% “prize”, losers get 75% “penalty” in years 12 - 16
• Estimated the impact of more efficient drug development (production) costs for each scenario
• Monte Carlo simulation with 1,000 trials

* Estimated from Year 1
Scenario 0
(p trial success = 0.5)

NPV (in $100 millions)

Cumulative Frequency

Base case
CED case
CED with reduced costs
Scenario 2
\( (p \text{ trial success} = 0.9) \)
Conclusions

• Loss of revenue during CED decreased eNPV from base case
• eNPV partially, but not completely, restored by better predicting “winners”
• Improved production efficiency had little impact on eNPV
Treatment Decisions: Are you like the average?
Will “niche” indications decrease eNPV?

- Number of treated patients will decrease
- Effectiveness in treated patients will increase
- Value-based pricing could maintain economic value
- Additional value created by avoidance of adverse events in patients who are not treated because they are unlikely to respond

Niche indications will NOT NECESSARILY decrease eNPV