Pragmatic Clinical Trials

Health Care Payers' Views of PCT Features and Trade-offs: A Preliminary Look

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One-hour, structured interviews
Formulary decisionmakers from ~ 15 payers
Diverse array of payers
- National insurers
- MCOs
- Medicare Part D plans
- Medium-sized insurers
- Public payers

Study's Definition of “Pragmatic Clinical Trial”

- Randomized, prospective clinical trial with at least two of these traits:
  1. Head-to-head comparators
  2. Broader patient population
  3. Outcomes of interest to patients, prescribers and payers
  4. Fewer data collection elements
     + to reduce burden of data collection

Potential trait # 5: More real-world clinical care than in RCTs

Five Themes

1. To Date, Payers Consider PCTs to be Rare Birds

- Most payers struggled to think of PCTs they had used in decisionmaking
- Even “classic” PCTs – ALLHAT and CATIE – did not come readily to mind for many
- Some payers did cite a head-to-head trial funded by a drug company

2. Payers Differ in Ranking of Traditional RCTs vs. Novel PCTs

- Some reject the idea of ranking—RCTs and PCTs are incommensurate
  - “RCTs – the gold standard for demonstrating whether a drug works” while
  - “PCTs provide evidence on the types of patients for which a drug works”
### 2. Payers Differ in Ranking (continued)

- Some would usually prefer a PCT over an RCT
  - PCTs’ “real-world,” payer-relevant evidence tips the scale vs. RCTs’ virtues
    + PCT: Head-to-head comparisons and/or broader patient populations vs.
    + RCT: Internal validity/infering efficacy from data
  - A few would do away with traditional RCTs if that were possible

### 3. External Validity Trumps Internal Validity

- Assessing the internal/external validity trade-off, payers often prefer PCTs to traditional RCTs
  - Broader population makes PCT clinical results relevant
  - Payers really want heterogeneity in Phase IV trials

- Or maybe not?
  - PCTs with too much heterogeneity lose some allure
  - Heterogeneity = OK in Phase III if there’s strong evidence of efficacy prior to Phase III PCT

### 4. PCTs May Have a Darker Side

- PCTs’ appeal = greater relevance to payers’ decisions about new drugs for their own mix of patients

- Nonetheless, payers are wary of PCTs
  - Pharma gets new ways to tweak trial designs to give their own drugs a hidden advantage

### 5. Thumbs Up to PCTs

- Most payers we interviewed believe PCTs lead to more informed decisionmaking
  - at least if PCTs are properly designed

- Most payers want to see the spectrum of types of evidence regarding drugs’ comparative effectiveness
  - RCTs, PCTs, observational studies, company-supplied decision models

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Stay Tuned

As PCTs Evolve
... and the Debate Continues