Payer and Decision Maker Use of Observational Studies for Health Care Reimbursement Decisions

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BACKGROUND

- As cost-containment pressures in the United States (US) intensify, the evidentiary hurdles to justify reimbursement and coverage for new drugs will continue to grow.
- Safety and efficacy data generated through randomized controlled trials (RCTs) to support regulatory bodies' marketing authorization requirements also provide critical evidence to decision makers.
- However, RCTs have certain limitations inherent in registration trials (e.g., head-to-head data typically are not available; trials measure efficacy or ongoing benefit-harm rather than effectiveness).
- Therefore, safety and efficacy data from RCTs alone are no longer adequate to meet the needs of all health care decision makers (i.e., payers, physicians, and patients).
- The demand for effectiveness data to fill the gaps left by RCTs is driving a need for robust, complementary sources of data.
- However, the perceived need for and acceptance of real-world, clinical, patient-centered, and/or economic outcomes through observational studies varies across stakeholders, organizations, and geographic regions.

OBJECTIVE

- To better understand how decision makers use results from observational studies to inform health care reimbursement decisions and/or market access for health care products.

METHODS

- We conducted desktop research of published literature, HTA reports, and third-party Web sites, to identify the types of observational studies most valuable to health insurance and managed care organizations in their reimbursement decision making.
- In addition, we conducted nine qualitative one-on-one interviews with payer decision makers from the RTI-HS US Commercial Payer Advisory Panel:
  - 1 medical director from a national plan
  - 5 medical directors from regional plans (northeast, southeast, midwest, mountain west, Pacific coast)
  - 1 medical director from an integrated health care system (northeast)
  - 1 pharmacy director from a regional pharmacy benefit manager (northeast)
  - 1 pharmacy director from a national pharmacy benefit manager
- Through qualitative one-to-one telephone interviews with our payer network, we gained insights into how the following types of observational studies were used and rated their importance in their decision-making process in comparison with RCTs:
  - Retrospective claims analysis: internal plan data
  - Retrospective claims analysis: external administrative health insurance claims data (e.g., MarketScan, PharMetrics/IMS)
  - Retrospective claims analysis: integrated plan data (e.g., Kaiser Permanente, Geisinger)
  - Prospective longitudinal survey
  - Prospective observational study or registry
  - Cross-sectional survey

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RESULTS

- Nine US payers, representing a wide spectrum of plan types and regions, answered nine questions about their use of observational study data.

What type of observational study data do you use?

- Most payers indicated that they most commonly review retrospective claims database analyses.
- With the exception of 1 medical director, all plans conducted internal retrospective claims database analyses at least semi-routinely.

How often do you use observational data and when?

- The most common response was that payers informally review observational study data daily or weekly, particularly internal retrospective claims analyses, with more formal reviews occurring every 2 to 3 months.
- Formulary placement reviews of drugs already on formulary are typically conducted when new observational study data become available.
- One payer with a closed formulary indicated that tier placement on his plan was based solely on price; therefore, new observational study data did not impact drugs already on formulary but could be influential in placing a previously omitted drug on formulary.

Which observational study designs are most robust?

- Rather than focusing on study design (e.g., prospective, retrospective, or cross-sectional studies), payers discussed transparency, the need for publication in a peer-reviewed journal, reproducibility with data from their own plan, sufficient powering of the study, adequate bias control (e.g., propensity score matching), whether the study is actionable in their plan, and the credibility of the study.
- When probed about the criteria for study credibility, payers were highly skeptical of observational studies performed completely internally by pharma, and most were unwilling to accept data on file (i.e., nonpublished, manufacturer generated) for observational study results.
- Several payers indicated that a credible third party, such as a university or independent research organization, should conduct these types of observational studies.

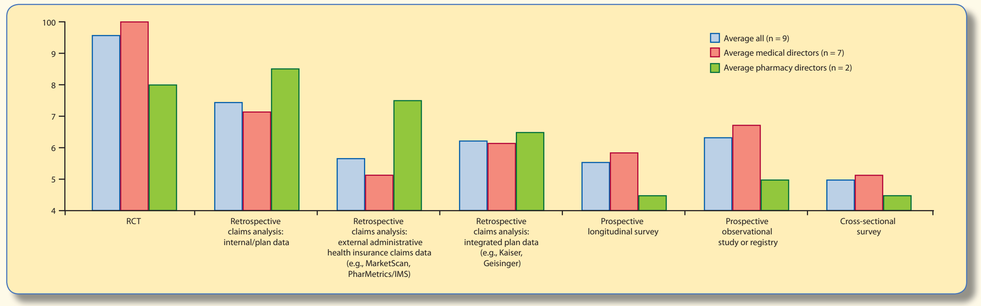
How high a priority is observational data in your organization?

- Payers rated the importance of observational study data in decision making for their organization on a scale of 0 to 10, where 0 is not a priority at all and 10 is an extremely high priority. Responses ranged from 3 to 10, with an average score of 5.1.
- Payers who review observational study data more frequently rated these data as more important than payers who reviewed observational data less frequently.
- One of the payers who rated the importance of observational study data as higher than average indicated that his score would have been even higher if he felt as though he could trust the data and results from observational studies.
- Another payer cited the importance of observational study data in providing information on compliance, particularly disease areas where compliance is paramount (e.g., diabetes and HIV)

Ratings of Specific Study Types

- Figure 1 displays payers' ratings of specific study types from 0 to 10, where 0 is a study type with no value in decision making and 10 is a study type with the highest value in decision making.

Figure 1. Payers' Value Ratings of Study Type



- Payers rated the following study types as having the highest values:
  - RCTs
  - Retrospective claims analysis using internal plan data (~20% < RCTs)
  - Prospective observational studies or registries (~33% < RCTs)

Differences Between Medical Directors and Pharmacy Directors

- There were notable differences in the responses of medical directors and pharmacy directors.
  - All 7 medical directors rated RCTs with a 10, while both the pharmacy directors rated RCTs with an 8.
  - One pharmacy director rated retrospective claims analysis using internal plan data higher than RCTs, while the other pharmacy director rated retrospective claims analysis using internal plan data on par with RCTs.
  - The medical directors placed more value on prospective studies than retrospective claims analyses; the pharmacy directors placed more value on retrospective claims analyses than prospective studies.
  - Cross-sectional surveys were consistently rated as having the least value among all payers.

Does your organization have a standard procedure for evaluating observational studies?

- The vast majority of payers stated that there was not a standardized procedure for evaluating these types of studies.
- If a standardized procedure was identified, the responses varied:
  - One payer indicated that the "standard procedure" is that he reviews these studies.
  - Another payer indicated that evaluations of observational studies are done only through the pharmacy and therapeutics committee (P &T committee).
  - The payer from an integrated health care system indicated that observational studies are evaluated and conducted through the research division and are often funded with a grant from pharma.

At your organization, who would be involved in evaluating observational data?

- The most common answer was research analytics. Other responses varied.
- Two payers indicated that the same people involved in a typical drug review would be involved (i.e., pharmacy directors and medical directors).
- Two payers indicated that they were the primary reviewer of observational study data.
- A third payer indicated that he was the primary reviewer of medical technology, while a staff pharmacist would review therapeutic observational studies.

If pharma gave you observational data, how would you use it in decision making?

- Most payers indicated that an additional level of critical evaluation was needed when observational data were provided by pharma.
- The study needs to be published and transparent, preferably in conjunction with a third party (university or independent research organization), and be reproducible in the health plan.
- Several payers indicated that along with ample RCT data, observational study data can be used to fill in the gaps and can be used in tier placement.
- Payers also noted that observational studies are particularly helpful in rare diseases or where RCT data are unavailable.

Do you use observational study data for risk-sharing agreements or value-based contracting?

- The vast majority of the payers interviewed indicated that they do not set up risk-sharing agreements or value-based contracting.
- Some payers indicated that in the future observational study data could be used to set up these types of agreements.

Would medicine dossiers that include observational study data help give preferred formulary placement?

- Most payers indicated that observational studies were not essential but were nice to include.
- Observational studies need to be published, to be considered.
- Some payers indicated that observational studies can help around the margins and may play a role in tier placement, depending on how compelling the observational study data are and how credible the study is.
- Some payers also indicated that they conduct a lot of internal research in addition to the medicine dossier.
- One payer indicated that he never sees the medicine dossier directly, but rather pharmacy puts together a packet of information that is reviewed before it goes to the P &T committee.
- The format of the material was not as important as the quality of the data and organization of the data.
- Another respondent indicated that a "toolkit" approach to comprehensively packaging the data may be more important in specialty areas and that very few branded drugs were on tier 2.

DISCUSSION

- Understanding the role of observational data in commercial payers' decision making is of critical importance.
- Most registration trials are either placebo controlled or are noninferiority; this lack of head-to-head comparative data can limit decision makers.
  - Supplementing the RCT data with robust observational data is an appropriate decision-making tool that is used by many payer decision makers in the US.
  - Caveats as to the perceived trustworthiness of the data that must be considered include minimization and control of bias, appropriate data sources, credible third-party participation, and peer-reviewed publication.
  - As Fleurance and colleagues (2010) note, "Observational studies can link together data sets that offer a wealth of information about real-world interventions and outcomes."
- As cost constraints become more pronounced, current open formularies likely will shift to that of a more closed system in the US.
  - Future decision making likely will be driven by the combination of RCTs and robust comparative effectiveness research, including data from observational studies.
  - Several respondents noted that in the cases of specialty products and biologics, a toolkit approach to providing pertinent RCT plus observational data from multiple study types would potentially be compelling for decision making on coverage policies.

CONCLUSIONS

- Payers rate RCTs highest across the board; however, all payers acknowledged that well-conducted observational studies could fill the gaps in real-world understanding and could provide critical decision-making evidence if the studies are actionable or results oriented for the plan.
- Data from observational studies are used to describe patient segments, understand treatment patterns and resource utilization, and provide effectiveness data that supplement clinical trials efficacy and safety data and help support market access decision making.
- Robust observational data play a valuable role in decision making at the US commercial plan level and will likely play a larger role in future coverage decisions.

REFERENCE

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