United States Decision Maker Perceptions of Data From Observational Studies and Other Health Economics and Outcomes Research

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BACKGROUND

- Policy makers and proponents of health care system reform routinely argue that more explicit appraisal of the clinical effectiveness and comparative value of new and existing interventions is essential for meeting the goals of sustained innovation, cost control, and improved quality in the health care system.
- Traditionally, a new health care intervention is evaluated on the basis of efficacy and safety data generated by randomized controlled trials that are designed to fulfill regulatory requirements. However, clinical trials designed for regulatory purpose do not always address questions concerning the comparative effectiveness of a new therapy relative to other existing treatment options (Subedi et al., 2011).
- With growing interest in incorporating real-world evidence in health care decision making, it would be important to assess United States (US) health care stakeholder perceptions of how health economics and outcomes research (HEOR) data and other data derived from observational studies can be utilized for health care decision making.

OBJECTIVE

- To understand US health care stakeholder perceptions of how observational studies and other HEOR data can be used to inform the evaluation of emerging therapeutic options.

METHODS

Participants

- Fifteen US stakeholders were interviewed: 5 payers, 6 clinicians, and 4 hospital administrators participated in one-on-one telephone interviews. Interviewees were recruited from all regions of the US (Northeast, Southeast, Midwest, and West) to obtain a nationally representative sample.
- Participating payers were pharmacy and therapeutics (P&T) committee members representing plans ranging from 0.65 million members to 35 million members:
  - One medical director from a national plan
  - Two medical directors from regional plans
  - One medical director from an integrated plan
  - One pharmacy director from a national plan
- Participating clinicians:
  - Three physicians who were serving or had previously served in a P&T committee decision-making capacity at large hospitals (≥ 500 beds).
  - Three key opinion leaders with input on hospital P&T committees.
- Participating hospital administrators:
  - Two hospital administrators who were serving as hospital P&T committee members at large hospitals (≥ 500 beds).
  - Two hospital administrators who were serving as parties responsible for quality initiatives and Joint Commission accreditation with a decision-making role on hospital P&T committees (≥ 500 beds).

RESULTS

HEOR Data of Interest

- Stakeholders were asked to rate their familiarity with and the usefulness of different types of HEOR data in the evaluation of new therapies on a scale of 0 to 10, where 0 is not useful and 10 is extremely useful (Figure 1).
- Clinical efficacy and safety postmarketing assessments had the highest rating in terms of usefulness, followed by adherence, persistence, and cost-effectiveness data across all stakeholders.
- Hospital administrators found budget-impact analysis data to be much more valuable than other stakeholders did.

Registry Data

- Stakeholders were queried about familiarity and usefulness of registry data (Figure 2).
- Clinicians found registry data more useful than other stakeholders.
- Registries examining disease progression and registries examining therapies were similarly useful to stakeholders.
- Although registry data typically are not available at the time of initial review of a drug by a health plan or hospital formulary committee, annual/semiannual review of drug classes by health plans and hospital formulary committees may include registry data.
- Stakeholders indicated that the purpose of registry data is useful for confirming results of the registration trials and may have some impact on formulary placement.
- Safety data from registries were thought to be of particular importance to stakeholders.

> Compare [registry data] with [registration trials]…should confirm…leads to confidence in prescribing. —US Clinician

Figure 1. Stakeholder Rating of Familiarity and Usefulness of Various Types of Data From Nonregistration Trials

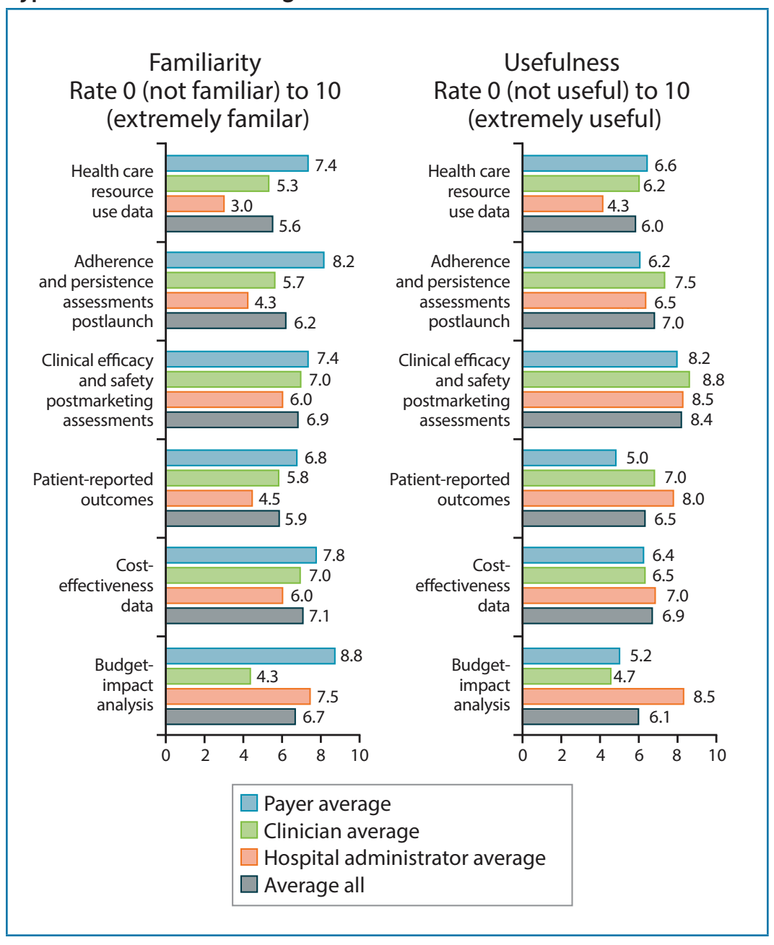
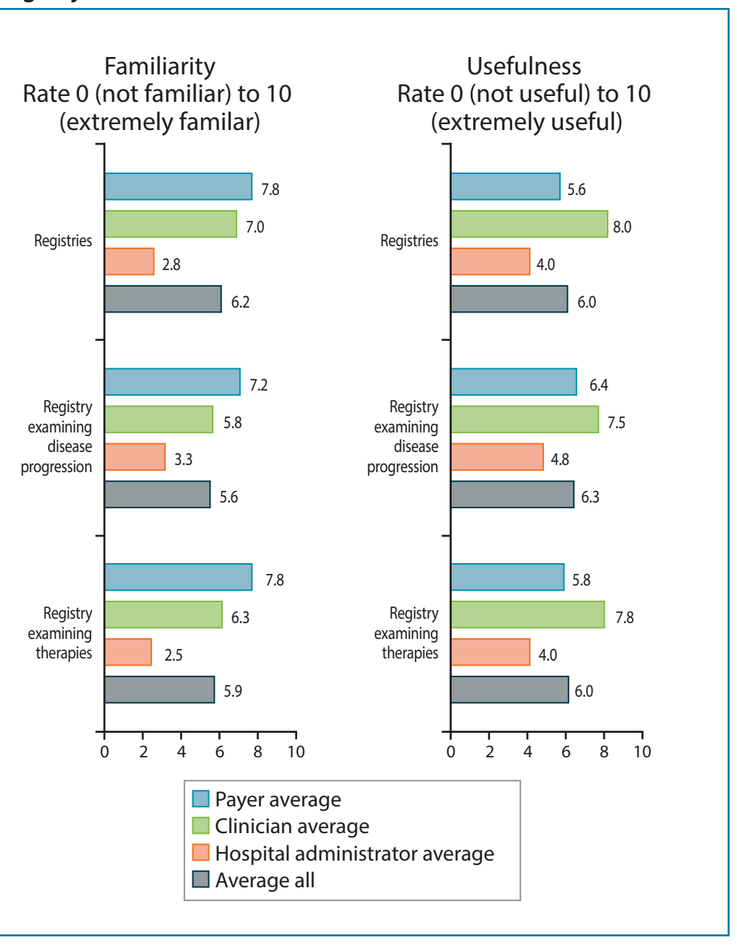


Figure 2. Stakeholder Rating of Familiarity and Usefulness of Registry Data



- When asked to choose between a large-scale registry or administrative claims data in the real-world evaluation of health care costs and utilization, adherence, clinical effectiveness, and safety, clinicians and hospital administrators preferred a large-scale registry, while payers' preferences varied.
  - Payer D indicated that neither is preferred, but registries tend to be more rigorous.
  - Stakeholders reiterated that quality, reliability, and addressing areas of need (i.e., answering influential research questions) are much more important than the study type.

> Neither is preferred, but registry is a little more rigorous… Really want pragmatic or head-to-head [studies]. —US Payer

Sponsorship

- Stakeholders preferred that studies be funded by a nonprofit organization rather than sponsored by manufacturers (Figure 3); however, they recognized that pharmaceutical companies have resources that nonprofit organizations lack.
- Stakeholders were concerned with bias in pharmaceutical-sponsored research in general; however, bias was even more of a concern with health economics research.
  - Among the 15 stakeholders, 11 indicated that sponsorship has an impact on the credibility of health economics research because costs are directly involved.
- Nevertheless, stakeholders indicated that they are seeking data that they can trust to help them make better, more-informed decisions. Hence, sponsorship is less important than study design, rigor, transparency, peer-reviewed publication, and journal quality.

> Rank order of credibility is: first = integrated health plan, second = academic, third = academic, but Pharma sponsored, fourth and really more like sixth place = Pharma internal study. Pharma should fund institutes that have good credibility in order to obtain seal of approval. —US Payer

> Protocol and scientifically and ethically sound are more important [than sponsorship]. Sometimes Pharma is the only source of funding. Some people won't look at pharma-sponsored research no matter what. I don't think this could be overcome no matter what Pharma does. —US Clinician

> Third party is better, and we understand why Pharma doesn't do more head-to-head, but head-to-head is what would make impact. —US Payer

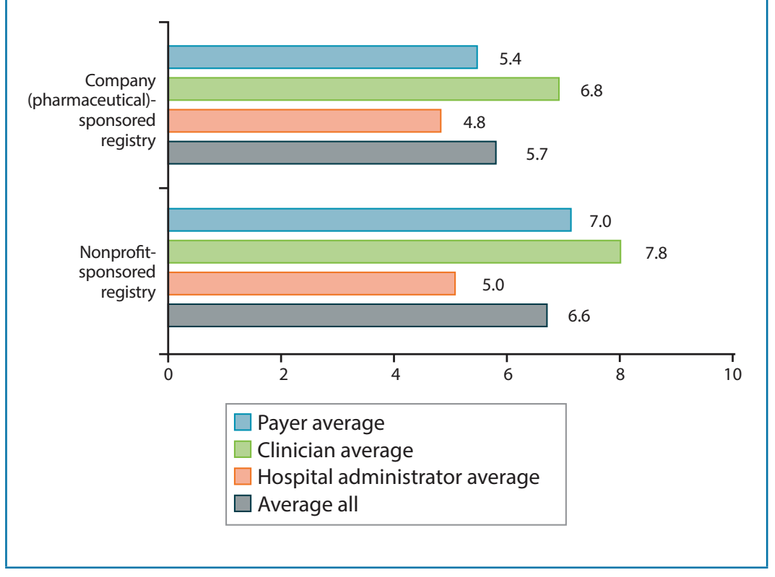
Data Gaps

- Real-world outcomes and head-to-head comparisons were the most commonly mentioned data gaps.

> Not going to put one drug in a preferred tier over another (except for contracting discounts) unless head-to-head data says so. —US Payer

- Clinicians would like to see more studies providing insights on patient treatment decisions, confidence in prescribing, and subpopulation data.
- On the other hand, payers and hospital administrators would like to see more data on costs, cost offsets, resource utilization, readmissions, and real-world outcomes.

Figure 3. Stakeholder Rating of Registry Data by Sponsorship



CONCLUSIONS

- Stakeholders are seeking more comparative effectiveness research addressing relevant research questions to better inform decision making.
- Comparative effectiveness research will have more impact on formulary decision making in the future.
- Rigor, transparency, and control for pharmaceutical-company bias are important factors for data credibility.
- Payers and hospital administrators rely on clinical experts to evaluate the efficacy and safety of new therapies, and they are more interested in studies examining costs, cost offsets, and resource utilization.

REFERENCE

Subedi P, Perfetto EM, Ali R. Something old, something new, something borrowed…comparative effectiveness research: a policy perspective. J Manag Care Pharm. 2011;17(9 Suppl A):S5-9.

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