

# Evidence Used During Pharmaceutical Technology Assessment

Nathan West, MPA\*; Musetta Leung, PhD; Michael Halpern, MD, MPH; Eric Faulkner, MPH • RTI International, Research Triangle Park, NC

## 1. Background and Study Objectives

Recent health reform legislation has called for an increase in the use of comparative evidence for decision making. In the current environment, there is limited understanding of the process and criteria employed in assessments of pharmaceutical evidence, including assessments by private and public health plans.

### Objectives

- To better understand the types of evidence considered and how they are used by health care payers and payer intermediaries to evaluate prescription drugs and biologics for possible formulary or preferred drug list inclusion
- To examine the extent to which payers consider the quality of the different types of evidence in their coverage decisions
- To compare these findings by health care payer attributes, including organization type and respondent type
- To supplement knowledge gaps that a comprehensive literature review could not address regarding current practices in pharmaceutical technology assessment (PTA)

## 2. Methods

- We conducted semi-structured 1-hour telephone interviews with key decision makers at payer organizations between November 2009 and March 2010.
- We contacted 34 organizations, 24 of which agreed to participate in our telephone interviews (71% response rate).
- Respondents included medical directors (N = 12) and pharmacy directors (N = 15). For some organizations, we interviewed both the pharmacy director and the medical director separately.
- Participants were asked to describe their PTA process and to rate the importance of the sources and types of evidence they review on a scale of 1 (not important) to 5 (very important).

## 3. Findings

### Respondent Characteristics

Table 1. Description of Participating Organizations

	Organizations Interviewed	Coverage Area	Approximate Number of Individuals Covered
National health plans	8	National	3 million to 35 million
Regional health plans	9	East, South, Midwest, West	600,000 to 3 million
Pharmacy benefit managers	3	National	10 million to 60 million
Medicaid agencies	4	South, Midwest	800,000 to 10 million

- The number of people covered varied widely among the national health plans. The smallest national plan covered 3 million individuals, and the largest plan covered approximately 35 million.
- Most of the national health plans provide Medicare Advantage and Medicaid managed care lines of business in addition to their large portfolio of commercial lines of business.
- The PTA process for these Medicare and Medicaid products is integrated into the decision-making process in place for products covering the commercial population.

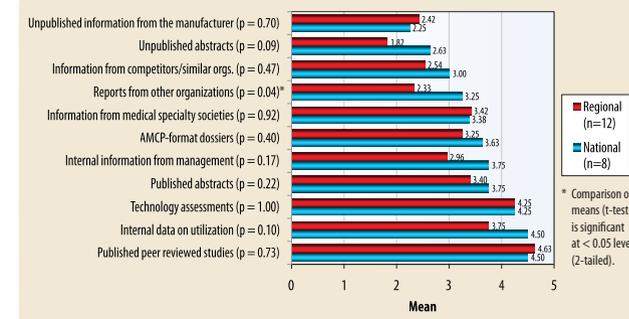
### Sources of Evidence

Table 2. Descriptive Statistics of Respondent Ratings of Importance Regarding Sources of Evidence (1 = not important; 5 = very important)

Sources of Evidence	N	Mean	Standard Deviation	Min	Max
Published peer-reviewed studies	26	4.64	0.63	3	5
Technology assessments	26	4.10	0.72	3	5
Internal data on utilization	25	4.08	0.95	1	5
Published abstracts	25	3.56	0.87	1	5
Information from within the organization (e.g., management)	24	3.44	1.17	1	5
Information from medical specialty societies	27	3.33	1.00	1	5
AMCP format dossiers	25	3.32	0.90	1	5
Reports from other organizations	26	2.73	1.00	1	4
Information from similar entities	27	2.61	1.27	1	5
Unpublished information provided directly by the manufacturer	26	2.12	0.98	1	4
Unpublished abstracts	26	2.08	1.02	1	4

- All respondents rated published peer-reviewed studies at least a 3, with a mean rating of 4.6; in the interviews, respondents noted that not all journals have the same credibility.
- Respondents also gave high scores for technology assessments and internal data on utilization (mean score of 4.1 for both).
- The majority of payers indicated that internal data on utilization, when available, were important during their PTA review and are used "all the time."
- AMCP dossiers were rated as having moderate importance.
- Unpublished abstracts and unpublished information from the manufacturer received the lowest mean rating of importance.

Figure 1. Comparison of Ratings by Sources of Evidence, by Plan Type



- We compared the responses between national and regional health plans to ascertain whether plan size may affect how sources of information are considered.
  - National health plans placed more weight on internal data compared to regional plans (4.5 vs. 3.8; p = 0.10).
  - National health plans gave more importance to evidence reports from other organizations (such as Medicaid Drug Effectiveness Review Project [DERP] reports) compared to regional plans (3.3 vs. 2.3; p = 0.04).
- We also compared the responses by the respondent's role in each organization. Medical directors and pharmacy directors may consider the importance of evidence sources and types differently.
  - Medical directors considered technology assessment to be a more important source compared to pharmacy directors (4.5 vs. 3.7 p = 0.01).
  - Pharmacy directors may consider information from within the organization and internal data on utilization to be more important because they may consider the two items to be similar (3.8 vs. 2.9) p = 0.06.

### Types of Evidence

Table 3. Descriptive Statistics of Respondent Ratings of Importance Regarding Types of Evidence

Types of Evidence	N	Mean	Standard Deviation	Min	Max
Randomized controlled trials	26	4.54	0.58	3	5
Review articles	27	3.63	1.04	1	5
Systematic reviews or meta-analyses	26	3.54	0.90	1	5
Professional society guidelines	27	3.46	1.02	1	5
Prospective observational or secondary data studies	27	3.43	0.93	1	5
Retrospective observational or secondary data studies	26	3.15	1.01	1	5
Budget impact	25	3.10	1.35	1	5
Cost and economic studies	25	2.86	1.06	1	5

- Respondents placed the highest value on randomized controlled trials (RCTs) (mean = 4.5).
- Review articles, systematic reviews or meta-analyses, and professional society guidelines were rated as important, but not as important as RCTs.
- Some respondents made the distinction that budget impact and cost and economic studies were weighted more heavily if they were based on internal data rather than information provided by the manufacturer.
- We found no statistically significant differences between medical directors and pharmacy directors regarding types of evidence.
- While not statistically significant at p < 0.05, national health plans rated review articles as more important compared to regional health plans (4.3 vs. 3.4; p = 0.06).

## 4. Discussion

- Study respondents seldom employed a singular or standardized approach to evaluating evidence (e.g., grading the evidence), but they do methodically review the evidence because they have strong preferences for some types and sources of evidence over others.
- Interviewees suggested the evaluation of evidence is a subjective process, "an art rather than a science."
- Across health plans, the number of individuals involved in compiling and evaluating the evidence ranged from multiple individuals in one national plan to a single medical director in one regional plan.
- Compared to regional plans, national plans reported less dependence on technology assessments performed by external organizations.
- Payers, across plan types, are becoming increasingly sophisticated in their assessment of published research that characterizes the efficacy/effectiveness of biopharmaceuticals.
- Comparative effectiveness research (CER) received mixed reviews from payer organizations. Payers expect that CER will be increasingly important in the future, affecting tier placements and utilization management of pharmaceuticals. However, respondents' opinions varied greatly regarding methods for CER, prioritization/government focus of CER resources, and interpretation of results.
- Many respondents were positive toward government-sponsored CER and eager for head-to-head data involving relevant comparators instead of placebos, although a few viewed existent CER as suboptimal regarding appropriate comparators or meaningful outcomes in a timely manner.
- Personalized medicine is an emerging area that payers sense will expand in coming years, but they have yet to make broad changes to plan operations based on personalized medicine advances in the near future.
- Respondents predicted passage of health care reform will have substantial effects on their plan operations, but they do not yet know what the effects will be as relates to their existing PTA processes.

## 5. Study Limitations

- This study lacked a random sample. We used a purposive sample of respondents and employed multiple uses of convenient sampling (e.g., established contacts, professional recruiting service).
- Interview protocols followed a semi-structured format, so many questions were designed to foster a more open-ended discussion and an opportunity for us to probe for more lucid information. Hence, we limit the number of methodologically derived conclusions that can be made based on our sampling approach and qualitative analysis.
- Future studies may benefit from surveying a larger randomly selected pool of PTA decision makers, such as other medical and pharmacy personnel involved in the P&T committee and formulary decision-making process. In this study, the population covered by these plans represented about 125 million, or approximately 40% of the U.S. population.

## 6. Conclusions

- Our findings confirmed that technology assessment plays a vital and influential role in the development of coverage and patient access policies, but our findings also brought to light key differences in the ways payer organizations evaluate evidence and make formulary decisions. Differences also existed between the role pharmacy directors and medical directors play in the PTA process.
- Payers have seen an increase in the types and sources of evidence for technology assessment; as a result, payers have become more critical in their evaluation and synthesis of the available information by ensuring that only relevant studies of quality are included for discussion and that decisions are made based on "best evidence."
- Greater transparency of the PTA process is one aspect of ensuring that information continues to be developed by evidence generators and systematically applied to coverage decisions using the evidence base.

## Acknowledgments

Funded by the National Pharmaceutical Council.

## Contact Information

\*Presenting author: Nathan West, MPA

Phone: 919-541-6816 · Fax: 919-990-8454 · E-mail: nathanwest@rti.org

Presented at: International Society for Pharmacoeconomics and Outcomes Research 15th Annual International Meeting, Atlanta, GA, May 15-19, 2010

## For further information:

RTI International, 3040 Cornwallis Road, Research Triangle Park, NC 27709  
www.rti.org

RTI International is a trade name of Research Triangle Institute.