How Do U.S. Payers Use Economic Models Submitted by Life Sciences Organizations?

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KEY POINTS . . .

This paper explores how company models are used by US payers and determines how and when models can make an effective contribution to improving formulary decision making.

Two surveys were conducted with payers who were registered in the AMCP eDossier System; October 2014 and March-April 2015.

Insights were gathered on payers' perceptions and use of industry-provided models.

The Role of Economic Models

Life sciences companies invest considerable resources in producing submissions to payers that demonstrate the cost-effectiveness of their products. Although dossiers contain a review of the relevant clinical data, a key feature is a pharmacoeconomic (PE) model, comparing the cost-effectiveness of the new drug with a relevant alternative, usually the current standard of care in the jurisdiction concerned. Some dossiers may also include budget impact models, if required and/or requested.

In jurisdictions with a well-defined structure and process for conducting technology assessments, the purpose of models, and their use by payers, is clear. For example, in the United Kingdom, the National Institute for Health and Care Excellence (NICE) has methods guidelines for manufacturers on how to produce their submission [1] and publishes detailed reports, indicating how the data and model were reviewed, the key considerations in reaching a decision, and recommendations on the appropriate indications for the use of the product. (http://www.nice.org.uk/guidance/published?type=ta).

Models can be useful as a source for the clinical data, for background information on the disease, to repopulate with one's own data and as a basis for constructing one's own analysis.

However, in jurisdictions with multiple payers, such as the US, the reimbursement landscape is much more complex and the precise nature of the use of submissions, especially PE models, is less clear and probably quite variable. Some standardization is achieved through the use of the Academy of Managed Care Pharmacy (AMCP) format for formulary submissions [2,3] and dossiers for some products are available through the AMCP eDossier System ("System") (http://amcp.edossiers.com). Nevertheless, very little is currently known about the use of PE models in the US Therefore, the purpose of this paper is to explore the use of company models by US payers and to determine precisely how and when models can make an effective contribution to improving formulary decision making.

Surveys of US Payers

Health care decision makers who were registered with the System were surveyed on their current use of pharmacoeconomic data provided by manufacturers, focusing in particular on the use of models. More than 1,200 payers are registered to use the System; mostly from managed care organizations (38%), but also from other settings, such as pharmacy benefit management (21%), hospital and care facilities (27%), and government institutions such as Veterans Affairs (4%) [4].

Two surveys were conducted: in October 2014 covering general issues, and one in March-April 2015 focusing on some specific issues concerning the use of models. In both cases a series of multiple-choice questions was used. (Copies of the surveys are available from the authors.) The number of payers responding to the surveys was 67 and 112 respectively. Forty-seven payers responded to both surveys and in each case the distribution of responders' work settings was similar to that of the registered users of the System in general. The responses were analyzed and discussed in workshops at the ISPOR meetings in Amsterdam (first survey) and Philadelphia (second survey).

Processes Followed by US Payers

Timing of assessments

In general the timing of payers' assessments of new products surrounded launch, with 78% of respondents to the first survey stating that they assessed products within 3 months of expected regulatory approval.

Figure 1. Range of Resources Available

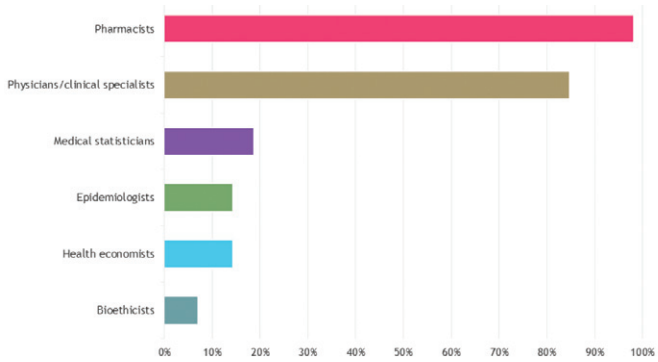


Figure 2. Payer Uses of Industry Models

Resources available to conduct assessments

Several skills are required to conduct assessments of the clinical- and cost effectiveness of drugs. Figure 1 shows the range of skills that respondents to the second survey reported were available to them. Perhaps the most interesting is that only 14% of payers reported having access to health economists; although it is likely that a number of pharmacists working on formulary management had training in health economics during their university education.

Consulting and using industry models

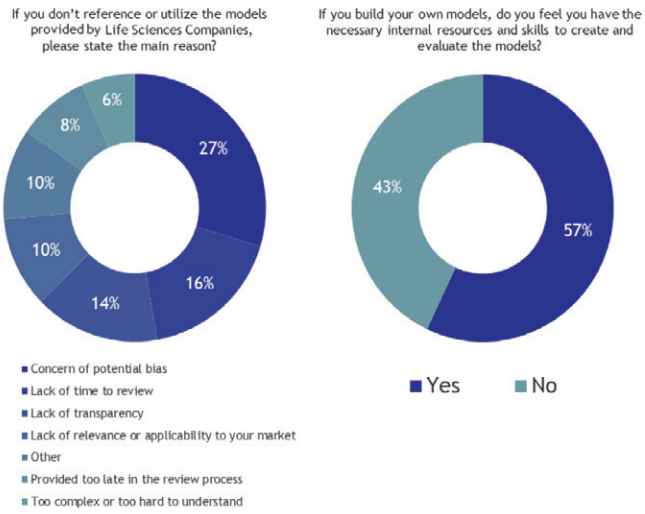
In the first survey, respondents were asked about the type of models/analyses their organization used most often in making reimbursement decisions, by giving them a menu of choices including administrative claims data analyses, budget impact models, cost-effectiveness/cost-utility analyses and cost-minimization analyses. Forty-five percent (45%) of respondents indicated that they used cost-effectiveness models most often in their reimbursement decisions, compared to 28% for budget impact models and 15% for administrative claims data analyses. Only 18% of respondents said that they never consulted industry models. A number of attendees at the Amsterdam workshop were surprised at this stated level of model use, as it is widely believed that cost-effectiveness models are not used by the vast majority of US formulary decision makers. Therefore, more detailed questions were asked in the second survey about respondents' use of industry models, which normally contain both cost-effectiveness and budget impact components. In the second instance, only 13% of respondents indicated they consulted industry models 'often', although 63% responded that they consulted them 'sometimes.' Twenty-four percent (24%) 'never' consulted industry models, which was slightly higher than the response to the same question in the first survey.

When considering how models were used, in the first survey, 22% of respondents indicated that they 'considered the (model) results as presented,' but 16% used electronic models 'to input their own data' and 39% used the industry models 'as a starting point for their own analysis.' In the second survey, the reasons why payers use models were explored in more detail. These responses are shown in Figure 2. It can be seen that respondents used models for multiple reasons, many of which are not necessarily related to using cost-effectiveness criteria in their decision making.

Reasons for not consulting industry models

As can be seen in Figure 3, when asked in the first survey, payers had concerns both about reliability (e.g., potential bias, lack of

Figure 3. Model Usage



transparency) and relevance (e.g., models not applicable to the local situation, or not timely). In the second survey, payer responses to the question 'What are the main deficiencies in industry models?' echoed these findings, with the most frequently reported reasons being biased assumptions (49%), timeliness of model availability (39%), not enough details being provided (35%), and models too complicated (33%).

Undertaking own analyses

It is one thing to be dissatisfied with industry models, but what are the alternatives? In the second survey, 18% of responders said that they 'often' construct their own cost-effectiveness models and 29% often construct budget impact models. In Figure 3 it can be seen that for those building their own models, 43% felt that they did not have the necessary internal skills and resources to create and evaluate models. A sub-group analysis of the second survey results indicated that payers who never consult industry models were also less likely to have access to epidemiologists, medical statisticians, or health economists. Around half of this same sub-group indicated, that in addition to not consulting industry models, they do not build their own models either. However, many payers in the second survey said that they consulted a wide range of 'trusted information sources' in making their formulary decisions. (See Figure 4).

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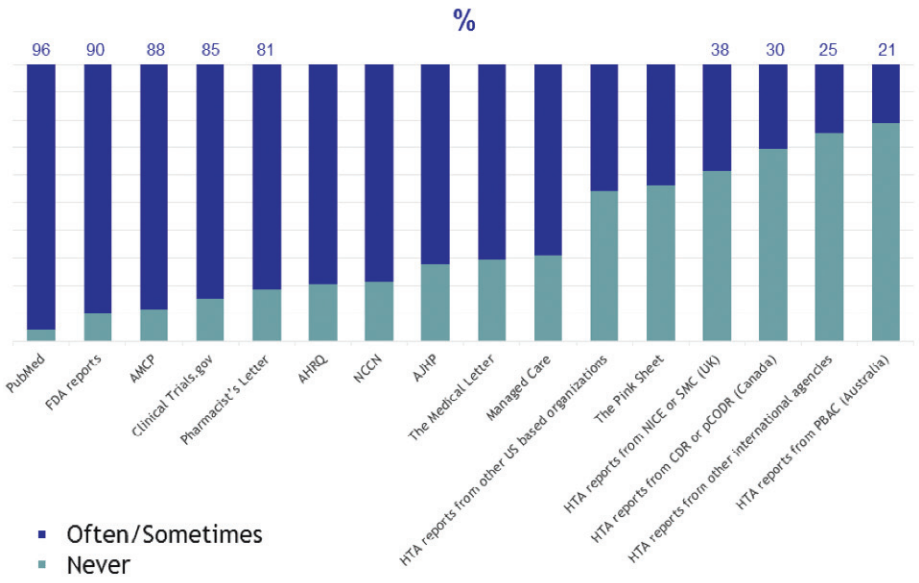
Figure 4. Trusted Information Services



AHRQ indicates Agency for Healthcare Research and Quality; AJHP, American Journal of Health-System Pharmacy; AMCP, Academy of Managed Care Pharmacy; CDR, Common Drug Review; FDA, US Food & Drug Administration; HTA, health technology assessment; NCCN, National Comprehensive Cancer Network; NICE, National Institute for Health and Care Excellence; PBAC, Pharmaceutical Benefits Advisory Committee; pCODR, pan-Canadian Oncology Drug Review; SMC, Scottish Medicines Consortium; and UK, United Kingdom.

Looking to the Future

The two surveys confirmed the view that the ways that US payers use the information provided by manufacturers are many and varied. A substantial proportion of the respondents to the surveys did consult industry models, but used them in several ways. 'Using' a model is not confined to considering the cost-effectiveness results. Models can also be useful as a source for the clinical data, for background information on the disease, to repopulate with one's own data, and as a basis for constructing one's own analysis. However, payers indicated a number of concerns about both the reliability and relevance of the models that were submitted to them.

There are a number of ways in which the current situation could be improved. Given that payers had concerns about the timeliness of models, it is important that manufacturers make these available early, within 3 months of regulatory approval of their products. In addition, in response to the comments about potential bias and lack of transparency, manufacturers should make their models as transparent as possible, conducting several sensitivity analyses of their own, but also allow payers to explore the impact of different assumptions and to input local data.

In addition, payers should make sure that they have access to the appropriate skills to critically assess industry models. This does not necessarily mean employing health economists or medical statisticians, but making sure that current staff are conversant with the appropriate methods, or seeking alliances with academic centers having the appropriate expertise. One of the reasons why organizations like NICE in the United Kingdom and the Pharmaceutical Benefits Advisory Committee (PBAC) in Australia have fewer concerns about industry bias is that they are confident in being able to detect it if it exists.

Also, given some of the complexities of using industry models, it is unlikely that payers will extract the maximum value for their decision-making by just studying a paper copy, or even an electronic version. Sometimes more interaction between payers and manufacturers may be useful during the formulary decision-making process; perhaps to request additional analyses or to discuss particular assumptions, provided this can be conducted in a way consistent with FDA regulations.

In all surveys there are often difficulties in interpreting the responses. The most surprising response in these surveys was the high percentage of individuals claiming to use cost-effectiveness/cost-utility analyses despite being offered a menu of analyses containing approaches, such as budget impact models and cost-minimization analyses, that could be confused with full cost-effectiveness analyses. Given the responses concerning the lack of internal skills and resources to develop or evaluate models, we also doubt whether many formulary decision makers in the US currently scrutinize cost-effectiveness models in the same way as the large public decision makers in Australia, Canada and the United Kingdom.

The second survey clarified that respondents 'used' industry models in a number of ways, many of which did not imply detailed scrutiny as part of the decision-making process. Many also used the information in industry models to conduct their own analysis. We did not ask what these analyses consisted of, but it is possible that many were simple comparisons of drug costs and clinical effects, perhaps also considering cost offsets in cases where these were substantial. Therefore, these approaches may be considered to constitute 'cost-effectiveness analyses' by the respondents in the surveys, although they would be aware that they differ from the kinds of analyses typically presented by manufacturers.

It is impossible to say whether this apparent interest in 'cost-effectiveness' signifies a future trend. In this context it will be interesting to survey formulary decision makers in the future, to assess their reactions to the existence of independently conducted cost-effectiveness analyses in the US, such as the drug assessments being carried out by the Institute for Clinical and Economic Review (http://www.icer-review.org). It will also be interesting to assess whether the existence of these assessments changes US decision makers' level of use and appreciation of industry models.

In conclusion, we believe that these surveys have increased our understanding of how US payers use the information submitted to them by manufacturers. Like all surveys, they rely on self-report

and we currently have no independent verification of the views that respondents expressed. It is also likely that those responding were more interested in the topic than the population of payers as a whole. Nevertheless, there was a considerable level of consistency in the responses between the two surveys and there is no obvious reason why the responses would differ from payers' real opinions.

Acknowledgements

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References

[1] National Institute for Health and Care Excellence. *A Guide to the Methods of Technology Appraisal.* 2013. Available at: http://www.nice.org.uk/aboutnice/howwework/devnicetech/guidetothemethodsoftechnologyappraisal.jsp. [Accessed December 5, 2013]. [2] Academy of Managed Care Pharmacy. The AMCP Format for Formulary Submissions. Version 3.0. J Manag Care Pharm 2010;16(Suppl. 1-a) [3] Academy of Managed Care Pharmacy. The AMCP Format for Formulary Submissions. Version 3.1. 2012. Available at: http://amcp.org/WorkArea/DownloadAsset.aspx?id=16209. [Accessed October 29, 2015]. [4] AMCP eDossier System. Registered user profile, current as of May 1, 2015. ■

Additional information:

The preceding article is based on survey results originally presented in workshops at the 17th ISPOR Annual European Meeting, Amsterdam, November 2014, and 20th ISPOR Annual International Meeting, Philadelphia, PA, USA, May 2015.

To view the presentations in which the survey is taken from the ISPOR 17th Annual European Congress in Amsterdam, The Netherlands, go to: http://www.ispor.org/Event/ReleasedPresentations/2014Amsterdam#workshoppresentations.

This topic will be presented at the ISPOR 21st Annual International Meeting in Washington, DC, USA, during Issue Panel 1: "Payers' Use Of Independent Reports In Decision Making – Will There Be An ICER Effect?" See pages 30-31 for further meeting details.