FROM THE JOURNALS

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HEALTH POLICY ANALYSIS

Challenges with Forecasting Budget Impact: A Case Study of Six ICER Reports

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In determining coverage policies, budget impact models (BIM) continue to be an important decision-making tool for many payers despite the inherent challenges in predicting future costs. This article highlights the issue within the context of U.S. formulary decision-making. Retrospective analysis of pharmaceutical sales data was used to estimate actual patient utilization to compare against budget impact results as modelled and reported by an independent body, the Institute for Clinical and Economic Review (ICER) prior to formulary decision. Based on inclusion criteria for types of medical technologies of interest that had been the focus of an ICER review, six ICER studies conducted prior to 2016 were selected for this analysis. Three BIM outputs were collected (aggregate therapy cost, therapy uptake and price) and compared against real-world estimates generated using drug sales data. Two categories of BIM estimates were considered, "predictive" and "contemporaneous". The first category covered newly approved drugs, and used forecasted future uptake. Note that prior to 2016, according to ICER guidelines, the uptake was assumed to be "unmanaged" – ie without "restraint on utilization" by insurers). The second category investigated treatments already on the market and measured their current managed uptake and budget impact. Representing the former and latter categories, four and two ICER reports were included, respectively.

In order to generate corresponding real-world estimates for results provided in the selected ICER reports, sales data from the IQVIA National Sales Perspective and National Prescription Audit were used. The primary outcome was the annual aggregate treatment cost for the drug included in the ICER BIM analysis. In the study, the aggregate treatment cost was the product of the estimated therapy cost for a single patient, and total uptake for one year. Real-world estimates were constructed to be consistent with the definitions of those used in the ICER report. In terms of year of analysis, most ICER reports did not state the year modelled. In those situations, the authors calculated estimates for the first calendar year following the report.

The analyses found that there were large differences in the real-world data based retrospective estimates compared to the earlier modelled predictions especially for the "predictive" modelled studies, where an "unmanaged uptake" assumption was used. In these, the predicted uptake exceeded ex post real-world estimates by an average of 25-fold. In addition, the modelled aggregated treatment cost exceeded the real-world data, by an average of 36-fold. Prices in the models exceeded those in the real world data estimates by 15%. In the category of "contemporaneous" studies, the modelled uptake estimates were less divergent, but still exceeded real-world estimates by 7.6-fold, while aggregate treatment cost exceeded by 8.6-fold. Interestingly, price estimates were 24% lower than reflected by real-world data.

The authors attribute the overestimation by the models especially within the predictive studies to the ICER's assumption of "unmanaged uptake" which, since 2016, is no longer used by the ICER organization in its studies. Beyond this, the authors acknowledge that it is impossible to ascertain how much of the differences are due to methodological differences or other factors. In the 2 contemporaneous studies, results were expectedly closer to the authors' real-world estimates but still larger by several fold. The authors discuss several reasons for this including the possibility of the ICER report itself influencing policy-making and in turn use and access which they term the "ICER effect".

Although the generalizability from six studies is difficult, this study is of interest as it examines the process of assessment itself. Introspection of the process is equally important in order that the system of assessment and methods used can be improved. Although the study was conducted within a U.S. payer setting, the results provide important lessons to researchers and decision-makers globally. Given the constraints on the health care budget, budget impact modelling continues to be key in decision-making in many settings. High budget impact is often a reason for population restrictions on the use of a health technology. Yet the results and applicability of the model results have rarely been examined after the primary decision. To this reader, although the study does not provide an easy solution to the challenges faced in budget impact modelling particularly in assigning assumptions for novel therapies, it highlights that decision-makers should be aware of and understand the assumptions used within the models as they make critical decisions which determine patient access. It also underlines the value of process reviews and reassessment of technologies, continued transparency in assessment methodology, data source use and decision-making. These can generate understanding and help spur improvements in formulary decision-making.