Cost-effectiveness data

Are neurologists in practice equipped to interpret them?

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Neurologists in clinical practice endure multiple demands on their time. Excellent and safe clinical care is their foremost priority; however, several ancillary calls also require their attention. Increasingly complex regulations, nonuniform insurer systems, and marketing claims all erode into precious time necessary for clinical scholarship. Generators of these nonclinical demands answer to mandates different from ours. Payers value risk-sharing schemes based on cost-effectiveness.¹ Similar priorities influence industry and payer decisions about pricing and coverage for new drugs or technologies. Industry, while keen on promoting innovation, cannot decouple its mission entirely from profit. Clinical competency is our ascendant mission and it should be distant from other mandates. Historically, in the United States, a combination of politics, plenty, and people's will have isolated all stakeholders, not just clinicians, from cost concerns. Nevertheless, we can ill afford to be ignorant of the basics of health economics. High cost of targeted therapies, enlarging beneficiary pools, and competing technologies are affecting health policy priorities, and secondarily, clinical decisions also. Now, we in the United States should start to face unpleasant truths about resource restraints.²

In this issue of *Neurology® Clinical Practice*, 2 articles, one by Cutter and Wang³ and the other by Young et al,⁴ provide us with helpful and timely advice in interpreting some key aspects of cost-effectiveness studies. They caution us against projection of short-term benefits to long-term gains in chronic diseases, and accepting the merits of a *p* value without the benefit of knowing the sample sizes. When different treatments are compared, the claim to superiority may depend on the comparator used, including the base rates of events in the placebo arm. A single set of sterile data is insufficient; a contextual reference to whether the data translate to clinically important differences is necessary. Statistical significance need not result in a minimally significant clinical improvement. These discrete messages alert us to the strengths and guard us from the wily charms of cost-effectiveness studies. Cutter and Wang stress their points using repurposed data from published good-quality articles. Cost-effectiveness studies often rely on modeling and administrative data. Young et al discuss lucidly the value of modeling studies, but only touch briefly on the use of administrative databases. They do not hesitate to comment on the uncertainties associated with modeling. Administrative databases are a mother lode of data; however, they are subject to the inaccuracies of claims submission and incompleteness of diagnostic (ICD) coding.⁵

Why do we need to understand the basics of cost-effectiveness analysis?

We seldom factor in cost-effectiveness while providing Medicare Parts A and B covered services; however, cost concerns tend to dominate the privatized Part D drug coverage tiering decisions. A similar situation applies to private payer pharmacy benefits; often they parallel Medicare

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coverage decisions. That cost-effectiveness is not central to Medicare A/B coverage has long been a source of debate, and even despair, without a clear future.⁶ For practicing neurologists, an awareness of the basics of cost-effectiveness analysis (CEA) is important as they try to decipher payer policies on drug or technology coverage and denials. When our requests to cover new drugs or technology are denied or substantially modified, we need to know the basis for such an action. Possibly it resulted from a payer's misread or misinterpretation of a CEA, or perhaps it is our inability to parse the methods section. Being aware of the payer's point of view, tenable or otherwise, will enhance one's position during appeals and discussions.

What do we do if we are presented with CEA data?

Should we be market-friendly early adopters or skeptics rejecting all CEA? Neither position is acceptable in any specialty. Procedure-intensive cardiology recommends a "show me" the benefit mindset.⁷ Could we requisition the services of Cutter or peers every time? Their current advice may be inapplicable when new CEA studies, using different methodologies, importune us to make critical patient care decisions.

Practicing neurologists, and clinicians in general, are not mavens in statistical methodology, nor are they expected to be.⁸ Remaining current and competent in the clinical sciences leaves little time for developing nuanced expertise in statistical and analytical methods. Will we be able to take laudable heuristic CEA tours as we prepare to appeal critical insurer policy decisions and evaluate product superiority claims and conflicting benefits of competing treatments?

What resources do clinicians have when the intricacies such as modeling, multivariable analysis, and mathematical details overwhelm us?

We need help from reputable objective resources that evaluate cost-effectiveness data and come up with recommendations. We could refer to a transparency of reporting standards checklist such as the Consolidated Health Economic Evaluation Reporting Standards (CHEERS) to increase the credibility of an economic evaluation.⁹ We may consult a free citation registry of cost-effectiveness analysis that is currently available, but its nonsubscription content does not include interpretations or recommendations (<https://research.tufts-nemc.org/cear4/Home.aspx>). Ultimately, however, for trustworthy answers, we should look to our respective specialty societies to guide us. Position statements or synthesized recommendations are necessary. In this regard, the American Academy of Neurology's repertoire of guidelines and recommendations is commendable. They should now expand to include cost-effectiveness analyses of selected therapies. Navigating alone, without biostatistical help, or time to research primary data sources, is not the preferred path. As Cutter and Wang point out, deconstructing cost-effectiveness analysis is "more than simple."

REFERENCES

1. Towse A, Garrison LP Jr. Can't get no satisfaction? Will pay for performance help? Toward an economic framework for understanding performance-based risk-sharing agreements for innovative medical products. *Pharmacoeconomics* 2010;28:93–102.
2. Neumann PJ, Rosen AB, Weinstein MC. Medicare and cost-effectiveness analysis. *N Engl J Med* 2005;353:1516–1522.
3. Cutter GR, Wang G. Cost-effectiveness analyses: beauty and the beast? *Neurol Clin Pract* 2013;3:436–439.
4. Young KC, Kelly AG, Holloway RG. Reading a cost-effectiveness or decision analysis study: five things to consider. *Neurol Clin Pract* 2013;3:413–420.
5. Sarrazin MSV, Rosenthal GE. Finding pure and simple truths with administrative data. *JAMA* 2012;307:1433–1435.
6. Neumann PJ, Chambers JD. Medicare's enduring struggle to define "reasonable and necessary" care. *N Engl J Med* 2012;367:1775–1777.
7. Krumholz HM. Medicine should be more like Missouri. *Circ Cardiovasc Qual Outcomes* 2009;2:289–291.
8. Concato J. Is it time for medicine-based evidence? *JAMA* 2012;307:1641–1643.
9. Husereau D, Drummond M, Petrou S, et al. Consolidated Health Economic Evaluation Reporting Standards (CHEERS) statement. *BMJ* 2013;346:f1049.

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