

Lessons for Health Technology Assessment: It Is Not Only about the Evidence

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Keywords: health-care decisions, HTA.

Introduction

Lessons from health technology assessment (HTA) organizations around the world are emerging, and only some of them are about evidence. Other lessons pertain to governance, communication, trust, politics, and the persistence of local “practice patterns” for evidence evaluations. This should not come as a surprise. HTA organizations worldwide have different constituencies and organizational structures, and exist amid different cultural traditions, political systems, and fiscal climates.

European versus American HTA

Broadly speaking, health technology assessment to inform health policy decisions has received a more favorable reception in Europe than in the United States, although to be sure, controversies persist in Europe and the receptivity to HTA varies across the continent. In general, the idea of establishing public HTA organizations has been a more natural fit with the more centralized, government-funded, and administered health-care systems of Europe.

European sensibilities on HTA are evident in the European Union Commission’s High Level Group on Health Services and Medical Care conclusion in November 2004 that, “HTA has become a political priority and there is an urgent need for establishing a sustainable European network for HTA” [1]. The EUnetHTA that was created as a result was envisioned as a sustainable European Network for Health Technology Assessment to inform policy decisions, and to connect public national HTA agencies, research institutions, and health ministries, enabling an exchange of information and support of policy decisions by member states [1]. With over 60 partners (including national and regional HTA agencies, as well as research institutions, international organizations, such as the Cochrane Collaboration), it reflects a government-focused vision for the usefulness of HTA (although it should be noted that neither EUnetHTA nor its members are actual government employees in most countries).

Within Europe, differences in practices exist across HTA organizations [2–4]. Some agencies are much more transparent than others about their deliberations, for example. There are also differences in terms of how HTA organizations set priorities, the degree to which stakeholders are permitted to provide input, how results are communicated, how HTA organizations interact with national reimbursement authorities, and how explicitly entities use decision analytic models and cost-effectiveness analysis.

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10.1111/j.1524-4733.2009.00558.x

As just one example, policymakers in northern European countries, such as The Netherlands, Sweden, and the United Kingdom, have been more enthusiastic users of cost per quality-adjusted life-year thresholds than their counterparts in central and southern Europe. Even among the countries using cost-effectiveness analyses, local experts have expressed different preferences for certain methodologies (e.g., the inclusion of friction costs in cost-effectiveness analyses in The Netherlands) [5].

American traditions and the American health-care environment have created a different climate for HTA in the United States [6]. The current economic crisis notwithstanding, cross-national surveys have consistently revealed that Americans generally tend to favor less government control compared to citizens of Western European countries [7]. Compared to Europe, the United States has a more privately oriented and decentralized health-care system, with significantly more cost sharing for its citizens [8].

The idea of centralized “big-government” HTA has historically received a much more hostile reception in the United States. A National Center for Health Care Technology existed for a short time but was eliminated by the Reagan administration in the early 1980s [9,10]. The Congressional Office of Technology Assessment, which conducted numerous and generally well-regarded health technology assessments, was removed by the Republican Congress elected in 1994. The Agency for Healthcare Research and Quality was threatened with extinction in the 1990s in part because of what was perceived as overly prescriptive clinical practice guidelines [11,12].

The American HTA organizations that have evolved and even flourished in this inhospitable environment are a varied lot, characterized by a strong private sector influence and carefully bounded authority. Large private health plans, such as Blue Cross/Blue Shield, Kaiser Permanente, and Aetna have developed their own health technology expertise and practices. Private organizations conducting HTA for the marketplace, such as ECRI Institute and Hayes, Inc., are also well established. Smaller private health plans rely on external expertise, but also conduct their own assessments, which tend to be opaque affairs, with no open meetings or appeals processes or published minutes about the rationale underlying decisions.

Public HTA organizations in the United States exist but play a circumscribed role, focusing on clinical (not economic) evidence and tailoring assessments to the populations under the agency’s purview. They tend to be more open and explicit than private HTA organizations about their processes and deliberations. Examples include evidence reviews conducted by the federal Medicare program, which evaluates 10 to 15 national coverage decisions each year and posts decisions and the rationale underlying them on the Medicare website [13]. Similarly, the drug class reviews conducted by the Drug Effectiveness Review

Project, an alliance of 14 state Medicaid agencies and several nonprofits that produce evidence reports, are available on its website [14].

The American landscape, thus, holds some unique challenges for HTA organizations, given the decentralized and privately based health-care system, the multitude of health plans, and different cultural attitudes toward the appropriate role of government. Arguably, stakeholders—one might call them “interest groups”—hold more sway in the United States than elsewhere, which make public HTA initiatives more challenging to implement. As others have argued, the United States is characterized by “deliberatively obstruction-oriented political structure” that frustrates government programs, even if they reflect popular aspirations and values [15,16]. Advancing HTA in this climate will require a delicate balancing act.

Achieving Balance/Future Directions

Needs of Stakeholder

Despite regional differences, the needs of HTA stakeholders worldwide are, by and large, universal. Payers want an evidence-based and politically acceptable process that balances efforts to increase access to effective new technologies against fiscal constraints. Manufacturers desire transparency, timeliness, and a reasonable degree of predictability. That is, to the extent possible, they want to reduce uncertainty around HTA, so that they understand the “rules of the road” and can plan accordingly.

To be sure, all HTA organizations profess to make evidence-based judgments. Moreover, thanks to the Internet, all have rapid access to the same published evidence (and often to the same or similar unpublished evidence). Still, guidance is needed to ensure that HTA organizations adhere to good evaluation practices and are accountable to their constituents.

Guidance for HTA organizations worldwide might be divided roughly into two categories: evidence-related lessons and non-evidence-related lessons. In terms of evidence, HTA institutions have always grappled with questions of how to synthesize and make sense of the totality of available information, particularly information from nonrandomized sources, and information about the economic value of medical care [17]. If the modern era of evidence evaluation matured in the 1960s to 1980s with the advent of randomized clinical trials (RCTs) and the beginnings of formal efforts to integrate evidence from nonrandomized sources, the field might be said to have entered a postmodern era in the last decade or so, characterized by more sophisticated attempts to synthesize the clinical evidence and to measure value and cost-effectiveness explicitly. Looking ahead, policymakers should keep three areas at the forefront of HTA: focusing on value and economic efficiency, incorporating real-world data; and developing better procedural rules around HTA implementation.

Focusing on Value and Economic Efficiency

Worldwide, there has been a steady, if somewhat uneven, march towards the formal consideration of cost-effectiveness in HTAs. In Europe, some countries (e.g., the United Kingdom, The Netherlands) have well-established procedures for considering cost-effectiveness in reimbursement decisions, while others (e.g., France) do not consider health economic information formally, although cost-effectiveness analysis may be recommended in manufacturers’ submissions [2].

In the United States, while there has been some progress, the trend toward using cost-effectiveness analysis has been slower to develop. American HTA organizations tend to separate the evalu-

ation of clinical evidence from economic evidence. This is explained as a political accommodation in a country where open consideration of cost-effectiveness remains largely anathema [18]. Unlike many of their European counterparts, American policymakers have been reluctant to use cost-effectiveness analysis openly, which likely reflects cultural and political influences, and, at least in part, the absence of a national health-care system which mitigates against the consideration of societal resource allocation decisions [18]. Nevertheless, US health policymakers in the private and public sectors continue in quieter fashion to develop strategies to use evidence of comparative value, striving to balance fiscal realities while avoiding the charge that they are explicitly rationing needed care [19]. For example, the US Medicare program has undertaken a number of projects that make use of cost-effectiveness information to inform coverage and payment policy, although not to deny services out rightly [20].

For payers worldwide, open consideration of economic efficiency raises challenges. Nevertheless, the lack of procedures for considering economic evidence forthrightly also creates problems. It contributes to mistrust, because observers assume that costs are considered surreptitiously in reviews. Considering costs separate from the clinical review tends to focus ultimate reimbursement decisions on a drug’s price rather than its overall value [21]. Questions about the value of health-care strategies are best considered in an evaluation in which clinical and economic evidence are combined simultaneously.

The debate about appropriate HTA practices would benefit from more openness and clarity about cost-effectiveness. It would also profit from a broader discussion of the value of health-care strategies, including diagnostic tests, medical procedures, and care delivery, rather than a narrow focus on drugs and devices. Finally, the debate on value would benefit from attention to the need for disinvestment of inefficient technologies, as well as investment in efficient ones [2].

Incorporating Real-World Data

The international medical community has long recognized that RCTs, while providing a gold standard of efficacy evidence, are of limited generalizability, typical community-based medical practice, and are expensive to conduct [22]. Efforts are underway to formalize the collection and evaluation of “real world” data, sometimes described as data for decision-making that are not collected in conventional RCTs. As the recent International Society for Pharmacoeconomics and Outcomes Research (ISPOR) Task Force on Real World Data emphasized, sources of data other than from RCTs can contribute to the evidence base in important ways by demonstrating how drugs and devices work under conditions or in populations not studied in trial, or relative to interventions not included in the study [22].

Despite these developments, there is a need for more clarity about which methods and data to use to answer particular questions [23]. A particular challenge relates to how to pool data from nonrandomized sources. Studies have shown that even for the pooling of data from randomized trials in meta-analyses, data extraction techniques can be prone to errors that can negate or even reverse the findings of the study [24,25].

Good Process Matters

Finally, there are a number of non-evidence-related lessons that involve the importance of good “process” for technology evaluations. The notion of “best practices” for conducting health technology assessment continues to evolve, although consensus is coalescing around several dimensions [26]. Good process means independence for those conducting assessments; transparency

about the criteria used to judge evidence as well as the evaluation decisions and the rationale underlying them; openness about the composition of the committees performing assessments; explicit timelines for completing assessments; opportunities for stakeholder input; and clear rules for appealing decisions.

Furthermore, a key recent process innovation relates to the registration of RCTs. Many medical journals now require investigators to deposit information about trial design into an accepted clinical trials registry before patient enrollment begins [27]. The idea is to ensure that information about the existence and design of clinically directive trials are publicly available in order to alleviate concerns about bias. Despite concerns about the burden of such a requirement, the response has been very strong: ClinicalTrials.gov, for example, contained 40,000 trials as of April 2007 with 200 new trial registrations weekly [27]. A key question for the future—and one that could help bolster the integrity of HTAs—is whether registries can extend to nonrandomized evidence and even decision analytic models. Possibly, such evidence can be registered before studies are begun, or at least data and models can be made available on the Web in some form for those who want to analyze them.

A final point concerns the importance of trust and good communication between HTA organizations and product manufacturers. Some friction between drug companies and HTA organization about the content and process surrounding evidence evaluations will always exist. Nevertheless, statements that one often hears suggest a wide gulf:

Things drug company officials often say about HTAs:

- Evidence-based medicine is code for cost containment;
- HTA organizations care only about RCTs;
- Absence of evidence is not evidence of absence;
- HTA organizations ignore my models; and
- HTA processes are not transparent.

Things HTA officials often say about (and to) drug companies:

- We are focused on *value* not on costs;
- We *do* use nonrandomized evidence;
- We don't trust *your* nonrandomized evidence;
- We do not trust your models either; and
- We are more transparent than we used to be.

Of course, HTA organizations and drug company perceptions about evidence reflect their perspectives as evaluators (and sometimes buyers) and sellers, and their different agendas. As American author and professor of sociolinguistics Deborah Tannen has pointed out, communication frequently reflects tacit agendas [28]. In the case of drug companies and HTA organizations, the conversation is about evidence but reflects an unspoken conversation about money and value. HTA organizations want to serve as responsible fiscal stewards; drug companies seek to maximize shareholder value. What seems notable about the communication surrounding HTA is the lack of trust on both sides. A reasonable question is whether we can establish HTAs processes, in which the discussion is solely about the evidence. For reasons enumerated above, the answer is almost certainly not entirely. The tacit agendas will always exist. But more consensus about HTA processes would help foster a more open and honest dialogue.

Conclusions

Differences in health systems, political traditions, national income, and local practice patterns will continue to translate into HTA differences across countries. We should not be surprised

that HTA organizations worldwide vary on so many dimensions, that they sometimes reach different conclusions, and that non-evidentiary factors play an important role. Still, some lessons for HTA are universal: the need to develop rigorous scientific evidence evaluations, as well as procedures that are transparent, fair, predictable, and efficient. There remains considerable room for improvement in moving HTA towards more predictability and rationality.

This will, of course, require resources as well as leadership, not only among government officials, but among individuals in professional organizations, including the ISPOR membership. The goal should be to improve the science and to reduce uncertainty. Citizens deserve high-quality evidence evaluations, and those making investment decisions about technology deserve to know rules of the road.

Source of financial support: ISPOR provided a modest honorarium.

Peter Neumann has no conflicts to declare.

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