

The Growing Importance of Health Technology Assessments (HTAs) in Reimbursement Decision-Making

By Julia Green, BS, Research Associate; Floortje van Nooten, MSc, Senior Research Associate and European Manager; and Robert M.A. Thwaites, MA, MCom, Senior Executive Director, Europe and Senior Scientist, Center for Health Economics and Science Policy; and William Lenderking, PhD, Senior Research Leader, Center for Health Outcomes Research

In order to decide whether a new drug or medical technology should be funded, a wide-ranging evaluation is typically needed. A formal health technology assessment (HTA)—the systematic evaluation of evidence gathered to consider the medical, social, economic, and ethical implications of the development, diffusion, and use of medicines, devices, and other health-related technologies—can help structure and facilitate this evaluation. Governments and payers are increasingly relying on HTAs to help with funding and reimbursement decisions.

This development has provided significant challenges for companies, particularly pharmaceutical companies. Not only is the use of HTAs expanding rapidly (there are now over 40 national agencies globally, each with its own standards and procedures for HTA evaluation), but

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evaluation methods are continually evolving as technologies advance and health

care structures and organizations change. HTA considerations are assuming an increasingly important role in the process of drug development, with pharmaceutical companies being required to produce evidence of effectiveness and cost-effectiveness in order to gain market access and reimbursement in a number of markets.

In order to understand the landscape for HTAs across the world, United BioSource Corporation (UBC) conducted a review of HTA requirements in 10 markets. Three key issues were examined for each country: initial market access (including market authorization and reimbursement), pricing, and continued market access. The use of HTAs and the typical process of market access are different across countries.

Achieving market access for a new product typically involves a number of steps. In all countries, approval by a regulatory body is required before a product is authorized for use. This approval depends on the safety, efficacy, and quality data for a new product or indication. If a product is to be funded or reimbursed, additional conditions must be fulfilled before a product can be launched or marketed. These criteria vary among countries but can include effectiveness, safety, drug price, budget impact, and cost-effectiveness, and can be assessed at a national level, sub-national or regional level, or in the case of the United States, by the payers. For pricing, the most common alternatives are that either the manufacturer has discretion in setting the price of the product or the manufacturer negotiates with the appropriate department within the ministry of interest to determine the price. Continued approval for a certain price or expanded or continued access to the market may require additional post-marketing evidence from the sponsors. Below, we provide three short examples of how HTA is conducted in the United Kingdom (UK), Japan, and the United States (U.S.). These countries were selected as case studies because they represent both the broad variability in HTA processes globally and also reveal similarities.

The National Institute for Health and Clinical Excellence (NICE) in the UK is well established and arguably the most stringent HTA body in the world. Both cost-effectiveness and clinical evidence are important factors in the Institute's reviews and recommendations, with clear requirements for literature review and epidemiological information, and set standards for complex, indirect comparison analyses and economic evaluations. Reviews are conducted by a large committee of academics and researchers drawn from universities in the UK and can take six to nine months to complete. Reviews can entail independent reconstruction of economic models submitted by sponsors, among other steps.

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Recommendations are then made based on explicit thresholds for cost-effectiveness.

In contrast, HTAs have not yet played a major role in Japan or the U.S. Japan's primary criterion for obtaining favorable reimbursement is clinical benefit. Health economic data may be filed along with clinical data, although this is not listed as an essential component of the submission. Once marketing approval has been granted, application for reimbursement must be filed with the Ministry of Health, Labor and Welfare (MHLW) in order to be listed on the National Health Insurance reimbursement list (i.e., a national formulary). The reimbursement price for new treatments is usually determined on the basis of comparison with existing drugs from the same category in Japan and other markets, although new drugs can receive premiums for innovation, usefulness, and market size. If no comparable price is available for a new drug, the price calculation is based on cost-plus methodology, whereby production/import costs, promotion expenses, general administrative expenses, distribution expenses, operative profit, and consumption tax are summed in order to determine pricing. In 2007, the MHLW; the Education, Culture, Sports, Science and Technology (MEXT); and, the Economy, Trade and Industry (METI) passed a "5-Year Strategy for the Creation of Innovative Pharmaceuticals and Medical Devices" with the aim of boosting the Japanese pharmaceutical/medical devices industry and streamlining review processes. Their goals included an accelerated review process, higher standards for clinical trials, and the expansion and improvement of review staff. Although these strategies mark modifications in the Japanese health care system, change is expected to be slow.

Unlike the UK or Japan (and many other countries), the U.S. has no central authority or consortium of ministries responsible for formal assessments of new therapies. A product can be marketed with FDA approval alone, although comparative effectiveness evaluations are beginning to assume greater importance. Payers control access through formulary listings on different tiers, usually with generic medications on Tier 1, preferred branded products on Tier 2, and other branded products

on Tier 3. Each tier is associated with different co-payments, with Tier 3 requiring the greatest contribution from the patient. Currently, the most important private organizations involved in HTA-like evaluations are large health plans such as Wellpoint, with cost-effectiveness analyses and budget impact models often included in these HTAs. There is no formal requirement for demonstration of cost-effectiveness for public insurers (Medicare and state Medicaid). Although the U.S. health care system is likely to change over the next few years, the initial focus may be more related to coverage expansion than methods of decision making for new treatments.

Similarities across countries in how HTA is applied seem to be related to general methodological considerations (e.g., the use of cost-effectiveness data, the acceptability of modeling), whereas the differences reflect both national and cultural values around health care and some of the details around assumptions and the type of evidence required (e.g., discount rates applied, cost-effectiveness threshold, economic perspective adopted). Given the nature of decision making in health care systems, these detailed differences will continue to reflect the local diversities that are present for each country.

An important focus of HTA will continue to be geared towards supporting countries' efforts to contain health care costs. Next to price cuts, clawback systems (i.e., repayments to the government when financial gain arises from unforeseen trading benefits, discounts or parallel trade) and rebates, other measures will be put in place. For example, some risk-sharing schemes (where the drug manufacturer shares some financial responsibility for the health outcomes produced) have already been created in the UK, and it is expected that patient access schemes, risk-sharing schemes, and co-pays will continue to expand as a way for payers or agencies to shift the cost burden (and also the risk burden) on to other parties.

Furthermore, the evidence to justify the value of a product will become increasingly important, with stricter requirements for initial market access as well as continued reimbursement for the product after market launch. HTA studies may need to be

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completed earlier in the drug development lifecycle; a number of countries (Australia, for example) are exploring how to bring the timing of reimbursement decisions in line with the timing of decisions on market approval. Transparency in the HTA process across markets will also assume increased importance, allowing for greater accountability in the use of HTAs in decision making.

Finally, greater collaboration and discussions among the health authorities of different countries are expected. NICE, for example, is passing on advice to an increasing number of developed, as well as developing, countries interested in health care reform. For the foreseeable future, HTAs are likely to play an increased role in the adoption and reimbursement of new products around the globe. Sponsors will need to consider HTA requirements as another important factor in drug development programs in order to ensure market access.

For more information, please contact
Julia.Green@unitedbiosource.com,
Floortje.Vannooten@unitedbiosource.com,
Rob.Thwaites@unitedbiosource.com, or
William.Lenderking@unitedbiosource.com.