



THE USE OF HEALTH TECHNOLOGY ASSESSMENTS (HTA) TO EVALUATE MEDICINES – KEY PRINCIPLES FROM GSK

Market-based pricing for reimbursed pharmaceuticals, in which companies are free to set prices and there are no supply-side or demand-side controls, remains the industry's preferred solution to meeting the needs of patients and society's demand for better medical treatment. However, markets in Europe tend instead to be characterized by monopsonistic payer structures, i.e. markets where buyers rather than sellers control large parts of the market, over-regulation, poor resource allocation, slow access for new medicines, and focus on cost rather than value.

Given the absence of genuine market conditions, the pharmaceutical industry is committed to engaging with governments and other payers to discuss processes and principles that will enable the development of pricing systems that reflect the value of products and reward innovation. One key activity where industry believes such principles can be applied is Health Technology Assessment (HTA).

Health technology assessments (HTA) refer to the process of using existing evidence to evaluate the clinical effectiveness, cost-effectiveness and broader impact of a health technology on patients and the healthcare system. Mechanisms to evaluate the clinical and/or cost-effectiveness of medicines are used in a majority of European Union member states. Evaluations have a critical impact on pricing and reimbursement decisions and, in the case of the United Kingdom, on prescribing guidance.

GSK recognises the desire of governments to develop mechanisms to assess the clinical and/or cost-effectiveness of medicines. However, in the current cost-driven climate, there is a risk that evaluation mechanisms will run counter to what should be their key objective: identifying medicines that bring the greatest benefit to patients, ensuring early access to these medicines, allowing choice among medicines of value and ensuring efficient healthcare through objective high-quality assessments. There are examples of evaluation leading to increased uptake and patient choice in multiple areas such as diabetes, cancer, cardiovascular. However, evaluation systems often remain a lottery at national level and are utilised with the objective of restricting choice.

The wider environment within which HTAs are conducted requires implementation of G10 Recommendations 3 and 6 in all member states¹. Recommendation 3 calls on member states to ensure that the time taken between the granting of a marketing authorisation and pricing and reimbursement decisions be fully consistent with Community legislation. Recommendation 6, which proposes that price controls on those medicines that are not reimbursed by the state should be lifted, should allow companies to set market prices for these products at launch, thereby preventing access delays of innovative medicines.

¹ In May 2002 the High-Level Group on Innovation and Provision of Medicines (G10) under the chairmanship of Commissioners Liikanen and Byrne released a report containing 14 recommendations for improving the competitiveness of the European pharmaceutical industry while encouraging high levels of public health protection. (http://pharmacos.eudra.org/F3/g10/docs/comprep_nov2000.pdf)



Member states and the European Commission should view HTA as a means to achieve better health outcomes, rather than a means to delay or even exclude innovative medicines from reaching patients.

If the potential opportunity presented by HTA is to be realised, an understanding between the pharmaceutical industry and governments on the functioning of HTA mechanisms is crucial. In order to achieve this understanding, a number of key principles must form the basis of evaluation mechanisms, regardless of the particular shape they take.

Key Principles

1. HTAs should be based on a clear, sophisticated and differentiated view of what constitutes value

The current debate on so-called 'me-too' drugs is not helpful. It oversimplifies the nature of therapeutic progress in medicines and overlooks the realities of pharmaceutical R&D processes. There should be more clarity and consensus on the criteria against which therapeutic progress (or value) can be identified throughout a product's lifecycle. The measures of value can include: mortality and morbidity data, side-effects, tolerability, predictive surrogate parameters, pharmaceutical form, route of application, compliance, ease of use, impact on the healthcare service, disease severity, medical need, quality of life, and patient preferences.

Improvements under any of these heads may constitute innovation that is of value to sub-groups of patients, and systems which restrict access to such improvements damage the choice available to doctors and patients, and hence damage the delivery of optimal health outcomes.

2. HTAs should be transparent and balanced

Where HTAs are focused on delivering guidance, the evaluating body should be independent of the payer. Evaluation systems should be clear and consistent with regards to methodology, criteria used and data required – this would include clear timeframes for the evaluation and any decisions arising from it. Processes need to be in place to ensure efficient and independent handling of appeals. The grounds for appeal should extend to a different interpretation of evidence, and parties entitled to appeal should include pharmaceutical companies. HTA guidance should enable physicians sufficient freedom to address individual situations.

When HTA is part of the reimbursement process, the requirements will necessarily be different, although transparency and balance remain essential. The Transparency Directive should apply with regard to deadlines, assessment criteria and appeal processes.



3. HTAs should be based on early and inclusive dialogue, including with patients

Industry should be able to understand and predict what authorities expect in terms of therapeutic added benefit and what kind of benefit is deemed worth paying for. This will require better dialogue between industry and authorities, which should start prior to the marketing authorisation. Dialogue should be structured around disease priorities, unmet medical and disease-management needs, and a clear understanding by payers and industry of which benefits are particularly relevant to patients and healthcare professionals in a given therapeutic area. Realistic and relevant criteria and study objectives should be agreed at all stages.

The HTA process should be inclusive, allowing at least an advisory function for the medical sector, patients and the pharmaceutical industry. The views, experiences and expertise of patients must be integrated into the evaluation process to allow for a better evaluation of the balance between benefits, costs and risk. Physicians and other clinical experts must also be involved in assessment and decision-making - decisions should not be made without input from specialists in the therapy area on the full range of benefits delivered.

4. Evaluations should be flexible to allow new data to be considered

A 'one size fits all' approach to the timing of appraisals fails to take account of the complexity of conducting assessments and ignores differences in treatments and therapeutic areas. The data generated for registration of medicines is seldom adequate to fully show a new medicine's effect on the treatment of a particular disease and its impact on the healthcare system as a whole. Often the sort of data needed to confirm cost-effectiveness and clinical effectiveness is data on real-life clinical use of a medicine. This can only be collected once a medicine has been on the market for a period of time. Pharmaceutical companies should therefore be able to submit health outcomes information to the relevant government bodies throughout a product's lifecycle. This evidence should receive appropriate attention and reward from payers. Systems should be established to enable the real-life benefits of medicines to be evaluated so that they can be incorporated into post-launch assessments of medicines.

5. Risk-sharing and flexibility is required in handling uncertainty

The fact that data may be incomplete at the time of launch creates a temporary uncertainty as to the full therapeutic value of a new product in use. Attention needs to be given to the design of new policies that would give payers and industry a flexible partnership approach to handling this uncertainty. For example, perhaps products, once they have received marketing authorisation, should be able to enjoy early, reimbursed launch, on the understanding that the provision of further data may lead to changes in reimbursement (which could 'benefit' either the payer or supplier).



Proper implementation of these partnership approaches would, however, be key to allow a proper handling of further data. In particular: payers and healthcare bodies would need to cooperate with industry to set up and maintain efficient data-collection infrastructures; and, in order for any risk-sharing process not to become a protracted series of price re-negotiations, payers should collaborate with industry to set up agreements which enable both parties to look at desired outcomes, timeframes, criteria, data collection capabilities, and volume targets.

6. Comprehensive understanding of the benefits of a drug in disease management is needed

Perspectives on a drug's value should be broad. Even where added benefit is identified objectively on the basis of agreed criteria, it may not be taken sufficiently into consideration within the framework of disease management needs and priorities. A comprehensive look, involving the opinions and experiences of the medical profession and individual patients or patient groups is needed to identify where unmet need in the real-life management of a particular disease exists, how quality treatment can be increased and how the optimal, appropriate and efficient use of a drug can be ensured.

7. Payers should commit to rewarding added value

Where payers seek value for money, pharmaceutical companies require money for value. With evaluation processes playing an increasingly important role in reimbursement decisions, governments must commit to rewarding medical advances. The reward society gives to an innovative medicine must reflect its added therapeutic value. Reward for innovation can come in different forms – such as price-setting or readjustment, volumes, therapeutic guidelines recognising new therapy, as well as speed of access.

8. Positive HTA outcomes should be implemented

Where the outcome of an evaluation is positive, payers, whether at national or local level, should commit funding to encourage implementation. Commitment to implementation and consistent prescribing is an important factor in evaluation mechanisms.

9. HTA should apply to all healthcare interventions

Evaluation and assessment should not be discriminatory by applying only to innovative medicines, but should where appropriate be applied to other forms of healthcare interventions.



Factors which would undermine the optimal outputs of the HTA process

1. A single, Euro-wide assessment

There should not be forced, harmonised EU evaluation. The outcome of an evaluation reflects local circumstances, different medical practice and healthcare delivery, specific healthcare priorities, and payer choices on what to consider and what to fund, influenced by the national pricing and reimbursement process and available resources.

2. HTA and regulatory review must not converge

The HTA should be separate from the regulatory review for the grant of a marketing authorisation. Regulatory review, whether through the EMEA or through a national regulatory authority, must be based on objective and scientifically verifiable criteria of efficacy, safety and quality. HTA must not become a fourth hurdle in marketing authorisation.

3. Silo Budgeting

Evaluations should take a balanced view of both cost-effectiveness and clinical effectiveness data. This means that the specialism of pharmaco-economics cannot be used as the sole measure of added benefit. It is an insufficient tool for measuring the full consequences of innovation, and increases payers' temptation to make a direct, mechanistic link between price and cost-effectiveness. A narrow focus on pharmaco-economics will ignore the indirect benefits of a new therapy, such as productivity gains, and reduction in caregiver and personal time costs. The priorities of the patient population, the nature of the therapeutic market and availability of alternative treatments, the perspective of medical specialists, affordability concerns and effects on macro-economic growth should all be recognised in decisions about price and reimbursement.

Silo-budgeting – the assessment of costs and benefits within a narrow operational cost-centre – is inimical to the true objective of HTA, which is to help decision-makers obtain the maximum health gain and economic benefit from health-care investment. Yet this broader view – healthcare expenditure as an investment not a cost – is rarely taken by healthcare administrators. More discussion must be stimulated of the wider macro-economic aspects of healthcare decision-making if “silo budgeting” is not to put at risk the optimisation of health-gains.



4. Cherry-picking

The UK's National Institute for Clinical Excellence (NICE) was created for the purpose of ending postcode prescribing and to increase uptake of new medicines. While NICE is increasingly becoming a guiding model for HTAs, governments have so far abstained from replicating NICE in its entirety. This is sometimes a question of countries lacking financial resources to set up and run an organisation such as NICE. However, there is a risk that countries will only introduce those elements that best allow evaluation mechanisms to serve as cost-control bodies. 'No-go' areas in this respect are the lack of arbitration procedure, evaluation bodies that are composed of 'payers' only, unrealistic or excessive timelines, and ad hoc changes in the criteria or comparators required by payers.

5. Inefficient reward for proven added value

A situation in which payers will not agree to a price increase even where sufficient data has been provided to prove superiority of a product must be avoided. There also needs to be careful consideration of access to wide patient populations to ensure that sufficiently significant data can be collected during the data-gathering period.

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