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POSITION PAPER

THE USE OF HEALTH TECHNOLOGY ASSESSMENTS (HTA) TO EVALUATE MEDICINES

KEY PRINCIPLES

Market-based pricing for pharmaceuticals remains the industry's preferred solution to meeting the needs of patients and society's demand for better medical treatment. Competitive markets are the most efficient way of allocating resources and rewarding innovation.

Given the absence of genuine market conditions in many countries, 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 activity where industry believes such principles can be applied is Health Technology Assessment (HTA).

HTAs 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, always separately from product registration. Evaluations have a critical impact on pricing and reimbursement decisions and, in the United Kingdom (and to a lesser extent other countries), on prescribing guidance.

Industry 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 objectives: 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, and cardiovascular. However, evaluation systems often remain a lottery at national level and are utilised with the goal of restricting choice.

The wider environment within which HTAs are conducted requires implementation of G10 Recommendations 3 and 6 in all member states[1]. Recommendation 3 calls on member states to ensure that the time taken between the granting of a marketing authorisation and pricing and reimbursement

[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. Both Health and Competitiveness Councils subsequently endorsed the recommendations. However, not all of them have been implemented. http://pharmacos.eudra.org/g10/docs/G10-Medicines.pdf

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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.

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 new 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**

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 administration, 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 focussed 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. Pharmaceutical companies should have a right to appeal. HTA guidance should enable physicians sufficient freedom to address individual clinical situations without adverse impact on reimbursement, formulary inclusion or patient co-payment.

When HTA is part of the pricing and 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 process of HTA 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-

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making - decisions should not be made without input from specialists in the therapy area on the full range of benefits delivered.

4. Evaluations should 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 show fully 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. 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 should be able to enjoy early, reimbursed launch, on the understanding that the provision of further clinical outcomes 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 in-market data-collection infrastructures. In order for any post-marketing review not to become a protracted series of price re-negotiations, payers should collaborate with industry to set up the approaches through which both parties may understand and evaluate real-world benefits and impact of medicines.

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 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.

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8. HTA outcomes should be implemented

Negative evaluations tend to be adhered to. 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.

10. Assessment should take place at national level

Whereas an international discussion of methodology and approaches towards assessing HTA data is desirable, assessment of individual products or therapies should be done at a national level. 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.

11. HTA should remain separate from regulatory review

The HTA should be separate from the regulatory review for the grant of a marketing authorisation, as it is currently the case. 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.

12. Evaluations should take into account indirect benefits

Evaluations should take into account 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.

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