Merck’s Position Statement – Health Technology Assessment (HTA)

Why it Matters

Policy makers globally recognize the importance of good health for economic development and are striving to bring quality health care to the population. In this context, value for money is an important consideration for governments and payers. Health Technology Assessment (HTA), increasingly used in the broader context of Relative Effectiveness Assessment (REA), is a process that uses evidence to evaluate the clinical efficacy, cost effectiveness and broader impact of a health technology on patients and the health care system.

HTAs need to account for national economic, organizational, social and ethical priorities, and consequently need to be country-specific.¹ For an HTA, health technologies comprise not just biopharmaceuticals, devices, diagnostics and treatments, but also other clinical, public health and organizational interventions. Depending on the scope of the evaluation, HTA can be approached from several different levels. “Micro-level” HTA is based on the evaluation of specific technologies, such as innovative medicines and technological devices. “Macro-level” HTA takes a more holistic view, focusing on the efficiency of organizational processes or health systems, and is used as evidence to support health policy and macroeconomic decision-making.²

As the effective and efficient use of health care resources and innovations becomes an increasingly important global issue, HTA serves as a scientific and multidisciplinary mechanism for governments and payers to make informed decisions about how to allocate limited resources and new technologies. In the absence of a universal definition of what constitutes value, “micro-level” HTA can potentially serve as an access barrier for patients to the uptake of innovative drugs. “Macro-level” HTAs may serve as a more complete way to analyze health systems, including policies, financing and regulatory approaches.³ The primary goal of HTA should not be a budget-based assessment of a single therapy. Rather, it should approach technology assessment with a patient-centered focus,⁴ evaluating how to best ensure access to new and innovative therapies.

Merck Position

Health Technology Assessment is a complex and evolving issue that requires transparency, inclusion of all stakeholders, and careful evaluation. If the opportunity presented by HTA is to be fully realized, Merck suggests the following key principles underlie evaluation mechanisms, regardless of the form the HTA evaluation process takes:

1. HTAs should be patient focused, and be based on clear, sophisticated and differentiated definitions of value.

2. HTAs should be built on early, inclusive dialogue, including patients, the biopharmaceutical industry, health care providers, and governments.

3. Evidentiary requirements should be regionally harmonized, with an understanding that the way HTA bodies evaluate this data will depend on local context and needs.

4. “Micro-HTA” can undermine governments’ efforts to provide timely access to new health technologies, and when used, must be transparent and uniform.

5. “Macro-HTA” by HTA bodies can improve health care quality and outcomes, by making decisions across health systems.

6. Evaluation frameworks should be carefully assessed, and remain flexible to allow consideration of new data.

7. The HTA evaluation process should be predictable, transparent, and well-balanced.

8. Voluntary parallel track submission pathways should be opened, but HTA and Regulatory review must not converge.

9. Risk-sharing and flexibility is required, given uncertainties in the evaluation process.

(Expanded principles below will be linked to the above shorter background on HTA)

1. HTAs should be patient focused and be based on clear, sophisticated and differentiated definitions of value.
   
   - HTAs need to be patient-centered, allowing for substantive patient input. We believe that HTAs should identify and promote health technologies that are of value to patients and allow for weighted patient participation in the HTA evaluation process—including a permanent role of patients in decision-making. We support initiatives, such as the European Patients’ Academy on Therapeutic Innovation (EUPATI), that educate patient advocates to ensure their voice is better heard in the HTA process.

   - Countries should avoid using HTA to create access barriers to innovative medicines, and instead utilize HTA to inform on health outcomes and patient satisfaction. It’s important that the voices of patients and advocates be heard in all steps: from early guidance, to assessment, to decision-making.

   - Merck believes that HTAs should result in higher rewards for higher-value, most innovative medicines.

2. HTAs should be built on early, inclusive dialogue, including patients, the biopharmaceutical industry, health care providers, and governments.
   
   - Biopharmaceutical developers need to understand what authorities expect in terms of therapeutic added benefit and what benefit they judge worth paying for. This will require earlier dialogue between industry and authorities prior to marketing authorization. Dialogue should focus on determining benefits that are particularly relevant to patients and health care providers. Realistic criteria that consider the limitations companies face should be agreed upon at all stages.

   - Merck understands that HTA systems in many countries are in development and we are committed to working with governments and payers toward ensuring HTAs fulfill their role – to accelerate patients’ access to innovative technologies, medicines and interventions, while taking into consideration constraints on budgetary resources.

   - The implementation of a national HTA system is labor intensive and requires strong technical expertise, adequate financial resources and a well-functioning information system. If governments decide to implement a central HTA body, they should carefully identify the resources and capacity needed. Merck is committed to supporting countries to develop pragmatic HTA evaluation approaches which align with national public health priorities.
3. Evidentiary requirements should be regionally harmonized, with an understanding that the way HTA bodies evaluate this data will depend on local context and needs

- An important and urgent goal of HTA bodies should be the regional harmonization of evidentiary requirements. The evaluation of this evidence will be based upon country-specific context, but national HTA bodies and HTA networks need to clarify and harmonize evidence expectations. We encourage the efforts of organizations such as EUnetHTA and HTA Network in harmonizing European evidentiary standards and assessment methodologies to be more reliable, timely, and transparent, while also recognizing the need for country-specific inputs. This has led to recommendations to "globalize the evidence [and] localize the decision." 5

- Merck advocates in favor of HTA bodies that tailor their HTA framework to account for local contexts, including societal values; and not simply "copied" on existing European HTA systems. An HTA evaluation framework used in one region may be inappropriate for use in another, given local differences in culture, economies, epidemiology and other factors. For emerging countries to effectively utilize HTAs, they must first build health system capacity, including infrastructures, good governance, regulatory capabilities, financing and partnerships.

4. “Micro-HTA” can undermine governments’ efforts to provide timely access to new health technologies, and when used, must be transparent and uniform

- Merck acknowledges governments’ need to evaluate the clinical and/or cost-effectiveness of medicines. These assessments are not intended to overshadow governments’ main priorities to provide timely access to new health technologies, allow patients and practitioners to choose medicines with the greatest benefit, and to ensure cost-effective health care through stringent evaluations. Despite this, a narrow “micro-HTA” can undermine governments’ efforts as it may not provide adequate guidance in context of the entire health care system. Systems that restrict access to therapeutic improvements affect the choices available to doctors and patients, which may negatively influence health outcomes.

5. “Macro-HTA” by HTA bodies can improve health care quality and outcomes, by making decisions across health systems

- Merck supports HTA bodies in the use of “macro-level” HTA, which utilizes evidence-informed decision-making to improve the health care quality and outcomes of populations. This approach will ensure that policy decisions are guided by a transparent scientific and systematic approach.6

- Using a more holistic view of HTA, policy makers and payers will have a better idea of how to systemically achieve increased value from available health resources, and will be in a better position to make appropriate decisions on allocating them.7

- Merck advocates for "macro-level" HTA to be applied to health systems as a whole, looking at infrastructure, delivery systems, workforces, insurance schemes, policies and other health-related interventions.7,8

6. Evaluation frameworks should be carefully assessed, and remain flexible to allow consideration of new data.

- Merck understands that re-assessment of national HTA systems to evaluate their benefits, impact on resources allocation, and cost is necessary. In line with remaining transparent, this should be approached very carefully and methodically, allowing input from the

---

8. Voluntary parallel track submission pathways should be opened, but HTA and Regulatory review must not converge.

- Regulatory approval and technology assessment require independent bodies and separate evidence in their evaluation processes, but these bodies need to ensure that evidence generation mechanisms and methodologies during product development do not conflict. Merck advocates open discussion and agreement between all stakeholders about how areas of divergence in evidentiary requirements should be dealt with during product development, as well as the development of parallel track submissions with regulatory agencies and HTA bodies.

- While communication between the two bodies is paramount, it should focus on evidence requirements and generation methodologies, not during the evaluation of a particular therapy. The two processes must remain independent, as the regulatory review (benefit–risk) is fundamentally different than HTA evaluation (relative effectiveness).

9. Risk-sharing and flexibility is required given uncertainties in the evaluation process.

- Available data may be incomplete at launch as a result of limited patient populations and/or limited resources for generation, therefore pragmatic and creative solutions to reimbursement and HTA evaluation are necessary. 'Risk-sharing' has been utilized in both the context of outcomes-based and financial-based agreements, particularly through several well-developed channels in the European Union.³

- This process can provide benefits to payers and the biopharmaceutical industry, allowing access to innovative therapies while sharing associated risks. This pathway to market should remain voluntary, and allow payers and industry to collaborate on agreements that enable both to evaluate timeframes, volume targets, and data collection capabilities.

About Merck
Merck is a leading science and technology company in healthcare, life science and performance materials. Around 40,000 employees work to further develop technologies that improve and enhance life – from biopharmaceutical therapies to treat cancer or multiple sclerosis, cutting-edge systems for scientific research and production, to liquid crystals for smartphones and LCD televisions. In 2014, Merck generated sales of € 11.3 billion in 66 countries. Founded in 1668, Merck is the world’s oldest pharmaceutical and chemical company. The founding family remains the majority owner of the publicly listed corporate group. Merck, Darmstadt, Germany holds the global rights to the Merck name and brand. The only exceptions are the United States and Canada, where the company operates as EMD Serono, EMD Millipore and EMD Performance Materials.

Merck’s biopharma business
With headquarters in Darmstadt, Germany, Merck’s biopharma business offers leading brands in 150 countries to help patients with cancer, multiple sclerosis, infertility, endocrine and metabolic disorders as well as cardiovascular diseases. Merck discovers, develops, manufactures and markets prescription medicines of both chemical and biological origin in specialist indications. We have an enduring commitment to deliver novel therapies in our core focus areas of neurology, oncology, immuno-oncology and immunology. For more information, please visit http://biopharma.merckgroup.com/en/index.html