

Takeda's Position on Health Technology Assessment

Background

Takeda's mission is to strive towards better health for people worldwide through leading innovation in medicine. We act on our mission by championing our corporate values: Patient, Trust, Reputation, and Business. We help save lives and improve the quality of life of patients globally and build trusting relationships with all stakeholders in society, which drives our business.

Our patient-centric philosophy and customer orientation impact the way we view Health Technology Assessment (HTA). HTA determines the value of health solutions to patients and society based on their innovation and relative benefit. HTA aims at evaluating in a transparent and unbiased manner the medical, social, economic, ethical and organizational issues, including direct and indirect impact, of health technologies. Such technologies include drugs, biologics, devices, medical procedures, healthcare delivery and management systems (e.g., disease management programs and payment systems) that increase the productivity and efficiency of total healthcare service.

We advocate for the appropriate use of HTA through rigorous, transparent, data-driven methodologies in the evaluation of patient and societal benefits derived from innovative healthcare solutions. By applying such methodologies, HTA can accelerate delivery of innovative health technologies, better aligning patient, provider, and payer decisions with the best available science.

Takeda firmly believes that approaches to the analysis of evidence to support decision-making on health technologies should observe a common set of HTA evaluation principles. Despite differences between country-specific and regional procedures, these principles apply to both the evidence generation and its application to decisions, namely, relative effectiveness, medical risks and benefits including their economic, social and ethical implications as well as treatment and payment decisions.

Takeda's position

Takeda outlines the following principles as a framework to guide the design and implementation of HTA, seeking dialogue and cooperation with all key stakeholders to advocate patient outcomes while ensuring proper evaluation of innovative healthcare technologies.

HTA exists to balance patient outcomes with the associated economic impact through multifaceted approach.

This is a delicate and complex balance to achieve, one that impacts patients, their families, communities, and our society at large. Focusing on patient needs first is our mission and responsibility.

HTA and its associated processes have been in practice in many countries, yet its record of driving patient outcomes while rewarding innovation has been mixed. We participate in discussions related to HTA to advocate for rigorous, transparent, data-driven methodologies, and we believe that HTA can play a valuable role to drive medical and scientific progress which can drive better patient outcomes.

Taking into consideration our expectations and those of our stakeholders, we have identified the following guiding principles that should be consistently applied to HTA.

Advocate for better patient outcomes

Takeda believes that improving patient outcomes should be at the centerpiece of HTA. Consistent with this view, we believe that it is critical that the HTA process takes into consideration the patient perspective and gives equal importance to patient reported outcomes. Furthermore, HTA should not cause delays in drug evaluation timelines or reimbursement following drug approval but rather ensure an efficient system that prioritizes patient health outcomes. Finally, HTA should not be used as means to restrict patient access to innovative medicines for budgetary purposes.

Incentivize and reward innovation

Incentivizing and rewarding innovation is vital for the healthcare ecosystem. HTA must take into consideration that innovation and scientific progress involves risks and investments of time, human capital, and financial investments with varying expected payoffs.

R&D investments may lead to incremental improvements or paradigm shifts in health outcomes. Progress in healthcare often relies on numerous small steps in order to reach major breakthroughs, which move the entire healthcare ecosystem forward and more importantly

Improve the lives of patients

Establishing a transparent, consistent, and predictable link between demonstrable healthcare impact and corresponding drug reimbursement will act to encourage and reward investments in the discovery and development of new innovative healthcare solutions, ultimately driving better patient health outcomes.

Pursue optimal implementation

Minimize burdens

While HTA should seek the highest quality and most accurate results, governments and private payers should minimize incremental burden of HTA such as administrative burdens in data collection and bureaucratic costs so

as to appropriately allocate resources towards research and development. On the other hand, HTA should prioritize the topics for which there are clear evidence gaps and which are of greatest importance for society, based on a priority-setting process that has broad stakeholder input.

Account for national healthcare system differences

HTA must be tailored to the unique geographic and healthcare system context each country faces. Since there is no one-size-fits-all approach, policy makers should recognize the strengths and weaknesses of each country's system and process and seek an optimal approach when introducing HTA.

Ensure a transparent governance model

HTA requires an unbiased and a transparent governance model. Such a model must seek to engage all key stakeholders in dialogue and remain independent of decision makers.

Independent of decision makers

HTA should be conducted independent of payers and pricing and reimbursement agencies. The role of the agencies should be to assess the therapeutic value of various healthcare technologies and their cost effectiveness in a transparent manner. This approach allows an objective and unbiased assessment of healthcare technologies and subsequent recognition and reward of innovation.

Engage all stakeholders

The healthcare industry comprises a complex stakeholder landscape, including public and private payers, patients, healthcare professionals, manufacturers, policy makers, and academics. The acceptance of HTA hinges on extensive stakeholder consultation throughout the process to ensure that costs and benefits are appropriately balanced and perspectives included.

Acknowledge holistic value

HTA must acknowledge holistic value by transparent, multifaceted and robust data-driven methodologies that may be updated and revisited as new information arises.

1. Agencies should first assess the therapeutic value of healthcare technologies take into consideration of not only clinical outcomes but also direct and indirect benefits, quality of life criteria and patient preferences.
2. Agencies should then assess the economic value of healthcare technologies taking into consideration of direct and indirect costs of all relevant technologies including operating expenditures, productivity losses and/or gains, societal impact of the disease, potential treatment, potential savings in medical and health related costs etc.

The above approach should be based on accepted, scientifically rigorous databases and expertise which may



extend beyond clinical trial data to include actual outcomes, such as real-world data, and not be limited to clinical trial data alone. e.g. post-marketing surveillance, observational data, pharmacovigilance or diseases registries.

March 2016