Benchmarking the impact of HTA on new medicines development and coverage decision making

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Introduction

Health Technology Assessment (HTA) is increasingly used to evaluate new medicines in order to inform coverage decision making and guide allocation of healthcare resources. Beyond the traditional regulatory requirements of quality, efficacy and safety, HTA considers the effectiveness, appropriateness and cost of medicinal products and technologies. Pharmaceutical companies are under pressure to adjust their drug development and submission strategies to accommodate both regulatory and HTA requirements for commercial success.

In addition, the variability in HTA organisations and methodologies that are utilised in HTA appraisal and coverage decision-making processes in different countries results in a complex and challenging environment and it is therefore important for companies to incorporate a clear understanding of HTA requirements into early strategic planning to mitigate risk.

As a result of this, CIRS (Centre for Innovation in Regulatory Science) initiated an annual benchmarking project to meet the needs of participant companies for comparative data and information in order to understand and quantify the impact of HTA on clinical development programmes, reimbursement timing and outcomes in different jurisdictions.

This poster summarises the methodology of this long-term benchmarking project, and the preliminary results from 2011-2013 studies.

Methodology

- Define the performance metrics
  The study questionnaire was developed in collaboration with nine multinational pharmaceutical companies to benchmark the HTA process for following individual products. Appropriate performance metrics were agreed upon by all participants to enable meaningful comparison between companies.
  The questions were comprised of eight main topics: three topics on the global development of new products and five topics on the roll-out phase in different jurisdictions. Figure 1 illustrates the schematic outline of the questionnaire. Based on the 19 questions, 62 key metrics were collected for each product, which were a combination of both quantitative metrics (for example, milestone events) and qualitative indicators (for example, scientific advice activities).

- Confirm the study scope and criteria
  The data collection focused on current products entering into pivotal trials to enable the most up-to-date snapshot of HTA-related activity in clinical development, as well as on licensed products to enable comparison between market access outcomes across jurisdictions. The data was captured separately with the long-term aim of being able to track products through phase II development and across roll-out to multiple jurisdictions over the coming years.

The jurisdictional data collection focused on both national level and regional level recommendations for each product. The key jurisdictions included in the study were determined by the study participants: Australia, Canada, New Zealand, Brazil, China, India, England, France, Germany, Italy, Japan, Korea, Spain, Turkey, Denmark, France, Germany, Italy, Japan, Korea, Spain, Turkey, Belgium, Canada, France, Germany, Italy, Japan, Korea, Spain, Turkey, and the USA.

- Establish a data collection protocol
  To ensure the accuracy, timeliness and security of data collection, an online data collection tool was designed to facilitate the provision of high-quality and comparable data across pharmaceutical companies.

Results

Data on 19 products that entered phase III and 30 products achieving first worldwide regulatory approval from 2009-2012 were collected and analyzed.

- For the phase III projects, 63% received HTA scientific advice, of which 61% occurred during phase II. Company-sponsored advisory boards were the most frequent source of advice as well as the most influential on the development programme. (Figure 2) The main reason HTA-related scientific advice was not implemented was due to the prioritisation of regulatory requirements.

- The majority of the phase III projects (69%) included an active comparator in the development plan; the main choice of comparators was “the gold standard comparator for the indication” (Figure 3). In addition to inclusion of active comparators, HTA-related requirements were implemented in development programmes. The inclusion of requirements was extremely heterogeneous, with the main requirements being patient-reported outcomes (84%), HTA-acceptable endpoints (74%), and cost-effectiveness analysis (74%).

- Additional comparators for local HTA submission were requested by all jurisdictions except the USA (Figure 4). The main reasons for requesting additional comparators were “local standard of care” and “least costly therapy”. England and France showed the highest percentage of products being reimbursed as per the regulatory label (50% and 55% respectively).

- For licensed products, the median time from regulatory submission to reimbursement decision varied from 639 days (Australia) to 846 days (Italy). For most jurisdictions, there was a gap between the regulatory approval and HTA submission.

Conclusion

Current benchmarking studies show that companies are actively taking scientific advice and incorporating HTA requirements into their development process, although they are still challenged by divergence in HTA processes and requirements across jurisdictions.

By continuing to develop this project into 2015 and beyond, the benchmarking database will become more robust and as it matures, could be used as a tool to help companies to achieve greater understanding of the diversity in HTA systems, to make the process more predictable and in particular, to identify and learn from outlier products and HTA review.

Purpose of the study

To give participating companies insight into how HTA requirements are impacting drug development and payer decision making in the context of new medicines being brought to market

Objectives

- Define performance targets to help focus on ongoing performance improvement;
- Identify activities and designs through early incorporation into development programmes that best address the HTA needs;
- Provide a clear understanding of the HTA systems in various jurisdictions;
- Improve the timeliness, transparency, and process predictability of HTA review.

Figure 1: Schematic outline of project structure

Figure 2: Type and impact of HTA-related scientific advice during development

Figure 3: Five key reasons for the choice of active comparators included in the development

Figure 4: Types of additional HTA-related information required by each jurisdiction

Figure 5: Median time to roll-out key jurisdictions

Bibliography