ASCERTAIN

WHAT ARE GLOBAL COST-EFFECTIVENESS MODELS AND HOW DO THEY WORK?

WHAT IS COST-EFFECTIVENESS ANALYSIS IN THE EVALUATION OF MEDICINES?*

Cost-effectiveness analysis looks at how the **health benefits** and **economic costs** for a new medicine compare to one or more treatment options in a country. It can be used to assess whether a new medicine provides **good value for money** for the healthcare system. The analysis is used by policy makers to help maximise health gains (improvements in physical, mental or social health) within the constraints of limited health budgets.

WHAT ARE GLOBAL COST-EFFECTIVENESS MODELS?

Global cost-effectiveness models are tools used to estimate the **long-term costs** and **health benefits** of a medicine in different healthcare systems. A standard model is first developed using clinical research and common assumptions. It is then **adapted for individual countries** or settings by adding local information, such as treatment practices, healthcare costs, disease patterns, and health technology assessment (HTA) requirements. This allows the model to generate results that are relevant to each local healthcare environment.

An example of a standard model is one that can **map out different treatment options** and what might happen after each choice. It would use common assumptions such as estimates of treatment costs, likelihood of recovery, or risk of side effects.

* For more information on this subject please see other educational materials developed under ASCERTAIN such as "What is a QALY and how is it used in decision-making on medicines?" and "How are costs and medicine effectiveness part of decision-making in access?" on our For Patients page at **https://www.access2meds.eu/for-patients/**

WHO IS USING THESE MODELS AND HOW?

Primary users of the global cost-effectiveness models include:

- **Pharmaceutical and device manufacturers** who make and use these models to support regulatory approvals, HTA submissions, pricing and reimbursement decisions, and negotiations with payers.
- **National HTA agencies and payers** may review or adapt these models to assess whether a medicine represents good value for money and should be funded from national or regional health budgets.
- **Researchers** in independent institutes, universities or international organisations (such as the World Health Organisation) may develop, test, and improve such models, assess healthcare interventions, and contribute to methodological development, guidance, and good practices that can support wider and more consistent use of these models.
- **Patient groups** can also use these models to evaluate or just better understand health interventions and advocate for a better value of care.

The models have a variety of uses which could include:

a. HTA (Health Technology Assessment)

HTA is a systematic process used to **assess the value of a new medicine compared to existing alternatives**. Depending on the country and the decision-making framework, it can include information on clinical effectiveness and safety, patient-relevant outcomes, economic consequences, budget impact and wider organisational or societal or ethical considerations.

Global cost-effectiveness models can support this process by providing a **consistent way to estimate the long-term health benefits and costs of a medicine**. Using a common model structure and evidence base can improve consistency and comparability across countries, while still allowing key inputs to be adapted to local conditions, such as treatment pathways, patient populations, costs, comparators, and specific HTA requirements. This can also reduce duplication of work and support the development of more consistent modelling practices across settings.

The role of cost-effectiveness in HTA varies between countries, with some countries not considering it at all or others looking at it in combination with budget impact, unmet medical needs, disease severity, and broader policy priorities.

b. Pharmaceutical pricing and reimbursement

In some countries, when a new medicine receives a positive recommendation following a HTA (meaning the medicine is considered to offer real value), the medicine developer enters **negotiations with decision-makers** to establish the price and type of reimbursement. The information contained in the cost-effectiveness analysis (adapted from the global cost-effectiveness model to a country's context) submitted by the manufacturer **can be used to establish a price** that rewards innovation while reflecting the value it brings in comparison to existing medications and the impact on the overall health budget.

c. Investment optimisation

In this category, data from global cost-effectiveness models can be used to help decision-makers **identify innovations with the greatest impact**. This can support priority setting, strategic investment decisions and more efficient use of limited healthcare budgets, to enable available resources to generate the greatest benefits.

KEY CHALLENGES ASSOCIATED WITH ADAPTING GLOBAL COST-EFFECTIVENESS MODELS

The ease and ability to adapt the global cost-effectiveness models into local contexts, is essential for their use. Key difficulties adapting the models can include:

a. **Lack of local data and data heterogeneity** – relevant local data may be unavailable, incomplete, or structured differently across countries, making it difficult to use the same inputs and data-processing approach.

b. **Differences in opinion and interpretation** between stakeholders, over methodological requirements.

c. **Clinical and healthcare system differences** (clinical practices, medicine/ treatment guidelines, patient population demographics).

d. **Model restrictions** (many global cost-effectiveness models are proprietary and thus restricting full access to them).

e. **Timeline estimation and long-term extrapolation** (making long term predictions based on short term data, making results depend on assumptions about future effectiveness).

f. **Differences in comparator and outcome** (local comparator for which no data exists, local outcomes such as aspects of quality of life not considered).

GOOD PRACTICES TO TACKLE CHALLENGES

Although not universally applicable, some good practices do exist to mitigate some of the challenges described above. They can include:

a. **Early identification of local sources of data and local system differences**, including differences in data availability, structure, treatment pathways, and clinical practice.

b. **Adherence to local HTA, reimbursement and other local decision-making requirements**, identifying country-specific methodological requirements early and incorporating them explicitly in the model.

c. **Transparency on model assumptions**, clearly documenting assumptions, data sources, and country specific changes so they can be understood and checked. Open-source approaches can further support transparency.

d. Using a **common core model** with **limited and explicit local adaptation**.

e. **Quality assurance and validation**, both the clinical assumptions and the technical implementation of the model, particularly when adapting it across different settings.

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