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How to: Develop an Early Cost-Effectiveness Model

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In the sixth installment of our "How to" series, Lumanity's Margarita Kulakova, Daniel Gladwell, and Jake Horgan explain how early cost-effectiveness models can be used to inform the value demonstration strategy of a medicinal product – through insights gained around its likely position in the treatment landscape, key drivers of value, potential evidence gaps, and more.



What is an early model?

An early cost-effectiveness model is usually developed during the early stages of a technology's lifecycle, typically before or during Phase II / III clinical trials. Alternatively, it can be constructed at the beginning of market access or commercialization planning to inform pricing research and guide evidence generation strategies.

Early models rely on simplified structures and assumptions, using limited and often uncertain evidence. Early models draw on preliminary data, target product profiles (TPPs), or proxy sources, while maintaining flexibility to adapt as more robust data become available. Their primary goal is not to produce definitive cost-effectiveness results, but to guide strategic decisions during product development.

Why do we need an early model?

The purpose of an early model is to support the manufacturer in making decisions regarding a new therapeutic. Models provide critical insights into the key drivers of economic value and help inform future evidence generation strategy. Key uses include the following:

1. Support global launch decisions and establish a pricing strategy

Early modeling supports companies in shaping pricing and reimbursement strategies by understanding what pricing range might be acceptable to payers. By running multiple scenarios, the manufacturer can generate economically justifiable price points based on varying willingness-to-pay (WTP) thresholds – signaling whether launch is likely to be viable in countries more focused on cost-effectiveness.

Early models also guide the strategic positioning of a product. This can include the identification of patient subpopulations for which better value for money can be expected.

2. Identify critical value drivers

Sensitivity analyses within early models reveal which parameters most influence cost-effectiveness outcomes. This helps inform a manufacturer's decision-making regarding target populations, pricing strategies, and prioritization of additional research.

3. Guide trial design and additional evidence generation

By identifying parameters that are critical to cost-effectiveness, early models can help manufacturers refine the design of clinical trials. For instance, they can highlight the need to collect detailed data on specific endpoints, include particular comparators, or analyze certain subgroups separately. They also help prioritize future evidence generation activities – including real world evidence (RWE), literature reviews, payer interviews, and expert elicitation.

4. Inform future HTA model structure

Early models also assess how the clinical trial endpoints can be translated into key model drivers such as long-term survival, utilities, healthcare resource use, and costs.

They also help to validate clinical assumptions before significant resources are committed to fully developing materials for an HTA submission.

How to develop an early cost-effectiveness model?

Early models are usually built under limited budgets, tight timelines, and high levels of uncertainty. Therefore, the modelling approach must be comprehensive, transparent, and flexible. Developing an early model includes the following steps:

Step 1: Defining PICO

The conceptualization of an early model typically begins with establishing the PICO framework: population, intervention, comparator(s), and outcome(s).

- Defining the patient population requires consideration of key subgroups, as cost-effectiveness may be more favorable in populations with higher unmet need
- For comparators, it is important to include not only currently established therapies but also those treatments that are likely to become future standards of care. Additionally, changes in medical practice, such as improvements in diagnostic techniques or modifications in hospital management, should also be anticipated
- Relevant outcomes determine the core health states within the model. These should be based on key disease progression indicators and informed by clinical trial endpoints, but there may be other clinically meaningful events to consider. For instance, long-term disease consequences not directly captured in clinical trials may need to be incorporated

Step 2: Choosing the model structure

The model structure should avoid unnecessary complexity that might obscure results or rely on overly speculative assumptions. However, the structure should also remain flexible enough for scenario testing and for additional inputs to be incorporated as more evidence becomes available.

Step 3: Identifying evidence

The TPP containing preliminary assumptions on comparative efficacy is usually the primary source of information for the early model. Other key sources include literature searches, clinical expert interviews, early trial data, burden-of-illness studies, and RWE (based on registry data, for example). Proxy data from similar conditions can be utilized to inform model parameters – this is particularly relevant for rare diseases.



Key considerations

Early models offer a structured approach for evaluating the commercial viability of a new product. However, given that they rely heavily on assumptions, scarce data, and hypothetical TPPs, early models should not solely dictate development strategies. Rather, they should inform broader strategic decision-making alongside highlighting data gaps, refining trial designs, and guiding data collection priorities.

Step 4: Estimating cost-effectiveness and the value drivers

An early model can establish a product's economically justifiable price (EJP): the price ceiling at which a product would be considered cost-effective. An EJP is calculated based on the incremental clinical benefits, expressed in quality-adjusted life years (QALYs), multiplied by the decision-maker's WTP threshold, plus any cost savings generated.

Areas of uncertainty and key drivers are identified through sensitivity and scenario analyses, which are central to testing the robustness of model outcomes and evaluating which assumptions drive the results most strongly.

Step 5: Refine evidence generation plan

The final step is to use the results (particularly those from sensitivity and scenario analyses) to prioritize future evidence generation to address identified data gaps. All of these efforts help improve the robustness of a future HTA model and thus avoid future pitfalls.

About the authors

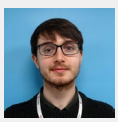
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