

Planning with Insights Even the Best Economic Models Need Validation

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Learning objectives



Appreciate the value of incorporating **insights from key stakeholders** during the development of an economic model via a case study



Discuss key model components where insights matter



Understand **timeframes** for ideal engagement with key stakeholders to validate key model components



Agenda



- Introduction
- Modelling overview
- Illustrative case study: Why insights matter?
- Life science company perspective
- Payer perspective
- Discussion

Our speakers



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**Vice President,
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Access strategy
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**Executive Director,
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Smita Kothari
PhD, MBA

Life science company
perspective

**Executive Director, Global
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MSD

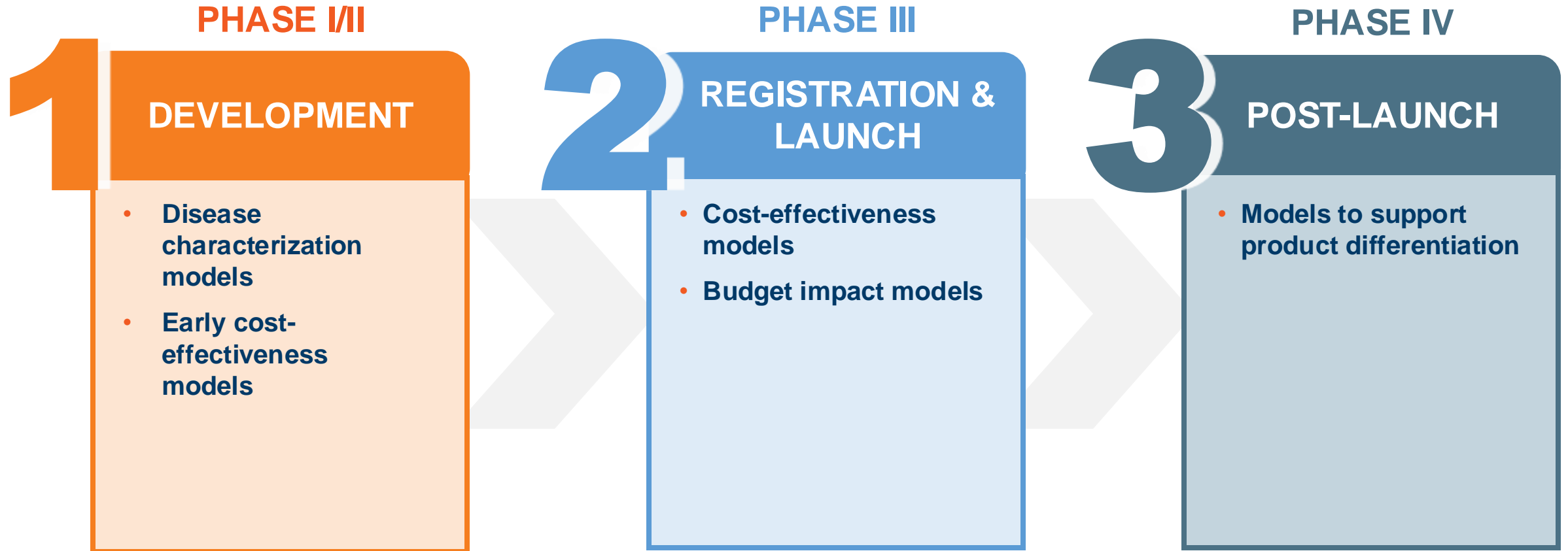


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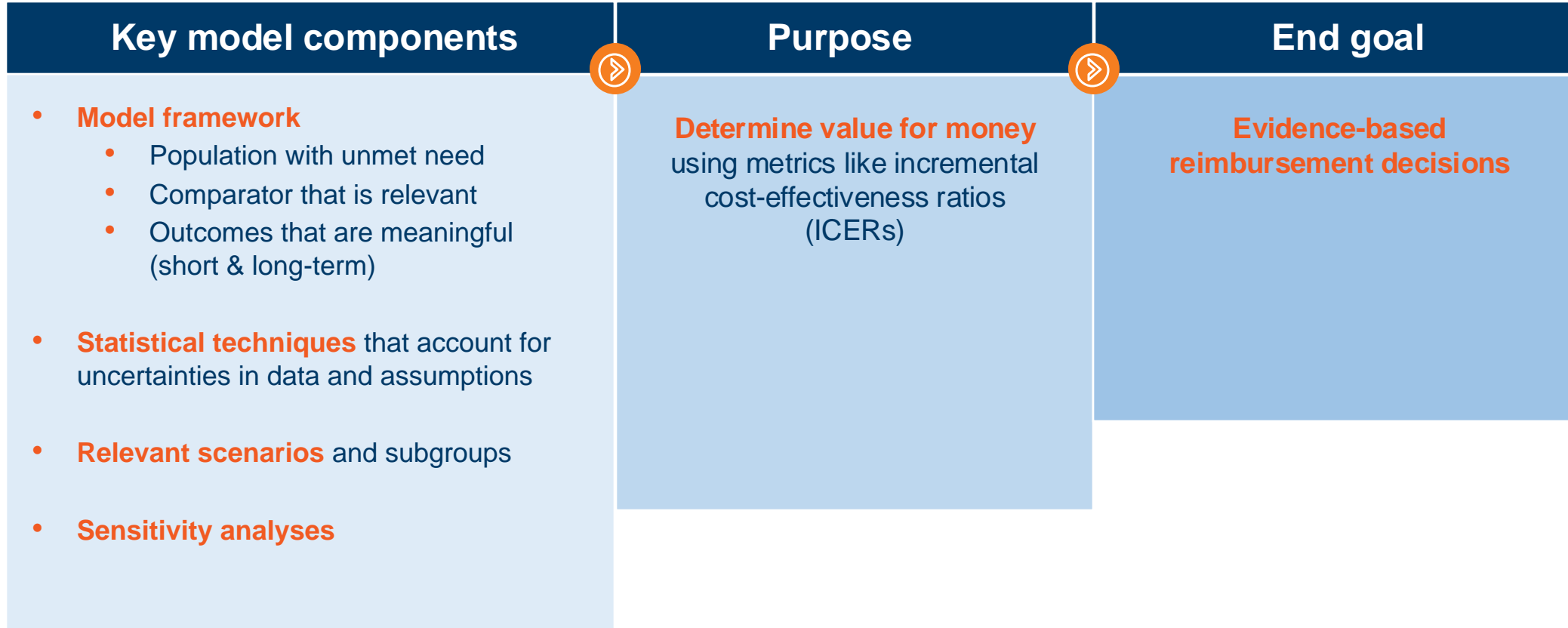
Payer
perspective

**Independent Advisor on
HTA and Market Access**

Models support decision-making across different stages of drug development



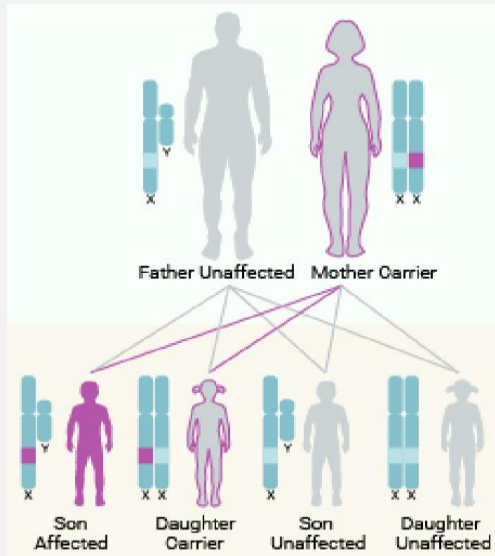
Insights from key stakeholders matter and underpin its scientific robustness and relevancy



Case study: Development of strategies via insight generation to enhance a global cost-effectiveness framework for DMD (1 of 2)



DMD disease background & burden



- DMD is a genetic disease diagnosed in childhood and causes premature death, usually by 30.
- DMD patients incur significant direct and indirect costs, with increasing costs as disease becomes more severe.



Understanding DMD disease progression

DMD economic model drivers

Maintaining a patient in **ambulatory states** is associated with better clinical and economic outcomes.



health state costs,



utilities, and



caregiver burden

A patient remaining in **non-ambulatory health state** will incur high costs and low QALYs. **Once a patient is non-ambulatory, a treatment that delays or halts disease progression** but does not improve health states will have an unfavorable impact on the CE ratio.



health state costs,



utilities, and



caregiver burden

Case study: Development of strategies via insight generation to enhance a global cost-effectiveness framework (2 of 2)



Challenge/Solution

In partnership with the life science company, Genesis Research Group conducted an HTA/payer council which included payers, patient advocates, and clinicians to:

- Address modeling challenges for cost-effectiveness evaluation
- Evaluate existing evidence package, identify evidence gaps, build recommendations/plans for mitigation



Key takeaways

KEY TOPICS

Disease natural history

- Natural history data appeared inconsistent with the clinical course of DMD due to insufficient mortality data
- Utility measures lacked granularity to differentiate non-ambulatory health states

Meaningful endpoints

- The prognostic value of NSAA was not well understood by payer/HTAs

ISSUES

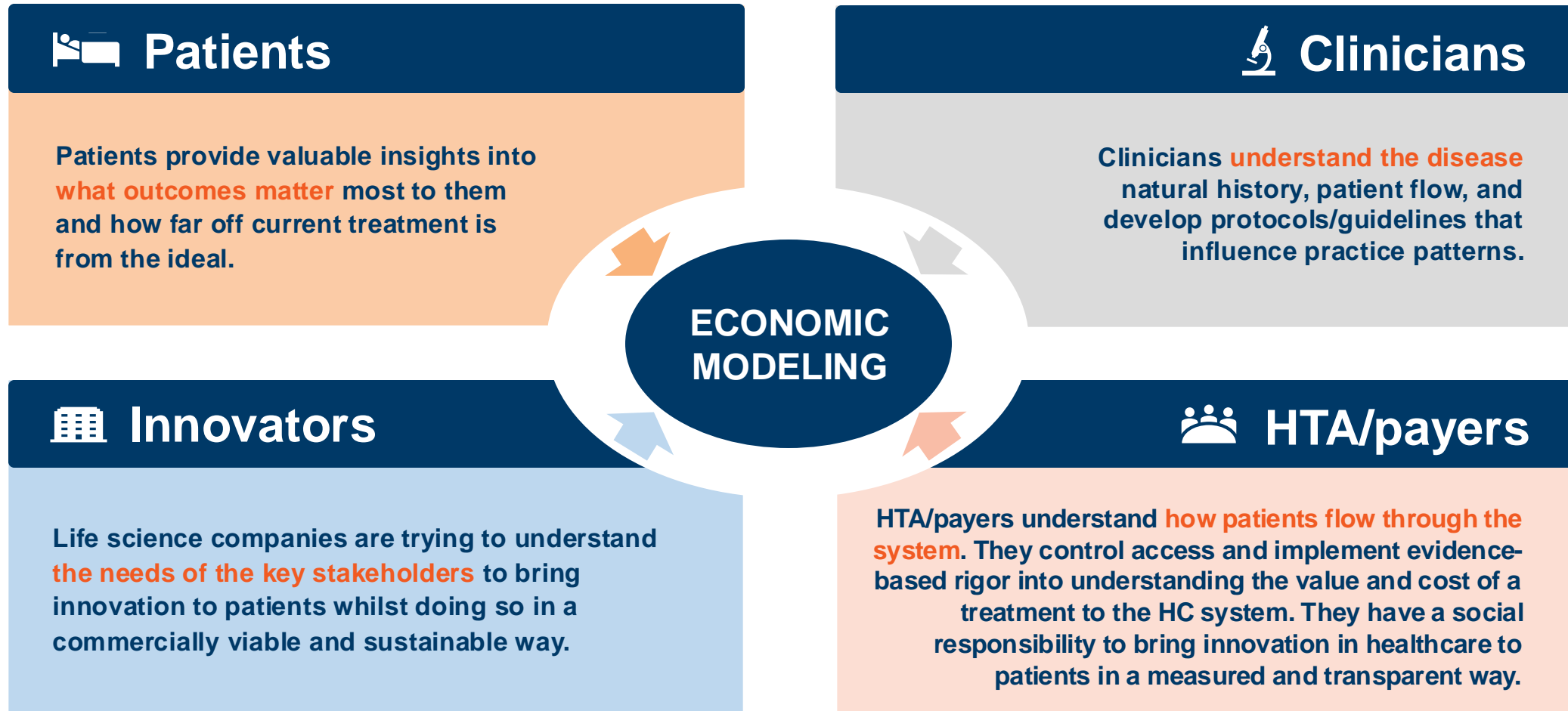
RECOMMENDATIONS

- Calibrated DMD natural history data for the model to account for mortality data
- Conducted sensitivity analysis to explore alternative NH model assumptions and data source for DMD mortality estimates.

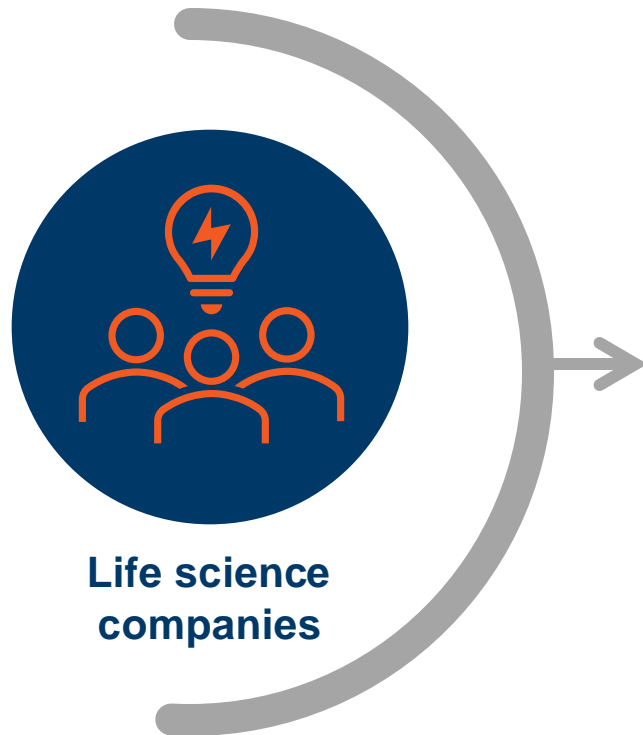
- Built an evidence generation and communication strategy to further establish the true prognostic value of NSAA.

- Building insights in early stages of model development prior to Phase III readout assisted the life science company in understanding model's face validity, e.g., are the movements in health states credible?
- Early planning helped the organization to build an appropriate strategy to enhance the cost-effectiveness model.

Key stakeholders in the healthcare system bring different perspectives to inform key model components

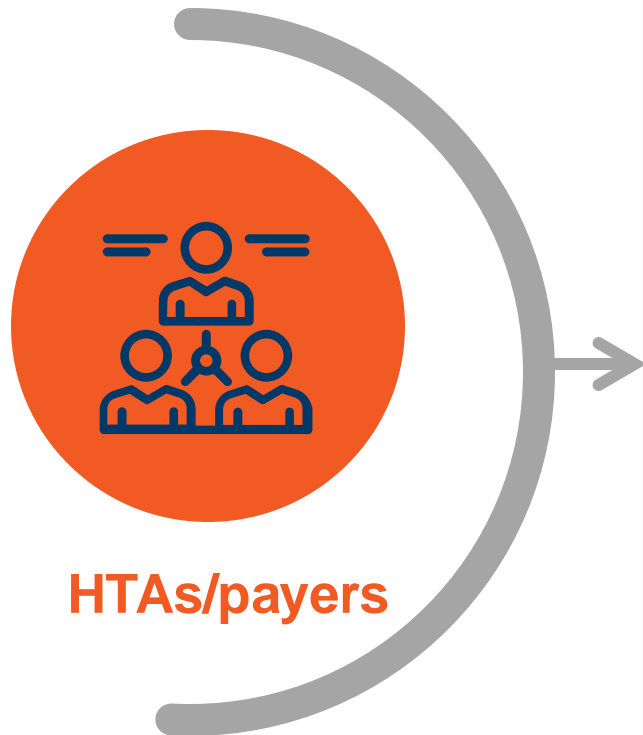


Key considerations: Economic model development with key stakeholders in mind



- 1. Characterizing a disease and its natural history in model framework** – requires systematic reviews, analog/landscape assessments and insights.
 - **Key questions:**
 - Do we understand the disease natural history?
 - Has a disease model been published?
 - Is there a model framework previously accepted by HTA/payers?
 - Does the model framework have face validity?
- 2. Determining the model inputs** – requires assessment of the evidence package in context of the PICO.
 - **Key questions:**
 - What is the population with unmet need and relevant comparator, etc.? Does the population reflect usual clinical practice?
 - Are the endpoints in the clinical trial meaningful and relevant to clinical practice?
 - What are the evidence gaps in the model inputs?
- 3. Understanding global market heterogeneity** – requires insights on how best to address variations in assessment methodology and differences in payer archetypes.
 - **Key questions:**
 - What country-specific evidence should be generated?
 - Do we need to adapt the model in context of the assessment methodology for a specific HTA?

Key considerations: Common missteps and how to avoid



1. Early engagement

Given the timelines for HTA decision-making processes, **preparation for HTA submission needs proactive strategies to anticipate changes in payer expectations and demonstrate value of asset using economic model.**

- **Key question:**

- Has the life science company leveraged formal and informal opportunities to obtain insights?

2. Validation of the model framework and input:

- **Key question:**

- Is there a good understanding of HTAs/payer perspective on the PICO, relevant sensitivity analyses, assumptions to address uncertainty, etc.?

3. Alignment of affordability of the intervention:

- **Key question:**

- Is there alignment between manufacturer objectives with payer affordability constraints?

The versatility and flexibility of proactive validation for models

Utilizing **insights from key stakeholders** to validate key model components, but also clinical trial designs and pricing assumptions, is critical to successful launch strategy development.

Validation approach by stage of development

Early
<Ph2

Late
>Ph2



Process-driven approach

Early scientific advice with HTA bodies is recognised as the **official route to validating both clinical and economic assumptions**. This is a single-time processes usually done once a product is advancing through phase 2 clinical trials



Desk-based approach

Desk-based work utilizes **targeted and systematic** literature reviews, **HTA analyses, pricing evolution**, and other approaches **to understand the landscape**, and validate assumptions. This can be done at early and late stages but relies on updates to publicly available data.



Stakeholder-based approach

Stakeholder primary research is critical all stages, to validate what payers, clinical experts, and patients believe are the **opportunities and limitations** in the market – this should be done, and reassessed, throughout the development of a product



Engaging with key stakeholders early for insights will assist in building scientifically robust and relevant economic models

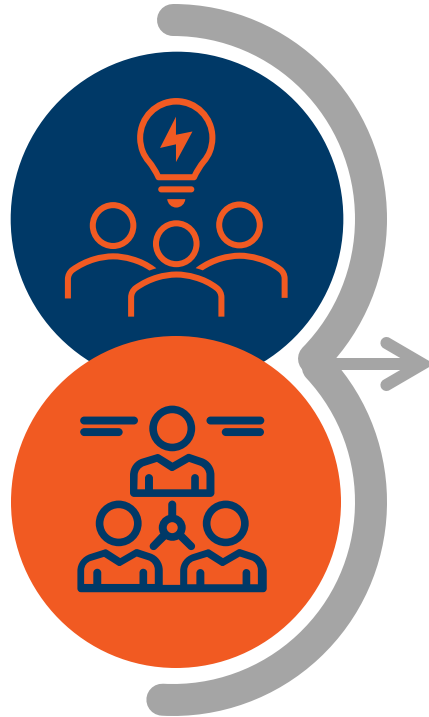
Key lesson:

Model framework/
input and assumptions

+

Stakeholder
insights

Improved model
validation with
greater impact and
optimal outcome



- Early engagement with key stakeholders to validate the model framework and the evidence package for model inputs allows time to build strategies and recommendations/plans for mitigation.
- Understanding HTA/payer insights, along with the clinician and patient perspective, is critical when articulating the economic value of an asset.
- Engagement with HTA/payers will assist in appropriate implementation of country specific variations in the economic models.



Questions?
