Novel value elements: Developing and applying a pragmatic value framework in early stages of product development

Background

- Traditionally, the value of pharmaceutical products is assessed based on direct health outcomes and healthcare costs.
- There is an increasing recognition of the importance of including broader impacts, including novel value elements and a societal perspective.
- Value frameworks can help to conceptualise value comprehensively, including benefits for patients, their families and carers, and society.
- There are multiple value frameworks in the existing scientific literature. A recent systematic review identified a total of 48 different value assessment frameworks published between 2008 and 2019 and designed to support health technology assessment (HTA) and decision making (Zhang et al., 2022). However, most of them were developed for specific countries, they do not reflect all the relevant perspectives and may not be suitable for assessing value from early stages of development.

Aim

We aimed to establish a comprehensive value framework to consistently determine and communicate the value of pharmaceutical interventions reflecting the perspectives of patients, payers, healthcare systems, and society, in early stages of product development.

Methods

- A targeted literature review of value frameworks and novel value elements was conducted 1) to collate an inventory of value elements, 2) to assess progress in their measurement, and 3) to understand their incorporation into HTA/payer guidelines.
- The outcomes of the literature review and operationalisation were peer-reviewed by two external experts in the HEOR community.

Results

- Figure 1 and Table 1: We created a comprehensive value framework that reflects the latest literature on value and perspectives. The inventory was organised across 6 domains. We found that at there are methodologies to measure almost all value elements and for some there exist ways to incorporate into HTA/payer decision making. The framework also captures the current state of play in terms of recognition by HTA agencies as per scientific literature- this synthesised published data from HTA guidelines of 46 countries by Breslau et al., 2023.
- Figure 2: The value elements and framework could form the basis of a process to assess interventions as early as in early phases of development. The process would involve the systematic assessment a new intervention in terms of their potential to have broader value. This value profile of a new therapy could be compared to any existing comparator. The incremental differences can help to identify broader value drivers and assist with evidencing these in the ongoing clinical development process.
- We tested this potential application process by analysing a hypothetical intervention in an early-stage neurodegenerative disease, where there is currently only symptomatic therapy. The process identified 5 main value drivers that could be evidenced throughout the clinical development to support communication and evaluation of the value profile of a technology.

Conclusion

- The identified value elements can form the basis of a value framework that can systematically identify value for all stakeholders.
- Assessment of value should be iterative as new evidence or methodologies evolve
- A comprehensive assessment of value elements can inform clinical and evidence development from an early stage and help communicating value to all stakeholders including payers for their decision making.



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Value framework

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Table 1: Value elements, their perspective, and their recognition in HTA guidelines

		Value elements	Types of costs and outcomes	Stakeholder/ Perspective	Is it recognised in 46 local HTA guidelines?
	1A C	Clinical efficacy	Health Outcome	Patient	20+ agencies
	1B C	Clinical safety	Health Outcome	Patient	20+ agencies
	1C Q	Quality of Life	Health Outcome	Patient	20+ agencies
	1D T	Treatment cost	Direct healthcare costs	Payer/HC system	20+ agencies
	1E C	Cost offset to the health care system	Direct healthcare costs	Payer/HC system	20+ agencies
	1F F	Health system capacity	Non-direct healthcare costs	Payer/HC system	< 10 agencies
efits	2A V	Value of cure	Health Outcome	Patient	<10 agencies
	2B V	Value of knowing and reduction in uncertainty	Non-health outcome	Patient	< 10 agencies
	2C V	Value of hope	Non-health outcome	Patient	<10 agencies
	2D S	Stigma	Non-health outcome	Patient	<10 agencies
	2E E	Enablement value	Non-health outcome	Patient	None
	2F R	Real option value	Non-health outcome	Patient	<10 agencies
el	2G C	Carer/family spillover effects	Non-health outcome	Patient	20+ agencies
	2H F	-ear of contagion	Non-health outcomes	Population	<10 agencies
	2I I	Innovation and scientific spillovers	Non-health outcomes	Population	None
	2J I	Insurance value	Non-health outcome	Population	None
	2K F	Herd protection	Health outcomes	Population	<10 agencies
	2L P	Prevention of AMR	Health outcomes	Population	<10 agencies
nce	3A P	Process of care	Non-health outcome	Patient	<10 agencies
	3B A	Adherence-improving factors	Non-health outcomes	Patient	20+ agencies
s and ion	4A C	Direct out-of-pocket medical costs	Direct non-healthcare costs	Society	<10 agencies
	4B C	Direct out-of- pocket non-medical costs	Direct non-healthcare costs	Society	<10 agencies
	4C P	Productivity cost	Non-direct costs	Society	<10 agencies
	4D C	Costs of informal care	Non-direct cost	Society	<10 agencies
	4E C	Costs to other government sectors	Non-direct cost	Society	<10 agencies
	4F F	Forgone Tax	Non-direct cost	Society	None
	4G L	_egal/Criminal Justice	Non-health outcomes	Society	None
	4H E	Education	Non-health outcomes	Society	None
	4I E	Environment	Non-health outcomes	Society	10+ agencies
	4J I	Impact on economic growth	Non-direct cost	Society	None
	5A S	severity	Non-health outcomes	Society	20+ agencies
	58 U	Jnmet need	Non-health outcomes	Patient/Society	<10 agencies
	5C R	Karity	Non-health outcomes	Patient/Society	20+ agencies
	5D N	National health system priorities	Non-health outcomes	Patient/Society	<10 agencies
	5E E	equity and patient population demographics	Non-health outcomes	Patient/Society	None

An approach could be to examine a hypothetical disease-modifying treatment vs. a symptomatic treatment.

- **1.** Expectation: We tested this approach on a hypothetical therapy in an early-stage neurodegenerative disease and assessed the presence and absence of value across all value elements.
- 2. Comparison: The analysis then compared the hypothetical asset to the current standard of care. There is currently only symptomatic therapy available to patients. The following potential value driver elements could be found across all value domains and hence relevant for all stakeholders:
 - **Access health system capacity:** the therapy has the potential to free up healthcare resources over the treatment period and beyond.
 - there is no effective therapy.
 - Patient-level benefits real option value: the delay in disease progression allows patients to benefit from future, more effective therapies.
 - **Population-level benefits carer spillovers:** effects are expected to translate into improved quality of life for informal carers.
 - **Societal effects productivity:** the intervention is expected to help patients avoid early retirement, maintaining their productivity.
 - Health condition and treated population unmet need: the therapy treats a condition with significant unmet clinical need
- 3. Demonstration: The clinical development program could subsequently be adapted to ensure that evidence is generated for the demonstration and recognition of the full value profile of the therapy.



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Patient experience - process of care: the therapy may offer improvements in the patient's healthcare management, where

Population-level benefits - innovation: the therapy may stimulate innovation in treatment and diagnosis of the condition





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