Novel Pricing Models to Reward Pharmaceutical Innovations: From Cost-Effectiveness Analysis to Value-Based Pricing

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Objective

With highly effective but high cost innovative end-of-life, cancer, and rare disease therapies entering the market, there is an increased impact of health technology assessments (HTAs) in the US. The recent evaluation of Nusinersen (SPINRAZA) and Onasemnogene abeparvovec (ZOLGENSMA) by the Institute for Clinical and Economic Review (ICER) illustrated the importance of applying the conventional cost-effectiveness analysis (CEA) as well as including other perspectives of value to advance access to groundbreaking treatments. This presentation is to review the contradictory perspectives on CEA and to introduce the latest qualitative value framework and quantitative pricing models.

Method

We searched for expert opinions regarding value-based pricing (VBP) using keywords “cost-effectiveness”, “ innovation” and “ value-based pricing” from the latest literature and panel discussions, and critically appraised their usefulness in driving access to innovation. Viewpoints from the following conferences and seminar series were included: MassBio Value of Health Series 2019; ISPOR 2019; ISPOR Europe 2019; Next Generation of Value Assessment: Including the Patient Voice, 2019, National Health Council.

Results

Ways forward to incentivize innovation include 1) establishing new value frameworks, 2) employing different decision rules, and 3) developing novel payment models.

Figure 1: A fundamental cost-effectiveness analysis (CEA) model

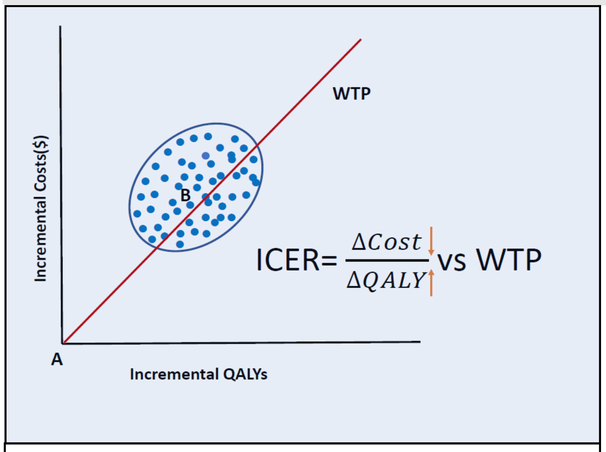


Figure 1: A, B represent two different treatments. The solid circles represent the uncertainty of the incremental cost-effectiveness ratio(ICER). Quality-adjusted life-year(QALY) compares health interventions’ impacts on both the quality and length of patients’ lives using a single metric. CEA measures and compares the efficiency of health technologies using a standard threshold (WTP). It ensures that the greatest possible health benefits were achieved given the available budget.

Contradictory perspectives on the cost per quality-adjusted life-year (QALY) matrix

Supporters of CEA

- Innovation may charge a high price. However, it will save overall costs in the long run (ex. one-time treatment).
- Personalized treatments target populations that respond well, thus will improve QALY.
- Personalized treatments can create extra value for risk-averse individuals by reducing the uncertainty they face about the treatment.
- HTAs provide a higher WTP for innovative treatments. A higher cost-effectiveness threshold would support a higher value-based price.

Criticism of CEA

- Limitation of QALY: Methods in utility measurement are not consistent across studies; Patient QoL is unlikely to be constant over their lifetime; Utility assessment for children, elderly, or disabled population is challenging; QALY generally neither addresses the heterogeneity of patient preferences nor engages patients, who increasingly are taking on an active and engaged role in the US health care.
- CEA measures technology efficiency but not resource allocation or budget impact. High efficiency does not mean affordability.
- CEA does not differentiate clinical benefits from an innovative technology or a “me too” drug. “Me-too” could be priced as expensive as new mechanisms of action.
- CEA does not evaluate the effect on society, the infrastructure of care, or other novel elements of value.
- The use of CE thresholds does not account for short-term budget impact and potential opportunity costs.

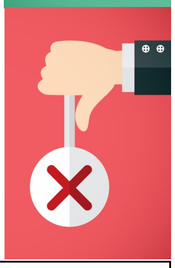
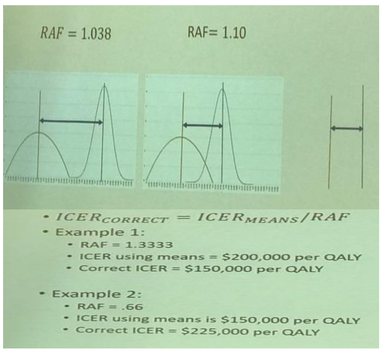


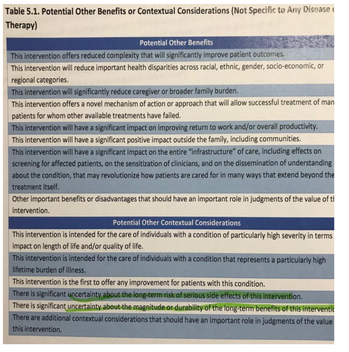
Table 1. Employing different decision rules

Country	HTA Body	Thresholds
Australia	PBAC	Implicit thresholds: • Research on previous decisions suggests there is an implicit CE threshold between 45,000 and 75,000 AUD that should be used for rare diseases or oncology drugs, and a threshold below 45,000 AUD should be used for all other therapies
Canada	CADTH/pCODR	Implicit thresholds: • Research on previous decisions suggests there is an implicit CE threshold of 50,000 CAN dollars
France	HAS, TC	No threshold
Germany	GBA, IQWiG	No threshold
Sweden	TLV	Implicit thresholds: • Research on previous decisions suggests variable thresholds based on disease severity from €80,000 to €112,000 for non-severe and severe diseases, respectively
UK (England)	NICE	Explicit thresholds: • Drugs could go through a fast track appraisal when they have a very high value for money (ie, up to £10,000) • Drugs are considered cost-effective when the ICER is within a threshold range of £20,000–£30,000 per QALY • Drugs used for end-of-life treatment, such as certain cancer therapies, permit an ICER of up to £50,000 per QALY • Drugs for very rare diseases are considered cost-effective up to £300,000 per QALY

(1a): Payers are typically willing to pay more for medicines for ultra-rare diseases and curative therapies (higher threshold)

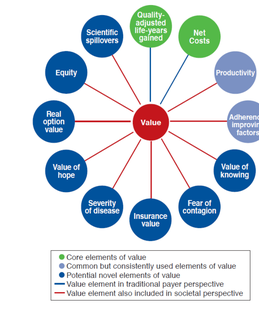


(1b) Incorporate uncertainty into healthcare technology evaluations by measuring RAF to adjust ICER

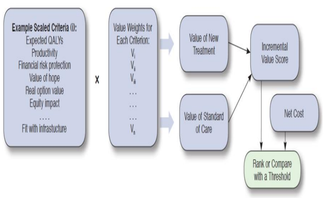


(1c) ICER report of value of SMA gene therapies

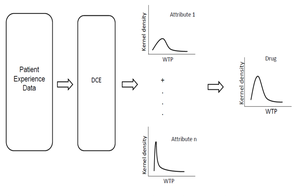
Figure 2: Novel decision framework and models



(2a): The value flower of ISPOR Task Force Report.

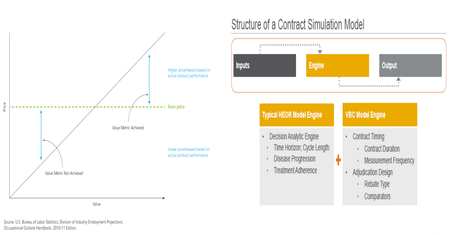


(2b): Multi-criteria decision making

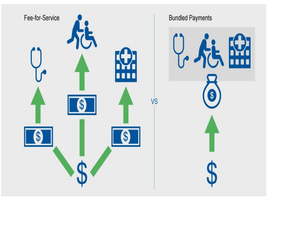


(2c) Patient Experience Framework by Discrete Choice Experiments and ML model to account for heterogeneity of patient preferences in value assessment

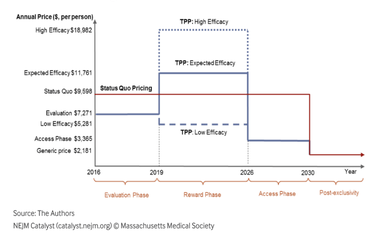
Figure 3: Novel value-based payment models



(3a) Pay-for-performance: payers and pharma companies agree to link payment for a medicine to value achieved in real-world performance.

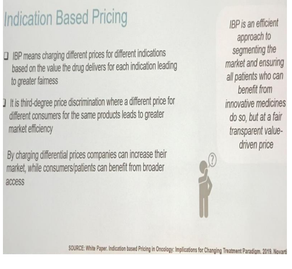


(3b) Bundled payment: Instead of paying for each service separately, bundled payment provides a single, pre-determined payment across an episode of care

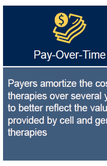


Source: The Authors
NEJM Catalyst (catalyst.nejm.org) © Massachusetts Medical Society

(3c) Three-Part Pricing: Phase 1: Evaluation. Prices are set low, with minimal copayments and access restrictions, to encourage adoption and develop real-world evidence rapidly. Phase 2: Reward. Prices are set based on the effectiveness established in the Evaluation Phase, which rewards manufacturers for their innovation. Phase 3: Access. Prices are reduced to facilitate widespread adoption.

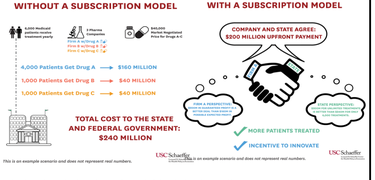


(3d) Indication Based Pricing



(3e) Pay-Over-Time model

(3f) Risk-sharing model



(3g) Subscription-based model for infectious disease, ex. Hepatitis C

Discussion & Conclusion

Assessing the value of innovation such as short-term curable and transformative therapies often presents important challenges, including increased uncertainty at the time of launch paired with high upfront costs, time divergence between costs and benefits, and concerns about affordability and fair sharing of economic surplus. The ISPOR Special Task Force suggested expanding the notion of “value” by including severity of disease, value of hope, insurance value, real option-value, reduction in uncertainty, reduced fear of contagion, equity, and scientific spillovers in addition to the traditional cost per quality-of-life matrix. However, criticism has been raised about its double-counting in some of the elements. “Multi-criteria decision making” and “Discrete experiment by patient experiences” are two other models to assess value quantitatively. While payers generally have a higher willing-to-pay for curative therapies, and the cost-effectiveness ratio could be mathematically adjusted based on outcome uncertainty, these remedies still could not overcome the budget constraints of the healthcare system. “Three-part-pricing,” “pay-over-time,” and “ subscription-based” models partially address the affordability issue. Payers generally lack incentives to pay VBP for cures because they cannot ensure that they will reap the long-term economic benefits as patients frequently move between plans. The “ risk-sharing” model could be one of the solutions. The novel HTA frameworks and models could potentially differentiate real innovation from “me too”, facilitate removing low-value care from the system, and relocate budgets to transformative products.

For complete list of references , please contact the presenting author: zou1@stu.mcphs.edu