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## Using Patient Experience Data and Discrete Choice Experiment to Assess Values of Drugs

### The Opportunities and Challenges of Rethinking Our Approach to Value Assessment in Healthcare

As America's healthcare system continues to evolve, it is critical that our perception of care and its value to patients evolve with it. In the past, value assessments have marginalized patients' perspectives in favor of other, more easily quantifiable variables. Unfortunately, this approach to value assessment hasn't been able to capture individual health states or preferences because it fails to engage with the most important stakeholders: the individuals receiving the care.

Take, for example, the quality-adjusted life-year, also known as the QALY. Explained at the most basic level, the QALY is a measurement of how an intervention improves a patient's quality and quantity of life. The QALY aims to encapsulate the value of healthcare interventions in a single index number, where 1 equates to 1 year of perfect health and 0 is associated with death.

From the patient perspective, assessing the value and impact of care through a summary metric is akin to summarizing a 200-page novel in a single word. Although many experts acknowledge the limitations of the QALY metric, they often throw their hands up and assert that patient perspectives are just too difficult to quantify as a practicable metric.

But things are beginning to change.

This year, health economists and health services researchers rolled up their sleeves to offer alternative approaches to measure value as part of the Pharmaceutical Research and Manufacturers of America Foundation's 2019 Challenge Awards. The awards presented researchers with a single prompt:

*"What are innovative, patient-centered approaches to contribute to healthcare value assessment that move beyond the inherent limitations of analyses based on the quality-adjusted life-year metric?"*

Researchers responded with myriad novel, innovative, and practical approaches to value assessment that enhance or mitigate past the QALY and allow deep engagement with patients. Perhaps more important, the volume of substantive submissions undermined the idea that successfully incorporating the patient voice into healthcare assessments was too difficult.

Of all the approaches offered, 4 winning submissions were selected based on their innovative and pragmatic approaches to value assessment. Although each of the approaches differs in methodology and design, a common theme throughout is the realistic way in which they account for the perspectives of patients. In one selected model, for example, the authors propose to inform value assessment with learnings from patient-focused drug development »

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(PFDD), a program instituted by the FDA to capture the experiences, perspectives, and needs of patients based on their unique symptoms and medical histories. Another approach argues for using patient stakeholder groups and existing patient registries to better incorporate patient perspectives.

One of the submissions takes value assessment a step further, arguing that conventional approaches to value measurement that emphasize the average (mean) of healthcare outcomes are flawed. According to the authors, because patient care is about more than just average outcomes, we should incorporate the statistical variance and skewness of treatment outcome distributions to more effectively capture the wide spectrum of patient experiences.

Interestingly, many of the selected winners offer methodologies that aren't constructed from scratch. Instead, they expand on existing infrastructure, such as the PFDD, which is already purposed to capture patient perspectives on a medicine's effectiveness. Another winning submission discusses the advantages

of applying multiple-criteria decision analysis—a long-standing decision-making tool used in other industries—given its ability to capture multiple dimensions of value that often go unmeasured.

But beyond just the winning approaches, many, if not most, of the submissions present realistic methods that expand the scope of value assessment, engage with patients, and innovate beyond the QALY.

It is clear that ongoing efforts such as the Challenge Awards that seek to move beyond the QALY are making meaningful headway in improving value assessments for patients. We should applaud the worthy goals of these efforts to fundamentally rethink what constitutes value. •

**Eileen Cannon**

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## Using Patient Experience Data and Discrete Choice Experiment to Assess Values of Drugs

*Surachat Ngorsuraches, PhD*

### INTRODUCTION

Value assessments of drugs have come under the spotlight in the US healthcare system. Various value assessment frameworks have emerged in recent years, yet to date, none are positioned to support healthcare decision making.<sup>1</sup> Despite the wide use of the quality-adjusted life-year (QALY) metric in value assessment, the QALY remains controversial because it is a single-dimensional, generic health measure. Furthermore, the QALY generally neither addresses the heterogeneity of patient preferences nor is a framework developed through engaging patients, who take on an increasingly active and engaged role in the US healthcare system.<sup>1,2</sup> Therefore, a critical need exists for innovative approaches to value assessment that are more centered on the patient and move beyond analyses based on the QALY metric. I propose the patient experience value framework, a new patient-centered approach to contribute to value assessment that includes multiple drug attributes and addresses heterogeneity in patient preferences.

### PROPOSED APPROACH

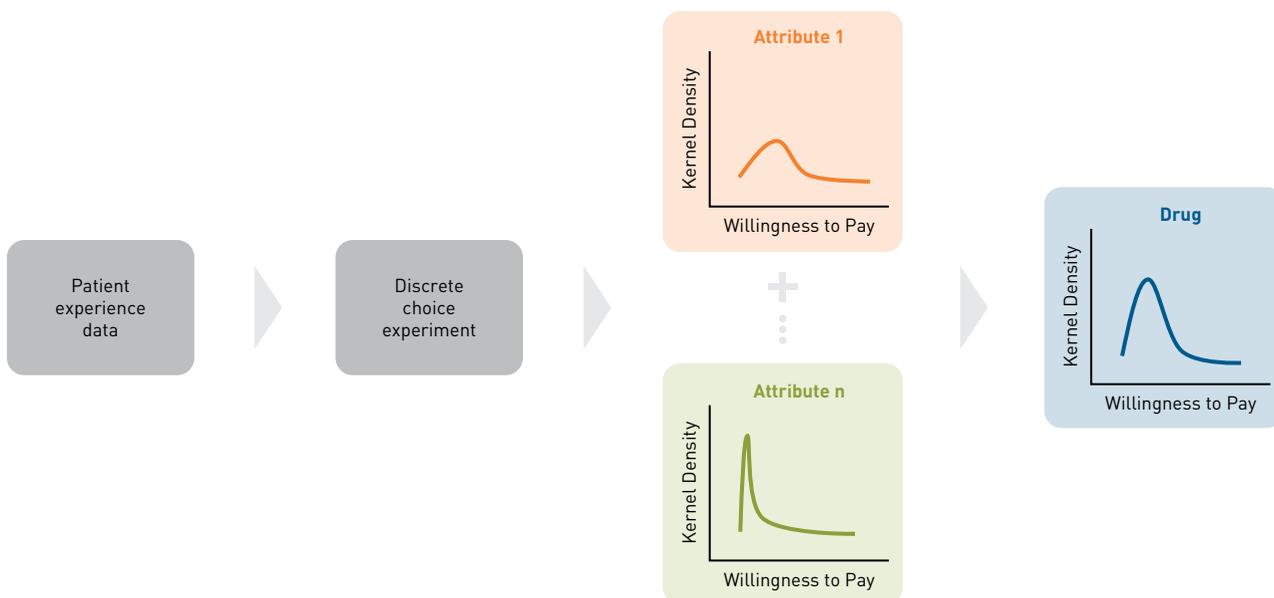
The proposed patient experience value framework utilizes patient experience data, generated using FDA guidance, to develop discrete

choice experiments (DCEs) to assess the value of drugs and account for heterogeneity of patient preferences (**Figure**).

#### **Patient Experience Data**

During the fifth authorization of the Prescription Drug User Fee Act, the FDA conducted dozens of “disease-specific, patient-focused drug development (PFDD) meetings to systematically obtain patient perspectives on specific diseases and their treatments.”<sup>3</sup> In 2016, the 21st Century Cures Act was signed into law. The law incorporates the PFDD into the FDA's decision-making process. Patient experience data are addressed in this act. The patient experience data primarily include information about patients' experiences with a disease or a condition and patient preferences regarding treatments of such disease or condition.<sup>4</sup>

As part of the continuation of PFDD efforts in accordance with the 21st Century Cures Act, the FDA is developing a series of guidance documents that focus on practical approaches and methods to address in a stepwise manner how to bring the patient's perspective or patient experience data into drug development and regulatory decision making.<sup>5</sup> The first draft guidance, “Patient-Focused Drug Development: Collecting Comprehensive and Representative Input,” was made available for public comment in June 2018.<sup>6</sup> This document

**Figure.** Patient Experience Value Framework for Value Assessment

includes general consideration for collecting patient experience data, methods for collecting and analyzing patient experience data, and operationalizing/standardizing data collection and data management.

The FDA's guidance on patient experience data provides methodologically sound data collection tools in clinical trials. I propose to apply this guidance to identify drug attributes that are important to patients. Previously, DCE studies used a variety of methods, including only literature review, to identify drug attributes. These drug attributes were clinically important, but they may not have captured what patients care about. From a patient perspective, the inclusion of irrelevant clinical attributes during assessments can lead to an overestimation or an underestimation of a drug's value.<sup>1</sup> Applying the FDA's guidance to value assessment provides a methodology for engaging patients and identifying the drug attributes that are most important to them.

### Value Assessment by DCE

I propose utilizing DCE with certain model specifications to derive patient preference, preference heterogeneity, and the distribution of the value of drugs. DCE is a rigorous method that offers the opportunity to incorporate patient preferences into value assessment processes. The International Society for Pharmacoeconomics and Outcomes Research Good Research Practices Task Force issued 2 reports that guide users on how to design DCEs.<sup>7-9</sup> First the DCE describes various choice sets by important drug attributes and their levels obtained from methodologically sound patient-experience data. However, a cost attribute is required for the value assessment purpose. An efficient design is recommended for drawing a subset of all possible combinations of selected attributes and levels to generate choice

sets.<sup>8,10</sup> Each choice set consists of a certain number of hypothetical drugs (alternatives) described by the selected attributes and levels. A questionnaire is then developed to include the choice sets. Patients are asked to select 1 alternative from each choice set.

Using random utility theory, patients' responses for each choice set are observed and analyzed in DCE. A mixed logit (ML) model can be used because it is a flexible and computationally practical econometric method for DCE.<sup>11</sup> In addition, the model allows the incorporation of preference heterogeneity. The utility function ( $U_{nsj}$ ) of the ML model is:

$$U_{nsj} = \sum_{k=1}^K \beta_{nk} X_{nsjk} + \varepsilon_{nsj}$$

where  $n$  = patient,  $s$  = choice set,  $j$  = alternative,  $k$  = attribute,  $X_{nsjk}$  = the full vector of observed attributes relating to individual  $n$  and alternative  $j$  on choice set  $s$ ,  $\beta_{nk}$  = the vector of individual-specific coefficients of attribute  $k$ , and  $\varepsilon$  = an error term.

Furthermore, the ML model can estimate individual-specific preferences from the mean of the parameters within the subpopulation of individual patients who select the same choices from the same choice sets. The model can also estimate the distribution of preferences for the attributes. Preference heterogeneity can be identified from the significant standard deviations of coefficients. From the ML model, the kernel density plots of the distribution of the individual-specific willingness-to-pay (WTP) estimates conditional on observed choices for each of the drug attributes can be developed.<sup>12</sup> The kernel density plot shows the proportion of patients for each WTP estimate point. Finally, the individual-specific WTP estimates from each of the drug attributes are added to provide the distribution

of the individual-specific WTP estimates or values of the drug.<sup>13</sup> In other words, the distribution provides the proportion of patients who are willing to pay for the drug at specific amounts.

## CONCLUSIONS

The US healthcare system can benefit from a variety of value assessment approaches. The patient experience value framework approach combines methodologically sound patient experience data and DCEs to assess the value of drugs in a patient-centered way. This method offers advantages over traditional QALY-based approaches and can complement existing value frameworks. •

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