Integrating the Patient Perspective into the Development of Value Frameworks
There has been growing concern about increased health-care spending, which has spurred activity around appropriate ways of assessing the value of treatment options. In addition to the challenges this poses to individual patients, payers and product developers alike, patient advocacy groups face a dilemma: they want to support new innovations that will help their constituents, but they also want to make sure that the value of these new treatments are based on criteria that matter to patients and that patients will be able to afford them.

Several organizations have recently developed or intensified their development of frameworks that attempt to objectively assess the value of individual therapies and other health-care services. However, most of them were not created with significant input from patients or patient organizations to their models or definitions of value. FasterCures has long been engaged in work that strives to bring the patient perspective to the medical research and development process and, in partnership with Avalere, is committed to developing the first value framework that truly includes patient perspectives and addresses individual patient value considerations.

The health-care system needs new tools and frameworks to:

- help providers and patients assess the value of different therapy options based on their specific perspectives and needs,
- allow public and private purchasers to better guide patients to the right health plans and services within those health plans, and
- aid pharmaceutical and medical device companies to appropriately target and price their therapies.

Avalere and FasterCures held a workshop at FasterCures’ 2015 Partnering for Cures meeting to discuss the growing trend of creating value frameworks and determine what is still needed in this area. The workshop’s audience—primarily made up of patients, patient advocates and pharmaceutical and medical device manufacturers—uncovered three key gaps and challenges in existing value frameworks. First, participants strongly expressed that the patient voice is largely lacking from current value frameworks, and there is an imminent need to develop a framework that considers the patient perspective on value. Second, participants felt that patient value considerations need to be thoroughly understood and assessed. Finally, to create a robust and viable framework that addresses the patient’s value perspective, all major stakeholders need to be involved in its development.

As a follow-up to the workshop, this report serves as a call to action regarding how to move forward to address these timely issues. To lay groundwork, we first provide an overview of these existing, newly developed value frameworks. We then outline key findings from the workshop geared toward refining five key value considerations from the patient’s perspective. We conclude by presenting an action plan for developing a patient-perspective value framework.

### Overview of the Approaches Taken by Newly Developed Value Frameworks

Avalere analyzed four value frameworks—developed by the American Society of Clinical Oncology (ASCO), the Institute for Clinical and Economic Review (ICER), Memorial Sloan Kettering (MSK) and the
National Comprehensive Cancer Network (NCCN)—that have recently been published. We briefly summarize them in Table 1 and provide more detail below.

Table 1: Summary of Current Value Framework Approaches

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<th>ASCO Value Framework</th>
<th>ICER Value Assessment Framework</th>
<th>MSK DrugAbacus</th>
<th>NCCN Evidence Blocks</th>
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<tr>
<td><strong>Output</strong></td>
<td>“Net Health Benefit” score</td>
<td>“Value-Based Price Benchmark” for a drug</td>
<td>“Abacus Price” for an oncology drug</td>
<td>“Evidence Blocks” for cancer regimens</td>
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<tr>
<td><strong>Inclusion of patient perspective</strong></td>
<td>Patients not included in development, but early versions of the framework were distributed with patient advocates</td>
<td>Families USA was the sole patient organization included in the Value Assessment Development Group</td>
<td>Involvement of patients and patient advocates in the Abacus development process is unclear</td>
<td>Evidence Blocks are created by panels of multidisciplinary expert clinicians—do not seem to directly involve patients or patient advocates in the process</td>
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The ASCO Value Framework

ASCO developed a framework to support decision making regarding the comparative value of new cancer therapies to standard regimens within randomized controlled trials (RCTs).1 In this framework, the value of a new therapy—or its “net health benefit score”—is analyzed based on three factors: its clinical benefit, toxicity and additional benefits such as improvements in the palliation of symptoms compared to a standard regimen. The net health benefit score is calculated as follows:

1. A therapy’s clinical benefit score can add up to 80 points and is drawn from changes in overall survival, progression-free survival or response rates compared to a standard regimen.
2. A therapy’s toxicity can either add or detract 20 points from the net health benefit score when compared to the toxicity and tolerability of a standard regimen.

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3. Finally, a therapy can gain 30 bonus points for **additional benefits**, compared to a standard regimen, such as improvements in the palliation of symptoms and treatment-free intervals.²

Though ASCO states that this framework is intended to provide medical oncologists with information to supplement the shared decision-making process, this framework does not yet include a patient-facing tool. Moreover, it is unclear how well the patient perspective was captured and incorporated throughout the process of creating the framework; ASCO’s methodology states that earlier versions of the framework were distributed at meetings with stakeholders including patient advocates. Finally, it is important to note that ASCO’s approach does not allow for comparisons across or beyond individual RCTs.

ASCO has invited public comment on its framework and is planning on publishing further iterations of the framework based on the feedback received.

**The ICER Value Assessment Framework**

ICER created its framework to assess the value of medical services, including drugs, devices and procedures based on three factors: care value, potential budget impact and the provisional value to the health system.³ Based on these three factors, the ICER framework produces a “value-based price benchmark” for each service.

First, care value is determined based on four components:

1. **comparative clinical effectiveness**, which reflects a therapy’s comparative net health benefit and the level of certainty in the evidence;
2. **incremental cost per outcomes achieved**, which reflects cost per quality-adjusted life year;
3. **other benefits or disadvantages** that were not included in clinical effectiveness, such as whether the method of administration encourages adherence; and
4. **contextual considerations** including ethical, legal or other issues that affect the relative priority of illnesses and therapies, such as whether other acceptable treatments exist.

Second, the therapy’s potential budget impact is calculated based on the estimated net change in total health-care costs over an initial five-year period.

Finally, an intervention’s provisional health system value is assessed based on whether it could treat a population with reasonable long-term value and with short-term costs that would not exceed growth in the national economy.

To create its framework, ICER convened a Value Assessment Development Group. However, Families USA was the sole organization representing patients or consumers included in the group out of 16 participating stakeholders. Moreover, ICER’s framework and data are not intended for a patient audience and do not include a patient-facing tool.

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With a $5.2 million grant from the Laura and John Arnold Foundation, ICER intends to review 15 to 20 products between 2016 and the beginning of 2017, near their time of Food and Drug Administration (FDA) approval. As a result, payers may use ICER’s value determinations as a basis for negotiating pricing and coverage policy.

Memorial Sloan Kettering Cancer Center’s DrugAbacus

Memorial Sloan Kettering’s DrugAbacus measures value according to six components: the value of a life year, toxicity, treatment novelty, research and development costs, rarity of the disease it targets and the population health burden the targeted disease causes. This tool provides a cost-benefit analysis for 54 cancer drugs that were approved by the FDA between 2001 and 2015. The DrugAbacus measures:

1. the value of a life year according to the improvement in overall survival,
2. toxicity based on both the number and severity of side effects that patients on the drug experience compared to the severity of the side effects they would experience if not on the drug,
3. treatment novelty based on whether a treatment has a novel mechanism of action or delivery,
4. research and development costs according to the number of subjects enrolled in the approval trials,
5. the rarity of the targeted cancer based on its projected incidence, and
6. the population health burden based on the estimated years of life lost due to the cancer in the U.S. population.

Memorial Sloan Kettering’s methodology for creating the DrugAbacus and its involvement of patients and patient advocates in the process is unclear. However, the tool itself suggests that it was not intended for a patient audience but rather for the providers who care for them.

The NCCN’s Evidence Blocks

NCCN’s Evidence Blocks assess the value of the regimens in its 66 cancer treatment guidelines according to efficacy, safety, quality and quantity of evidence, consistency of evidence and affordability. The Evidence Blocks measure:

1. efficacy according to the prolonging of life, the lack of disease progression and the reduction of symptoms that an intervention creates;
2. the safety of a regimen according to the relative likelihood of side effects from an intervention that is scored highly but with fewer side effects;
3. the quantity and quality of evidence related to an intervention based on the number of existing relevant clinical trials;
4. the consistency of evidence according to the consistency of clinical trial results; and

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5. the **affordability** of an intervention based on its entire cost, including the cost of the drug, supportive care and hospital care.

Though the NCCN Evidence Blocks are intended for all users of the NCCN Guidelines, including patients, the blocks are created by panels of almost exclusively multidisciplinary expert clinicians.

**Workshop Results: Conceptualizing and Refining the Patient Perspective on Value**

*The Original Patient-Perspective Value Considerations*

Avalere constructed the Partnering for Cures workshop to focus on five key patient-perspective value considerations:

1. **value of the treatment to the individual patient**, including an assessment of the clinical, functional and quality-of-life benefits and harms of a treatment that accrue to the patient over the long term;
2. **the cost of the treatment to the patient**, such as out-of-pocket spending, which will vary depending on an individual’s coverage and plan benefits;
3. **the strength of evidence**, which provides valuable information to patients because it guides them in understanding the reliability of the evidence being presented to them and to what extent a treatment is likely to show the benefits it is purported to have;
4. **shared decision making** (SDM), which refers to the consideration of a patient and family member’s personal values and preferences when deciding on a treatment option; and
5. **the usability of the information** provided by a value framework—for example, whether there is a patient-facing tool associated with the framework—that ensures access to meaningful, understandable information for SDM purposes.

**Value of the Treatment to the Individual Patient**

Many workshop participants felt that “value to the individual patient” was the most important consideration for a patient-centric treatment assessment tool. However, participants felt that none of the existing frameworks consider value from the individual patient’s perspective—a component that should be included in future frameworks. Participants generally voiced the need to further unpack the meaning of “value” and provide a more granular definition. Two suggestions were made for how to better define value.

First, participants felt that value considerations vary depending on whether one takes a condition-specific perspective or a more general perspective that cuts across conditions. For example, for patients with long-term and chronic illnesses, there is a need to consider a treatment’s long-term effects as well as how it will fit into an already complex treatment regimen. Thus, several participants felt that the current value framework landscape is too focused on end-of-life and cancer care and called for the
development of a more disease-agnostic framework that can also be relevant for patients with chronic illnesses.

Second, participants generally voiced the importance of considering “whole-person” value beyond just clinical value. By whole-person value, we refer to the effects that a therapy might have on a patient’s day-to-day experiences and functions, such as symptoms alleviated, potential side effects, the method and site of administration, whether child care or time off of work will be necessary, or whether support services will be available. Whole-person value involves considering how a treatment fits into the overall experience of a patient. Thus some participants expressed a need to include patient experience information in value frameworks.

Cost of Care to the Patient

To best assess the value of a treatment to a patient, individual costs must be considered alongside clinical value. Workshop participants highlighted various gaps and challenges in existing value frameworks, such as the need to:

- incorporate the most current prices of treatments;
- include considerations of additional costs associated with the therapy, such as imaging, rehabilitation and supportive care; and
- consider potential opportunity costs, such as lost wages, time off of work and transportation costs.

Some participants also felt that assessing the affordability of a treatment for the patient can be challenging as it is subjective and dynamic. There is a need to explore how a physician can truly assess what is considered affordable to an individual patient, especially as affordability may change depending on several factors, such as the time of year relative to out-of-pocket maximums, the type of coverage held year-to-year and changing formulary inclusions/exclusions.

Strength of Evidence

Workshop participants discussed how value frameworks can better assess the evidence for how a treatment will affect an individual patient while accounting for heterogeneity in the patient population. Namely, when applying evidence for a treatment’s efficacy, how can providers consider different disease experiences and the variability in responses to a treatment that different patients can have?

“The challenge I see in most of these models is that they’re so focused on an individual therapy. The reality is, especially for a chronic disease, that’s not a patient’s experience. A patient needs to think about the several therapies that they’re on and how they’ll all come together.” – participant at Partnering for Cures workshop

“One of the biggest gaps between evidence generation and evidence application in actual clinical care is the variability of response: subgroup effects and heterogeneity among the patient population. I don’t see that being addressed in any of these models.” – participant at Partnering for Cures workshop
To better account for the heterogeneity of the patient population, several participants expressed the need to adequately include the patient voice in evidence generation and explore how, for example, the same treatments affect different age groups or are used differently in daily practice. Other suggestions included allowing patients to test different treatments and relying on the patient-provider relationship and shared decision making to parse out different responses to treatment.

**Shared Decision Making and Usability of Information**

SDM and usability of information can be considered in tandem, as the information needs to be accessible to patients for them to adequately engage in SDM with their providers.

When assessing different treatment options, SDM refers to the incorporation of individual patient and family member values into the decision-making process and patient-provider conversations. Common examples discussed during the workshop that can affect a patient’s choice of therapy included whether a certain therapy would enable a patient to attend his or her child’s wedding or whether it would affect a patient’s ability to have children in the future.

To these ends, future value frameworks need to ensure that cost and quality information is accessible, understandable and usable to patients. Though several regulatory barriers exist for pharmaceutical and medical device manufacturers for sharing value-based information that is not in the label, current available information on the cost and quality of therapies is largely not intended for a patient audience. Therefore, there is a significant gap in the market for tools and strategies that help inform patients on the value of different therapies and that can be used by providers to drive conversations with patients and ultimately aid their decision-making processes.

**What Next? Ensuring the Patient Voice is Solicited and Integrated: Developing a Patient-Perspective Value Framework**

During the workshop, FasterCures heard the audience’s needs for a value framework that includes a more robust and representative patient perspective and addresses patient value considerations. To meet this need, FasterCures and Avalere Health have created a one-year action plan to develop a patient-perspective value framework through a rigorous, patient-centered process and methodology. We outline the four-step action plan below.

**Phase I: Creating Two Multi-Stakeholder Work Groups**

Phase one of the action plan involves creating a workgroup of 8-10 people/organizations committed to fully engaging in the framework development as well as a steering committee of 15-20 people/organizations that will be engaged in concept vetting and pressure testing at key stages of the framework development process. Key to this effort is the equal involvement of varied stakeholders to ensure that it is appropriately vetted, pressure tested and adopted by patients, providers, payers and
developers. We will ensure strong participation from patients and patient advocates in both stakeholder groups.

**Phase II: Developing the Key Features/Considerations for a Patient-Perspective Value Framework**

Phase two of the action plan involves soliciting input from our stakeholder groups on the current state of activities in the field; patients’ value considerations; concerns, challenges and barriers in the field; and key framework features and considerations, including specific use cases for piloting. We will then convene an in-person working session with our workgroup to gain consensus and draft the key framework components and features. The draft framework component and features will then be vetted, pressure tested and finalized with our steering committee in a series of feedback processes and conference calls.

**Phase III: Drafting and Pressure Testing the Patient Perspective Value Framework**

Phase three of the action plan involves leveraging existing work and conducting further background research to draft the patient-perspective value framework in the form of a public-facing report. The draft value framework will be refined by the workgroup in a series of working sessions, and it will be pressure tested with the steering committee through a series of feedback processes and conference calls.

**Phase IV: Conducting Promotion and Outreach**

We will present the draft patient-perspective value framework at the November 2016 Partnering for Cures meeting, and we will solicit feedback from meeting participants. After the meeting, we will publicly release the draft framework for public comment. We will incorporate public input into the final patient-perspective value framework and publish it on both the Avalere and FasterCures websites. Finally, with input from our stakeholder groups, we will develop a strategy for piloting the final framework across different therapies and use cases.

**Conclusion**

We’ve heard time and again from all sectors of the healthcare industry that they want to involve patients in the process, yet many are still making decisions without appropriately soliciting patients and ensuring that their input is being applied and used. It is time to start asking patients about their perspectives and fully incorporating them into the decision-making process. The landscape analysis, workshop and action plan have now brought us to a point where we can start the hard – and necessary – work of creating a true patient-perspective value framework.

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