



Advancing Patient Centered Approaches to Value-Based Care

*A Roadmap to Increased Patient
Engagement in Value Assessment*

Partnership to Improve Patient Care

www.PIPCpatients.org





Introduction

Value frameworks and similar value assessment tools are playing an increasingly prominent role in health care as insurance companies, Medicare, state agencies, and other stakeholders all are working rapidly toward "value-based" payment models. But will they pay for care that patients value? The Partnership to Improve Patient Care (PIPC) was founded on principles of patient-centeredness that cannot be achieved without engaging patients and people with disabilities. For the transition to a value-based health system to achieve outcomes that matter to patients, patients must have a seat at the table in defining value through the development of the value frameworks and tools.

Over the past few years, PIPC has listened to its patient partners about their engagement experiences in the development of value frameworks and tools. We recognize that underlying the lack of meaningful patient engagement in determining value, is the fact that patients and value framework developers often have very different perspectives. Outcomes that matter to patients define value to the patient, while value framework developers – particularly those targeting payer-level decisions rather than physician-patient decisions -- are often focused on average value from the payer perspective and short term costs that are useful to the actuarial value framework of insurers seeking to determine what they will or will not cover. Value to the patient is a relevant consideration in creating a value-based health system that is also patient-centered.

This issue brief builds on existing calls for patient engagement by providing a guide to developers seeking to create value frameworks and tools that can become the foundation of a truly patient-centered health system. The paper describes best practices in procedures for conducting value assessments that are patient centered; it does not address the equally important issues related to methods used for value assessment.

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Part One

Opportunities for Value Framework Developers to Effectively Engage with Stakeholder Groups

As optimizing the delivery of healthcare services has become increasingly tied to assessing the value of potential interventions, a growing number of organizations have developed value frameworks that offer new approaches to inform healthcare decision-making. The Institute for Clinical and Economic Review (ICER), American Society of Clinical Oncology (ASCO), and National Comprehensive Cancer Network (NCCN) have all recently engaged in the generation of distinct value frameworks. These tools take different approaches to assessing clinical and economic evidence to inform treatment and prescribing decisions by physicians and their patients, and in some cases, pricing or policy decisions made by private payers. To some degree, characteristics of these emerging value assessment tools reflect similarity to the more formal and structured health technology assessment (HTA) processes utilized in other countries.

New approaches to assess value have the potential to support well-informed, patient-centered healthcare; however, there is wide variability in approaches to incorporating stakeholder perspectives, particularly from patients, into the assessments as well as the development, refinement, and dissemination of the value frameworks.

Different value assessment tools are focused on different types of decision-making. Some, like ASCO and NCCN, focus primarily on physician and patient decision-making. Others, like ICER, prioritize payer-level decisions. At the same time, some value assessment organizations recognize the importance of considering the patient perspective and advancing the goal of patient-centeredness.

While the goal of patient engagement is commendable, it is not easy. It requires methods that accommodate the distinct, and varying, perspectives of patients on value of interventions. And it requires processes that engage patients and obtain and respond to their input. This paper focuses on key decision points in the value assessment process where patient engagement and input are important, and describes best practice procedures for achieving this.

It is critical that value assessment organizations utilize a formal, publicly described process for integrating the patient and other stakeholders' perspectives into frameworks to ensure their perspectives on value are reflected. A sound process is the essential foundation in achieving patient-centeredness in value assessment, but a sound process must also be matched by validated and transparent methods. Furthermore, increased transparency and stakeholder participation may lead to greater buy-in and utility of these tools, which is advantageous for developers. This issue brief outlines seven critical points in value frameworks where opportunities exist for increased stakeholder engagement. Furthermore, this brief identifies examples of best practices where value framework developers have successfully engaged in a meaningful way with patients and other stakeholders and provides opportunities for improvement.

Seven Key Opportunities for Improvement through Increased Stakeholder¹ Engagement

- Obtaining Stakeholder Input in Priority Setting
- Identifying and Employing Patient-Relevant Outcomes and Endpoints
- Structure and Use of Advisory Panels
- Soliciting and Responding to Stakeholder Comments During Conduct of Value Assessments
- Ensuring Transparency in Approach and Methods
- Engaging Patients in Dissemination of Results
- Structured Assessment Review Cycles

¹Key Stakeholders as used in this document are defined as: patients, caregivers, patient advocacy groups, clinicians, payers and policy makers

Part Two

Obtaining Stakeholder Input in Priority-Setting

As the first step in the assessment process, priority-setting can be defined as the process through which value assessment organizations determine which health care interventions (treatments, tests, services, etc.) will be reviewed. This step is essential to establishing an objective, trustworthy program and addresses patient-relevant priorities and assesses value across the continuum of patient care. With that in mind, value assessment organizations should seek to include representatives from all key stakeholder groups (i.e., patients, caregivers, patient advocacy groups, clinicians, payers and policymakers) beginning with the initial phase of topic selection and prioritization. An explicit priority-setting process enables stakeholders to effectively engage in determining which treatments or healthcare technologies will be evaluated.

Both patient groups and value assessment organizations will benefit from a transparent and inclusive priority-setting process. As noted in the guiding principles of the Institute of Medicine's (IOM) 1992 report, "Setting Priorities for Health Technologies Assessment: A Model Process," any priority-setting process "must be—and must appear to be—objective, open, and fair" and the process must "invite input from a variety of interested parties."² Additionally, incorporating patients and other stakeholders into the early steps of the review process will ensure that the value assessment organizations' research agendas reflect topics that are important to stakeholder and patient groups. Patients and providers will be better equipped to make informed topic recommendations with access to information about the range of drug and non-drug options discussed throughout the priority-setting process. Organizations will also benefit from the unique perspectives and expertise offered by patient groups. Ensuring stakeholder input in topic selection and prioritization will result in value assessment organizations choosing to review the most appropriate drug interventions and services based on current treatment patterns in the marketplace.

Consistent with the IOM's model for a priority setting process², the Patient-Centered Outcomes Research Institute (PCORI) has made strides to incorporate multiple stakeholder perspectives into all stages of the research prioritization and agenda-setting process. Investigators, patients, and other stakeholders have the ability to directly provide PCORI with potential research questions and participate in Advisory Panels. The procedure for evaluating investigator-initiated research proposals details patient and other stakeholder's involvement during every phase of the research process. For example, patients are brought into the discussion beginning with the proposal development and participate in the process of reviewing PCORI applications.³ Patients and other

² Donaldson MS, Sox HC, eds. *Setting Priorities for Health Technology Assessment: A Model Process*. Washington, DC: National Academy Press; 1992.

³ PCORI Merit Review Process. Available at: <http://www.pcori.org/funding-opportunities/merit-review-process>

stakeholders can also directly engage with PCORI to solicit research questions and request funding through topic-specific research proposals. Proposals are then selected for funding after careful review by the PCORI Board of Governors, Science Oversight Committee, Advisory Panels (comprised of practicing and research clinicians, patients, and experts in scientific and health services research, health services delivery), and relevant workgroups.⁴ PCORI has designed a process that provides patient and other stakeholder groups with several opportunities to meaningfully participate and share their perspectives in topic selection and research agenda setting.

By contrast, ICER lacks a clear process for priority setting and should establish one that is consistent with the IOM model. The PCORI example shows that following this model is possible, and demonstrates that ICER could benefit from following a similar approach. Topics selected for ICER evaluation are often drawn from horizon scans of new and emerging therapies and recently completed, comparative effectiveness reviews. Additionally, in ICER's current process, stakeholders have the option to propose a topic for a future review. Still, despite soliciting suggestions from the public, the process through which ICER selects the final group of treatments to evaluate is not transparent or open to public discussion. This lack of transparency makes it difficult for patient and stakeholder groups to truly understand the rationale behind topic selection and why certain topics were selected over others. Without greater transparency and stakeholder input, topics may not appropriately reflect the preferences and needs of clinicians and patients. Historically, ICER has released many of its assessments to coincide with the release of high-profile, high-cost FDA approvals of innovative treatments in the therapeutic class it plans to assess. By providing patient and stakeholder groups with transparent opportunities to participate in topic selection, ICER could gain public support by choosing topics that reflect greater stakeholder participation throughout its research selection process.⁵

⁴ How We Select Research Topics. PCORI. Available at: <http://www.pcori.org/research-results/how-we-select-research-topics>.

⁵ Topic Selection. ICER. Available at: <https://icer-review.org/methodology/icers-methods/topic-selection/>.

Part Three

Identifying and Employing Patient Relevant Outcomes and Endpoints

Value means different things to different stakeholder groups. Patients often weigh dimensions of value according to their individual circumstances, needs and preferences. To that end, value assessment organizations should proactively consult with patients and their caregivers throughout the assessment process and especially at the point of study design to better understand outcomes and endpoints that are the most relevant and important to affected patients (e.g., health-related quality of life, functional status, palliation of symptoms, survival, and productivity). To optimize the dimensions of patient preferences that are included, value assessment organizations must recognize the significance of the patient perspective and work to incorporate patient-relevant outcomes in their determinations of value throughout the assessment process and especially at the point of study design.

ASCO recently demonstrated its ability to adapt and address patient perspectives related to value in its May 2016 update to its value assessment framework. ASCO announced that in the next iteration of its framework they will incorporate patient-relevant outcome measures. The announcement was made in response to over 400 comment letters submitted by various stakeholders (e.g., patients, patient advocates, physicians, and manufacturers). As a significant start, ASCO incorporated quality of life into its calculation of net health benefit, marking a step towards addressing some of the concerns regarding a lack of patient-centeredness within the first version. In calculating the net health benefit, ASCO's framework will award bonus points not only for symptom palliation and treatment-free survival, but also award points for statistically significant improvements in quality of life. The combination of considering symptom palliation and quality of life differentiates this approach from other value frameworks.⁶

Furthermore, PCORI has institutionalized a practice of including patient-relevant measures in research proposals. PCORI's Methodology Committee developed a set of methodology standards that dictate that patient-relevant outcomes are factored into the proposals.⁶ The methodology standards recommend that patient-reported outcome (PRO) measures are used in lieu of, or in addition to, measures derived from other sources.⁷

Using these approaches as examples, ICER has an opportunity to refine its value framework by more consciously integrating patient-relevant measures into their value assessment processes,

⁶ ASCO Value Framework Update. ASCO. May 2016. Available at: <https://www.asco.org/about-asco/press-center/news-releases/asco-value-framework-update>.

⁷ PCORI Methodology Standards. PCORI. Available at: <http://www.pcori.org/research-results/research-methodology/pcori-methodology-standards#AssociatedwithPatient-Centeredness>.

through consistent consideration of outcomes that have meaning for patients. For example, while the current ICER framework includes sections that aim to consider “advantages/disadvantages” and “contextual considerations,” ICER’s framework does not explicitly define how this process could be used to further integrate the patient perspective into the determination of “Care Value.” In addition, while ICER considers cost from the perspective of the healthcare system, it does not consider patient out-of-pocket costs, a consideration that is of great concern to patients.⁸ In April 2016, the Cancer Support Community responded to ICER’s draft report on multiple myeloma by stating, “[t]he patient perspective is not incorporated into the ICER’s value framework, incorporating instead elements that are certainly relevant when exploring the efficacy and safety required for regulatory approval, but absent is the guidance of real world experience and preferences of the patients.”⁹ The Cancer Support Community’s comments align with those of other comment groups asking ICER and other value assessment frameworks to consider those endpoints that truly matter to patients.

⁸ Evaluating the Value of New Drugs and Devices. ICER. 2015. Available at: <http://icer-review.org/wp-content/uploads/2014/01/Slides-on-value-framework-for-national-webinar1.pdf>.

⁹ Institute for Clinical and Economic Review (ICER) Assessment of Drug Therapies for Multiple Myeloma. Cancer Support Community. April 2016. Available at: http://www.cancersupportcommunity.org/sites/default/files/uploads/policy-and-advocacy/letters-submitted/csc_comment_letter_to_icer.pdf.

Part Four

Structure and Use of Advisory Panels

Advisory panels represent a key opportunity for gaining input from patients and clinical experts with relevant clinical expertise. However, wide variability in how they are structured and used may limit the degree of diverse representation. Some value framework developers and HTA organizations utilize advisory panels to facilitate greater stakeholder representation and encourage a diverse perspective in the value assessment process. Value framework developers should ensure optimal representation from patients, practicing physicians, and researchers with clinical and research experience in the clinical area being assessed. However, simply having a patient representative on an advisory panel is not necessarily sufficient for meaningful engagement. It is critical for organizations to consider who participates on advisory panels, when and how often these entities are consulted, and the weight with which their input is considered. Additionally, advisory panel meetings should be open and transparent, announced in advance, afford an opportunity for input from the public, and provide a public record of its proceedings. As a best practice, value framework developers should not only ensure that patients are included throughout the process through venues such as advisory panels, but also design advisory panels that are diverse in the perspectives represented, while also representing the relevant expertise needed for meaningful input on the subject matter.

As examples, PCORI and the National Institute for Health and Care Excellence (NICE) actively engage with stakeholders through advisory panels. PCORI Advisory Panels are comprised of practicing and research clinicians, patients, and experts in scientific and health services research, health services delivery.¹⁰ NICE uses the Citizens Council which has 30 members recruited to reflect the diversity of the adult population in the UK. Members of the Citizens Council are asked for their views on specific moral or ethical questions important to NICE. These considerations form the “social values” that underpin NICE’s work.¹¹

On the other hand, while ICER also has its own advisory panels, the majority of panel participants are policy experts and representatives from payers and industry. Not one of the 8 seats on ICER’s advisory board is represented by a patient group.¹² ICER needs to proactively engage with clinical advisors who are board certified in the therapeutic area under study immediately after topic announcement so they can provide input into the scoping document. ICER does hold public meetings that include opportunities for panel votes and a policy roundtable; however, there is a need to include disease specific experts and greater patient representation on the advisory panels.

¹⁰ Join an Advisory Panel. PCORI. Available at: <http://www.pcori.org/get-involved/join-advisory-panel>.

¹¹ Citizens Council. NICE. Available at: <https://www.nice.org.uk/get-involved/citizens-council>.

¹² ICER’s Governance and Advisory Boards. ICER. Available at: <http://icer-review.org/wp-content/uploads/2016/02/Governance-and-Advisory-Board-Members-July-2016.pdf>.

Part Five

Soliciting and Responding to Stakeholder Comments During Conduct of Value Assessments

A standard and transparent process for addressing stakeholder feedback ensures appropriate consideration of stakeholder input. In order to provide this input, patients and stakeholder groups require a clear process to delineate how comments should be made and an understanding of how feedback will be addressed. Value framework developers should ensure that comment opportunities allow a sufficient time frame for review and consideration to provide input and that the comments and all responses are made publicly available to allow interested stakeholders, including the patient community, to understand how and why their input has or has not been incorporated. As a best practice, value framework developers should also provide an explanation of how comments were or were not addressed, and ensure that this information is publicly accessible and lacks ambiguity.

The Agency for Healthcare Research and Quality (AHRQ) provides an example of a structured and transparent process for addressing stakeholder feedback. The AHRQ TA Program invites peer review comments and public review comments and posts them on the TA Program website within three months after the associated final report is posted on this website. The report authors' targeted responses to the comments (the "disposition of comments") are also posted on the same Web page as the associated comments.^{13,14}

While ICER does have a process in place for interested stakeholders to provide comment during the assessment process, the open comment periods are often too short to provide sufficient and meaningful feedback. Other organizations, on the other hand, stipulate more adequate time frames for comment periods. For example, PCORI's statute specifies that public comment periods range from 45 to 60 days in length.¹⁵ Further, while public comments on ICER's draft reports are posted to its website, public comments on the draft scoping document are not publicly available. The Multiple Myeloma Research Foundation has stated that it "has no idea whether ICER addressed any of these concerns or ignored them completely. To be clear, the MMRF has not contributed to and in no way endorses, supports or affirms the views reflected in the ICER Draft Report."¹⁶ ICER needs to provide an explanation in its final reports of how input was or was not considered, and provide accurate information of organizations' views so as not to mislead the public. The comment opportunity on the ICER draft scoping document was recently expanded from one to two weeks; however to date,

¹³ AHRQ Technology Assessment Program. Available at: <http://www.ahrq.gov/research/findings/ta/index.html>

¹⁴ AHRQ Lifecycle for a Topic Nomination for Research. Available at: <https://www.effectivehealthcare.ahrq.gov/index.cfm/submit-a-suggestion-for-research/what-happens-to-my-suggestion-for-research/>

¹⁵ Patient-Centered Outcomes Research and the Authorization of the Patient-Centered Outcomes Research Institute (PCORI). Available at: http://www.pcori.org/sites/default/files/PCORI_Authorizing_Legislation.pdf

¹⁶ Giusti, Paul. Multiple Myeloma Research Foundation. Comments on the ICER Draft dated April 7, 2016. Available at: https://icer-review.org/wp-content/uploads/2016/05/MWCEPAC_MM_Public_Comment_050516.pdf

ICER has not made public comments on the draft scoping document or their responses to comments available to the public. The public has two weeks to submit written comment on the draft report and often the draft evidence reports that ICER releases are more than 150 pages in length, making it difficult for stakeholders to respond meaningfully in a short period of time. This point is highlighted by the American Society of Hematology acknowledging that “[b]ecause of the very short turnaround time associated with this review, ASH’s comments are brief.”¹⁷ Comments are made publicly available after the final report is issued. ICER releases a summary of comments and how they addressed them, but the document is not considered to be comprehensive.

In another example, there is also a lack of opportunity for stakeholder feedback in the Drug Effectiveness Review Project (DERP). DERP coordinates with a group of state Medicaid and public pharmacy programs to produce comparative, evidence-based research products to inform policymakers and other stakeholders making coverage decisions. DERP organizations and clinical advisors act as proxies for patients’ views on which health outcomes would be most relevant. DERP does not directly engage patients in the process for identifying outcomes, instead soliciting public feedback by establishing comment periods. However, the public comment opportunities are consistently short across all of the opportunities which does not give stakeholders sufficient time to review and provide robust feedback.^{18,19} Furthermore, patient organizations are often not the intended audience of the reports nor do they help patients make individualized care decisions.²⁰

The National Comprehensive Cancer Network (NCCN) framework could also improve its standard process for addressing public comments on their value assessment framework, known as Evidence Blocks, within their established guidelines. The NCCN Evidence Blocks were developed by NCCN staff in consultation with disease specialist clinicians and are intended as a visual representation of five key measures that provide important information to health care providers and patients about specific recommendations contained within the [NCCN Clinical Practice Guidelines in Oncology \(NCCN Guidelines®\)](#).²¹ To develop the Evidence Blocks, NCCN panel members scored each measure using a standardized scale from 1 to 5, with 1 being the least favorable and 5 the most favorable.¹⁹ Development was therefore restricted to NCCN members. While they accept public comments on the framework on an ongoing basis, it is not clear how comments are considered nor the process for potentially integrating comments into the framework. If input into the development of the framework is largely contained to NCCN clinicians and NCCN members, it misses an opportunity to more clearly integrate patient perspectives, limits the scope of meaningful feedback, and ultimately is less relevant to achieving outcomes that matter to patients.²²

¹⁷ Abrams, Charles and Kenneth Anderson. 20 April 2016. American Society of Hematology Comment Letter to Steve Pearson. Available at: https://icer-review.org/wp-content/uploads/2016/05/MWCEPAC_MM_Public_Comment_050516.pdf

¹⁸ DERP Timeline. Available at: <https://www.ohsu.edu/xd/research/centers-institutes/evidence-based-policy-center/derp/about/derp-structure/timeline.cfm>

¹⁹ DERP Report Timeline Key Dates. Available at: <http://www.ohsu.edu/xd/research/centers-institutes/evidence-based-policy-center/derp/documents/upload/04a-DERP-Report-Tracking-Public.pdf>

²⁰ Drug Effectiveness Review Project (DERP). The Center for Evidence Based Policy. Available at: <http://www.ohsu.edu/xd/research/centers-institutes/evidence-based-policy-center/evidence/derp/index.cfm>

²¹ National Comprehensive Cancer Network. NCCN clinical practice guidelines in oncology (NCCN Guidelines®) with NCCN Evidence Blocks™. NCCN website. <http://www.nccn.org/evidenceblocks/>

²² As of December 5, 2016, NCCN is in the process of making revisions to its Evidence Blocks to promote patient engagement and patient-centeredness

Part Six

Ensuring Transparency in Approach and Methods

It is imperative for end users of value assessment frameworks to understand the underlying assumptions that affect results, determine whether they have a strong evidentiary foundation, and be able to assess the implications of alternative assumptions. Value frameworks should provide transparency in methods in order to allow patients to see how patient-relevant factors are considered, position other organizations to replicate findings, and provide patients and providers, as decision-makers, confidence in the findings. This should include transparency in types of data used, economic models and assumptions. Additionally, a critical component of “transparency” is that the organizations developing value assessments allow for external assessment and validation of the methods used to derive their calculation of value. Value framework developers should follow robust methodologic guidance established through consensus documents, where available, in order to facilitate external validation and encourage transparent assessment of each organization’s approach to value assessment.

One example of a well-tested and validated approach is that of the European Society for Medical Oncology (ESMO). In their model, ESMO developed a validated and reproducible tool to assess the magnitude of clinical benefit for cancer medicines, the ESMO Magnitude of Clinical Benefit Scale (ESMO-MCBS). In developing its framework, ESMO consulted a robust team of faculty and field tested its methods with more than 50 expert biostatisticians. In developing its value assessment tool, ESMO ensured that several different stakeholders provided their perspective to ensure the model’s methodology was well tested and validated. Furthermore, as part of the established process, the ESMO-MCBS criteria are reviewed and revised on a regular basis.²³

ICER has the potential to benefit from following a similar approach. To date, ICER has been inconsistent in posting research protocols to Open Science Framework and lacks transparency of overall model design and related assumptions. Finally, ICER should establish a long-range plan to validate that the estimates and findings from the reports it generates on newly approved treatments actually align with results that are eventually demonstrated in the real world, with respect to cost-effectiveness and value to the health system. We would encourage ICER to assure all stakeholders that it has a process for validation of implied value that can be used to improve the utility of its reports over time and trigger revisions as needed to more accurately reflect real world experience. Insights could be gained from the *Initiative on Value Assessment Frameworks* being led by the International Society for Pharmacoeconomics and Outcomes Research which includes a focus on value to the patient in the development and dissemination of high-quality, unbiased value

²³ Cherny, N, Sullivan, R, Dafni, U et al. A standardized generic, validated approach to stratify the magnitude of clinical benefit that can be anticipated from anti-cancer therapies: the European Society for Medical Oncology Magnitude of Clinical Benefit Scale (ESMO-MCBS). *Ann Oncol.* 2015; 26: 1547–1573

assessment frameworks.²⁴

²⁴ International Society for Pharmacoeconomics and Outcomes Research, Initiative on US Value Assessment Frameworks, see <https://www.ispor.org/ValueAssessmentFrameworks/Index>

Part Seven

Engaging Patients in Dissemination of Results

Once a framework has been developed and output and findings have been produced, the natural next step is to establish a means of dissemination to support decision-making. With that in mind, a value framework should communicate information and results in ways that are relevant to the intended audience, reflective of patients' needs and preferences, and clear in terms of research protocol. For example, PCORI mandates that the process for conducting research and evaluating evidence be made transparently available to the public to ensure optimal understanding of research conclusions.²⁵ Patients and other stakeholders should be able to understand what the evidence means for them in order to avoid potential misuse and devaluation of the information. To accomplish this, value framework developers should ensure that patients and other stakeholders are involved in designing tools for the dissemination of results and implementation into decision-making between patients and providers. As a start, the final report should make clear the factors that are important to patients (e.g., ease of administration, dosing schedule) and clearly communicate the limitations, assumptions, and uncertainties of the findings that impact treatment decisions.

Examples of organizations that are facilitating the use of their findings in a way that can be used to help patients and physicians make better decisions include ASCO, NCCN, and PCORI.

In ASCO's first update to its value framework, ASCO 2.0 Value Framework, ASCO announced its plans to deploy the framework in a software application that would allow patients to modify the weights attributable to each of the framework's elements. ASCO foresees the framework will be deployed as a software application to help patients decide among their treatment options.²⁶ In another example, NCCN's Evidence Blocks™ provide a visual tool that may make it easier for clinicians and patients to interpret and arrive at their own conclusions on the value of a therapy. Finally, a key focus area for PCORI is understanding and showing how these highly technical reports can get distilled into useable information for patients. The PCORI Patient and Family Engagement Rubric describes ways that engagement in research can occur. The rubric is structured according to the three phases of research—planning, conduct, and dissemination of study results. Each section includes potential engagement activities, tips for how to demonstrate those activities in an Engagement Plan, and brief examples from successful applications.²⁷

²⁵ Patient-Centered Outcomes Research and the Authorization of the Patient-Centered Outcomes Research Institute (PCORI). 42 U.S.C. § 1320e(h)(3). Available at: http://www.pcori.org/sites/default/files/PCORI_Authorizing_Legislation.pdf

²⁶ Schnipper, Lowell, et al. (2016). Updating the American Society of Clinical Oncology Value Framework: Revisions and Reflections in Response to Comments Received. American Society of Clinical Oncology, doi: 10.1200/JCO.2016.68.2518. Retrieved from: <http://ico.ascopubs.org/content/early/2016/05/25/JCO.2016.68.2518>

²⁷ PCORI Engagement Rubric. PCORI (Patient-Centered Outcomes Research Institute) website. Published Feb. 4, 2014. Updates June 6, 2016. Available at: <http://www.pcori.org/sites/default/files/Engagement-Rubric.pdf>

In addition to these examples, it is also important that organizations establish a mechanism to monitor the use of the output that is disseminated to ensure that stakeholders are finding utility of the findings in real-world practice settings. This will be a key step in the evolutionary process of integrating value assessment tools into practice settings, and to support shared decision-making between patients and their provider. It is important that assessment framework developers collect feedback and solicit end-user input to guide the implementation process to ensure that the information aligns with the tools' intended use.

With these patient friendly dissemination models in mind, ICER and DERP are two examples of organizations that have an opportunity to improve their process of communicating findings in a meaningful way to patients and their providers. ICER posts reports to its website that are theoretically available to payers, clinicians, and patients. However, due to the technical nature of the final reports, the utility for their implementation at the point of care are highly limited and appear to be more oriented towards serving the payer and policymaker community. ICER goes as far as to create an "Action Guide" and a "Patient Decision Aid;" however, these tools are not tested as to the utility for shared decision-making between patients and their providers.²⁸ Furthermore, as of July 2012, DERP Reports and Products were deemed proprietary and only available to participating organizations within the program.²⁹ Participating organizations currently include 13 state Medicaid agencies.³⁰ Once a report is produced, only participating organizations have access to the full report. If non-participating organizations or the general public wish to learn more about the findings, they have to make a request to DERP. Following a request, an investigator will present an overview of findings and answer questions, but will not share the full report.³¹

²⁸ ICER Patient Materials. Available at: <https://icer-review.org/programs/patient-materials/>

²⁹ DERP Disclaimer. Available at: <http://www.ohsu.edu/xd/research/centers-institutes/evidence-based-policy-center/evidence/derp/documents/index.cfm>

³⁰ DERP Participating Organizations. Available at: <http://www.ohsu.edu/xd/research/centers-institutes/evidence-based-policy-center/derp/participating-organizations.cfm>

³¹ McDonagh et al.: Methods for the Drug Effectiveness Review Project. *BMC Medical Research Methodology* 2012 12:140.

Part Eight

Structured Assessment Review Cycles

Value assessment tools aim to inform decision-making; for that stated goal to be achieved, it is important that the most up-to-date evidence is used and assessments are updated as new evidence becomes available. Value assessment organizations should review and update assessments on a regular basis to ensure the assessments reflect current clinical practice and consider all available evidence.

DERP presents a helpful example of a structured process for systematic reviews of its reports. Reports are considered for update on an annual basis. The consideration process includes a scan of the literature using the same search strategy as the Final Report but limited to Medline. In addition, new drugs, safety alerts, and indications in the drug class are identified. Updated reports (along with all abstracts of articles identified in the search) are reviewed by participating organizations, and a decision is made whether or not to proceed with an update. If a decision is made to update a report, the key questions are reviewed for relevance and for potential changes. The process then repeats in the same fashion as an original report.³² DERP's thorough report helps to ensure that the most up-to-date evidence is considered in its evaluation of therapies and services.

The AHRQ Evidence-based Practice Center (EPC) program has instituted a similarly robust process to update their systematic reports. The stakeholder impact of each active report is assessed annually and categorized as high, moderate, or low. For reports with the highest stakeholder impact, the AHRQ EPC program commits resources to regularly update these reports. For reports of moderate stakeholder impact, the Scientific Resource Center gathers data on relevant new research relevant for a report topic and assesses whether the report's conclusions are outdated. The AHRQ EPC program then posts this information on the Effective Health Care (EHC) Program Web site. Reports in the lowest tier of stakeholder impact are assessed annually for stakeholder impact for three years and may be moved to a higher tier based as a result. These reports do not undergo regular content updating. Reports that remain in the lowest tier for 3 years are archived. This process is reviewed and revised on an ongoing basis.³³

NICE is another organization that has a process in place to ensure that the most up-to-date evidence is incorporated into its reports. When NICE publishes guidance, it proactively suggests time for its review. This is the length of time after publication after which NICE will consult with relevant organizations on a proposal about whether or not the guidance needs to be updated, and if so, how

³² DERP Systematic Reviews. Available at: <http://www.ohsu.edu/xd/research/centers-institutes/evidence-based-policy-center/derp/documents/methods.cfm>

³³ AHRQ. How Are Evidence-based Practice Center Systematic Reviews Updated? Available at: <https://www.effectivehealthcare.ahrq.gov/index.cfm/submit-a-suggestion-for-research/how-are-epc-systematic-reviews-updated/>

to update the guidance. The length of time between guidance publication and review consideration varies depending on the available evidence for the technology, and knowledge of when ongoing research will be reported. Guidance may also be reviewed before the suggested review time when there is significant new evidence that is likely to change the recommendations.³⁴

ICER has not communicated a process to reassess therapies it has reviewed. To date, ICER has provided little detail as to whether it intends to develop a review process and if so, how they intend to initiate one. ICER has the potential to benefit from following a similar approach to reassessing its findings outlined by DERP, AHRQ, or NICE.

³⁴ NICE Guide to the processes of technology appraisal. 2 September 2014. Available at:
<https://www.nice.org.uk/process/pmg19/resources/guide-to-the-processes-of-technology-appraisal-pdf-72286663351237>

Part Nine

Recommendations & Examples of Best Practices

Key Opportunities for Engagement	Best Practices
1. Obtaining Stakeholder Input in Priority Setting	Value assessment organizations should seek to include representatives from all key stakeholder groups beginning with the initial phase of topic selection and prioritization, and should describe an objective process for priority-setting.
2. Identifying and Employing Patient Relevant Outcomes and Endpoints	Value framework processes should seek input on the outcomes and endpoints that are the most relevant and important to patients, and work to incorporate patient-relevant outcomes in their determinations of value. Assessments should appropriately consider both short term and long term outcomes.
3. Structure and Use of Advisory Panels	Value framework developers should not only ensure that patients are included throughout the process through venues such as advisory boards and voting panels, but also design advisory boards and voting panels that are diverse in the perspectives represented and include adequate representation from clinical experts and practicing physicians.
4. Soliciting and Responding to Stakeholder Comments During Conduct of Value Assessments	Value framework developers should ensure that comment opportunities allow a sufficient time frame for review and consideration and that the comments submitted are made publicly available to allow interested stakeholders, including the patient community, to understand how their input was (or was not) considered. As a best practice, value framework developers should also provide an explanation of how comments were addressed, and ensure that this information is publicly accessible and lacks ambiguity.
5. Ensuring Transparency in Approach and Methods	Methods for assessing value should be grounded in sound, recognized methods and be subject to meaningful and rigorous peer review. Value frameworks should provide transparency in methods in order to allow patients to see how patient-relevant factors are considered, position other organizations to replicate findings, and provide decision-makers confidence in the findings. This should include transparency in types of data used, economic models and assumptions made, with reliance on consensus document, where available.

6. Engaging Patients in Dissemination of Results	An optimal value framework should communicate information and results in ways that are both relevant to the intended audience and reflect patients' needs and preferences in order to help the intended end-user make decisions. Value framework developers should ensure that patients and other stakeholders are involved in designing approaches to the dissemination and implementation of results. Developers should also establish mechanisms to insure that their tools are being utilized based on intended use.
7. Structured Assessment Review Cycles	Value assessment organizations should review and update assessments on a regular basis to ensure the assessments reflect current clinical practice and consider all available evidence.