ASSESSING VALUE: PROMISE & PITFALLS

Health Care Stakeholders Identify a Path Forward on Value Assessment
Introduction

The National Pharmaceutical Council (NPC) brought together health care stakeholders on September 29, 2016, to examine one of the most critical issues in health care today: how to measure the value of a medical treatment and its impact on patient care.

Hundreds of participants representing biopharmaceutical manufacturers, payers, patients, providers and other experts gathered in Washington, D.C., in person and online, for a conference, Assessing Value: Promise & Pitfalls. Participants examined the state of value assessment tools, identified ways to advance the field and engaged in dialogue on how to use assessment frameworks to make health care decisions.

NPC President Dan Leonard, who kicked off the conference, said, “With so many new life-saving and life-improving treatments for cancer and medicines to treat rare or chronic conditions, it is more important now than ever to be able to capture and communicate the value of these groundbreaking medicines.

“Moving in parallel to these scientific breakthroughs is a massive health care system transition—one shifting from a focus on paying for the volume of health services to a focus on paying for the value of health benefits accrued to the patient. In this changing environment, how do we measure value? And how can we make sure that patients have a full voice in how value is measured?”

A number of value assessment frameworks have been developed in an attempt to address these questions, but the field of value assessments is still relatively young. However, Mr. Leonard stressed, it is important to realize that even these early value assessments could have tremendous impact on treatment decisions as well as coverage and reimbursement decisions. It is therefore of great importance that health care stakeholders come together to shape the development of value assessment going forward, he noted.

To date, organizations such as the Institute for Clinical and Economic Review, National Comprehensive Cancer Network, Memorial Sloan Kettering Cancer Center, American College of Cardiology-American Heart Association and the American Society of Clinical Oncology have developed frameworks, which were designed as decision-making aids with different end users in mind.

NPC President Dan Leonard highlights the importance of value assessments in his opening remarks.

Mr. Leonard noted that this is an important point because one framework cannot meet the needs of all stakeholders. Different frameworks will be needed to answer different questions.

“There is much more work that needs to be done before these frameworks are ready for widespread adoption and use,” he said.
Framing the Discussion

Why is assessing value in health care important? Why is it important to do it right? These two questions were at the heart of a presentation by NPC Executive Vice President and Chief Science Officer Robert Dubois, MD, PhD. “Clearly understanding value, measuring value, is important, but it has to be done right,” said Dr. Dubois.

Dr. Dubois raised four key challenges with assessing the value of health care treatments:

1. **Beware of what you ask for and the consequences.** According to a Kaiser Family Foundation survey, a majority of Americans say they want to see more government intervention in health care. Value frameworks will be used to guide health care decisions, making this a double-edged sword. The United Kingdom and Germany have similar systems for making choices, which at times have limited patient access to beneficial treatments. There needs to be a tradeoff between cost, better patient care and innovation.

2. **Be careful what you call value assessment.**
The concept of “value” is different from “budget impact,” which is the cost per patient times the number of patients. Value and budget impact are also different from “affordability,” which refers to society’s willingness to pay. Terms—and assessments—need to be kept separate.

3. **Today’s frameworks have much to teach, but improvements are needed.** The existing frameworks are at a fledgling stage, and none is ready for broader use or adoption. The methods need testing, the evidence is often limited, all benefits are not always considered, the assessments may lack a system-wide perspective and the output can be confusing for the end user.

4. **Be careful in deciding how a framework can be used.** The results of a framework’s output could be misinterpreted and seriously “affect patients in the allocation of resources.”

There is much that we need to learn before these frameworks “are ready for prime time” or broader use in the United States, Dr. Dubois explained. In order for the field to advance, there are six issues that value assessment developers and stakeholders “need to wrestle with before we’re ready to really, really say we’re there”:

- The type of evidence that is used—and how it is used—should be a critical underpinning for all assessments.
- Frameworks need to have deeper involvement of patients and their perspectives.
- One size doesn’t fit all. Frameworks need to have flexibility to include a variety of inputs.
Frameworks should be transparent; end users should be able to see the evidence that was used and know how the model was built so results can be reproduced.

Cost effectiveness should be considered from both a health care and societal perspective, such as considering productivity improvements and the burden on caregivers.

Budget impact is different than value assessment. If the budget impact is estimated, then it should be considered separately from value determinations.

Dr. Dubois acknowledged that these challenges wouldn’t be solved in one day, and added that the “dialogue that begins today is going to continue for a long time in the future.”

Moving Value Frameworks From Fledgling to Functional

Cliff Goodman, PhD, Senior Vice President and Director of the Center for Comparative Effectiveness Research at the Lewin Group, headed a team that evaluated how the five major value assessment frameworks align with NPC’s Guiding Practices for Patient-Centered Value Assessment. These Guiding Practices outline 28 specific elements for value assessment, which are broken out into six key aspects: assessment process, methodology, benefits, costs, evidence and dissemination and utilization. The Guiding Practices also highlight seven methods for sound budget impact assessment, but make clear that budget impact should be considered separately from value, as budget impact is not a measure of value.

The five frameworks analyzed were developed by the Institute for Clinical and Economic Review (ICER), National Comprehensive Cancer Network (NCCN), Memorial Sloan Kettering Cancer Center (DrugAbacus), American College of Cardiology-American Heart Association (ACC-AHA) and the American Society of Clinical Oncology (ASCO).

During his presentation, Dr. Goodman made several key points:

- **Intended audiences.** These frameworks were designed for different purposes and audiences, with ACC-AHA, ASCO and NCCN developed primarily for clinicians and payers; ACC-AHA expects that its assessments will reach payers as well. ICER and DrugAbacus’ assessments are intended for policymakers, payers and industry. Framework developers should be clear about their intended audiences and how the framework addresses users’ interests.

- **Transparency.** Limitations in transparency, such as evidence sources, methods and management of stakeholder feedback, can diminish the credibility and utility of value frameworks.

- **Stakeholder input and feedback.** Clear, timely and responsive provisions for stakeholder input and feedback are recognized globally as standard attributes of publicly accountable health technology assessment programs and related efforts involving health and economic evaluations. It also is necessary to demonstrate responsiveness to such input.

“Regardless of who your primary target audience is, put patients at the table from the start.”

Dr. Cliff Goodman, Senior Vice President and Director of the Center for Comparative Effectiveness Research, Lewin Group
● **Patient involvement.** To date, frameworks vary widely in the extent to which they seek or reflect patient involvement. Patients are ultimately affected by the value-based decisions of other stakeholders, so it is very important to engage with patients and consider their perspectives throughout the framework development and assessment processes.

● **Expert involvement.** The credibility of value assessments depends in part on the types and extent of expert involvement. As frameworks use more advanced methods to evaluate different types of evidence, conduct extensive economic modeling and serve more diverse users, they should revisit their mix of expertise and ways in which experts are involved in the processes.

● **Types of interventions.** Among these frameworks, the majority of focus is on drugs and biologics, leaving stakeholders without value assessments for devices, diagnostics, surgical procedures and other interventions. That limited focus will bias the basis for informed decision-making.

● **Evidence sources and quality.** The data that is selected and used as part of a value assessment can bias results. Frameworks should be transparent about the sources and types of evidence that are used, note their limitations and consider updating an assessment should new evidence become available.

● **Costs and other economic aspects.** There is wide variation among the value frameworks with respect to cost analyses and cost-effectiveness thresholds. This highlights the importance of transparency in the assumptions, evidence and methods used, but also the recognition of their impact on the findings of value assessments and how these findings will be applied.

● **User preference entry.** Value frameworks vary widely in enabling user input. All of the frameworks can be more user friendly and provide good user interfaces. To become more user friendly, frameworks should enable entry of user preferences by their primary target audience, as well as by others who might be affected by the findings. Most framework developers have generally indicated an interest in enabling this input.

● **Potential misinterpretations and misuse.** There is utility in having multiple frameworks address the same topic from different perspectives, but frameworks must be clear about their intended uses and assumptions to avoid confusion. They must make a concerted effort to ensure their work is transparent and comprehensible, and minimize or correct misinterpretation or misuse.

Dr. Goodman stated, “[T]hey might be called something else tomorrow, but value frameworks and the thinking of analyzing health and economic benefits for individuals and populations, that’s not going away.” As he noted, frameworks are a response to increasing demand for evidence and analysis. They should continue to evolve and change, benefitting from better alignment with good practices, especially when it comes to transparency, stakeholder engagement and methodological rigor.

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Shelley Fuld Nasso, Chief Executive Officer, National Coalition for Cancer Survivorship, looks on as Dr. Stephen Noga, Senior Medical Director for Global Medical Oncology Affairs, Takeda Oncology, makes a point during a panel discussion.
Multiple Myeloma: A Case Study

Dr. Goodman also presented results from research that compared how four existing value assessment frameworks reviewed treatments for multiple myeloma. The research looked at the patient population for each value framework, treatments analyzed, the evidence base, clinical outcomes of interest, measures of cost/affordability and the main output of the value assessments. Given the variety of inputs and methodologies, each framework arrived at different results.

In particular, this analysis of four value frameworks (NCCN, ASCO, ICER and DrugAbacus) and their assessments of multiple myeloma treatments found:

- A major concern is the relative timing for assessments of new treatments. For some treatments, although NCCN and ICER looked more broadly at the evidence, only one clinical trial was available at the time each framework was applied to assess multiple myeloma treatments.

- Patients with multiple myeloma value some outcomes that may be lesser or non-priorities for some or all frameworks, including:
  - health-related quality of life;
  - ease of use;
  - management of toxicities and side effects (including low-grade, chronic side effects); and
  - financial toxicity (i.e., patient cost burden of therapies).

- Updates to multiple myeloma assessments are necessary, given that treatments and the evidence base for this disease are rapidly evolving.

Dr. Goodman outlined next steps based on lessons learned from this analysis, which include:

- Considering the assessment timing for conditions such as multiple myeloma. He recognized, however, that there will be tradeoffs of user demand for timely findings and sufficiency of the evidence for credible findings. He cautioned that in the absence of head-to-head comparisons, use of indirect comparisons may weaken findings.

- Reaching out to multiple myeloma patients and clinicians early and maintaining ongoing outreach to better understand:
  - patient-centered and clinically relevant outcomes;
  - comparators that are relevant to therapeutic options for patients and clinicians; and
  - how patients, clinicians and others who are not the primary target audiences of an assessment may be affected, directly or indirectly, by how stakeholders will use the results of those assessments.

- Ensuring that frameworks are explicit and otherwise transparent about multiple methodological aspects, e.g.:
  - how and why particular regimens were selected for assessment;
  - the sources of evidence used (including who can submit evidence);
  - protocols and criteria for inclusion/exclusion of evidence;
  - how data are entered and used in scoring, equations, algorithms, models, etc., with examples included with the methodology; and
integration of expert stakeholder and patient input.

- Making sure that frameworks are more explicit and otherwise transparent about additional aspects, e.g.:
  - intended audience(s) and purpose(s);
  - limitations of frameworks and output; and
  - guidance on use of frameworks.

- Incorporating provisions for prompting assessment updates (periodically or with the availability of new evidence). Dr. Goodman pointed out how the various frameworks address this challenge:
  - NCCN’s process is well designed for rapid response to new evidence, though it does not involve in-depth systematic reviews or economic modeling.
  - As ASCO develops methodology, it should consider a process for assessment updates.
  - ICER does not currently update assessments and should consider doing so, especially when data/evidence are limited at the time of initial assessment.
  - DrugAbacus does not update the data in its tool. Recently, it added indication-based pricing for four drugs. It should consider expanding that feature to more drugs and updating the data on a regular basis.

Key Takeaways From Panel Discussions

Panel discussions were held following each of Dr. Goodman’s research presentations. Throughout the course of these conversations, key themes emerged:

- **More deeply involving patients and their perspectives is required.** Patient engagement from the start of a value assessment—and development of a framework—is extremely important. Several panelists expressed frustration that patient needs are often not considered in the assessment process.

- **Value is different from budget impact and should be treated separately.** Joshua Ofman, MD, MSHS, Senior Vice President, Global Value, Access & Policy, Amgen, noted that across the US, “we’re really having a conversation about budgets disguised as a conversation about value.” Participants recognized that budget analysis is important, but it is a measure of resource use, not of value, and should be kept separate. Combining the two analyses could create confusion.

- **It’s not that we shouldn’t calculate budget impact analysis; it is clearly something very important and something that payers need. But I do think it should be separate from the value analysis.”**

  *Dr. Peter Neumann, Director, Center for the Evaluation of Value and Risk in Health, Tufts Medical Center*

- **The existing frameworks could benefit from greater collaboration.** Panelists do not believe there will be one universal value framework for all diseases, or even for one disease such as cancer, as one size won’t fit all. However, there can be greater collaboration and alignment among frameworks. Opportunities for collaboration and alignment include sharing of data and resources, creating a curated database and exchanging ideas about standard sets of outcomes that can be more broadly applied. There also is potential for cooperation regarding good practices for transparency, stakeholder engagement and methodological rigor.

- **Value assessments should focus broadly on all aspects of the health care system, not just on medications.** Focusing only on medications—which are one component of the health care system—does not provide a complete perspective of the system. It also does not contribute to the development of value assessments for alternative interventions; limiting the scope of available assessments will bias decision-making. Value assessments need to consider all aspects of the health care system, such as diagnostic tests, procedures, hospitalizations and office visits.
- **Framework developers feel responsible for minimizing misuse and misinterpretation.** There is potential for value frameworks to be misused or misinterpreted by an unintended audience, and framework developers feel responsible for ensuring that their work is used as intended. Keys to minimizing problems are checks and balances, transparency and being receptive to feedback. If an assessment is being misused or misinterpreted, developers see this as a signal to go back and modify or improve it.

- **Assessments should be regularly reviewed and updated to keep pace with and account for medical innovation.** Changes in technology and the evidence base can cause an assessment to become outdated, which could adversely affect patient care. Frameworks should have provisions for prompting assessment updates, either periodically or upon availability of new evidence. ASCO, for example, envisions having all of the information and evidence about disease regimens for a specific indication curated in a library, loaded into software and made instantaneously available via a mobile device. This shared resource would be continuously updated in real time.

- **In some situations, value frameworks could actually help expand patient access to a treatment.** A perception of some stakeholders is that value frameworks are intended to limit care. In one example, a payer could utilize a framework to demonstrate the high value of a treatment in a new population, which could expand use of the drug to that population.

- **Payers expressed interest in innovative contracting approaches based on value.** Medical directors from health plans said they do not have a problem paying for high-cost drugs that have high value; the issue is paying when there is not good value. Contracting linked to value is of interest.

- **Market forces are encouraging providers to think differently about value.** Increasingly, it is not just payers and patients thinking about value. As payment incentives change and providers are now being rewarded for value, they have greater interest in understanding and measuring value.

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“The budget impact conversation is a really important conversation to have … [but] it unfortunately treats biopharmaceuticals like a consumption good rather than an investment good.”

*Dr. Joshua Ofman, Senior Vice President, Global Value, Access & Policy, Amgen, and NPC Board Member*
“I think it is incumbent upon us to be as transparent as possible and also to make our methods as reproducible as possible.”

Anna Kaltenboeck, Program Director and Senior Health Economist, Center for Health Policy and Outcomes, Memorial Sloan Kettering Cancer Center

Humana Senior Vice President and Chief Medical Officer Dr. Roy Beveridge, National Patient Advocate Foundation Executive Vice President for Patient Advocacy Gwen Darien, the National Coalition for Cancer Survivorship’s Ms. Fuld Nasso and Takeda Oncology’s Dr. Noga participate in a panel discussion moderated by NPC’s Dr. Dubois (center).

Next Steps in Framework Evolution

At the end of the day, Dr. Goodman and Dr. Dubois noted that there is still much work to be done in the evolution of value frameworks, especially if they are meant to be tools that will help health care decision-makers. They echoed several critical points raised by participants throughout the course of the conference, such as ensuring that patients and their perspectives are included in the assessment process, being transparent about the types of evidence and methods that are used, incorporating user preferences, viewing cost effectiveness both from a health care and a societal standpoint and carefully considering whether and how a budget impact assessment is done.

Value assessments will affect innovation.

All stakeholders expressed a desire to continue to drive high-value scientific innovations. Although the current value frameworks are imperfect, participants felt they will be a factor that will affect innovation. Biopharmaceutical innovators said that the emphasis on value already impacts research and development decisions, considerations about a company’s pipeline and portfolio and pricing decisions. In addition, this will affect the type of evidence that they will need to develop as part of their research process.

The Lewin Group’s Dr. Goodman and NPC’s Dr. Dubois discuss next steps in the evolution of value assessment frameworks.
“If we get it right, value assessments can be valuable and useful tools for practitioners, patients and payers. ... [If] we get it wrong, these tools could have unintended consequences that could limit patients’ ability to get the innovative treatments they need.”

Dan Leonard, President, NPC

As Mr. Leonard said, “These early value assessments could have a tremendous impact on patient treatment decisions, as well as on coverage and reimbursement decisions. So it’s important for us to bring all health care stakeholders together to help shape the development of value assessment going forward.

“If we get it right, value assessments can be valuable and useful tools. But if we get it wrong, these tools could have unintended consequences that could limit patients’ ability to get the innovative treatments they need.”

Although the conference itself was just one day, the dialogue—and NPC’s engagement with stakeholders—will continue, particularly as the field of value assessment evolves and matures. NPC is proud to play a constructive role in this ongoing conversation.
Program Participants

NPC would like to thank our conference speakers and cosponsors for their participation in this important dialogue. Our cosponsors included the Alliance for Aging Research, Caregiver Action Network, Friends of Cancer Research, Global Liver Institute, Healthcare Leadership Council, National Black Nurses Association, National Health Council, National Patient Advocate Foundation, National Psoriasis Foundation and Personalized Medicine Coalition.

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Additional Resources

The National Pharmaceutical Council has developed a number of resources about value assessment, including guiding practices, interviews with thought leaders and public comments, all of which are available on our website.

National Pharmaceutical Council Website:
www.npcnow.org

Guiding Practices for Patient-Centered Value Assessment:
www.npcnow.org/guidingpractices

Current Landscape: Value Assessment Frameworks:
www.npcnow.org/publication/current-landscape-value-assessment-frameworks

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