Value Assessment Frameworks

Elisabeth Oehrlein, PhD, MS

October 31, 2019

10:15 AM – 11:30 AM

Session Overview

Overview and Introduction to Value Assessment

- What is value assessment?

- Why should patients engage?

- What resources are available to help patients and patient groups engage?

Panel Discussion

- Catherine Davis Ahmed, VP, Policy and Outreach, The FH Foundation

- Annie Kennedy, Senior Vice President, Legislation & Public Policy, Parent Project Muscular Dystrophy

- Ashley Valentine, Co-Founder and President, Sick Cells

Q&A

What is value assessment?

Value

VALUE ASSESSMENT

Sometimes called a "Health Technology Assessment" or HTA

Multidisciplinary process
- Reviews clinical evidence compared to existing care

Cost effectiveness

Social and ethical impacts

VALUE FRAMEWORK

A tool used by some organizations to evaluate new treatments
- It produces a value assessment, report, or recommendation

May be used to guide reimbursement, shared-decision making, or other decisions

Value Framework Developers or Value Assessment Bodies*

Organizations that conduct a value assessment using a value framework as a guide

US examples

Value Framework Developers or Value Assessment Bodies*

Organizations that conduct a value assessment using a value framework as a guide

US examples

|	ACC-AHA	ASCO	DrugAbacus	ICER	IVI	NCCN	PPVF
Target Audience	Clinicians/ patients	Clinicians/ patients	Primarily payers; secondarily policy makers, clinicians, patients	Primarily payers; secondarily policy makers, clinicians, patients	Payers, policy makers, clinicians, patients	Clinicians, patients	Payers, policy makers, clinicians, patients
Services Addressed	Drugs, devices, other interventions	Drugs	Drugs	Primarily drugs, limited extension to other medical services	Drugs (could be extended to other health care services)	Treatment regimens, primarily drugs	Drugs (could be extended to other health care services)
Conditions Addressed	Cardiovascular	Oncologic	Oncologic	All conditions, particular focus on new drugs anticipated to be high impact	All conditions	Oncologic	All conditions

Westrich K and Buelt L. Current Landscape: Value Assessment Frameworks. Washington, DC: National Pharmaceutical Council; Fall 2019. Available from: https://www.npcnow.org/system/files/research/download/npc-current-landscape-value-assessment-2019-final-web.pdf
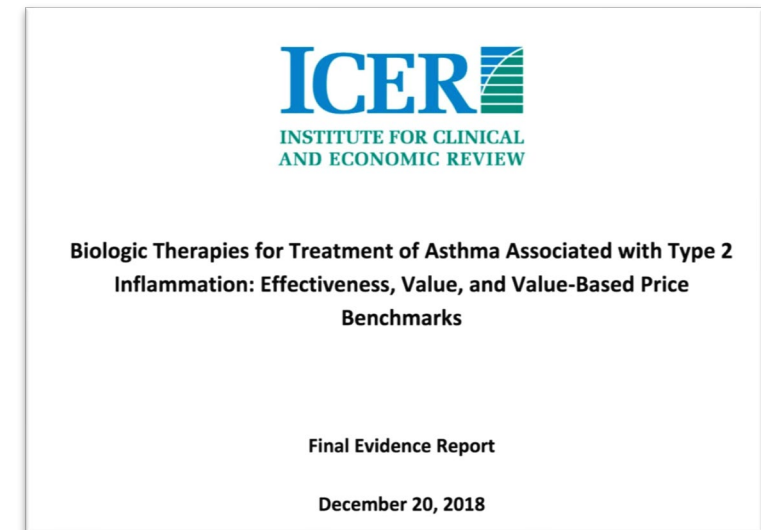
Terms in Context Example

Value Framework Developer

Value Framework

Overview of the ICER value assessment framework and update for 2017-2019
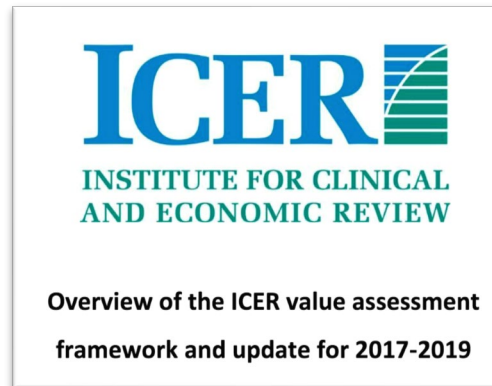
Mini textbook on their approaches to:

- Comparative effectiveness research
- Cost effectiveness analysis
- Patient engagement

Methods applied to a specific topic →

Biologic Therapies for Treatment of Asthma Associated with Type 2 Inflammation: Effectiveness, Value, and Value-Based Price Benchmarks

Final Evidence Report

December 20, 2018

Value Assessment

- "Value" Recommendation

*(health technology assessment body)

Why should patients engage?

Value to Whom?

Patient-Centered Value Assessment

Goal of patient-centered VA is for patients to have access to treatments they need at prices they can afford. Patient-centered VA exists when patients have been engaged, heard, understood, and respected throughout the entire process, and their input is incorporated and guides decision-making.
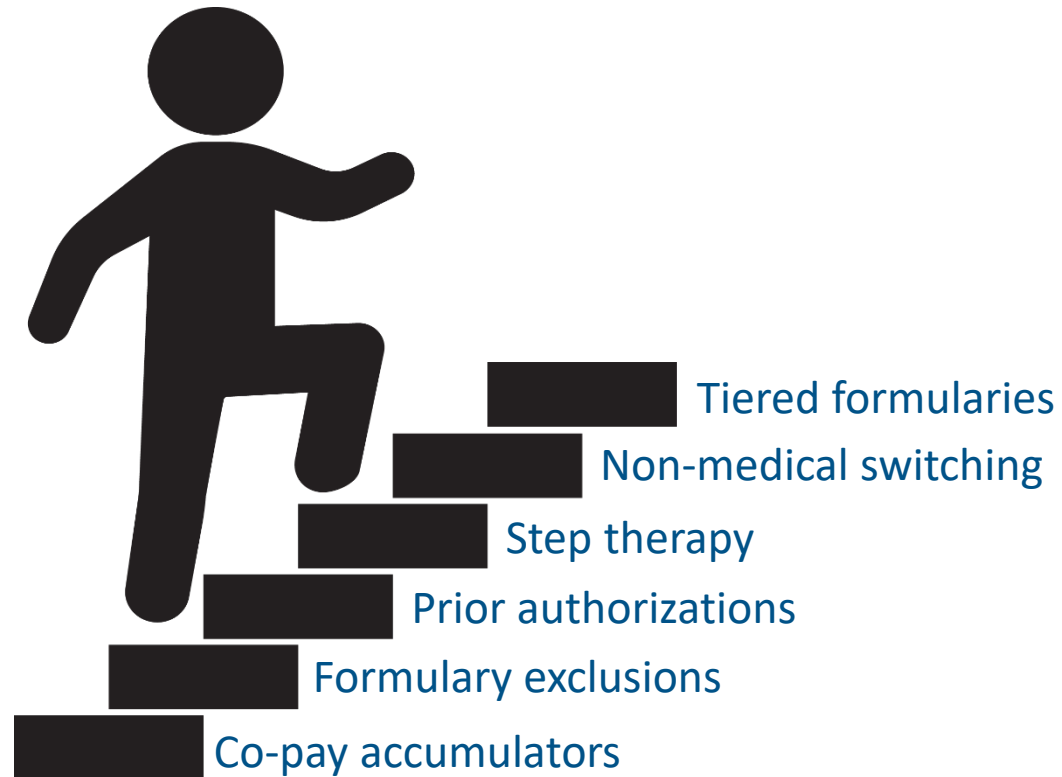
Why Engage?

- Many assumptions go into a value assessment
  - VA bodies may not be getting their facts straight regarding patient lived experiences, such as:
    - Desired outcomes
    - Costs
    - Treatments

- Make these reports a source of information on their condition

- Push value assessors and the researchers to innovate

Potential Impact on Patient Access and Outcomes
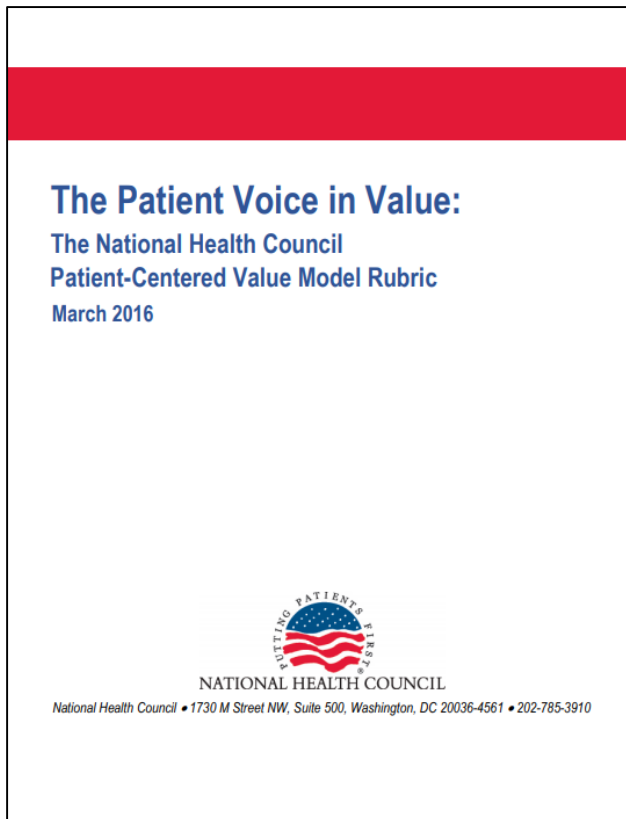
Patient perspectives and patient-centered evidence needs to be incorporated into decision-making

Tiered formularies

Non-medical switching

Step therapy

Prior authorizations

Formulary exclusions

Co-pay accumulators

Tools and Resources

Value Model Rubric

The Patient Voice in Value:

The National Health Council
Patient-Centered Value Model Rubric

March 2016

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National Health Council • 1730 M Street NW, Suite 500, Washington, DC 20036-4561 • 202-785-3910
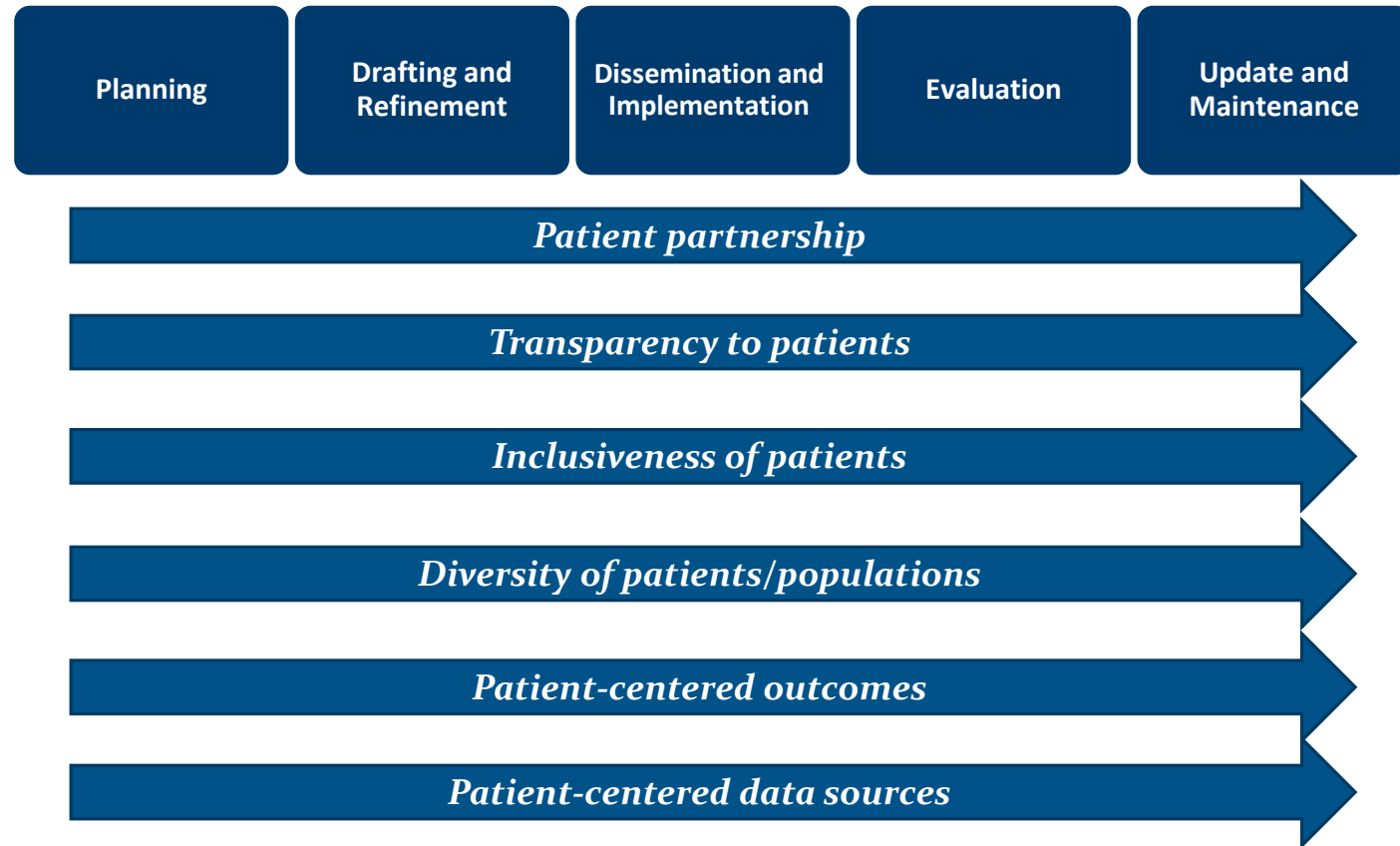
	Characteristics of Meaningful Patient Engagement in Model Development	Characteristics of Patient Centeredness in Model Development
	High ← → Low	High ← → Low
Patient Partnership **Transparency** **Inclusiveness** **Diversity** **Outcomes** **Data Sources**	▪ Direct relationships and partnerships are **bidirectional**, **reciprocal**, and **continuous**. ▪ Communications are open, honest, and clear. Engagement goals, participants, methods, desired impacts, and actual impacts are clearly outlined and transparent.	▪ Created by engaging, informing, and actively listening to people with chronic conditions. ▪ Respectful of and responsive to patient preferences, needs, and values in context of their own social worlds.

Domains of a patient-centered value model

Planning	Drafting and Refinement	Dissemination and Implementation	Evaluation	Update and Maintenance

Patient partnership

Transparency to patients

Inclusiveness of patients

Diversity of patients/populations

Patient-centered outcomes

Patient-centered data sources

Rubric Notes

- Groups support the intent behind value frameworks
- Do not wish to waste resources on unnecessary treatments
- But, patients want information to make informed decisions on the basis of what is or is not <u>valuable to them</u>
- Rubric is intended to assist all stakeholders and support developers in conceptualizing plans for meaningfully engaging patients
- Rubric should be considered a guide for patient-centeredness good practices
- Living document, to be refined over time on the basis of feedback from patient, patient group, framework developer, and others

NHC Get Ready Checklist

The National Health Council:

Value Framework Get-Ready Checklist
For Patient Organizations
September 2016

NATIONAL HEALTH COUNCIL

National Health Council ● 1730 M Street NW, Suite 500, Washington, DC 20036-4561 ● 202-785-3910

Companion piece to NHC's Patient-Centered Value Model Rubric

Get-Ready Activity	✔
Step 1. Advance Preparation – As Early as Possible	
Know which organizations are developing a framework for your disease of interest (or similar diseases); it will help you to be better prepared. These organizations should come to regard you as a partner in this process, but some may still need to be convinced of the value of your input. Being prepared in advance will help you demonstrate that value. The steps below help you to begin to prepare.	
A. Search: Who are the framework developers working in your area of interest?	
o Find the organizations known for developing frameworks. Right now, those organizations include ICER, ASCO, NCCN, and MSK. They are the most well known and talked about.	
o Network with other patient organizations and relevant health professional associations to learn from their past experiences with developers and to find out if new framework-development organizations are appearing on the scene.	
o Some health insurance plans and pharmaceutical companies also develop economic models. Find out about them in the published literature, in the press, or through conversations with other patient organizations or organizations representing providers who treat your condition of interest.	
o Do your homework to identify and learn about these organizations; gather and share information among patient groups as you learn more.	
o Look at developers' websites. Is your disease or a relevant treatment listed? Has the organization developed a framework or model in the past that is relevant to your area of interest? Is it going to update that work? Is it planning to develop a new framework or model?	
o Sign up to receive alerts and newsletters so you know what work is planned for the future. Some organizations put out lists of projected topics for the coming year.	
B. Assess: Understand the processes of each framework-development organization working in your area of interest.	
o Does this organization's existing process already seek patient input? Are there clear opportunities for engagement? Look for policies, calls for comment, and calls for nominations. You don't have to be limited to just those opportunities, but you should be aware of what they are.	
o What does their announced or typical timeline look like? Are your opportunities to engage clearly outlined? Track those dates and prepare for them. If no dates are published, use the schedule of released frameworks to help you develop a work plan for meeting deadlines.	
o Who is the target audience for their frameworks? Payers? Physicians? This is often stated outright. Knowing this can help you to understand and, if necessary, refute approaches used or assumptions made.	

A step-by-step guide for patient advocacy organizations to engage in the development, implementation, and assessment of value frameworks.

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Step 1. Advance Preparation

Search: Who are the framework developers working in your area of interest?

Assess: Understand the processes of each framework-development organization working in your area of interest.

Learn: Familiarize yourself with the developer's website, materials, and past value-related work.

Investigate: What is the FDA review timeline for products in your disease area, and how will it impact the timeline for value framework development?

D. Investigate: What is the FDA review timeline for products in your disease area, and how will it impact the timeline for value framework development?	
○ Is a new treatment for your disease of interest under FDA review? Some model developers focus on new products and anticipate economic model inputs based on FDA approval.	
○ When is the new treatment(s) expected to be approved? These dates can be critical to economic model release as the price of the product, which will be incorporated, may not be available until the product is launched. The product launch will be predicated on the FDA review timeline. Some economic models are developed prior to approval, but many will rely on product pricing at launch.	

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Step 2. Decide Whether to Engage

Articulate: Everyone involved should understand the issues.

Evaluate: Internally evaluate your organization's appetite for engagement.

Get-Ready Activity	✔
Step 2. Decide Whether to Engage	
Engaging in value framework and economic model development, and value assessment processes can be resource intensive and time consuming. Your organization should carefully make a decision about whether to engage and to what extent, guided by need, urgency, resources, and capacity. If you choose not to engage, the consequences should be carefully considered.	
A. Articulate: Everyone involved should understand the issues.	
o Create a short paragraph on why this value work is important to your organization. Why should you engage?	
o Vet this paragraph with colleagues, your organization's Board of Directors, medical board, external research and clinical experts, and others who have relevant experience. As applicable, gain their feedback.	
o Define the resources your organization will commit to this activity and how frequently you will revisit this commitment.	
o Communicate to your whole organization – staff, leadership and constituents – that you are taking this process seriously and want patients to be involved. Use various communications channels to state this such as your website, blog, e-news, print publication and social media. These channels can announce specific ways patients can participate.	
B. Evaluate: Internally evaluate your organization's appetite for engagement.	
o What are the overall goals of your organization? How do your organization's goals align with this issue? Would you consider engaging on value frameworks your role?	
o What resources do you need to engage? What resources do you have?	
o Do you have the capacity to take this on? Do you need outside help? Outside help may include external research and clinical experts. Who else will be on the team? What does it take to get them up to speed?	
o Do you want to engage with this developer at this time, on this topic?	

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Step 3. Consider Coalitions and Partnerships

Mobilize: Bring together relevant organizations and stakeholders

Get-Ready Activity	✔
Step 3. Consider Coalitions and Partnerships When possible, engaging with key organizations and stakeholders can make this process easier and more impactful.	
A. Mobilize: Bring together relevant organizations and stakeholders	
○ Reach out to provider groups, other patient groups, and other stakeholders in your field as well as other groups in similar disease areas for their input.	
○ Look for their comment letters, press releases, media quotes, and any articles.	
○ A united voice of like-minded individuals and groups is more powerful than a lone voice. You can also work in a collaborative fashion to stress different things in a complementary approach.	
○ Develop strategies for how you might work together.	

Step 4. Refine Your Strategy

Assemble: Your strategy is best substantiated with data and facts.

Gather: Information from your community is a key asset.

Search: Fill gaps in data where you are able.

Articulate: State your intentions clearly.

Re-evaluate

Message: Have your message ready to deliver.

Prepare: Be prepared for some negativity toward patient groups.

A. Assemble: Your strategy is best substantiated with data and facts.
o What do you have in-house that can inform a framework, model or assessment? Gather and organize the facts and figures you already have at your organization; keep them up-to-date and accessible. Data may not always be scientifically rigorous and results should not be misrepresented or exaggerated, simply factual.
o Sources of data should be inclusive of patient experiences and narratives. This may include patient stories and experiences as well as survey or registry data, both published and unpublished.
B. Gather: Information from your community is a key asset.
o For data you do not yet have on hand, gather insights and information from your patient community.
o Do you know your community's opinions, preferences, experiences, and views?
o Conduct interviews, focus groups, and surveys to gather the data that can inform the framework.
C. Search: Fill gaps in data where you are able.
o Your patient registry may have valuable data that can inform a framework or model. Tap that resource.
o Use external sources such as literature and web searches for other useful data.
o An FDA patient-focused drug development meeting and "Voice of the Patient" report may provide valuable data. Check the FDA website for those resources.
D. Consult: Experts can help you organize and understand the data.
o Tap your organization's scientific committee early so they are alerted and ready when you need them.
o Consult with outside economic experts who may be familiar with your disease of interest.
o If you do not fully understand something, ask for help or obtain training.

Step 5. Engage with Developers

Approaching a developer: Have a game plan mapped out.

Objective(s): Define your objectives for the conversation.

Communication with Developers

A. Approaching a developer: Have a game plan mapped out.
o Reach out to the contact you have identified at the developer organization(s) with a letter of introduction describing your organization and its mission. Send the letter by email and mail. Or, reach out to a contact person you may have met or have already contacted for information.
o Explain that you would like to introduce yourself and state your objective(s) (e.g., you want to become more engaged in their framework development activities).
o Outline briefly your expertise and what you have to offer (e.g., knowledgeable individuals in your membership about the disease of interest, a list of the leading clinicians in the country with expertise about the disease, or a disease registry of patient-reported outcome data).
o Stress the uniqueness of the disease or issues the developer might not be familiar with that you want them to know about as they begin their review. Emphasize things like heterogeneity of the disease, that all patients don't experience the disease the same way, don't respond the same way to treatments, etc. Stress that medical literature can't tell them everything they need to know about your community, its needs, and treatment hopes.
o Follow up to arrange an appointment to speak. Request a one-hour call or meeting, but settle for a shorter time if one hour is not available in the reasonable future. You may need to call several times to make the appointment. Don't be discouraged.
o Take every opportunity possible to introduce yourself and your organization to the staff at the development organization. Introduce yourself at public meetings and express your desire to partner.

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Step 6. Follow Through

Follow-up: Demonstrate your continued commitment.

Visibility: Use every opportunity to be viewed as an important stakeholder

Communication: Keep your constituents informed.

Reliability: Commit for the long haul.

A. Follow-up: Demonstrate your continued commitment.
o Send a note of thanks that includes important points from the conversation and the next steps committed to by both parties.
o Continue to send letters or emails to the organization with information, encouragement when appropriate, and to repeat your asks as needed.
o Prepare well-thought-out comments when there are public comment periods.

B. Visibility: Use every opportunity to be viewed as an important stakeholder.
o Make note of every publicly available comment period and opportunities for engagement with value framework developers.
o Plan your schedule accordingly and plan to attend all meetings in person to strengthen your presence.
o Speak to the media to convey your message. But do this as planned and on your terms.
o Advocate when and where needed.

C. Communication: Keep your constituents informed.
o Keep your members, stakeholders, and other constituents informed along the way.
o Inform and active your patient community as needed, especially when you need volunteers.

D. Reliability: Commit for the long haul.
o Dedicate the time and resources to fulfilling any commitments you have made to the developer (e.g., serving on a committee, writing a review, providing data, etc.)

NHC Value Work Group

- Representatives from NHC patient-group members and non-member patient organizations by invitation
- Meets monthly by teleconference to share and learn from each other's experiences, and to stay up-to-date on topics related to value assessment
- Upcoming topics:
  - Communications on Value and Value Assessment
  - Multi-criteria decision analysis

A Dialogue on Patient-Centered Value Assessment

- Articulate a shared vision for what marks success in enhanced patient centricity in VA and

- Outline tangible, feasible actions toward achieving that success. The actions may be on the part of patient groups, VA developers, or both in collaboration

Patient groups	Value assessors	In collaboration
• Add health economists to their scientific advisory boards, just as clinicians are currently included on these boards.	• VA bodies can develop a VA-report section describing how patient input guided VA decision-making. Rationale for why patient input was not used in a report should also be described to help improve data in the future.	• Following an appraisal, the VA body and patient group can debrief on how submitted data were useful and not useful, and how data collection or presentation can be improved for the future.

Health Economics & Value Assessment Education

- Increase patient-community capacity to engage on value, especially regarding value frameworks and assessments, as well as other value-related research, programs, and initiatives.

CHAPTER 1
- Role of health economics
- Introduction to basic terms

CHAPTER 2
- The Mysterious QALY
- Budget-Impact Analysis

CHAPTER 3
- Diving into Value Frameworks
- What patients and patient groups need to know about Value Frameworks
- The NHC Get Ready Checklist

CHAPTER 4 (under development)
- Heterogeneity of Treatment Effect
- Sensitivity Analysis in Value Assessment

Upcoming NHC Tools and Resources

Glossary of Value Assessment Terms

Budget impact analysis: When a new product becomes available, an organization, like a health plan or hospital, will want to estimate the impact purchasing that new product will have on its budget. The estimate of that impact will need to consider the costs for specific treatments with usual care today versus an estimate of the cost of treatments with the new treatment added. Thestimates consider how many people will use each of the treatments available and the costs for thiose treatments.[1,2] See the NHC's module: Budget Impact Analysis

Burden of Illness: The combined costs, in dollars and other impacts, as the result of a disease. This includes pain, lost wages, caretaker costs, mental health effects and more.[3] Studies that measure the "burden" of illness aim to examine things like the cost to the individual patient, possibly the cost to the employer, payer or insurance company, or even the cost to society.[4] (Also known as "burden of disease")

Comparative effectiveness research (CER): CER compares the effectiveness of two or more interventions or approaches to health care, examining their risks and benefits. Comparing two or more interventions distinguishes CER from other types of clinical research, for example research where one treatment is compared to a placebo.[5] See the NHC's module Getting to Know the Lingo

Cost Effectiveness: In the context of pharmacoeconomics, cost effectiveness is studied by looking at the results of different interventions by measuring a single outcome, usually in units (for example, life-years gained, deaths avoided, heart attacks avoided, or cases detected). Alternative interventions are then compared in terms of cost per unit of effectiveness in order to assess how it provides value for money. This economic evaluation helps decision-makers to determine where to allocate limited healthcare resources. Cost effectiveness, however, is only one of a number of criteria that should be used to determine whether or not interventions are made available. Other issues, such as equity, needs, and priorities should also be part of the decision-making process.[6] See the NHC's module Unlocking the Mysteries of the Quality-Adjusted Life Year (QALY) and Getting to Know the Lingo

- Glossary of Value Assessment Terms
- Finalized Fall 2019

Upcoming NHC Tools and Resources

PICOTS Framework		
What is this?		
Population **I**ntervention **C**omparator(s) **O**utcome(s) **T**iming **S**etting		
Where is this information in a value assessment report?		
Information may be found in: • If an "analytical framework" is presented, it will likely include the outcomes being evaluated • A section on the evidence base • Value assessment may not specifically describe a PICOTS framework, but should describe the individual elements		
Relevant Modules or Resources		
• The Patient Voice in Value: The NHC Patient-Centered Value Model Rubric • NHC Clinical Outcome Assessment Series		
Considerations	**✓**	**Notes**
• Does the PICOTS framework seem reasonable? Were members of the patient community encouraged to contribute to the PICOTS framework?		
• Are all elements of the PICOTS framework described?		

• Are important subpopulations included? Are they analyzed separately as needed? Subpopulations may be defined by:
     o Age
     o Comorbid conditions
     o Etiology
     o Ethnicity
     o Gender
     o Genetics
     o Geographic location
     o Health literacy
     o Insurance coverage
     o Rural, suburban, urban location
     o Race
     o Socioeconomic status
     o Severity		

• Are the outcomes integrated into the value model should include those that patients have identified as important and consistent with goals, aspirations, and experiences?		
• Does the data used in the report reflect your community's opinions, preferences, experiences, and views?		
• Is data that you submitted incorporated? If not, is justification for why it was not incorporated provided?		

- The NHC Considerations Guide for Patient Organizations Developing Comments on Value Assessment
- Finalized Fall 2019

Upcoming NHC Tools and Resources

Pearls of Wisdom: Ways to Make An Impact
Beyond Public Comments

Examples:

- Engaging Health Economic Outcomes Researchers to assist with patient organization engagement

- Provide suggestions on voting questions

  - Many communities have participated meaningfully in the Roundtable meetings but then the Roundtable panel didn't know what to do with the information because questions were set prior to the meeting
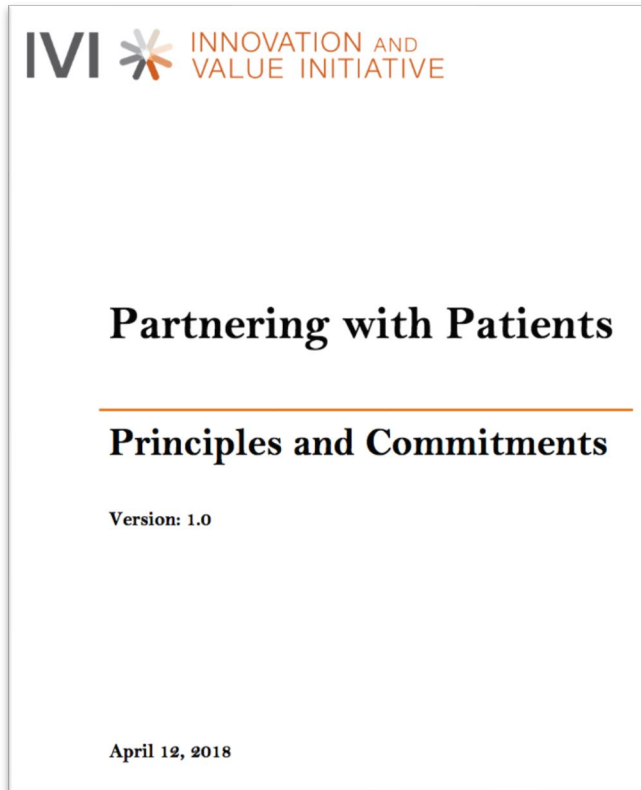
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ICER Patient Participation Guide

•In this guide:
  • What is ICER?
  • What does ICER do?
  • How you can participate in ICER's process

  https://icer-review.org/patient-participation-guide/

Topic and Timeline Announced Publicly

Give Open Input on a New Topic (Weeks 1-3)

A Draft Scoping Document is developed and posted.

Comment on the Draft Scoping Document (Weeks 4-6)

ICER reviews comments and posts a Revised Scoping Document.

A Draft Evidence Report and Draft Voting Questions are posted.

Comment on the Draft Evidence Report (Weeks 22-25)

A revised Evidence Report and Revised Voting Questions that include updates based on public comments are posted to the website.

Attend a Public Meeting (Week 30)

ICER's Evidence Report is publicly deliberated by one of three evidence review groups: CTAF, the Midwest CEPAC, or the New England CEPAC.

Final Evidence Report and Meeting Summary posted.

IVI Partnering with Patients Principles and Commitments

INNOVATION AND VALUE INITIATIVE

Partnering with Patients

Principles and Commitments

Version: 1.0

April 12, 2018

Principle 1: IVI will involve multiple patient stakeholders in value assessment.

Principle 2: IVI will employ robust methods to identify and involve patient stakeholders.

Principle 3: IVI will partner with patient stakeholders at all stages of research.

Principle 4: IVI will continuously evaluate and refine patient partnership action.

Principle 5: IVI will commit resources for continuous patient engagement.

European Patients' Academy (EUPATI) HTA resources

Experiences of patient advocates in HTA processes along with some questions and challenges with such involvement and work.

→ Patient experiences abroad

Guidance for patient involvement in HTA

8 Suggested patient involvement activities

    8.1 General HTA process

    8.2 Outreach and education

    8.3 Wider involvement

    8.4 For individual HTAs

    8.5 Identifying and prioritising which technologies to assess.

    8.6 Scoping (developing a framework for an individual HTA)

    8.7 Assessing and developing recommendations/guidelines

    8.8 Reviewing and disseminating HTA outcomes

https://www.eupati.eu/webinar/webinar-experience-patient-involvement-hta-processes/
https://www.eupati.eu/health-technology-assessment/guidance-for-patient-involvement-in-hta/

HTAi Ethical Considerations for Patient Groups Collecting Information

Issue	Consider
1. Need for activity	○ Do you already have information that can answer the HTA submission questions? ○ Have you found a gap in the available information? Does this gap mean you need to collect new information? ○ Have you planned and tested the way you will collect the information to make sure it meets your needs?
2. Inclusivity	Have you taken steps to reach out to as broad a population (including vulnerable groups) as feasible?
3. Informed consent	Is each person who is asked to take part competent to consent? If yes, have they been told: ○ how the information being collected will be used and shared? ○ who is collecting the information? ○ that they can refuse to take part, stop taking part at any time, or choose not to answer all the questions without this being held against them? ○ any perceived or potential conflicts of interest of the person(s) or group collecting the information? ○ what is involved in taking part (how much time, what will be discussed, possible use of their actual words or stories in the submission)? ○ the realistic potential benefits? ○ the risks or potential harm of taking part (such as distressing thoughts, sense of stigma)? ○ That they will not be able to be identified from the submission?

Issue	Consider

4. Ensuring anonymity and confidentiality	Have you put in place a process that makes sure that people taking part:
	
	 ○ cannot be identified in the submission, such as:
	    o not using the real names of those taking part
	    o using initials, letters or numbers
	    o not collecting any identifying information?
	
	 ○ are told the outcome of the HTA in a way that does not reveal to others that they took part?
	
	 Do the people taking part understand:
	
	 ○ the guarantees given about concealing their identity?
	
	 ○ how their information will be stored and kept safe?

5. Data protection and privacy	○ Does your patient group have a data protection policy you need to follow?
	
	 ○ Does your region/country have a data protection or privacy policy you need to follow?
	
	 ○ Have you informed the people collecting the information that:
	    o responses must be stored securely
	    o they must not discuss or report responses in a way that would allow someone to be identified?
	
	 ○ Have you locked the data you collected and reported in a drawer or password protected it?
	
	 ○ Have you backed up the data you collected and reported?

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Panel Discussion

Value Assessment Experiences

Moderator: Elisabeth Oehrlein, PhD, Senior Director, Research and Programs, National Health Council

Panelists:

- Catherine Davis Ahmed, MBA, VP, Policy and Outreach, The FH Foundation
- Annie Kennedy, Senior Vice President, Legislation & Public Policy, Parent Project Muscular Dystrophy
- Ashley Valentine, Co-Founder and President, Sick Cells