Moving Value Frameworks from Fledgling to Functional

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Overview

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- Purpose
- Methodology
- Summary of findings
- Limitations
- Toward improvement

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Background

- Five US value assessment frameworks
  - American College of Cardiology and American Heart Association (ACC-AHA)
  - American Society of Clinical Oncology (ASCO)
  - Memorial Sloan Kettering Cancer Center – DrugAbacus
  - Institute for Clinical and Economic Review (ICER)
  - National Comprehensive Cancer Network (NCCN)

Aligning with NPC's Guiding Practices

- These value assessment frameworks were underway prior to publication of NPC's Guiding Practices (GPs)
- As such, these frameworks were not designed to align with NPC's GPs
- Even so, various guidelines and best practices have long been available in such related areas as systematic reviews, evidence appraisal, health economic methods, and HTAs
- Some of these guidelines and best practices are also reflected in NPC's GPs

Works-in-Progress

- Developers of all of these frameworks characterize them as works-in-progress, evolving, and responsive to external feedback
- Even so, the ICER framework, NCCN Evidence Blocks, and DrugAbacus are operational and their findings are publicly available and cited by decision-makers

Purpose

1. Evaluate how the five major value assessment frameworks align with NPC's guiding practices (GPs) for patient-centered value assessment and to compare and contrast these frameworks across the GPs
2. Continue to guide the field in ensuring that value assessment frameworks meet a set of standards/good practices that helps to ensure that these frameworks support patient care and outcome

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Methodology

- Staff gathered available literature on the five frameworks
- Two reviewers independently rated each framework against NPC's 28 GPs, plus the 7 GPs for budget impact assessment for the one framework that conducts those, and provided rationale for each determination
- Senior staff member reviewed the two sets of ratings and discussed discrepancies with reviewers to reach consensus
- Draft findings were shared with representatives of each framework organizations with requests for suggested clarifications and other input
- Lewin also interviewed experts in the field who represent various stakeholders

Methodology (continued)

- Evaluation Determination Categories

Category	Symbol	Description
Fully met	●	The framework meets all components of NPC's guiding practice
Partially met	◐	The framework meets some component of NPC's guiding practice, but there are other components that are unknown or not met (include details in Rationale column)
Not met	○	Available information suggests that the framework does not meet the guiding practice
Cannot be determined	∅	Applies to the following scenarios: • The framework does not provide information related to this Guiding Practice • A component of the framework or assessment methodology is still under development
Not applicable	NA	The framework is not structured in a way that applies to the guiding practice (e.g., when a framework does not perform a budget impact analysis)

Summary of Findings: Assessment Process

	Guiding Practice	ACC-AHA	ASCO	Drug Abacus	ICER	NCCN
GP1	Proposed assessment topic, process and timelines should be announced in advance to enable stakeholder participation and feedback.	∅	∅	○	●	●
GP2	Interested stakeholders should be involved in the assessment process to represent all perspectives.	∅	∅	◐	●	◐
GP3	The scope of an assessment should be defined a priori and incorporate stakeholder input.	∅	∅	○	●	◐
GP4	Public comment periods should be included, with sufficient time to review materials and submit comments, and with transparency around how comments are addressed by the convening body.	∅	◐	○	◐	◐
GP5	Assessments should be regularly reviewed and updated to keep pace with and account for medical innovation. There should be a continuous open process for stakeholders to request a timely review of an assessment to account for new technology or other changes in the evidence base.	∅	∅	∅	○	●
GP6	Sufficient time, staff and resources should be dedicated to support a thorough and robust assessment process.	∅	∅	◐	●	●

Summary of Findings: Methodology

	Guiding Practice	ACC-AHA	ASCO	Drug Abacus	ICER	NCCN
GP7	Value assessments should focus broadly on all aspects of the health care system, not just on medications.	○	○	○	◐	○
GP8	Methods should be based on established health economic methodologies, consistent with accepted standards.	∅	○	○	◐	○
GP9	Methods, models, and assumptions should be transparent and assessment results should be reproducible.	⊗	●	◐	◐	◐
GP10	Base case assumptions must represent reality.	∅	◐	∅	◐	◐
GP11	Sensitivity analyses should be performed, taking into account input from external stakeholders. Where sensitivity analyses result in material changes to the interpretation of the result, a focused discussion should be included.	∅	○	○	●	○
GP12	Weights should be included to accommodate varying user preferences.	○	○	●	○	◐

Summary of Findings: Benefits

	Guiding Practice	ACC-AHA	ASCO	Drug Abacus	ICER	NCCN
GP13	The measurement of value should include a broad array of factors that are important to patients and society.	∅	◐	◐	●	◐
GP14	Clinical benefits and harms should be incorporated in a manner that recognizes the heterogeneity of treatment effect rather than the average response.	∅	○	○	●	○
GP15	The time horizon for value should be long-term, ideally lifetime.	∅	◐	●	●	●

Summary of Findings: Costs

	Guiding Practice	ACC-AHA	ASCO	Drug Abacus	ICER	NCCN
GP16	All health care costs and cost offsets should be included.	◐	○	○	●	○
GP17	The time horizon for costs should be long enough to incorporate the benefits of the treatment and the lower costs of medications when they become generic.	∅	∅	○	●	○
GP18	Costs should be representative of the net price most relevant to the user.	∅	○	○	○	○
GP19	Thresholds should be developed in a transparent manner, may vary by population and disease, and should undergo a multi-stakeholder evaluation process.	◐	NA	NA	◐	NA

Summary of Findings: Evidence

	Guiding Practice	ACC-AHA	ASCO	Drug Abacus	ICER	NCCN
GP20	Evidence should be identified in a systematic, transparent and robust manner.	●	○	◐	●	●
GP21	Stakeholders should be given the opportunity to submit relevant evidence, such as clinical trial and real-world evidence beyond the published literature.	○	○	◐	●	●
GP22	Best available evidence should be used for the assessment.	●	○	◐	●	●
GP23	Accepted methods should be used to assess quality of evidence, certainty of evidence and conflicting evidence.	●	∅	○	●	◐
GP24	Where evidence synthesis is warranted, formal analysis should be conducted, in accordance with accepted methodologies.	●	∅	○	●	◐
GP25	Subjective evidence should be used minimally, if at all, and its inclusion should be clearly labeled.	●	●	◐	●	◐

Summary of Findings: Dissemination and Utilization

	Guiding Practice	ACC-AHA	ASCO	Drug Abacus	ICER	NCCN
GP26	Assessment results should be presented in a manner that is simple for the user to interpret and apply.	∅	◐	◐	●	◐
GP27	Value assessment should clearly state the intended use and audience to avoid misuse.	●	●	◐	●	●
GP28	Press releases should only be issued for final assessments, include limitations of the assessment, and highlight areas where sensitivity analyses result in material changes to the interpretation of the results.	∅	∅	NA	○	●

Summary of Findings: Budget Impact Assessment

	Budget Impact Assessment Guiding Practice	ICER
BIA1	Budget impact assessments should examine all aspects of the health care system, not just medications.	●
BIA2	Budget impact assessments should be separate from value assessments.	◐
BIA3	Budget impact assessments should include time frames that are long enough to incorporate the benefits of the innovation and the lower costs of medications when they become generic.	◐
BIA4	Budget impact assessments should include realistic estimates regarding the uptake rate. Stakeholders may have done extensive assessments of potential uptake and should be given the opportunity to submit their results. A sensitivity analysis of different uptake rates should be conducted.	○
BIA5	Budget impact assessments should acknowledge the considerable uncertainty in the inputs by incorporating sensitivity analyses and reporting ranges around estimates.	●
BIA6	A BIA is simply an assessment of budget impact, and should not be judged against artificial affordability caps.	○
BIA7	Assessments of ways to address budget impact concerns should include all relevant stakeholders and consider all approaches.	◐

Limitations

- Meeting checklist criteria is necessary, but not sufficient
- Checklists can tell you whether something was done, but not how it was done or how well it was done
- More nuanced analysis is needed to determine quality
- This study represents a snapshot in time; all of the frameworks are works-in-progress that are continuing to evolve

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Toward Improvement

- Comparison of the processes, methods, and other attributes to NPC's GPs provides opportunities to highlight important directions for improvement
- Among such opportunities …

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Intended Audiences

- These frameworks were designed for different purposes and target audiences; for the most part, they are directed to:
  - clinicians and patients (ACC-AHA, ASCO, NCCN)
  - policymakers, payers, industry (DrugAbacus, ICER, ACC-AHA)
- However, regardless of a framework's primary target audiences, their value-based decisions will affect other stakeholders
- This should influence stakeholder engagement, problem formulation, presentation of results, anticipated impacts

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Transparency

- Limitations in transparency can diminish the credibility and utility of value frameworks
- How deep transparency? Executable models and associated computer code?
- Value frameworks have distinct opportunities to improve their transparency
- Related fields have robust, evolved process documentation that supports transparency (e.g., USPSTF, Cochrane, AHRQ EPCs, NICE manuals/handbooks)

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Stakeholder Input & Feedback

- Clear, timely and responsive provisions for stakeholder input and feedback are recognized globally as standard attributes of publicly accountable HTA, other programs
- Wide variation among frameworks in provisions for input and feedback
- Beyond making provisions for stakeholder input, it is necessary to demonstrate responsiveness
- While it may not be necessary to itemize disposition of each stakeholder comment, frameworks' credibility will be affected by their record of responsiveness

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Patient Involvement

- All of the frameworks express commitment to patients
- Although patient perspective is not primary for some frameworks, patients are ultimately affected by value-based decisions of other stakeholders
- Regardless of primary target audience … Put patients at the table from the start
- In initial scoping, gain patient input on pops./subgroups, interventions, comparators, outcomes, and costs of interest
- Not just one … Recognize diversity of patient communities, even within a particular disease/condition

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Expert Involvement

- The quality and credibility of value assessments depends in part on types and extent of expert involvement
- As frameworks pursue advanced methods to evaluate clinical, epidemiological, and economic evidence and conduct extensive economic modeling …
- They should continually revisit their mix of expertise and the ways in which internal and external experts are involved

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Types of Interventions

- Among these frameworks, the majority focus is on drugs and biologics
- There are various reasons why; however …
- An array of value assessments lacking in diagnostics, devices, surgical procedures, and programmatic interventions will bias the basis of informed health care decision-making

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Evidence Sources & Quality

- Disparity in evidence sources across the frameworks …
- Range from reliance on single RCTs to systematic reviews of RCTs, other clinical trials, obs. studies, conference abstracts, regulatory review dossiers, and more
- Selection of cost data often of limited relevance to user
- Evidence search protocols should be fully transparent regarding, their methods, sources, and criteria for evidence selection
- Frameworks should rate the quality of the evidence in a transparent manner using standard, accepted methods
- Address impact of evidence scope/limits on findings

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Costs, Other Economic Aspects

- Wide variation among the value frameworks with respect to cost analyses; among issues …
- Use of cost-effectiveness (CE) thresholds
- Two of the frameworks use CE thresholds with similar ranges … more likely to be used as rough benchmarks and incorporated into policies and decisions
- Attention to roles, standards for CE thresholds, e.g.,
  - social, economic, ethical basis
  - flexibility for certain pop. groups, diseases; early-stage leaps in success (big innovation)
  - who sets them

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User Preference Entry

- Value frameworks vary widely in enabling user input
- Frameworks should enable entry of user preferences where feasible
- There is intra- as well as inter-stakeholder variation in preferences for value parameters
- Enabled by advances, options in interactive technology

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Potential Misinterpretation, Misuse

- Having multiple frameworks address the same or similar topics from different stakeholder perspectives or using alternative methodologies can be informative; however …
- Frameworks with contrasting results can confuse users
- Stakeholders that are unable, or do not choose, to discern intended uses and underlying assumptions of frameworks may misinterpret or misapply their results
- Framework developers cannot be responsible for all uninformed or biased uses of their assessments; however …
- They should make concerted efforts to ensure that their work is transparent and comprehensible, and minimize or correct misinterpretation or misuse of their findings

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Here to Stay

- Value frameworks' prominence follows decades of evolution of HTA, PE, OR, CER, etc.
- They respond to increasing national and global demand for evidence and analyses of health and economic impacts of health care interventions
- These and other frameworks continue to evolve
- Stakeholders will continue to push for improvements
- By whatever name, assessment of value is here to stay

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Here to Stay (2)

- All of these frameworks will benefit from alignment with—and efforts to advance—good practices for transparency, stakeholder engagement, methodological rigor, etc.
- NPC's GPs and related guidelines/best practices should evolve to reflect and advance state of the art for meeting user needs and serving wider stakeholders

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Main To-Dos

- Patient involvement from the start
- Implications beyond primary target audiences
- Push transparency
- Stakeholder input and responsiveness
- Sourcing evidence to address the assessment
- Roles and standards for cost-effectiveness thresholds
- Roles and standards for budget impact analysis
- Enable user preference entry
- Toward unified methods?

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