

GUIDING PRACTICES FOR PATIENT-CENTERED VALUE ASSESSMENT

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Background

The National Pharmaceutical Council (NPC) first published guiding practices for value assessment in 2016. That was a time when multiple organizations had created unique value assessment frameworks, each for different purposes.

Since then, the US experience with value assessment has changed substantially, with activities increasingly focused on the value of pharmaceuticals. At the time of publication, the Institute for Clinical and Economic Review (ICER) now issues 6-8 assessments per year, and the Centers for Medicare and Medicaid Services' Drug Price Negotiation Program under the Inflation Reduction Act appears to some observers as a form of value assessment, even though (as of this writing) neither the CMS guidance nor statements from agency officials explain whether the government's goal is to lower drug prices, reduce patient out-of-pocket costs, or get more value for the government's money. In addition, the research methods supporting value assessment – particularly around the inclusion of the patient voice in these evaluations – have continued to evolve.

These have all factored into this update of NPC's guiding practices for US value assessment to help inform the growing importance of this tool to support pricing pharmaceuticals based on the value they provide to patients and society as a whole.

Value encompasses the balance of benefits and costs experienced by patients, their caregivers and families, and society over time. Artificially setting drug prices too low fails to account for the value they provide to patients, caregivers, healthcare systems, and society as a whole.

Value assessment comprises both elements that are science and elements that are judgment, or opinion. With this complexity, there is no single answer to a value assessment and it should be viewed as a tool, not a rule to set prices. Nonetheless, high-quality, comprehensive value assessments can inform both payer coverage policies as well as the price that is negotiated between payers and manufacturers.

NPC's updated guiding practices are aimed at facilitating these market-based, individual payer-manufacturer value-driven negotiations to best account for the diverse needs of payers' enrolled populations, local treatment practices, and stakeholder preferences.

Key tenets of our guiding practices address the full spectrum of value assessment uses and activities:

- Value assessment is a multi-stakeholder deliberative process and value-based prices should not merely be the output of a cost-effectiveness model or based on a single threshold.
- The assessment process should be systematic, objective, and transparent and should involve affected stakeholders throughout the assessment process to represent all perspectives. And, with the rapid pace of medical innovation, assessments should be regularly reviewed and updated to keep pace.
- Scientific methods supporting value assessments should focus broadly on all aspects of patient care and healthcare systems, not just on medications. Methods, models, and assumptions should be transparent and follow scientific best practices so that assessment results will be reproducible.
- Benefits of innovation included in assessments should capture the broad array of benefits important to patients and society. Doing so requires engagement with multiple stakeholders, evidence types, and analytic tools should be incorporated in the assessment to capture all relevant dimensions of value.
- Costs of treatment, all healthcare costs, and cost offsets should be included in assessments. Time horizons for costs should be long enough to incorporate the benefits of the treatment and the lower costs of medications when they face branded and generic competition.
- The evidence used throughout assessments should be identified in a systematic, transparent, and robust manner. The best available evidence should be used for the assessment and the evidence should be appropriate for the outcome evaluated; clinical trials and real-world evidence should be evaluated.
- Finally, assessment results should be disseminated in a manner that is transparent and easy for users to interpret and apply. Communications around value assessments should clearly state the intended use and audience to avoid misuse.

Introduction

As our healthcare system continues to move from a volume-based system towards a value-based one, there is increasing interest in assessing value for all components of healthcare.

Value assessments in the US are commonly conducted through interactions between payers and manufacturers for most drugs on formularies as well as by third-party groups on selected pharmaceutical products each year. These assessments are one of many inputs to complex payer decisions on patient access to effective treatments through their coverage and reimbursement policies. As such, incorporating patient perspectives and maintaining patient-centricity throughout the assessment process is critical. Furthermore, assessment processes should not delay patient access to innovation.

This is a constantly evolving area, in part because current practices do not capture many dimensions of value and because the process requires analytic and social value judgments. It is, therefore, crucial that good practices are established and updated to guide meaningful value assessments and to encourage further improvements. We acknowledge other efforts in good practice recommendations, including the Professional Society for Health Economics and Outcomes Research (ISPOR)¹ and others. The National Pharmaceutical Council (NPC) published our first guiding practices document in 2016. Since that time, value assessment research methods have continued to improve. It is these developments and the growing experience with value assessment that have motivated NPC's current guiding practices for US value assessment which span a full range of topics including the assessment process, scientific methodology and measures, and dissemination and use.

Value encompasses the balance of benefits and costs experienced by patients, their caregivers and families, and society over time. There are a multitude of specific benefits that constitute “value,” from reduced mortality, improved patient functioning and quality of life, treatment burden, patient and caregiver productivity, outcome equity, and societal value of scientific innovation to name just a few.^{2,3} There is no single answer to a value assessment. Nor should we expect one; the importance of the various elements of value will vary by stakeholder, especially different patient populations and individual payers, self-insured employers, and pharmacy benefit managers (PBMs). In addition, there is often high variability in the quantity and quality of evidence and analytic methods used in assessments which introduce uncertainty and can reduce credibility. Establishing good practices to guide value assessments can help ensure they are effective tools to support value in patient care and outcomes, rather than flawed tools that impede access and innovation.

The price of pharmaceuticals should be based on the value they provide to patients, caregivers, healthcare systems, and society as a whole. Clearly, comprehensive value assessments can inform both payer coverage policies as well as the price that is negotiated between payers and manufacturers. Importantly, it is this market-based, individual payer-manufacturer value-driven negotiation approach that best accounts for heterogeneity in the unique needs of payers' enrolled populations, local treatment practices, and stakeholder preferences.^{4,5} That is, price should not merely be an output of a cost-effectiveness model or based on a single threshold.

Value assessment is a multi-disciplinary organizational process that incorporates scientific tools and evidence. Our thinking explicitly acknowledges these aspects of value assessment by providing best practices not only for the scientific methods that inform value assessment but also for the critical decision-making processes that will necessarily vary from organization to organization.

The following sections of this report describe NPC's guiding practices for patient-centered value assessment in terms of the assessment process, scientific methodology, benefits, costs, evidence, and dissemination and utilization.

Assessment Process

1. **The rules for selecting pharmaceuticals for assessment should be systematic, objective, and transparent.** Whether an entire class of products or individual drugs are selected for assessment, the rationale and process of selection should be made public to ensure transparency and credibility.
2. **Proposed assessment topics, processes, and timelines should be announced in advance to enable stakeholder participation and feedback.** Announcing assessment plans in advance provides interested stakeholders with ample opportunity to set aside needed resources to provide input into upcoming assessments. Stakeholder experience and resources to provide timely and evidence-based feedback must be considered by the value assessment process.
3. **Affected stakeholders should be involved throughout the assessment process to represent all perspectives.**^{6, 7, 8, 9} Gaining involvement from the manufacturer and other interested stakeholders at the initiation of and throughout the assessment process - not only at the release of a draft report, for example - ensures all perspectives are considered and provides the opportunity to fully inform the assessment. Provider, patient, and caregiver perspectives are especially important and should be included as early as the topic identification stage.
4. **The scope of an assessment should be defined a priori and incorporate stakeholder input.**^{10, 11, 12} A scoping document should be created prior to an assessment which explicitly outlines the methods and sources for the assessment and includes the end-goal of the assessment, making recommendations on coverage policies. The scoping document should be based on input from patients, manufacturers and other interested stakeholders. Their input on draft key questions and scope prior to beginning an assessment ensures all perspectives are considered, the planned scope is fully vetted, and key questions are refined where needed.

Assessment Process

5. **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.**^{13, 14} Public comments should be included for all key documents and decision points in an evaluation, including scoping documents, analytic protocols, and draft reports. Allowing sufficient time for interested stakeholders to review materials and prepare comments ensures that stakeholders can thoughtfully and comprehensively respond to the comment request. Presenting all comments and submitted evidence in assessment reports – as well as providing transparency around how comments are addressed – builds credibility and trust in the process.
6. **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 technology advancements or other changes in the evidence base.¹⁵ Changes in patient management and the supporting evidence base can cause an assessment to become outdated, and those outdated results could adversely impact patient care and outcomes. Having a regular review cycle, along with a process for requesting an updated review when indicated, can ensure assessment results remain current and provide the timeliest information to guide shared decision-making and patient care.
7. **Sufficient time, staff, infrastructure and resources should be dedicated to support a thorough and robust assessment process.** The evidence and analytic methods supporting value assessments are complex and require substantial scientific and clinical expertise to ensure high-quality, robust inputs to the assessment process. Conducting assessments without sufficient time, staff and resources leads to assessments of inferior quality which could adversely impact patient care and outcomes.

8. **Value assessments should focus broadly on all aspects of patient care and healthcare systems, not just on medications.**^{16, 17, 18} Within each system, focusing on one component of an interconnected system does not provide a complete perspective on benefits to patients. Medications are one component of the healthcare system. Focusing only on medications while excluding the rest of the healthcare system (e.g., patient education or disease management programs unique to the system, access to infusion centers for infused drugs, procedures, diagnostic tests, hospitalizations, office visits), will result in an incomplete assessment. In some cases, the value derived from improved treatment, including patient populations with developmental or cognitive disabilities, may be measured in decreased reliance on or utilization of social services and supports (e.g., assisted living paid for by state or city programs, and vocational support). Such condition- and population-specific dimensions of value should be included in assessments where appropriate.
9. **Methods, models, and assumptions should be transparent and assessment results should be reproducible.** To build credibility and trust in an assessment, the methods, models (including all calculations), and assumptions included in the assessment should be transparent to interested stakeholders,^{19, 20} and they should be able to reproduce the assessment results on their own. When assumptions about clinical and economic impacts are necessary, the rationale for and impact of these assumptions is critical. Further, credibility of modeling and any recommendations will be greatest if the models themselves are made publicly available.²¹
10. **Economic tools used to inform assessments should be based on established health economic methodologies, consistent with accepted standards.** Health economic assessment is a very complex and sophisticated undertaking and many bodies of work and years of debate have shaped the methods. Following well-accepted and fit-for-purpose methodological standards^{22, 23, 24} is necessary to produce a meaningful and credible economic assessment.
11. **If cost-effectiveness (CE) models are used in an assessment, model inputs and assumptions must represent reality as best possible, with deviations fully documented.**²⁵ CE models are intrinsically limited and complex analytic tools; these characteristics can mask their inability to capture the full value of medical innovations. Despite their shortcomings, CE models are a common input to value assessments. A conventional best practice in health economics is to use a “base case” to represent what is known about a product, such as the results from a clinical trial. However, the base case often does not capture what we need to know about a product, e.g., what value do patients and payers realize within a particular healthcare system. It is critical that the assumptions inherent in the base case are realistic and accurate. Multiple scenarios should be modeled to best inform assessments and to illustrate the range of values possible.

12. If incremental cost-effectiveness ratios (ICERs) – typically generated by cost-effectiveness models – are employed in an assessment, ICER thresholds should not be directly tied to a “value-based” price. Cost-effectiveness analyses are often used as part of comprehensive value assessments, but, when used, should be treated as a tool, not a rule. However, it must be clear that the ICERs a model produces cannot be interpreted as yielding a “value-based” price; the gold standard for design and conduct of cost-effectiveness modeling mentions “prices” only in that more research is required to use CE models in pricing.²⁶ That research has not been conducted and any value assessment based on CE models is incomplete at best.
13. Comparative Effectiveness Research (CER), including model design, should follow consensus guidance and be fit-for-purposes of value assessment.^{27, 28, 29} Principles of good CER adopt elements of high-quality research methods, including: clear statements of objectives; transparency of process, data and methods; engagement of stakeholders and analytic perspectives reflecting multiple stakeholders; use of comparators relevant to current clinical practice; evaluation of outcomes relevant to the stated objectives; and, explicit treatment of heterogeneity and uncertainty. Comparisons against products not indicated for the condition under consideration should be rare and only in cases where clinical experts and guidelines suggest the off-label use is the existing standard of care (SOC). To the extent that off-label use is SOC, this should be explicitly noted as a caveat to the findings.
14. Sensitivity analyses should be performed to explore the impact of uncertainty for any scientific tool used to inform the assessment. These analyses should include input from external stakeholders with a rationale provided for the ranges used in the analysis. Where sensitivity analyses result in material changes to the interpretation of the results or revisions to the model, a focused discussion should be included.^{30, 31} In the specific case of CE modeling and its limitations, sensitivity analyses around key uncertainties will identify how results could vary under differing assumptions and will generate a range of potential results. Note that sensitivity analyses should not be an obscure addendum for so-called sub-group analyses or contextual considerations that are actually most meaningful to patients and payers. The implications for the user may vary across this range, so clear guidance will be needed to help them understand which assumptions are driving the differences and why.

15. **Patient preferences regarding the benefits and risks of a product should be included in the assessment.** Patient preference information (PPI)³² has been used to inform many aspects of value assessment, such as endpoint selection, supplementing patient-reported outcomes, and defining what benefits are most important to patients.³³ Inclusion of patient preferences in CE models is still a developing area, with current efforts focused on formally incorporating PPI into model structures and assumptions.³⁴ However, MCDA methods have certain advantages over CE models as an alternative approach to incorporating patients' perspectives.³⁵ Models should allow users the flexibility to test differing assumptions around patient preferences. At a minimum this should include the ability to make weighted adjustments to accommodate varying user preferences, to adjust the assessment assumptions and parameters to accommodate individual preferences for different outcomes and factors (e.g., patient preferences for clinical benefit vs. side effects), and make adjustments to represent different scenarios (e.g., payer ability to vary the population).
16. **Thresholds for determining a price in an assessment cannot fully reflect the diversity of patient and payer values.**³⁶ However, if optimal ranges for certain measures will inform the assessment, these should be developed in a transparent manner, will vary by population and disease, and should undergo a multi-stakeholder evaluation process. Optimal ranges for benefit:risk assessments or incremental cost-effectiveness ratios should be developed and applied in a transparent manner³⁷ and subject to a multi-stakeholder evaluation process³⁸ reflecting societal values related to disease conditions and innovation. No single threshold can or should be universally applicable.

17. **The measurement of value should include a broad array of benefits that are important to patients and society.**^{39, 40, 41} Patients and society value a variety of factors such as survival, public health and infection control, quality of life, health equity, the ability to participate in daily activities, caregiver burden, worker productivity and presenteeism, short-term disability, unmet need for diseases with limited or no treatments, burden of disease, and innovation. Omitting these factors when relevant to a value assessment provides an incomplete picture of a treatment's value and may consequently limit access to care for vulnerable patient populations.
18. **Multiple stakeholders, evidence types, and analytic tools should be incorporated in the assessment to capture all relevant dimensions of value. No single perspective or piece of evidence can capture the totality of value to patients and payers.** Analytic tools used in assessments (e.g., CE models) are continually evolving to include new dimensions of benefit:risk;⁴² these methodological improvements take time to accumulate and test prior to general acceptance among the research community. Therefore, the scientific tools used to support value assessments are always lagging behind clinical science (e.g., durable gene therapies) and the value that society places on them.
19. **Clinical benefits and harms should be incorporated in a manner that accounts for health disparities and recognizes the heterogeneity of treatment effect rather than only reporting or communicating the average response.**^{43, 44} Patients respond to treatments differently. In addition, some patient groups experience disparities in health status and access to care. Explicitly addressing these heterogeneity and distributional issues can make the assessment more meaningful for the full spectrum of patients and to payers' decision-making.⁴⁵
20. **The time horizon for value should be long-term, ideally lifetime.**⁴⁶ Many of the benefits of treatments, such as avoided events (e.g., heart attacks), show up in the longer term. To capture the full value of a treatment, the time horizon for clinical and care value should be long enough to capture these benefits, ideally covering a patient's lifetime. Long-term outcomes data from clinical trials are often unavailable at product approval, particularly for rare pediatric conditions or approvals based on surrogate endpoints. Nonetheless, value assessments should, at a minimum, include scenarios that assume key outcomes are durable in the long-run rather than assuming that benefits cease based on the follow-up period in a registrational trial. Evidentiary standards should be appropriate for evaluating all benefits and risks to patients and may therefore differ from the standards for regulatory approval.

21. [All healthcare costs and cost offsets should be included from the stated perspective of the analysis.](#)⁴⁷ Both the healthcare system and the societal perspectives should be included in value assessments.⁴⁸ Treatments may have up-front costs that lead to long-term improvements in patient health. Those improvements may have “cost offsets,” or reductions in resource needs, such as reduced hospitalizations. By including both costs and cost offsets, the full value of a treatment can be assessed and capture the total costs of care. Only considering the treatment costs but not the potential cost offsets would lead to an incomplete assessment of value. Arbitrary limits on the amount of cost savings to be included in an assessment should not be used in an assessment as this, perversely, defends inefficiencies in the standard of care and disincentivizes effective innovations.
22. [The time horizon for costs should be long enough to incorporate the benefits of the treatment and the lower costs of medications when they face branded competition and/or become generic.](#) Many of the cost-offset benefits of treatment, such as avoided hospitalizations, show up in the longer-term. To measure the full value of a treatment, the time horizon for costs should be long enough to capture these cost-offsets,⁴⁹ and to account for the lower costs of medications when generics and biosimilars are introduced.
23. [Costs should be representative of the net price most relevant to the stated perspective.](#)⁵⁰ Costs are a driving component of a value assessment, and care should be taken to ensure that costs are most representative of the actual price to achieve an accurate assessment. For biopharmaceuticals, following good research practices for measuring drug costs and how drug costs change over time (e.g., dynamic pricing) can help achieve this objective. Net prices should account for all discounts and rebates provided to payers.

24. [Relevant evidence should be identified in a systematic, transparent and robust manner.](#) To maximize credibility and trust in the assessment process, the procedures by which evidence is identified and included in the assessment should be objective, systematic, reproducible, and made public as part of the scoping process. Formal methods for conducting and reporting on literature reviews are needed. By extension, the rationale for not including submitted evidence should also be made explicit. Not following elementary scientific best-practices erodes trust in the process.
25. [Stakeholders should be given the opportunity to submit relevant evidence, such as clinical trial and real-world evidence beyond the published literature.](#)⁵¹ Stakeholders, particularly patients and patient groups, may have pertinent evidence that is not available in the published literature. Despite potential concerns around public dissemination of proprietary data which should be handled case-by-case, such data may prove useful input to the assessment process. To ensure the evidence base is as comprehensive as possible, stakeholders should be given the opportunity to submit this evidence for consideration prior to the initiation of the formal assessment itself. To the extent that key stakeholders possess useful evidence, assessment bodies should make an effort to review and assess that evidence using appropriate methods.
26. [Best available evidence should be used for the assessment and the evidence should be appropriate for the outcome evaluated.](#)^{52, 53} Understanding a treatment's impact on patient-centered outcomes is critical in an assessment of value. In certain circumstances, only randomized clinical trial evidence may be available. In others, real-world evidence may provide an additional understanding of how a treatment is used for typical patients, and its comparative assessment to alternative patient care options. It is uncommon that clinical trials – designed for regulatory review of efficacy and safety – yield adequate information on healthcare utilization and costs to support a full value assessment. This is a gap that RWE is best suited to fill.⁵⁴ For example, observational studies can have larger sample sizes than clinical trials, very long follow-up periods, and broader representation of both patients and practice patterns. By using information collected in real-world evidence research, we can enhance the understanding of what is currently known regarding the topic that is being investigated. Both high-quality clinical trial and real-world evidence should be considered in any value assessment.

27. [Accepted and appropriate methods should be used to assess quality of evidence, certainty of evidence and conflicting evidence.](#)⁵⁵ The results of an assessment depend on the evidence that underlies it. Evidence can be of varying quality and certainty, and the findings from individual studies can conflict with each other. To produce a meaningful and credible assessment, appropriate methods should be used to evaluate quality and certainty of evidence and to determine how to handle conflicting evidence. If formal grading rubrics are used they should be fit-for-purpose and most appropriate for the type of evidence (e.g., clinical versus epidemiological versus economic data). Results of the evidence grading process should be included in assessment reports to best inform subsequent decision-making. Procedures for evaluating evidence quality should be included in scoping documents and the results should be made available through the value assessment.
28. [Where evidence synthesis is warranted, formal analysis should be conducted, in accordance with appropriate methodologies.](#) Representing the totality of evidence on product benefits, risks, and costs for value assessments may require formal evidence synthesis methods, such as systematic literature reviews and meta-analysis. The process of synthesizing evidence is a complex one. When there is a need to combine multiple sources of quantitative evidence, scientifically sound methodologies should be followed in order to ensure a meaningful and credible assessment.
29. [Subjective evidence on clinical efficacy and costs should be used minimally, if at all, and its inclusion should be clearly labeled.](#)⁵⁶ In situations where high-quality cost or clinical efficacy evidence is lacking, subjective expert clinical opinions might be considered. Expert opinion can be very useful, for example in the case of conflicting clinical trial results. However, as a substitute for objective trial data, opinion may be biased by the expert's experiences or beliefs, making it less reliable. As such, it should be treated as lesser-quality evidence and its use should be minimized. Subjective expert opinion should be transparently labeled and the user should be made aware of the potential limitations. Further, referencing past assessments that used an assumption or expert opinion source should still be listed as an assumption or expert opinion source rather than only referencing the past assessments with implied resolution around the lesser-quality evidence used.

Dissemination and Utilization

30. [All novel evidence generated or synthesized by value assessment organizations should be made public and submitted for peer review.](#)⁵⁷ Comprehensive value assessments may require qualitative research with patients, novel RWE analyses, development of economic models, or new systematic literature reviews. In keeping with the requirements of transparency, these analyses should be made available in full to the public rather than opaquely referenced in a value assessment report. If the guiding practices outlined here are followed, the burden of peer review should be minimal.
31. [Assessment results should be presented in a manner that is transparent and easy for the user to interpret and apply.](#)^{58, 59} The process and output of a value assessment can be complicated. Presenting the results in a manner that can be easily understood and applied by the user is critical for the value assessment to achieve its intended impact. Technical results should be fully documented according to scientific standards⁶⁰ and any recommendations should clearly link back to the evidence, with caveats on any uncertainties and unknowns made explicit. Oversimplification is a risk and developing educational materials to assist the user in interpretation and application is recommended.
32. [Value assessment should clearly state the intended use and audience to avoid misuse.](#)⁶¹ With the broad interest in value assessments, there comes a risk that assessment results will be misused by an unintended audience. For example, a value assessment designed for payers may not be appropriate for shared decision-making between patients and their doctors. Safeguards against misuse should be highlighted, such as creating a guidance statement that is explicit about how assessments should be used and instances where use is not appropriate.
33. [Public communications of final results should clearly express the complexities of value assessments, including limitations of the assessment and areas where uncertainties and sensitivity analyses result in material changes to the interpretation of the results.](#)⁶² Value assessments are complex undertakings and produce results that are highly nuanced; there is no single “takeaway” well-suited to executive summaries, press releases, or other abbreviated communications. Importantly, public communication of draft value assessments is rarely appropriate since final reports are often materially different than earlier drafts. Like any credible scientific reporting, value assessment results should be communicated objectively and comprehensively.

- 1 Garrison LP, Neumann PJ, Willke RJ, et al. A Health Economics Approach to US Value Assessment Frameworks—Summary and Recommendations of the ISPOR Special Task Force Report. *Value in Health*. 2018;21(2):161-165.
- 2 ISPOR. What Is HTA? <https://www.ispor.org/heor-resources/health-technology-assessment-central/what-is-hta>.
- 3 Neumann PJ, Garrison LP, Willke RJ. The History and Future of the "ISPOR Value Flower": Addressing Limitations of Conventional Cost-Effectiveness Analysis. *Value Health*. 2022 Apr;25(4):558-565.
- 4 NPC. The Myth of Average: Why Individual Patient Differences Matter. <https://www.npcnow.org/myth-of-average>.
- 5 Drummond MF, Augustovski F, Bhattacharyya D, et al. Challenges of HTA in Pluralistic Healthcare Systems: An ISPOR Council Report. *Value Health*. 2022 Aug;25(8):1257-1267.
- 6 Drummond M, Schwartz JS, Jansson B, et al. Principle 10. Key Principles for the Improved Conduct of Health Technology Assessments for Resource Allocation Decisions. *International Journal of Technology Assessment in Health Care*. 2008. 24:3:250.
- 7 Luce BR, Drummond MF, Dubois RW, et al. Principles 2 and 3. Principles for planning and conducting comparative effectiveness research. *Journal of Comparative Effectiveness Research*. 2012. 1 (5):433.
- 8 Oortwijn W, Husereau D, Abelson J, et al. Designing and Implementing Deliberative Processes for Health Technology Assessment: A Good Practices Report of a Joint HTAi/ISPOR Task Force. *Value Health*. 2022 Jun;25(6):869-886.
- 9 Frank LB, Concannon TW. Inclusion In Health Technology Assessments: The First Step Toward Equity. *Health Affairs ForeFront*. 2021. 10.1377/forefront.20211104.341669.
- 10 Drummond MF, Schwartz JS, et al. Principle 1. 247.
- 11 Luce BR, Drummond MF, et al. Principle 2. 433.
- 12 Oortwijn W, et al. Designing and Implementing Deliberative Processes for Health Technology Assessment: A Good Practices Report of a Joint HTAi/ISPOR Task Force. *Value Health*. 2022 Jun;25(6):869-886.
- 13 Oliver A, et al. HTA and its influence on health-care priority setting. *Intl J of Tech Assess in Health Care*. 2004. 20:1. 9.
- 14 Drummond M, Schwartz JS, et al. Principle 10. 253.
- 15 Drummond MF, Schwartz JS, et al. Principle 13. 255.
- 16 Drummond MF, Schwartz JS, et al. Principle 3. 249.
- 17 Donaldson MS, Sox HC. Committee on Priorities for Assessment and Reassessment for Health Care Technologies. Setting Priorities for HTA. National Academies Press; 1992. Recommendation 2. Institute of Medicine.
- 18 Luce BR, Drummond MF, et al. Principle 6. 434.
- 19 Drummond M, Schwartz JS, et al. Principle 2. 248.
- 20 Luce BR, Drummond MF, et al. Principle 5. 434.
- 21 Neumann PJ, et al. A Call for Open-Source Cost-Effectiveness Analysis. *Ann Intern Med*. 2017 Sep 19;167(6):432-433
- 22 ISPOR Good Practices for Outcomes Research Index. http://www.ispor.org/workpaper/practices_index.asp.
- 23 Drummond M, Schwartz JS, et al. Principle 5. 250.
- 24 Luce BR, Drummond MF. Principle 9. 435.
- 25 Drummond M, Schwartz JS, et al. Principle 5. 251.

- 26 Sanders GD, Neumann PJ, Basu A, et al. Recommendations for Conduct, Methodological Practices, and Reporting of Cost-effectiveness Analyses: Second Panel on Cost-Effectiveness in Health and Medicine. *JAMA*. 2016 Sep 13;316(10):1093-103.
- 27 Berger ML, Sox H, Willke RJ, Brixner DL, Eichler HG, Goettsch W, Madigan D, Makady A, Schneeweiss S, Tarricone R, Wang SV, Watkins J, Mullins CD. Good Practices for Real-World Data Studies of Treatment and/or Comparative Effectiveness: Recommendations from the Joint ISPOR-ISPE Special Task Force on Real-World Evidence in Health Care Decision Making. *Value Health*. 2017 Sep;20(8):1003-1008.
- 28 Dreyer NA, Bryant A, Velentgas P. The GRACE Checklist: A Validated Assessment Tool for High Quality Observational Studies of Comparative Effectiveness. *J Manag Care Spec Pharm*. 2016 Oct;22(10):1107-13.
- 29 Luce BR, Drummond MF, et al. Principle 6. 434.
- 30 Drummond M, Schwartz JS, et al. Principle 8. 252.
- 31 Luce BR, Drummond MF, et al. Principle 11. 436.
- 32 Joint meeting of the Arthritis Advisory Committee and the Drug Safety and Risk Management Advisory Committee (March 21, 2021). Brief Overview of Patient Preference Information (PPI). <https://www.fda.gov/media/146925/download>.
- 33 Marsh K, de Bekker-Grob E, Cook N, Collacott H, Danyliv A. How to integrate evidence from patient preference studies into health technology assessment: a critical review and recommendations. *International Journal of Technology Assessment in Health Care*. 2021;37(1).
- 34 Lakdawalla DN, Phelps CE. Health Technology Assessment With Diminishing Returns to Health: The Generalized Risk-Adjusted Cost-Effectiveness (GRACE) Approach. *Value Health*. 2021 Feb;24(2):244-249.
- 35 Mendola ND, Oehrlein E, Perfetto EM, Westrich K, McQueen RB. Stakeholder perception of pharmaceutical value: A multicriteria decision analysis pilot case study for value assessment in the United States. *JMCP*. 2022 Oct;28(10):1190-1196.
- 36 NPC. The Myth of Average: Why Individual Patient Differences Matter. <https://www.npcnow.org/myth-of-average>.
- 37 Drummond MF, Schwartz JS, et al. Principle 15. 256.
- 38 Oortwijn W, et al. Designing and Implementing Deliberative Processes for Health Technology Assessment: A Good Practices Report of a Joint HTAi/ISPOR Task Force. *Value Health*. 2022 Jun;25(6):869-886.
- 39 Luce BR, Drummond MF, et al. Principle 7. 434.
- 40 Drummond M, Schwartz JS, et al. Principle 6. 252.
- 41 Neumann PJ, Garrison LP, Willke RJ. The History and Future of the "ISPOR Value Flower": Addressing Limitations of Conventional Cost-Effectiveness Analysis. *Value Health*. 2022 Apr;25(4):558-565.
- 42 Lakdawalla DN, Phelps CE. Health Technology Assessment With Diminishing Returns to Health: The Generalized Risk-Adjusted Cost-Effectiveness (GRACE) Approach. *Value Health*. 2021 Feb;24(2):244-249.
- 43 Luce BR, Drummond MF, et al. Principle 10. 436.
- 44 NPC. The Myth of Average: Why Individual Patient Differences Matter. <https://www.npcnow.org/myth-of-average>.
- 45 Basu A, Grieve R, Pritchard D, Stevens W. One size does not always fit all in value assessment. *Am J Manag Care*. 2019 Nov;25(11):540-542.
- 46 Mathes T, Jacobs E, Morfeld JC, Pieper D. Methods of international health technology assessment agencies for economic evaluations- a comparative analysis. *BMC Health Services Research* 2013, 13:371.
- 47 Drummond M, Schwartz JS, et al. Principle 7. 252.

- 48 Sanders GD, Neumann PJ, Basu A, Brock DW, Feeny D, Krahn M, Kuntz KM, Meltzer DO, Owens DK, Prosser LA, Salomon JA, Sculpher MJ, Trikalinos TA, Russell LB, Siegel JE, Ganiats TGet al. Recommendations for Conduct, Methodological Practices, and Reporting of Cost-effectiveness Analyses: Second Panel on Cost-Effectiveness in Health and Medicine. *JAMA*. 2016 Sep 13;316(10):1093-103
- 49 Hay JW, Smeeding J, Carroll NV, et al. Good research practices for measuring drug costs in cost effectiveness analyses: issues and recommendations: the ISPOR drug cost task force report – Part I. *Value Health* 2010;13:3-7. Recommendation 5. 6.
- 50 Hay JW, Smeeding J, et al. Recommendations 1, 4, 8. 6.
- 51 Drummond MF, Schwartz JS, et al. Principle 10. 254.
- 52 Drummond MF, Schwartz JS, et al. Principle 11. 254.
- 53 Luce BR, Drummond MF, et al. Principle 8. 435.
- 54 FDA: Drug and Device Manufacturer Communications With Payors, Formulary Committees, and Similar Entities Questions and Answers Guidance for Industry and Review Staff. <https://www.fda.gov/media/133620/download>
- 55 Luce BR, Drummond MF, et al. Principle 9. 435.
- 56 Balshem H, Helfand M, et al. GRADE guidelines: 3. Rating the quality of evidence. *Journal of Clinical Epidemiology* 2011;64:401-406.
- 57 Neumann PJ, et al. A Call for Open-Source Cost-Effectiveness Analysis. *Ann Intern Med*. 2017 Sep 19;167(6):432-433.
- 58 Drummond MF, Schwartz JS, et al. Principle 14. 255.
- 59 Oortwijn W, et al. Designing and Implementing Deliberative Processes for Health Technology Assessment: A Good Practices Report of a Joint HTAi/ISPOR Task Force. *Value Health*. 2022 Jun;25(6):869-886.
- 60 Consolidated Health Economic Evaluation Reporting Standards (CHEERS) 2022 Explanation and Elaboration: A Report of the ISPOR CHEERS II Good Practices Task Force. *Value Health*. 2022 Jun;25(6):1060.
- 61 Donaldson MS, Sox HC. Guiding Principle 2. 53.
- 62 NewsStory Review Criteria. Criteria 4. <http://www.healthnewsreview.org/about-us/review-criteria/>.