



# Aligning Health Technology Assessment with Efforts to Advance Health Equity





# ALIGNING HEALTH TECHNOLOGY ASSESSMENT WITH EFFORTS TO ADVANCE HEALTH EQUITY

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# Executive Summary

In the United States, value-based health care is a concept that has gained strong support among policymakers and payers. Despite significant investment in value assessment, the process and metrics historically used to value therapeutic interventions have worked against health equity. High value care must also be equitable care. This report provides recommendations that may assist HTA organizations, health systems, payers, and policymakers that want to center their value assessment work on health equity.

Researchers and health economists face a difficult history of the use of quantifiable algorithms to value health care that do not prioritize the delivery of equitable care. Going forward, it is critical that we not tether value assessment to the biases of the past. As part of nationwide efforts to end systemic racism, the recommendations in this report are intended to provide a roadmap for HTA organizations to better value the perspectives and experiences of people that have been historically devalued in the assessments used to value health care and not represented in the existing data. We urge reconsideration of current definitions of value to clarify the answer to the question, “Value to whom?” What brings value to the end users of our health care system — the patients — not just payers? This will require development of new methods and algorithms used to calculate value that inherently value health equity and that incorporate elements of value unique to different subpopulations.

The work of HTA organizations and others conducting value assessment impacts how treatments are covered, including their cost sharing. We urge organizations conducting value assessment to define value consistent with the values inherent in the nation’s civil rights and disability laws established to ensure everyone receives high quality care with the goal of health equity. The report’s recommendations focus on the need to address data gaps, methodologies contributing to health inequity, and the need for improved engagement, particularly among people excluded from the data.

## Recommended Focus for HTA Organizations and Others Engaged in Addressing Data Gaps:

- Identify explicitly the limitations of data as they relate to a value assessment’s conclusions for omitted subpopulations.
- Invest in addressing data gaps before conducting a value assessment, provide resources to fill data gaps during a value assessment, and allow time for the development of partnerships to support collecting representative data as part of an ongoing data collection process.
- Recognize the heterogeneity among patients based on their social identities, geographic communities, and other factors — such as access to health facilities — and incorporate that information into the base case assessment of a treatment’s value.

## Recommended Focus for HTA Organizations and Others Engaged in Testing and Validating New Methods to Eliminate Bias:

- Ensure methods incorporate the values of patients and people with disabilities, starting with systematic reviews and direct engagement with the patient and disability communities to ensure their values are considered in the base case.
- Avoid valuing treatments based only on averages and avoid reliance on QALYs, instead using methods that intentionally allow for consideration of patient differences.
- Retrospectively review previous value assessments with an overlay of real-world evidence and consideration of factors impacting health equity.
- Increase transparency of methods.

Recommended Focus for HTA Organizations and Others Seeking Meaningful Engagement from Diverse Stakeholders in the Patient and Disability Communities:

- Consider models from PCORI and participatory researchers to develop protocols for engagement that make equity and inclusion a high priority.
- Value engagement by providing engaged patient and disability partners with resources to participate effectively.
- Evaluate and improve engagement practices to correct any systemic disadvantages to engaged partners, clearly stating how affected patient and disability stakeholders were engaged in published HTA.

When the benefit designs that drive coverage and the clinical guidelines that drive provider decisions are built on biased HTA that relies on flawed science, incomplete data, and discriminatory algorithms, health inequities are a natural consequence. If HTA organizations and others want to do their part to address the cycle that perpetuates health disparities for certain subpopulations, data limitations must be addressed alongside improved methods capable of eliminating bias and recognizing social and structural determinants of health.



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# Part One

## *Introduction*

In the United States, value-based health care is a concept that has gained strong support among policymakers and payers. Despite significant investment of dollars, human resources, and time in health technology assessment (HTA), also known as value assessment, the process and metrics historically used to value therapeutic interventions have worked against health equity. Those contributing to this report agree that high value care must also be equitable care. Therefore, we have developed recommendations that may assist HTA organizations, health systems, payers, and policymakers that want to center their value assessment work on health equity.

The World Health Organization describes HTA as follows:

Health technology assessment (HTA) is a systematic and multidisciplinary evaluation of the properties of health technologies and interventions covering both their direct and indirect consequences. It is a multidisciplinary process that aims to determine the value of a health technology and to inform guidance on how these technologies can be used in health systems around the world.<sup>1</sup>

Yet, researchers and health economists face a difficult history of the use of quantifiable algorithms to value health care that do not prioritize the delivery of equitable care. Going forward, it is critical that we not tether value assessment to the biases of the past. As part of nationwide efforts to end systemic racism, the recommendations in this report are intended to provide a roadmap for HTA organizations to better value the perspectives and experiences of people that have been historically devalued in the assessments used to value health care and not represented in the existing data. We urge reconsideration of current definitions of value to clarify the answer to the question, “Value to whom?” What brings value to the end users of our health care system — the patients — not just payers? This will require development of new methods and algorithms used to calculate value that inherently value health equity and that incorporate elements of value unique to different subpopulations.

We applaud those HTA organizations and others conducting value assessment that are striving to do better and are seeking to play a role in efforts to achieve health equity by recognizing the historical biases in health care value assessment. This report is intended to assist organizations conducting value assessment to identify priorities for their scope of work as they participate in efforts to advance health equity. We look forward to their work to develop more concrete and actionable changes to the HTA process and to lending our voices to the new challenges that will inevitably arise from it. We understand that this will be an ongoing process and, working together, we are hopeful to point to tangible progress in the near future as organizations conducting value assessment prioritize health

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<sup>1</sup> World Health Organization, Health technology assessment, [https://www.who.int/health-topics/health-technology-assessment#tab=tab\\_1](https://www.who.int/health-topics/health-technology-assessment#tab=tab_1)

equity. We hope that this report conveys the urgency to respond to concerns about the implications of HTA on health equity.

# Part Two

## *The Problem*

It is, for many, a harsh reality that HTA is relied upon to make coverage decisions affecting affordability and access to care despite its inherent bias. This report and its recommendations highlight the flaws of the data that drive conclusions about health care value and the process that excludes certain voices from a role in value assessment. Its recommendations aim to help HTA organizations and others participate effectively in efforts to advance an equitable health care system in which all people can achieve the best possible outcomes relative to the newest science and innovations and to ensure that all people are reflected in the data that drives decision-making. We understand that a true commitment to health equity will require ongoing engagement in this work to address not only the issues that we raise, but the challenges that are identified and the new questions posed as part of this process.

We appreciate the recent work of Sick Cells and the Innovation and Value Initiative and their publication of a report that discussed the Institute for Clinical and Economic Review's (ICER) process for developing a value assessment for sickle cell treatments in 2019-2020 as a constructive example of the health equity challenges inherent in value assessment. The report provided examples underscoring the disproportionate power of HTA organizations, the exclusion of Black, Indigenous, and people of color (BIPOC) voices in HTA decision-making, and the impact of a centralized focus on a national population on representativeness of the data. They concluded that HTA organizations should give a meaningful voice to BIPOC communities and patients, improve the evidence base and HTA methods to remove implicit biases, and understand that HTA is a "product of and contributor to systems of inequity and bias."<sup>2</sup>

The work of HTA organizations and others conducting value assessment impacts how treatments are covered, including their cost sharing. We urge organizations conducting value assessment to define value consistent with the values inherent in the nation's civil rights and disability laws established to ensure everyone receives high quality care with the goal of health equity. Recently, policymakers, including the Centers for Medicare and Medicaid Services (CMS) under the Biden Administration, have expounded that health equity is one of their top priorities.<sup>3</sup> With health equity noted as a critical priority, it should be a top consideration for how we value health care and not just a check-the-box activity. If a foundational goal of health care policy and coverage decisions is health equity, then organizations conducting value assessment are potentially useful and important partners in

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<sup>2</sup> Mark Linthicum, MPP, et al, "Finding Equity in Value: Racial and Health Equity Implications of U.S. HTA Processes," published 2022, [https://sickcells.org/wp-content/uploads/2022/10/IVI\\_Sick-Cells\\_Equity-in-Value\\_2022.pdf](https://sickcells.org/wp-content/uploads/2022/10/IVI_Sick-Cells_Equity-in-Value_2022.pdf).

<sup>3</sup> CMS Strategic Plan: Pillar Health Equity, published 2022, <https://www.cms.gov/files/document/health-equity-fact-sheet.pdf>.



delivering high value care that eliminates premature morbidity and mortality and improves quality of life for all populations.

# Part Three

## *Health Equity is an Imperative*

**W**e believe health equity is an imperative and that the foundational step toward health equity is improving the research and science that drives our health care system. The reality is that certain populations are excluded, intentionally or unintentionally, from much of the research that feeds value assessment models. Instead, research tends to rely on population-level averages wherein comparative and cost effectiveness algorithms are derived from health utilities reflecting white males, and clinical trial data are infamous for their lack of diversity.<sup>4</sup> Value assessment organizations dealing with serious gaps in the data often try to ameliorate the issue by recognizing limitations of their conclusions. The challenge in this approach is that the process of value assessment is still based on flawed exclusive data that only serves to perpetuate inequity in how our health care system values treatments and services.

Health disparities — quantifiable differences in health-related outcomes — have been documented across many dimensions, including race, gender, age, location, disability status, and sexual orientation. These disparities are exacerbated by the unequal experiences of different subpopulations. The resulting health inequity is especially apparent along lines of race, with BIPOC populations experiencing significant barriers that lead to poorer health overall than white populations.<sup>6</sup> We recognize that racism is a prime culprit — to the point where racism is a public health crisis.<sup>7</sup>

### *Asthma Case Study*

Asthma provides a case study for health inequity. Black individuals are three times as likely to die of asthma. Black patients with asthma also visit the emergency department five times more often than white patients.

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<sup>4</sup> Willyanne DeCormier Plosky, et al, “Excluding People with Disabilities from Clinical Research: Eligibility Criteria Lack Clarity and Justification,” *Health Affairs*, published October 1, 2022, <https://www.healthaffairs.org/doi/10.1377/hlthaff.2022.00520>.

<sup>5</sup> National Minority Quality Forum, “Traditional Value Assessment Methods Fail Communities of Color and Exacerbate Health Inequities White Paper,” *National Minority Quality Forum, Inc.*, published 2019, <https://www.nmqf.org/nmqf-media/traditional-value-assessment-methods>.

<sup>6</sup> Elizabeth Baca, et al, “Activating Health Equity: A Moral Imperative Calling for Business Solutions,” *Deloitte*, published April 12, 2021, <https://www2.deloitte.com/us/en/insights/industry/health-care/developing-an-agenda-of-equity-in-health.html>.

<sup>7</sup> Office of Minority Health & Health Equity, “Impact of Racism on our Nation’s Health,” *Centers for Disease Control and Prevention*, published April 8, 2021, <https://www.cdc.gov/healthequity/racism-disparities/impact-of-racism.html>.

A significant study led by the Asthma and Allergy Foundation of America identified the causes of such inequity to include several factors. First, social determinants of health — such as economic stability, education, physical environment, social environment, and health care — were cited causes. Second, structural determinants of health — such as systemic racism, residential segregation and discriminatory housing policies, discriminatory hiring and promotion practices, stereotyping and stigmatization, explicit bias, and environmental injustice — were cited causes.<sup>8</sup>

Implementation of strategies to improve asthma health across all populations requires knowledge of the heterogeneity of treatment impact. Studies indicate that high quality asthma management requires recognition of ethnic-specific differences in bronchodilator drug responsiveness that exist between Mexicans, Puerto Ricans, and African Americans with asthma.<sup>9</sup> If health equity is the goal, underserved populations should have equal access to high-quality care that is effective for them. This requires value judgments that recognize social and structural determinants of health and that give value to reducing the existing inequality for people getting low value and low-quality care. For asthma, we know that health equity requires giving value to addressing historical environmental injustices and poverty that have served to exacerbate the prevalence of the condition, as well as recognizing the ethnic-specific differences that impact the effectiveness of treatment for varying subpopulations and, ultimately, providing the care that is most effective.

***Commitment from HTA organizations is needed to address health inequity.***

A genuine commitment to health equity is best demonstrated by HTA organizations working collaboratively to correct the biased methodologies and models that perpetuate health inequity. We want to encourage shared learning among HTA organizations and others that have a mission to be patient-centered and that have prioritized health equity considerations in value assessment.

As an example, the Innovation and Value Initiative (IVI) established a Health Equity Initiative in March 2022 as a 2-year initiative that aims to define gaps in knowledge and methodologies needed to advance value assessment that supports health equity. As other entities seek to similarly update their value frameworks to center on working toward health equity, we urge them to collaborate with entities such as IVI that have similar goals and whose commitment to transparency provides a model for other HTA organizations to follow. IVI's named goals include advancing an understanding of why health equity considerations are important; identifying gaps in procedural, data collection, and methods practices and their implications for health equity; developing "best practice" protocols to mitigate these gaps; and achieving multi-stakeholder consensus about areas for further research, scientific practice change, and policy development.<sup>10</sup>

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<sup>8</sup> AAFA, "Asthma Disparities in America: A Roadmap to Reducing Burden on Racial and Ethnic Minorities," *Asthma and Allergy Foundation of America*, published 2020, <https://www.aafa.org/asthma-disparities-burden-on-minorities.aspx>.

<sup>9</sup> Mariam Naqvi, et al, "Ethnic-Specific Differences in Bronchodilator Responsiveness Among African Americans, Puerto Ricans, and Mexicans with Asthma," *Journal of Asthma* 44:8, published July 2, 2009 <https://doi.org/10.1080/02770900701554441> and AAFA, "Asthma Disparities in America," 71.

<sup>10</sup> IVI, "IVI Launches Health Equity Initiative," *Innovation and Value Initiative*, published April 27, 2022, <https://www.thevalueinitiative.org/press-release-ivi-launches-health-equity-initiative/>.

ICER has also pledged to be part of change, stating, “We acknowledge we are part of that system, and we need to witness more, care more, act more. We hear the clarion call ringing out — to be part of the change needed to ensure that race is no longer a determining factor in life expectancy, that medical research dollars flow to combat conditions that impact communities of color, and that we achieve health equity by addressing the negative impact of social determinants of health.”<sup>11</sup> Almost two years after its statement on race in America in 2020, ICER launched an initiative to evaluate and advance health technology assessment methods that support health equity through a new grant from the Commonwealth Fund. ICER has stated its intent to evaluate procedural and methodological changes that could further support health equity goals in HTA. The findings from this effort are intended to guide ICER’s update to its value assessment framework and, similar to IVI’s efforts, inform the work of other HTA groups worldwide.<sup>12</sup>

ICER’s advisory group on health equity includes overlapping steering committee members with those working with IVI’s Health Equity Initiative. ICER and IVI therefore have an opportunity to access the insights provided by each other’s efforts in this space, and work collaboratively toward consensus and shared learning that would influence the field of health economics and correct the methods that drive value assessment. We urge all entities seeking to eliminate the bias in the algorithms used for value assessment to collaborate in a shared effort to advance the field of health economics.

To truly move toward health equity, it will be imperative that HTA organizations acknowledge the inherent bias against socially disadvantaged populations that exists in our health care system and reflect on all data sets they consider using in their models. These inherent biases are embedded deeply in our health care system and research ecosystem and a critical eye will be needed to determine which research building blocks are fit for purpose, as well as which will need to be discarded in order to move forward. For example, the Gail Model, a breast cancer risk prediction model, is known to underestimate breast cancer risk in Black women. Yet the NIH website continues to direct individuals to it with a warning that it “may underestimate risk in black women with previous biopsies and Hispanic women born outside the United States.”<sup>13</sup> Racism and inherent bias is not new, but as pockets of our health care system seek to tackle it head on, the advances in HTA to eliminate bias in their algorithms will facilitate those efforts.

For HTA organizations to be part of change requires taking stock of the work undertaken already to identify how existing methods are part of the problem and how they could be improved.<sup>14</sup> Knowing that some HTA organizations such as ICER and IVI want to do better, we urge them to sincerely

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<sup>11</sup> ICER, “ICER Statement on Racism in America,” *Institute for Clinical and Economic Review*, published 2020, [https://icer.org/news-insights/commentaries/race\\_in\\_america/](https://icer.org/news-insights/commentaries/race_in_america/).

<sup>12</sup> ICER, “ICER Launches Initiative to Evaluate and Advance Health Technology Assessment Methods that Support Health Equity,” *Institute for Clinical and Economic Review*, published July 11, 2022, <https://icer.org/news-insights/press-releases/hta-methods-health-equity-paper-2023/>.

<sup>13</sup> National Cancer Institute, “The Breast Cancer Risk Assessment Tool,” *National Cancer Institute*, published 2017, <https://bcrisktool.cancer.gov/>.

<sup>14</sup> Mark Linthicum, MPP, et al, “Finding Equity in Value: Racial and Health Equity Implications of U.S. HTA Processes,” published 2022, p. 21, [https://sickcells.org/wp-content/uploads/2022/10/IVI\\_Sick-Cells\\_Equity-in-Value\\_2022.pdf](https://sickcells.org/wp-content/uploads/2022/10/IVI_Sick-Cells_Equity-in-Value_2022.pdf).



recognize the systemically racist history of value assessment as a strong first step. For example, contributors to this report applauded the American Nurses Association’s apology for racism and its accounting of past actions as a strong start on a path forward toward forgiveness and reconciliation.<sup>15</sup> It provides a useful model.

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<sup>15</sup> ANA, “Our Racial Reckoning Statement,” *American Nurses Association*, published June 11, 2022, <https://www.nursingworld.org/practice-policy/workforce/racism-in-nursing/RacialReckoningStatement/>.

# Part Four

## *Addressing the Data Gaps*

Improved methodologies for assessing health technologies and services are limited by exclusive data that fails to represent all people. This data problem is well understood to create serious limitations for the conclusions of value assessment.<sup>16</sup> Organizations conducting value assessment have an opportunity to cite such known data limitations in their reports and assessments that clarify for health care decision-makers the subpopulations for whom the assessment is or is not credible, as well as to take concrete steps to fill the recognized data gaps, consistent with ISPOR's recommendation below:

*Item 20: Characterizing uncertainty: Describe methods to characterize any sources of uncertainty in the analysis.*<sup>17</sup>

Otherwise, the inherent bias of the underlying data and the limitations of the conclusions advanced by existing methods relying on that biased data will not be understood by those making decisions, whether as payers and policymakers designing covered benefits or as providers and patients making health care decisions.

For background, the National Minority Quality Forum joined several organizations in 2020 to review the existing literature about value assessment and its implications for health inequity. The report acknowledged that value assessments are largely based on population-level averages and rarely report results specific to minorities. Also, the metric for determining cost effectiveness, known as a quality-adjusted life year (QALY), assigns a lower value to the lives of patients with disabilities and chronic conditions. The report also recognized that the underlying health utilities that are used alongside the QALY as part of the algorithm for cost effectiveness are typically derived from homogeneous Caucasian non-Hispanic populations. Utility designs also may not incorporate outcomes that matter to patients, including social determinants of health that too often drive disparity in health among various subpopulations.<sup>18</sup>

As an example of the real-world impact of HTA, an assessment of the value of a treatment for asthma will be inherently biased if it fails to recognize that the standard treatment is not effective for ethnic-specific populations that also experience the highest burden of disease due to social and structural

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<sup>16</sup> [https://sickcells.org/wp-content/uploads/2022/10/IVI\\_Sick-Cells\\_Equity-in-Value\\_2022.pdf](https://sickcells.org/wp-content/uploads/2022/10/IVI_Sick-Cells_Equity-in-Value_2022.pdf)

<sup>17</sup> Don Husereau, et al, "Consolidated Health Economic Evaluation Reporting Standards (CHEERS) 2022 Explanation and Elaboration: A Report of the ISPOR CHEERS II Good Practices Task Force," *Value Health* 25:1, published June 2022: 10-31, <https://doi.org/10.1016/j.jval.2021.10.008>.

<sup>18</sup> National Minority Quality Forum, "Traditional Value Assessment Methods Fail Communities of Color and Exacerbate Health Inequities White Paper," 4.

determinants as described above. It will undervalue treatments for populations that are not represented in the data and therefore whose experience with treatment is not captured.

In some cases, HTA organizations and others engaged in value assessment have an opportunity to address the shortcomings of the data available to them, such as by conducting surveys that solicit needed information from subpopulations that may not be well represented in clinical trial data. For HTA to advance health equity, these more representative data of all people with lived experience with the condition or disease being studied should also inform the base case of cost effectiveness analysis. It has been a source of frustration for some patient advocacy organizations that the survey data collected by patient organizations to inform ICER's value assessments did not make it into the base case assessments of cost effectiveness but, if referenced at all, were limited to the contextual considerations of the report.<sup>19</sup> As articulated by Sick Cells in its recent report, omitting this evidence can systematically bias HTA results and exacerbate stigma and racism.<sup>20</sup>

Over time, HTA organizations could play a role in driving research to develop higher quality data reflecting the real-world experiences of patients by requiring their assessments to rely on data that meet a higher standard. Otherwise, evaluations of treatment impact are not considering the differential effectiveness among subpopulations and instead are relying on averages that bias the estimation of treatment value and exacerbate health inequities. Some data sources that could be useful for understanding treatment impact on subpopulations are not published or publicly available. While a solution to this challenge is outside the scope of this report, organizations conducting value assessment could influence researchers, including clinical trial developers, to build in patient-reported outcomes and patient preferences into their data collection in preparation for an anticipated future value assessment.

### ***Recommended Focus for HTA Organizations and Others Engaged in Addressing Data Gaps***

- **Identify explicitly the limitations of data as they relate to a value assessment's conclusions for omitted subpopulations:**
  - HTA organizations and others conducting value assessment should consider strategies to explicitly identify the gaps in patient data and recognize them clearly throughout a value assessment's results as limitations to the report's conclusions for the omitted subpopulations. Such limitations should not be confined to a single section of the report but clarified throughout it.
  - Disclaimers related to the limitations of a value assessment's conclusions should also not be limited to the report itself. Disclaimers should be included in ancillary materials associated with the release of a value assessment, so that other stakeholders (e.g., policymakers, media) who learn of the report's conclusions understand its limitations.

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<sup>19</sup> Mark Linthicum, MPP, et al, "Finding Equity in Value: Racial and Health Equity Implications of U.S. HTA Processes," published 2022, [https://sickcells.org/wp-content/uploads/2022/10/IVI\\_Sick-Cells\\_Equity-in-Value\\_2022.pdf](https://sickcells.org/wp-content/uploads/2022/10/IVI_Sick-Cells_Equity-in-Value_2022.pdf).

<sup>20</sup> Linthicum, 17.

- HTA organizations and others should consider strategies to work in collaboration with affected patient and disability stakeholders in the earliest scoping phase to assess the data available for use in a value assessment to determine whether it is sufficient to answer the questions being asked, describing the uncertainty as recommended by ISPOR. Where gaps exist, work collaboratively to seek out additional data sources or engage in a process of further data collection.
- **Invest in addressing data gaps before conducting a value assessment, provide resources to fill data gaps during a value assessment, and allow time for the development of partnerships to support collecting representative data as part of an ongoing data collection process:**
  - HTA organizations and others should consider whether resources slated to be invested in a planned value assessment may be better invested in data generation.
  - HTA organizations and others deciding to advance a value assessment without high quality data should consider engaging with patient groups and other stakeholders to fill data gaps, providing them with resources to conduct timely surveys and incorporate the results into the base case analysis of cost effectiveness and value.
  - HTA organizations and others should consider strategies to have an ongoing data collection process, facilitated by outside relationships with researchers and patient organizations, to ensure representative data are available in advance of conducting a value assessment. Alternatively, increased time could be allotted to conduct a value assessment to establish such partnerships and collect the needed representative data. Value assessment should not be conducted without appropriate, representative data.
- **Recognize the heterogeneity among patients based on their social identities, geographic communities, and other factors — such as access to health facilities — and incorporate that information into the base case assessment of a treatment’s value:**
  - HTA organizations and others should consider strategies allowing them to better stratify their work to identify the differential treatment impact of treatments under review based on the varying social identities of patients potentially benefiting from treatment and incorporate that knowledge into the base case as a strategy to advance health equity.<sup>21</sup>
  - HTA organizations and others should consider strategies specifically identifying the geographic communities that represent distinct social characteristics to assess how these populations’ intersecting social identities and environmental exposures may impact health, access to care, and outcomes. Additionally, HTA organizations and others should consider similar strategies that allow for incorporating that information into the base case so that the conclusions serve as a driver for health equity.<sup>22</sup>

<sup>21</sup> Jacquelyn McRae and Eberechukwu Onukwugha, “Why the Gap in Evaluating the Social Constructs and the Value of Medicines?” *PharmacoEconomics* 39:12, published August 30, 2021: 1365–1372, <https://doi.org/10.1007/s40273-021-01075-w>.

<sup>22</sup> McRae, “Why the Gap in Evaluating the Social Constructs and the Value of Medicines?” 1365 – 1372.



- HTA organizations and others should explicitly recognize factors that may skew the validity of value assessment conclusions such as how lower costs associated with standard care could result from underutilization of care, higher costs could result from overuse of emergency room services for populations without access to primary care, and differential access to pharmacies and community-based health workers could drive challenges for adherence to prescribed treatment and therefore impact associated treatment cost and health outcomes.<sup>23</sup>

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<sup>23</sup> McRae, 1365-1372

# Part Five

## *Addressing Methodological Flaws that Undermine Diversity, Equity, and Inclusion*

**H**TA organizations and others conducting value assessments cannot strive for inclusive excellence while excluding the needed inclusive data from value assessments — to do so is a violation of the principles of diversity, equity, and inclusion. We are similarly concerned that, without improved methods, organizations conducting value assessments are highly limited in their ability to incorporate factors such as social and structural determinants of health. IVI is one organization that we are aware is working to incorporate new HTA methods that are better able to capture heterogeneity of treatment impact into their major depressive disorder model, with a focus on social determinants of health and distributional cost effectiveness analysis — a step in the right direction and a process that requires more resources and more allotted time to conduct an assessment. We urge all organizations conducting value assessments to similarly dedicate resources, not only to collect and incorporate data on patient preferences among subgroups, but also to test and validate methods that are capable of using that data to better articulate the differences among patients and people with disabilities related to the treatment being assessed.

The research literature also suggests that incorporating intersectionality would lead to more equitable and accurate value assessments. Intersectionality theory captures the compounding effects of occupying two or more of these social identities (i.e., Black and female). Traditionally, cost effectiveness analysis has not been subject to sufficient examination through an intersectionality framework, yet real world research supports the conclusion that overlapping social identities such as race and class have real effects on health behaviors, measured preferences, and economic costs. We encourage HTA organizations and others to work to capture this heterogeneity in benefits and costs within a value framework. Omitting it provides inadequate information to decision-makers to evaluate the value of treatments for socially disadvantaged populations and biases their conclusions.<sup>24</sup>

Improved HTA methods have the potential to support precision medicine innovations and advance health equity by incentivizing the development of innovative treatments for subpopulations that have historically experienced health disparities. When organizations conducting value assessments partner with stakeholders to develop a concrete list of the valued outcomes for different subgroups, they are better able to understand the value associated with treating their conditions. Understanding those varying values, HTA organizations can help payers, providers, and policymakers ensure delivery of care that each subpopulation would deem most effective.

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<sup>24</sup> McRae, 1365-1372

Collaboration with patient and disability stakeholders who have lived experience related to the condition being studied is essential as HTA organizations seek to validate and incorporate new methods. There is no one-size-fits-all. For example, in the absence of diverse RCT data, an organization conducting value assessments could incorporate claims data as a source of information about the condition and its treatment, about the subpopulations being treated, and about the geographic locations of people being treated to better understand differences in their outcomes and the limitations of the system of care accessible to them. Real-world claims data could be compared to an earlier value assessment to determine its validity or its shortcomings for specific populations. Yet, for such a method to be reliable requires an understanding of the condition being studied that is most known to the patients and people with disabilities that are the affected stakeholders.

For example, when ICER reviewed treatments for sickle cell disease and took steps to incorporate Medicare claims data, the survey of sickle cell disease patients provided insight that most patients were being served by Medicaid – not Medicare.<sup>25</sup> Therefore, the data from Medicare were not necessarily reflective of the patient experience. Additionally, the survey found that pain crises were managed largely at home and not in a setting that would be captured by claims data.<sup>26</sup> ICER did not reflect pain events outside the hospital in its analysis of the cost effectiveness and value of treatment despite qualitative input from sickle cell patients.<sup>27</sup> Incorporating this knowledge into the base case would have produced a more accurate and nuanced assessment.<sup>28</sup>

We urge organizations conducting value assessments to advance retrospective reviews of their past reports using real world data. For example, in 2021, ICER used observational real-world evidence as a comparator to its earlier assessment of treatments for hereditary angioedema.<sup>29</sup> ICER’s stated goal was to remove some of the data uncertainty identified during the 2018 review. Especially when value assessments are launched before the availability of real-world evidence, such retrospective reviews conducted in partnership with affected stakeholders are essential to understand real world implications of treatment.

ISPOR, a professional society for health economics and outcomes research, plays an influential role in efforts to advance improved HTA methods. In 2022, the Consolidated Health Economic Evaluation Reporting Standards 2022 were published by the ISPOR CHEERS II Good Practices Task Force. The report included several recommendations that we identified as high priorities for improving the methods used by HTA organizations to address health equity:

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<sup>25</sup> Sick Cells, comment letter to ICER on draft evidence report for sickle cell disease, Feb. 2, 2020, page 1. [https://sickcells.org/wp-content/uploads/2021/06/Sickle-Cells-Public-Comments-to-ICER\\_final.pdf](https://sickcells.org/wp-content/uploads/2021/06/Sickle-Cells-Public-Comments-to-ICER_final.pdf)

<sup>26</sup> Sick Cells My Life with SCD Patient and Caregiver Survey Results: Inputs for the 2019 ICER Review, presentation to SCDA National Convention, 2020. [https://sickcells.org/wp-content/uploads/2022/08/SCDAA-Convention-2020\\_Presentation-Slides.pdf](https://sickcells.org/wp-content/uploads/2022/08/SCDAA-Convention-2020_Presentation-Slides.pdf)

<sup>27</sup> Linthicum, page

<sup>28</sup> Bradt P, Spackman E, Synnott PG, Chapman R, Beinfeld M, Rind DM, Pearson SD. Crizanlizumab, Voxelotor, and L-Glutamine for Sickle Cell Disease: Effectiveness and Value. Institute for Clinical and Economic Review, January 23, 2020, p. 89. [https://icer.org/wp-content/uploads/2020/10/ICER\\_SCD\\_Evidence\\_Report\\_031220-FOR-PUBLICATION.pdf](https://icer.org/wp-content/uploads/2020/10/ICER_SCD_Evidence_Report_031220-FOR-PUBLICATION.pdf)

<sup>29</sup> ICER, “Assessment: Hereditary Angioedema,” *Institute for Clinical and Economic Review*, last modified August 24, 2021, <https://icer.org/assessment/hereditary-angioedema-2018/>.

*Item 18: Characterizing heterogeneity: Describe any methods used for estimating how the results of the study vary for subgroups.*

*Item 19: Characterizing distributional effects: Describe how impacts are distributed across different individuals or adjustments made to reflect priority populations.*

*Item 26: Study findings, limitations, generalizability, and current knowledge: Report key findings, limitations, ethical or equity considerations not captured, and how these could affect patients, policy or practice.<sup>30</sup>*

To gain the trust of patient and disability stakeholders partnering in this work, HTA organizations and others conducting value assessments will need to prioritize increased transparency of their methods, the data that informs their assessments, and the algorithms that are used to make conclusions. One positive model is IVI's Open-Source Value Project, which uses a process for developing disease-specific value assessment models intended to promote "collaboration, exploration, and innovation." Their model materials, from methods documentation to the open-source code, are freely available for download and use, and all stakeholder input is publicly released.<sup>31</sup> This level of transparency allows the models to be accessible and usable to all stakeholders.

HTA organizations and others conducting value assessments have a meaningful opportunity to participate in the co-creation of non-biased tools in health care, to purposefully correct the value assessment of the past that has perpetuated health inequities, and to do better in the future. Otherwise, HTA risks being marginalized by dependence on flawed data and methods to serve profit-making enterprises over the people in need of health care.

***Recommended Focus for HTA Organizations and Others Engaged in Testing and Validating New Methods to Eliminate Bias***

- **Ensure methods incorporate the values of patients and people with disabilities, starting with systematic reviews and direct engagement with the patient and disability communities to ensure their values are considered in the base case:**
  - HTA organizations and others conducting value assessments should consider allocating resources to the conduct of a comprehensive systematic review of the condition for which a treatment is being assessed to gain insights from the literature about patient preferences that could be incorporated into the earliest scoping

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<sup>30</sup> Don Husereau, et al, "Consolidated Health Economic Evaluation Reporting Standards (CHEERS) 2022 Explanation and Elaboration: A Report of the ISPOR CHEERS II Good Practices Task Force," *Value Health* 25:1, published June 2022: 10-31, <https://doi.org/10.1016/j.jval.2021.10.008>.

<sup>31</sup> IVI, "Open-Source Value Project," Innovation and Value Initiative, last modified 2022, <https://www.thevalueinitiative.org/open-source-value-project/>.

document on which patients, people with disabilities, and other stakeholders will provide input.

- HTA organizations and others should consider strategies to advance methods identifying the values of a represented subgroup and incorporate those values meaningfully into the base case value assessment.
- **Avoid valuing treatments based only on averages and avoid reliance on QALYs, instead using methods that intentionally allow for consideration of patient differences:**
  - HTA organizations and others should develop, identify, and incorporate new methods for value assessment that allow for identifying and interpreting the heterogeneity of treatment impact, consistent with the ISPOR recommendations.
  - HTA organizations and others should cease using the QALY metric, which is documented to have discriminatory implications.
  - HTA organizations and others should explicitly state the distributional impacts of a cost effectiveness analysis in relation to a population's experience of social disadvantages, consistent with ISPOR's recommendations.
  - HTA organizations and others should consider strategies to conduct value assessment using methods that consider intersectionality based on social identities and, where data gaps exist, explicitly acknowledge the limitations for making reliable conclusions for a particular subpopulation.
- **Retrospectively review previous value assessments with an overlay of real-world evidence and consideration of factors impacting health equity:**
  - HTA organizations and others should invest resources in retrospectively reviewing their previous value assessments with an overlay of real-world evidence and improved societal understanding of social and structural determinants of health. This should be geared toward addressing their potential to drive health inequity where the earlier conclusions may have been biased, potentially changing the conclusions, improving health care decisions that rely on the value assessment, and allowing for a stronger understanding of the limitations of the conclusions for decision-makers.
- **Increase transparency of methods:**
  - HTA organizations and others should increase the transparency of their methods and consider strategies such as an open-source data model.
  - HTA organizations and others should consider the recommendations of ISPOR related to study findings, limitations, generalizability, and current knowledge, as well as take steps to report key findings, limitations, ethical or equity considerations not captured, and how these could affect patients, policy, or practice.<sup>32</sup>

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<sup>32</sup> Don Husereau, et al, "Consolidated Health Economic Evaluation Reporting Standards (CHEERS) 2022 Explanation and Elaboration: A Report of the ISPOR CHEERS II Good Practices Task Force," *Value Health* 25:1, published June 2022: 10-31, <https://doi.org/10.1016/j.jval.2021.10.008>.

# Part Six

## *Incorporating Best Practices for Engagement and a Meaningful Voice for Stakeholders*

**H**TA organizations, as well as others conducting value assessments, that are seeking to center their value assessments on advancing health equity will find many best practices in the engagement processes of participatory researchers and social work researchers. Comparable to steps taken by PCORI, incorporating these best practices into HTA would advance a culture shift that values the input of people with lived experience in the health system. Improved data and methods are difficult to achieve without this shift in culture.

For example, PCORI was created with the goal of doing research differently and centering its comparative clinical effectiveness research on measuring outcomes that matter to patients, recognizing the heterogeneity among patient subpopulations. In the years following PCORI's creation, its Patient Engagement Advisory Panel (PEAP) identified best practices for engagement and sought to directly address the challenges facing PCORI-funded researchers that had little experience with engaging patients and people with disabilities throughout the research process. The PEAP developed and published an Engagement Rubric for applicants that outlined very specific steps for engaging patients and other stakeholders in the planning, conduct and dissemination of a funded study.<sup>33</sup> Similarly, the PEAP published a Compensation Framework that called for fair financial compensation to the patients, caregivers, and patient/caregiver organizations' contributing to the research, reflecting the value of their commitments of time and effort.<sup>34</sup> Finally, the PEAP provided guidance to its funded partners on budgeting for these engagement activities.<sup>35</sup>

Building on this background, in 2021 the PEAP published Equity and Inclusion Guiding Principles that provided more specific recommendations, calling for “critical engagement with historically disenfranchised groups whose interests have not been consistently centered due to systemic devaluations based on race, ethnicity, income, geography, age, sexual orientation, disability, and other characteristics.” The PEAP identified inclusion as a key principle, demonstrated by authentic engagement and active listening, as well as attention to the beliefs and fears that could restrain participation, alongside acknowledgement of the historical and cultural context members bring to the team. The PEAP recommended avoiding terms like “minority, underrepresented, marginalized.” Their inclusion principle emphasized that stakeholders should engage as allies and representatives

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<sup>33</sup> PCORI, “Engagement Rubric for Applicants,” *Patient-Centered Outcomes Research Institute*, last modified June 6, 2016, <https://www.pcori.org/sites/default/files/Engagement-Rubric.pdf>.

<sup>34</sup> PCORI, “Financial Compensation of Patients, Caregivers, And Patient/Caregiver Organizations Engaged in Pcori-Funded Research as Engaged Research Partners,” *Patient-Centered Outcomes Research Institute*, published June 10, 2015, <https://www.pcori.org/sites/default/files/PCORI-Compensation-Framework-for-Engaged-Research-Partners.pdf>.

<sup>35</sup> PCORI, “Budgeting for Engagement Activities,” *Patient-Centered Outcomes Research Institute*, last modified October 2020, <https://www.pcori.org/sites/default/files/PCORI-Budgeting-for-Engagement-Activities.pdf>.

of individuals not invited to participate or who may be unable to engage due to burdens such as cost. A second key principle was equitable partnerships, calling for giving engaged stakeholders respect, opportunities for co-creation, meaningful roles, decision-making authority defined collaboratively with the research team, and equitable compensation. Trust and trustworthiness were a principle described as being earned and not static, requiring research team members to explicitly acknowledge as well as understand factors impacting trustworthiness (culture, experience), such as how research has played a role in constructing racial hierarchies and imposing trauma. Finally, the principles of accountability and actionability underscore the need for awareness that research team behaviors and decisions will naturally gravitate toward past norms unless consistently challenged toward equity and inclusion ideals. Strategies for improvement include ongoing evaluation of the partner experience with modifications as necessary for accountability and improvement that also consider power differentials on decision making.<sup>36</sup>

For value assessment, meaningful engagement is especially challenged by the limited resources of patient and disability stakeholders that stand to benefit from the studied treatment. Even larger patient organizations rarely have an epidemiologist or health economist on their staff. Advocacy organizations serving communities that are largely underserved in our health care system tend to be underserved themselves. Therefore, mounting a data collection process that is professionally designed with well-constructed questions and disseminated in a manner that is powered to get the input needed to be broadly representative is not an easy task, typically being far outside their normal scope of business. Yet, it is precisely the organizations representing people with lived experience that should be driving the data collection process because they are trusted and credible resources in their communities.<sup>37</sup>

We urge HTA organizations and others conducting value assessments to recognize the investment, partnership, technical assistance, and other resources that would enable organizations representing people affected by the studied treatment to themselves collect, curate, and generate the necessary data to effectively and productively participate in a value assessment process. For example, we suggest the research enterprise allow for and provide resources to pay for professional consultants and health economists with an expertise in health equity to contract with these representative organizations and to be ethically dedicated to them in their work with HTA organizations or other entities conducting a value assessment. An organization's contracted expert should also be given a seat at the table in the value assessment process. When these representative organizations have the capacity to participate meaningfully, whether directly by their staff and/or with assistance from their own dedicated health economist, a value proposition centered on the outcomes that are most important to improve health and advance health equity is more likely to emerge.

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<sup>36</sup> PCORI's Advisory Panel on Patient Engagement, "Equity and Inclusion Guiding Principles," *Patient-Centered Outcomes Research Institute*, March 2021, <https://www.pcori.org/sites/default/files/Equity-and-Inclusion-Guiding-Engagement-Principles.pdf>.

<sup>37</sup> Mark Linthicum, MPP, et al, "Finding Equity in Value: Racial and Health Equity Implications of U.S. HTA Processes, published 2022," page 20, [https://sickcells.org/wp-content/uploads/2022/10/IVI\\_Sick-Cells\\_Equity-in-Value\\_2022.pdf](https://sickcells.org/wp-content/uploads/2022/10/IVI_Sick-Cells_Equity-in-Value_2022.pdf).



PCORI recognized early on the need to improve the capacity for patient engagement and developed programs to build that capacity.<sup>38</sup> For example, through a Eugene Washington PCORI Engagement Award, the National Health Council's Value Work Group was able to develop a series of resources as part of their Value Classroom providing introductory information about health economics and other resources for patient and disability stakeholders engaged in a value assessment process.<sup>39</sup> The result is a useful set of tools from a trusted source that is focused on advancing value assessment centered on patients. HTA organizations and others that truly want their engaged partners in the patient and disability communities to participate meaningfully in their value assessment work should encourage them to take advantage of these kinds of resources developed to empower their effective participation in a value assessment process.

In addition to its methodological recommendations, the ISPOR CHEERS 2022 report also included recommendations related to the engagement processes of conducting HTA. The report recommended a strong focus on patient engagement in Item 21, calling on HTA organizations to describe “any approaches to engage patients or service recipients, the general public, communities, or stakeholders (such as clinicians or payers) in the design of the study.”<sup>40</sup> We are encouraged that ISPOR has also come to recognize the importance of patient engagement as part of the value assessment process.

***Recommended Focus for HTA Organizations and Others Seeking Meaningful Engagement from Diverse Stakeholders in the Patient and Disability Communities:***

- **Consider models from PCORI and participatory researchers to develop protocols for engagement that make equity and inclusion a high priority:**
  - HTA organizations and others conducting value assessments should consider PCORI's Engagement Rubric, Compensation Framework and guidelines to budget for stakeholder engagement as potential models for developing their own protocols for meaningful engagement of people with lived experience.
  - HTA organizations and others should consider PCORI's Equity and Inclusion Guiding Engagement Principles as a potential model for establishing processes that will facilitate increased and more meaningful participation from subpopulations that have historically been devalued or harmed by HTA, thereby building trust by explicitly recognizing the past harm posed by HTA and committing to work collaboratively to assure future HTA is centered on improving health equity.
  - As HTA organizations and others take steps to update their value framework and methods to be centered on health equity, we recommend the added engagement of participatory researchers with experience engaging patients and communities in their work in order to support consideration of best practices.

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<sup>38</sup> PCORI, “Eugene Washington PCORI Engagement Awards,” *Patient-Centered Outcomes Research Institute*, last modified July 19, 2022, <https://www.pcori.org/engagement/eugene-washington-pcori-engagement-awards>

<sup>39</sup> NHC, “Education: Value Classroom,” *National Health Council*, published 2021, <https://nationalhealthcouncil.org/education/value-classroom/>.

<sup>40</sup> Husereau, “Consolidated Health Economic Evaluation Reporting Standards (CHEERS) 2022 Explanation and Elaboration: A Report of the ISPOR CHEERS II Good Practices Task Force,” 10-31.

- **Value engagement by providing engaged patient and disability partners with resources to participate effectively:**
  - HTA organizations and others should consider strategies to equitably compensate engaged stakeholders representing patients and people with disabilities for their participation in a value assessment process.
  - HTA organizations and others should consider providing sufficient resources to engaged organizations representing the impacted populations of patients so that they are able to hire outside experts, such as a health economist, to work in the best interest of patients during the value assessment process.
  - HTA organizations and others should work to ensure that engaged stakeholders representing people with lived experience have access to training and resources, such as the NHC Value Classroom, that will increase their capacity to engaged in a value assessment process and assure that they are broadly representing those people with lived experience that are not participating.
  
- **Evaluate and improve engagement practices to correct any systemic disadvantages to engaged partners, clearly stating how affected patient and disability stakeholders were engaged in published HTA:**
  - HTA organizations and others should evaluate the partner experience and make modifications as needed to ensure that systemic disadvantages to any participants are addressed in real time.
  - HTA organizations and others should state clearly as part of a published HTA how affected patient and disability stakeholders were engaged in the design of the study, consistent with ISPOR's recommendation.

# Part Seven

## *Conclusion*

**W**hen the benefit designs that drive coverage and the clinical guidelines that drive provider decisions are built on biased HTA that relies on flawed science, incomplete data, and discriminatory algorithms, health inequities are a natural consequence. The combined efforts of all health care stakeholders will be needed to achieve progress in our work toward health equity, including people with lived experience, payers, providers, policymakers, and HTA organizations. It will be imperative for HTA organizations and others conducting value assessments to engage stakeholders from the patient and disability communities early and, in collaboration with them, identify at the onset of a value assessment the limitations that may be inherent within the data that drives its conclusions, ultimately sharing in the responsibility to advance efforts to fill those data gaps. If HTA organizations and others want to do their part to address the cycle that perpetuates health disparities for certain subpopulations, data limitations must be addressed alongside improved methods capable of eliminating bias and recognizing social and structural determinants of health.

Progress requires self-awareness of the past and present, as well as a commitment to ongoing work to identify and address traditional assumptions about the value of health care that may entrench health inequity. We understand that there is ongoing work to develop concrete and actionable changes to the HTA process by HTA organizations that recognize the need fix the systemic challenges described in this report. As HTA organizations and others strive to do better in the future by centering their work on health equity, we hope that these recommendations provide useful guidance to prioritize their future work.