

## **Medicare Drug Price Negotiation Program**

### **PIPC Recommendations for a Process Centered on Patients and People with Disabilities**

The [Inflation Reduction Act](#) (IRA) provides the Center for Medicare and Medicaid Services (CMS) with unprecedented new authority to reduce drug prices for America's seniors through the Maximum Fair Price (MFP) provisions. Importantly, the MFP provisions of the law also include provisions to protect patients and support patient-centeredness.

For over 10 years, the Partnership to Improve Patient Care (PIPC) has been at the forefront of the movement toward patient-centeredness in health care. CMS has a crucial opportunity to continue advancing this goal throughout the implementation of the Medicare Drug Price Negotiation Program. As CMS makes decisions under the Medicare Drug Price Negotiation Program to improve drug affordability, it is vital for the agency to center its decisions around patients and people with disabilities.

PIPC provides recommendations below that center on three pillars of patient-centeredness: 1) creating strong procedures for meaningful patient engagement; 2) establishing patient-centered standards and outcomes; and 3) rejecting the use of biased cost-effectiveness standards.

#### **Background:**

The IRA charges the Secretary of the Department of Health and Human Services (HHS) with undertaking a negotiation process to determine an appropriate MFP for some prescription drugs. In order to undertake this work, the Secretary is charged with developing and using a consistent methodology and process for negotiations. The IRA (Section 1194(e)) charges the Secretary with considering certain elements including whether a drug represents a therapeutic advance, comparative clinical effectiveness of the selected drug and its therapeutic alternatives, and whether the selected drug addresses unmet medical needs. As CMS builds a process for how it plans to undertake these new charges, it will be important that the agency is keeping beneficiary needs — patients and people with disabilities — at the forefront of its decision-making.

Some elements that are important to patients and people with disabilities are built into the statute. For example, the language that prohibits the use of comparative clinical effectiveness research in such a way that treats extending the life of an elderly, disabled, or terminally ill individual as of lower value than extending the life of an individual who is younger, non-disabled, or not terminally ill. The IRA clarifies that CMS cannot rely on discriminatory metrics, like the Quality-Adjusted Life Year (QALY), which is deeply important to the patient and disability communities. Other areas of importance — like a clear avenue for engagement throughout the process — are not spelled out, and it is critical that CMS put clear processes in place to protect the patient voice.

To ensure that the Medicare Drug Price Negotiation Program serves beneficiaries, it will be important to ensure that patients can access the care that shows them the most value, which is a therapeutic benefit. There is a robust body of ongoing research looking to understand therapeutic benefits to patients and how to conduct high-quality clinical-effectiveness research. As CMS develops its program, PIPC believes it will be important for CMS to utilize this research to ensure that CMS accurately captures therapeutic benefits to beneficiaries. PIPC encourages CMS to review the ongoing work being done to assess the

therapeutic benefit to patients and people with disabilities, as well as incorporate these findings into its process.

PIPC is pleased to share the perspectives and priorities of organizations seeking to advance definitions of treatment benefits and health care value that are centered on patients and people with disabilities, as well as recommendations reflecting common ground. We hope that this information is useful to CMS as the agency works to identify the data elements and sources of evidence appropriate for establishing therapeutic benefit and for comparing alternative treatments.

### **Executive Summary:**

As noted by stakeholders and policymakers — including Senator Bob Casey — during the debate around the passage of the IRA, engagement of affected patient and disability stakeholders is essential to the CMS process. Below are our recommendations, which focus on how CMS can establish a process that engages and incorporates the views of patients, people with disabilities, and caregivers. In support of our recommendations, we cite pre-existing work conducted in this area by patient and disability groups, disability policy experts, and federal agencies. We believe these recommendations will be useful to CMS in developing evidentiary standards and engagement practices that ensure the benefit to patients and people with disabilities are made central to decision-making.

### **CMS should establish a negotiation process that allows for continuous, robust engagement of patients and people with disabilities at multiple levels.**

- CMS should create an **ombudsman** for the Medicare Drug Price Negotiation Program that serves as a central point of input for patients and people with disabilities, similar to FDA’s Patient Affairs office or PCORI’s Director of Patient Engagement. The ombudsman should be an individual with significant experience in patient engagement, familiar with the organizations representing patients and people with disabilities, and responsible for ensuring a feedback loop that allows input to be disseminated to decision-makers at CMS and responses to be disseminated back to those providing input.
- **Solicit input from patients and people with disabilities early** in the drug price negotiation process, giving stakeholders time to collect and provide meaningful comments. CMS likely will need to begin seeking input from patients and caregivers very early in the process so that CMS can consider it along with other inputs before the agency makes an “initial offer” of a Maximum Fair Price. This could include, for example, CMS convening **public roundtables** of disease or treatment-specific experts from the patient and disability communities, as well as their caregivers, for each drug selected for MFP negotiation.
  - This process should look similar to the process used by FDA to engage patients as part of Patient-Focused Drug Development, both as part of externally-led meetings and FDA-led meetings.
  - Another potential reference point is the engagement process used by PCORI to identify the outcomes that the organization values. CMS should similarly engage patients and people with disabilities in establishing a predictable process for engagement related to its consideration of data elements about a selected drug, the evidence used in consideration of factors in statute used to assess therapeutic value, and its alternative therapies.

- CMS should share the non-proprietary evidence that they are considering for unmet need, comparative research and therapeutic advance and solicit feedback about its relevance to the needs, and outcomes and preferences of patients. CMS should also solicit other patient data sources from patients and people with disabilities that may have their own resources for collecting data.
- CMS should solicit input from **diverse** communities that can share information about the differences among subpopulations and their needs, outcomes, and preferences.
- CMS should provide **resources needed for effective engagement** of patients and people with disabilities should be provided.
  - Resources may include financial assistance to participate in meetings and roundtables or to generate real-world evidence through surveys, making meetings accessible to people with disabilities, providing materials in accessible formats, or allowing an extended time for input and comments.
  - This recommendation is consistent with best practices supporting engagement, particularly supporting engagement of those historically not engaged, as consistently reflected in the work of PCORI, PIPC, and NHC.
- CMS should **seek input on topics that are relevant to people with disabilities, patients and caregivers** and should clearly describe these topics to stakeholders in advance. This engagement could include, for example, feedback on relevant treatment alternatives, outcomes that matter to patients, and the relative importance of these outcomes.
- CMS decisions should be **sufficiently transparent** so that people with disabilities, patients, and caregivers can see the extent to which their input was considered in the agency’s decisions.
- CMS should **ensure that information gathered during public comment periods and meetings is reflected in the final guidance** that CMS will publish in advance of the first year of negotiations, advancing the principle of transparency that is supported across organizations.
- CMS should engage patients and people with disabilities **to assess any unintended consequences**, including the impact on access to treatment, cost-sharing implications, or otherwise.
  - As analyzed by PIPC, NCD, and DREDF, restricted access implications have been experienced in countries relying on methods for assessing value that fails to capture the real-world value to patients.

**When developing its offer for MFPs, CMS should ensure it is prioritizing assessing therapeutic benefit and considering value through the lens of how patients and people with disabilities experience and value their health care.**

- CMS should define **unmet need** based on the patient perspective and whether a treatment meets their needs, outcomes, and preferences in a manner unmet by other treatments, consistent with the PCORI’s statutory **charge** to address the “needs, outcomes and preferences” of patients.
  - Unmet need should be defined in a manner that acknowledges the experiences of people living with a condition who may value a treatment with fewer side effects, modes of administration that do not require travel, frequency of administration, etc. The CMS definition should prioritize how a treatment advances adherence and improved quality-of-

life as indicated by engaging patients and people with disabilities and use of patient-level data.

- Unmet need should not be defined by the averages, but instead take into consideration the subpopulations that may not benefit from existing therapies due to their unique characteristics or for whom those therapies are not accessible due to social determinants of health (SDOH).
- **CMS should define *comparative clinical effectiveness research*** in a manner consistent with the existing definition in the Affordable Care Act (ACA).
  - The ACA stated, “The terms ‘comparative clinical effectiveness research’ and ‘research’ mean research evaluating and comparing health outcomes and the clinical effectiveness, risks, and benefits of 2 or more medical treatments, services, and items...” making it clear that such research does not involve cost comparisons or cost-effectiveness.
  - The ACA also barred in Medicare, as well as PCORI, the use of QALYs and similar metrics that are historically used in cost-effectiveness analyses, further underscoring that such research should not rely on costs.
  - In determining what comparative effectiveness research to rely on, CMS should consider engaging patients and people with disabilities to understand their perspectives on the quality of the research available and whether it represents their preferred outcomes and experiences.
  - The comparator matters and should reflect a clinically comparable treatment as indicated by patients and their clinicians as opposed to selecting a comparator based on its cost, a lesson learned from countries such as Germany and a key component of efforts to advance innovative methods.
- CMS should determine whether a treatment reflects a ***therapeutic advance*** based not only on the clinical trial data but evidence that reflects **what patients and people with disabilities value about their care and outcomes**.
  - CMS will need to engage specific patient and disability communities with the condition treated by a selected drug to determine their specific priorities for improving their quality of life with treatment, a theme consistent in calls for improved patient engagement in research and decision-making.
  - Studies related to therapeutic advancements should reflect the diversity of the patients being treated and their differences.

**CMS should set standards for high-quality, patient-centered evidence that will drive investment in the development and testing of innovative methodologies that are inclusive and advance health equity.**

- Standards established by CMS should recognize and address the **shortcomings of historic methods**.
- CMS should rely on standards developed by leading patient and disability organizations to determine whether the evidence that it intends to rely on for the development of an initial MFP offer is centered on patients and people with disabilities.

- To determine what evidence meets CMS standards, the agency should look to organizations — such as those representing affected patients, people with disabilities, as well as experts among practicing physicians and providers — as potential reference points as they would be most familiar with the usefulness of the evidence base for making decisions.
- As previously stated, CMS should prioritize evidence that is patient-centered and captures value for patients, caregivers, and persons with disabilities.

**CMS should follow the NCD’s recommendation not to rely on QALYs or similar metrics as a factor for determining therapeutic benefit or “value.”**

- CMS should **clarify in guidance and/or regulations that it will not rely on QALYs or similar metrics.**
  - This recommendation is consistent with ACA’s statutory ban on the use of QALYs and similar metrics in coverage, reimbursement, and incentive programs in Medicare decisions.
  - This recommendation would also uphold the IRA’s requirement that the comparative clinical effectiveness research factored into determinations of therapeutic benefit do not discriminate.
- **CMS should avoid consideration of any evidence that is informed by QALYs or similar metrics such as the equal-value of life year gained (evLYG),** a metric recently created by the Institute for Clinical and Economic Review (ICER) to supplement the QALY that similarly discriminates based on age and has similar shortcomings for accounting for quality-of-life improvements.

**PIPC Recommendations for Advancing Patient-Centeredness in Drug Price Negotiation:**

PIPC’s recommendations are rooted in long-standing efforts by several organizations, both inside and outside government, to improve the evidence base for comparing treatments and measure their therapeutic benefit and “value.” We highlight the work conducted by PIPC, PCORI, FDA, NCD, IVI, NHC, and DREDF. They have each worked to identify how people across subpopulations and ages that live with disabilities and chronic conditions value their care, the elements of which may be useful for CMS and demonstrate where there is common ground among patients and people with disabilities, as well as experts in disability policy and research. A central theme among these organizations is the need to understand what people living with the conditions to be treated value and the outcomes they want from their care.

**Engagement of patients and people with disabilities should happen throughout the CMS process.**

There is broad consensus among policymakers and leaders in the field of patient-centered outcomes research that robust engagement of people with lived experience is essential to measuring the therapeutic impact of a treatment. For example, FDA has developed a [structure](#) and accumulated experience in efforts to engage patients. In developing an internal process, the FDA created the [Patient Engagement Collaborative](#), a group of patient organizations and individual representatives who discuss how to achieve more meaningful patient engagement in medical product development and other regulatory discussions at the FDA. The program aims to systematically obtain patient perspectives on specific diseases and their treatments. As part of [Patient-Focused Drug Development](#), the FDA’s focus is on clinical outcome assessments (COA) to measure outcomes of importance to patients, which represents a process consistent with the definitions of comparative clinical effectiveness research in

both the ACA and IRA. Additionally, the FDA strives to promote collection of patient experience data as means to identify patients as partners and co-developers for the selection of COAs. To achieve these goals, the FDA has created an [Office of Patient Affairs](#) as a central entry point for coordination with affected patients.

Similarly, PCORI has created several advisory panels to inform its comparative clinical effectiveness research, including a patient engagement advisory panel known as the [PEAP](#). Early in its creation, the PEAP recognized that the researchers funded by PCORI needed guidance on patient engagement and developed a Patient Engagement [Rubric](#) to guide them.

IVI is also invested in engaging patients as they work toward improved methods for assessing the value of treatments. IVI has invested in a diverse Board of Directors, Patient Advisory Committee, and Health Equity advisors as part of the ongoing development of their value assessment process utilizing a multi-criteria decision analysis. For example, IVI's [Rare Disease Initiative](#) is a collective effort with EveryLife Foundation for Rare Diseases, funded by PCORI. The project reflects a cooperative process to plan for the development of new approaches in patient research and value assessment, ultimately advancing our understanding of patient-centered outcomes in rare disease. The intent is to work together with patients and other stakeholders in a series of roundtable dialogues to inform project outcomes.

We sought to provide recommendations to CMS that build on this foundation of engagement. Understanding that patients and people with disabilities are unsure of the best avenues to engage with CMS outside of a formal comment period, we recommend an ombudsman position for the Medicare Drug Price Negotiation Program that would serve as a central point of input for patients and people with disabilities. We also urge CMS to establish a predictable and transparent process for engaging expert patients and people with disabilities who have lived experience, as well as their caregivers, in the assessment of each selected drug. Predictability is an important principle for organizations representing patients and people with disabilities that do not have significant resources to monitor CMS' activities and react quickly. To ensure CMS is receiving appropriate input from affected patients and people with disabilities, we urge a predictable process for engagement related to its consideration of data elements about a selected drug, the quality of evidence used in consideration of factors in statute used to assess therapeutic value, and alternative therapies. Diversity of input is also important if CMS is to understand the potential health equity implications of its work emanating from the differences among subpopulations related to their needs, outcomes, and preferences.

Because the CMS process may primarily involve the use of pre-existing evidence as opposed to the generation of new evidence, we urge CMS to share with affected patients and people with disabilities the non-proprietary evidence that they may consider for unmet need, comparative research, and therapeutic advance, as well as solicit feedback about its relevance to the needs, outcomes, and preferences of people with lived experience. CMS should also solicit other relevant data sources from patients and people with disabilities that may have their own resources for collecting real-world data. The most reliable source for evidence centered on patients and people with disabilities will be the organizations representing these specialized populations.

We appreciate that this data gathering will need to happen through a process that allows CMS to meet the MFP decision deadlines set in statute. At the same time, we believe it is vital for the agency to

achieve patient-centered procedures within these deadlines. Achieving this goal will likely require the agency to begin engaging patients and people with disabilities very early in the process.

In addition, patients and people with disabilities should be continuously engaged as CMS monitors for any unintended consequences, including the impact of decisions on access to treatment, cost sharing or otherwise. Analyses by [PIPC](#), [NCD](#), and [DREDF](#) show that restricted access implications have been experienced in countries relying on methods for assessing value that fail to capture the real-world value for patients. NHC has also worked to understand how the results of a value assessment may impact decision-making about care options. As part of [patient input](#), NHC identified unintended consequences such as challenges in access to care as a key component. For example, in a [dialogue](#) between patients and employers, a recommendation emerged in favor of an “access alert template” that could be filled in by patient organizations when they become aware of access challenges experienced by patients. Addressing access challenges early is essential to mitigate these concerns.

Resources needed for effective engagement of patients and people with disabilities should be provided — consistent with best practices supporting engagement — particularly supporting engagement of those historically not engaged, as consistently reflected in the work of PCORI, PIPC, and NHC. For example, PCORI’s PEAP provided [guidance](#) to researchers on compensating patients for their role in the research design and implementation process, as well as [budgeting](#) for patients to take on these roles.

For engagement to be meaningful, information gathered — whether as part of public comment periods or other engagements — should be reflected in the final guidance, regulation or decision, to advance the widely supported principle of transparency.

**CMS’ process should ensure the factors listed in the IRA to be considered in assessing therapeutic benefit are prioritized and considered through the lens of how patients and people with disabilities experience and value their health care.**

Over the years, PIPC and others have developed [recommendations](#) for policymakers focused on ensuring that the evidence on which decision-makers rely is centered on the needs, preferences, and outcomes of patients and people with disabilities. In [reauthorizing](#) PCORI in 2019, Congress responded to the need for high-quality information centered on patients and people with disabilities by giving PCORI explicit [authority](#) to collect and analyze the costs and burdens associated with conditions being researched. Since then, PCORI has engaged with expert stakeholders and the broader community to assess the components of “patient-centered value in health and health care.” It may be useful to CMS to review the most recent PCORI [report](#) which gathered views on the components and attributes of patient-centered value; measurement of patient-centered value and related data collection, and; gaps in information needed to measure patient-centered value in health and health care.

#### *Unmet Need*

Consistent with PCORI’s statutory [charge](#), we urge CMS to define the factor of unmet need, as called for in the IRA, based on patient perspectives of existing care and whether it meets their needs, outcomes, and preferences. Unmet needs should be defined in a manner that acknowledges the experiences of people living with a condition who may value treatment with fewer side effects, modes of administration that do not require travel, frequency of administration, etc.

The [FDA's definition](#) of “unmet need” is not analogous, as it was created to determine whether a new drug qualifies for Fast Track approval, making their definition less relevant to considerations related to therapeutic benefit drugs that have been on the market and widely used in the general population. A new CMS definition should prioritize how a treatment: 1) advances adherence; 2) improves quality-of-life as indicated by engaged patients and people with disabilities; and 3) and uses patient-level data.

Unmet need should not be defined by the averages of the aforementioned metrics, but instead take into consideration the subpopulations that may not benefit from existing therapies due to their unique characteristics or access issues related to SDOH. Engagement of subpopulations to better understand their unique experiences with health care is consistently [supported](#) among organizations leading the field of patient-centered research and health equity.

### *Comparative clinical effectiveness research*

When PCORI was [authorized](#) by Congress in 2010, its statute called for the institute to conduct comparative clinical effectiveness research, with an emphasis on the preferences, needs, and outcomes of patients, as well as acknowledgement of the differential outcomes among subpopulations, avoiding conclusions based on averages or QALYs. We urge CMS to define comparative clinical effectiveness research or comparative research consistent with the existing definition in the ACA, which states, “The terms ‘comparative clinical effectiveness research’ and ‘research’ mean research evaluating and comparing health outcomes and the clinical effectiveness, risks, and benefits of 2 or more medical treatments, services, and items...” Additionally, the IRA and ACA clearly indicate that comparative clinical effectiveness should not involve the use of QALYs and similar metrics. The [ACA](#) barred Medicare from using discriminatory metrics in decision-making and explicitly barred use of cost-per-QALY and similar metrics in both PCORI's comparative clinical effectiveness research and in Medicare decisions.

PCORI was created in [response](#) to the conduct of comparative effectiveness research that was heavily academic and not informed by the needs, outcomes, and preferences of patients and people with disabilities. Therefore, it was often not useful to health care decision-making. For CMS, engagement of patients and people with disabilities is essential to understanding their perspectives about the quality of the comparative research available and whether it represents their preferred outcomes and experiences. Moreover, the comparator matters and should reflect a clinically comparable treatment as indicated by patients and their clinicians. This is preferable to selecting a comparator based on its cost, a lesson learned from countries such as Germany and a key component of efforts to advance innovative methods.

In 2022, PIPC [researched](#) the German health care system in an effort to discern lessons applicable to the U.S. Some policymakers have referenced Germany's system as a potential framework for assessing therapeutic benefit that does not use discriminatory QALYs or similar metrics such as the evLYG. PIPC's analysis found drawbacks in the German system due to its limitations on acceptable evidence. These limitations create a host of issues for their final recommendations, such as the inclusion of inappropriate comparators that are not clinically similar, a failure to include real-world sources, the inclusion of restricted endpoints to show value that excluded health outcomes important to patients, a failure to capture how treatments differ among subpopulations, and a lack of meaningful patient engagement. While Germany's use of a clinical approach is a step in the right direction, their shortcomings would need to be addressed by CMS through the development of standards for acceptable, patient-centered comparative research.

## *Therapeutic Advance*

Whether a treatment reflects a therapeutic advance should not rely solely on the early clinical trial data that is relied upon in the FDA approval process, but should reflect what patients and people with disabilities value about their care and outcomes in the real world. It is important to recognize that the treatments being assessed by CMS are not new, making real-world evidence a reasonable expectation.

For example, the shortcomings of historic value assessments specific to cell and gene therapies were highlighted in 2020 as part of a report entitled, "[Value for Whom? Incorporating Patient Perspectives into Value Assessment for Novel Cell and Gene Therapies](#)," conducted by PIPC in partnership with the EveryLife Foundation for Rare Diseases. The report concluded that there were several challenges that needed to be addressed to achieve comprehensive, patient-centered value assessment for cell and gene therapies. For example, the duration of clinical benefits may not be known or understood based on available data. Especially for rare conditions, subgroup analyses are not considered, instead relying on averages and discriminatory QALYs to provide a generic measure of health or disease burden, as well as using utility scores that may not represent the patient experience. Outcomes that matter to patients such as productivity, caregiving, and hope are often omitted from considerations of value. These outcomes are important considerations for CMS as the agency solicits evidence and considers whether they are reliable for making decisions.

In determining whether a treatment reflects a therapeutic advance, the [work](#) of PCORI may be useful in identifying the attributes of patient-centered value. This includes attributes focused on improvements related to side effects, caregiver burdens, and more. Yet, CMS will need to engage specific patient and disability communities with the condition treated by a selected drug to determine priorities for improving their quality of life with treatment, a theme consistent with calls for improved patient engagement in research and decision-making. If evidence related to a therapeutic advance fails to reflect the diversity of the patients being treated and their differences, the direct engagement of patient and disability communities to understand their real-world experiences will be that much more important.

**CMS should set standards for high-quality, patient-centered evidence that will drive investment in the development and testing of innovative methodologies that are inclusive and advance health equity.**

Significant attention has been paid to improving methods for assessing and comparing treatments to be inclusive. Consistent with calls for patient-centered value assessment, PIPC, and allied organizations have long advocated for the Centers for Medicare and Medicaid Innovation ([CMMI](#)) to identify and operationalize the "patient-centeredness criteria" mandated under Section 1115A of the ACA. The statute calls for evaluation of alternative payment models (APMs) against patient-centeredness criteria, i.e. not measures solely relying on payer-centered benchmarks. Development of standards by CMS could also provide a strong start to the development of these criteria for APMs.

CMS leadership can drive innovation in the methodologies used to measure a therapeutic benefit to better capture value to affected patients and people with disabilities. While there may be no one-size-fits-all measure of therapeutic benefit, [NCD](#) recommended using nondiscriminatory methods in combination, [DREDF](#) recommends using them jointly, and [IVI](#) has advanced viewing value from different perspectives through its work on Multi-Criteria Decision Analysis. Academic entities and research

organizations will strive to meet CMS' standards as a representation of best practices, thereby improving the evidence base used to assess the effectiveness of health care. Lessons are available from entities – such as PCORI, IVI, and FDA – that are already working to foster an evidence base that is inclusive and that measures outcomes that matter to patients as a priority. Guidance from CMS on evidentiary standards would be an incentive for academia to follow their lead.

The development of standards by CMS would also provide a roadmap to entities assessing nondiscriminatory alternatives for valuing health care. The NCD recommended in their latest [report](#) that further research and development of alternative methodologies to the QALY are needed and should be led by entities such as PCORI. It will be important for CMS' standards to recognize and address the shortcomings of historic methods identified by PIPC and its partners, DREDF, NCD, and others to ensure that alternatives identified or developed meet those standards.

In establishing standards, CMS should identify the consensus among leading organizations and patient and disability experts, both in terms of the process used to develop an assessment of therapeutic benefit as well as the algorithms used to measure whether a treatment represents a therapeutic advance or unmet need. As the development of value assessments has increased, so have concerns about their failure to be centered on patients and people with disabilities and to achieve meaningful engagement from the patient and disability communities. In response, entities such as the NHC amplified their work related to health care value, patient engagement, and advancing real-world evidence. In 2016, as part of its [Patient-Centered Value Model Rubric](#), NHC identified six domains that established an early foundation for defining the patient voice in value and that continue to be referenced in evolving recommendations:

- **Patient Partnership.** Patients should be involved in every step of the value model development and dissemination process.
- **Transparency to Patients.** The assumptions and inputs into the value model itself – and each step in the process – should be disclosed to patients in an understandable way and in a timely fashion.
- **Inclusiveness of Patients.** The value model should reflect perspectives drawn from a broad range of stakeholders, including the patient community.
- **Diversity of Patients/Populations.** The value model should account for differences across patient subpopulations, trajectory of disease, and stage of a patient's life.
- **Outcomes Patients Care About.** The outcomes integrated into the value model should include those that patients have identified as important and consistent with their goals, aspirations, and experiences.
- **Patient-Centered Data Sources.** The value model should rely on a variety of credible data sources that allow for timely incorporation of new information and account for the diversity of patient populations and patient-centered outcomes, especially those from real-world settings and reported by patients directly. The data sources included should reflect the outcomes most important to patients and capture their experiences to the extent possible.

After its 10-year reauthorization and the appointment of a new Executive Director, PCORI began its development of an updated [Strategic Plan](#) through extensive stakeholder feedback, responding to calls for more targeted patient-centered outcomes research. To identify whether the evidence base reflects methodological best practices, CMS has an opportunity to look to PCORI's work to improve the research

infrastructure – including the science and methods of comparative clinical effectiveness research – in developing standards.

### ***Standards for Consideration***

**Partnership with patients and people with disabilities** should be explicit throughout the process of assessing the therapeutic benefit of treatments, allowing their input on the algorithms used to measure a therapeutic advance or an unmet need. This is a central principle expressed by PIPC and NHC and is being operationalized by PCORI. The FDA has similarly operationalized partnership and engagement in Patient-Focused Drug Development.

**Transparent and open-source models** for assessing therapeutic benefit are themes reiterated across organizations. IVI has made it a priority to operate an open-source model, following through on the NHC's identification of transparency as a domain of a patient-centered value framework, whereby assumptions and inputs at every step are disclosed to patients in an understandable way and in a timely fashion. Their [open-source value project](#) allows the public to access their methods and the data that drives their conclusions, and it prioritizes being inclusive, transparent, patient-centered, dynamic, innovative, and accessible. The NCD has similarly called out transparency as a positive feature of alternative methodologies, consistent with PIPC and DREDF. Transparency and acknowledgment are important themes, ideally leading to published work in collaboration with affected communities to understand for whom a value assessment is relevant – or not.

**Inclusion** is a key element of the engagement process for assessing therapeutic benefit, as well as for the evidence base that should reflect the diversity of people being treated. There is consensus among PIPC, partner organizations representing communities of color, IVI, and PCORI, that in order for health equity to be a priority and to be given weight in assessing therapeutic benefit, steps should be taken to ensure diversity among those engaged in the process and in the underlying research data. IVI has acknowledged the need to improve and test new methods for valuing health care that address historic discrimination and the need to openly explicate limitations for people not represented so they are not used to make decisions regarding them. Biased assessments that fail to be inclusive should not be deemed reliable sources of evidence for decision-making if the goal is to drive researchers to adopt innovative methods.

**Consideration of how a treatment can differ in its impact among subpopulations** is an essential element for addressing health equity. As discussed by PIPC and its partners, this includes the consideration of how different people may experience social determinants of health, varying access to health facilities, as well as differences based on gender, age, race, and ethnicity. By statute and in its strategic plan, PCORI has committed to emphasizing impacts on subpopulations in its funded studies.

**Consideration of outcomes that matter to patients as identified by patients** is a high priority across leading organizations. It is being operationalized in research through patient engagement activities conducted by entities such as PCORI, the FDA, and IVI. Patient-reported outcomes and other patient-driven measures allow for policymakers to understand how patients value treatment.

**Data sources centered on patients and people with disabilities** are needed to assess the therapeutic benefit of any treatment if it is to reflect the real-world experiences and needs of patients among diverse communities. That includes prioritizing the use of updated studies that reflect new information about a treatment’s impact in the real world. This principle was advanced in the statute that created PCORI, and is being operationalized within PCORI, the FDA, and IVI. It has been a long-standing priority for PIPC, as well as DREDF and NHC, that research looking at therapeutic benefit and “value” not rely solely on randomized clinical trial data. IVI’s work highlights the need for connecting data aggregation and data sharing activities with patient-designed resources for collecting real-world data, such as through registries. This approach is in contrast to just relying on the conclusions of randomized clinical trials conducted before real-world, patient-centered data was collected or used to inform health decisions. Similarly, NHC has [pushed](#) for health researchers, policymakers, and regulators to establish standards and structure for using real-world data (RWD) as evidence in regulatory and clinical decision-making that makes patients the primary focus. It has been a source of frustration for patients and people with disabilities that the research on therapeutic benefits and value often fails to incorporate RWD, instead drawing from randomized clinical trials to the exclusion of RWD that is viewed as less rigorous despite often being more relevant.

**If health equity is a priority, then it should be given weight** in assessing the therapeutic benefit of a treatment. In 2022, several organizations, including PIPC, the National Minority Quality Forum (NMQF), Global Liver Institute, Sick Cells, and the Preparedness and Treatment Equity Coalition (PTEC) collaborated in the development of a report entitled, “[Aligning Health Technology Assessment with Efforts to Advance Health Equity.](#)” The report called for new value methods and algorithms that inherently value health equity, finding that “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.” The report drew from PCORI’s [Equity and Inclusion Guiding Engagement Principles](#) that highlighted inclusion, equitable partnerships, trust and trustworthiness, and accountability and actionability, as well as [IVI’s](#) ongoing Health Equity Initiative to outline recommendations to health technology assessment (HTA) organizations to address data gaps. Specifically, the recommendations encouraged HTA organizations to include subpopulations and the differences in treatment impact among patients based on their social identities, geographic communities, and other factors, particularly in calculations of value and cost effectiveness. The report also recommended improved methods to eliminate the historic bias of health care value assessment that devalue care for people with disabilities – particularly people who are Black, Indigenous, People of Color (BIPOC) – by considering their values, avoiding reliance on averages and QALYs, increasing use of real-world evidence and consideration of factors impacting health equity, and increasing the transparency of methods. Lastly, the report recommended meaningful engagement from diverse stakeholders in the patient and disability communities that prioritize equity and inclusion, allocate resources to support engagement as well as evaluate and improve engagement practices to correct systemic disadvantages to engaged partners, alongside improved transparency about engagement practices. [Tactics](#) proposed by PIPC and others for ensuring health equity is given weight include:

- Consideration of real-world evidence inclusive of BIPOC;

- Consideration of the impact of social determinants of health on communities experiencing health disparities and the value of addressing their health needs and improving their health;
- Consideration of the historic under-investment in innovations addressing health disparities, particularly for conditions unique to BIPOC subpopulations.

**Consideration of the societal perspective** is needed to understand the benefits and value of treatments for society at large. For example, IVI has advanced consideration of societal benefits as part of its model. [PIPC](#) and [others](#) have also called for consideration of a societal perspective in consideration of the therapeutic value of treatments, such as for treating COVID-19.

**CMS should follow the NCD’s recommendation not to rely on QALYs or similar metrics as a factor for determining therapeutic benefit or “value.”**

We urge CMS to state early and often in guidance and/or regulations its intention not to rely on QALYs or similar metrics, committing to abide by the ACA’s statutory ban on the use of QALYs and similar metrics in coverage, reimbursement, and incentive programs in Medicare decisions and to uphold the IRA’s requirement that the comparative clinical effectiveness research factored into determinations of therapeutic benefit do not discriminate. CMS should avoid consideration of any evidence that is informed by QALYs or similar metrics such as evLYG, a [metric](#) recently created by the Institute for Clinical and Economic Review (ICER) to supplement the QALY that similarly [discriminates](#) based on age and has similar shortcomings for accounting for quality-of-life improvements. For example, Oregon disability advocates have [opposed](#) efforts by the Health Evidence Review Commission to simply redact references to QALYs in determining the health care treatments and services that will be covered on its prioritized list of services.

In 2019, the NCD published a report entitled, “[Quality-Adjusted Life Years and the Devaluation of Life with Disability](#).” The report raised significant concerns about the increasing reliance on QALY-based cost-effectiveness analyses by payers and policymakers due to the QALY’s inherently discriminatory methodology. In 2022, the NCD published a report on alternatives to QALYs, providing information on the benefits and risks associated of other models, particularly the risk of discrimination. The NCD emphasized the importance of patient preference data on how people value quality-of-life gains versus life-year extensions and its application in a manner that accurately reflects the different values among people with differing disabilities and coexisting conditions. NCD also emphasized public transparency of the data used to assess value as a benefit of new models such as multi-criteria decision analysis. The NCD has recommended that policymakers avoid the use of QALYs, consider using a combination of alternative methods, [bar](#) the use of QALYs across federal programs and [study](#) alternatives.

In 2021, DREDF published a report entitled, “[Pharmaceutical Analyses Based on the QALY Violate Disability Nondiscrimination Law](#)” which responded to a defense of QALYs. The report explained how using QALYs, even in tandem with alternative measures such as evLYG, violate disability nondiscrimination law. The methodological biases highlighted included the use of utility weights associated with different health states and the resulting devaluation of disabled life and the failure of QALY and evLYG to account for both the full value of life-extension and the value of quality-of-life improvement. DREDF viewed the following as positive for an alternative value framework: 1) using condition-specific measures more precisely evaluating symptoms and quality-of-life; 2) avoiding societal judgements that provide a biased view of quality-of-life for people with disabilities; and 3) using the

right therapeutic comparators as positives for an alternative model for valuing health care. DREDF applied similar values to its [work](#) against discriminatory state-based Crisis Standards of Care during the pandemic, noting that “many policies for rationing medical care were based on discriminatory assumptions about the life worth of people with disabilities (as well as higher weight persons and individuals of color who experience health disparities and higher incidences of a range of health conditions).” In its report, DREDF concluded, “there are alternative metrics in development that, when used jointly, can reduce reliance on discriminatory assumptions about people with disabilities and improve access to life-sustaining or life-improving treatments.”

## **Conclusion**

PIPC appreciates CMS’ consideration of our recommendations. CMS has an important task ahead in setting up a process to implement the negotiation provisions of the IRA. For CMS to meet its obligations to beneficiaries, it will be critically important that CMS is thoughtful in how it assesses therapeutic benefit to affected patients and that patients and people with disabilities are granted a seat at the table and a clear and robust path to engagement throughout the process. PIPC stands ready to serve as a resource to CMS and to make connections to other relevant stakeholders.