April 14, 2023

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CMS Deputy Administrator and Director of the Center for Medicare
Centers for Medicare & Medicaid Services
U.S. Department of Health and Human Services
7500 Security Boulevard
Baltimore, MD 21244-1850

Dear Deputy Administrator Seshamani:

Thank you for this opportunity to comment on the Initial Memorandum for Implementation of the Medicare Drug Price Negotiation Program. Our organizations represent the public stakeholders referenced in the guidance – the patients and people with disabilities impacted by this negotiation process. Our comments will focus on the role that we hope to play in ensuring that the agency centers its considerations on outcomes that matter to patients and people with disabilities as it implements this important new program to ensure drug affordability for individuals under Medicare.

The Maximum Fair Price (MFP) provisions of the Inflation Reduction Act (IRA) provide the Centers for Medicare & Medicaid Services (CMS) with significant new authority to reduce drug prices for Medicare beneficiaries. As your guidance recognized, the MFP provisions of the law also include provisions to protect patients and support patient centered action. CMS has the opportunity to continue advancing this crucial goal throughout the implementation of the Medicare Drug Price Negotiation Program. As CMS makes decisions to improve drug affordability, it is vital for the agency to center its decisions around patients and people with disabilities.

Specifically, this important new program gives CMS an opportunity to advance patient-centeredness in health care decision making while improving medical affordability through lower drug prices. While we commend the agency for the steps it has already taken in this direction, such as soliciting stakeholder input at the beginning of the decision-making process, we urge the agency to include additional measures to ensure the program is truly centered on the needs of patients and people with disabilities.

Our recommendations below center on three pillars: 1) creating additional procedures to meaningfully engage with patients and ensure that the evidence CMS relies on is transparent; 2) establishing patient-centered standards and outcomes; and 3) more definitively rejecting the use of Quality-Adjusted Life Years (QALYs) and other discriminatory cost-effectiveness standards. We believe these recommendations will be useful to CMS in developing evidentiary standards and engagement practices that ensure patient benefits are central to decision-making.

**We Urge Meaningful Engagement of Patients and People with Disabilities**

Allowing members of the public to provide input into the decision-making process, particularly the Medicare beneficiaries directly impacted by this work, will best position CMS to identify all available unbiased and nondiscriminatory evidence for the factors described in section 1194(e)(2). We appreciate that CMS is inviting patients and other public stakeholders to provide input in an initial 30-day period for information collection. Further, we are aware that CMS also released an information collection request (ICR) on Negotiation Data Elements which describes how CMS intends to collect the data described, including information relevant to section 1194(e)(2). We are reviewing this and will provide additional comments as pertinent. As CMS considers the tactics that will be used to gather information, we provide the following recommendations:

- CMS should create an ***ombudsman*** for the Medicare Drug Price Negotiation Program to act as a central point of input for patients and people with disabilities, similar to the Food & Drug Administration's (FDA's) Patient Affairs Office or the Patient-Centered Outcomes Research Institute's (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 that input is disseminated to decision-makers at CMS and responses are given back to those providing said input.
- ***CMS should incorporate additional procedures to obtain and respond to 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 should go beyond written comments provided through a single, open-ended Information Collection Request, and 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 the FDA to engage patients as part of Patient-Focused Drug Development, both as part of externally led meetings[1] and FDA-led meetings.[2]
    - 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 to establish a predictable process for engagement related to its consideration of data elements about a selected drug, the

[1] "Externally-Led Patient-Focused Drug Development Meetings." U.S. Food and Drug Administration, FDA, 29 July 2022, https://www.fda.gov/industry/prescription-drug-user-fee-amendments/externally-led-patient-focused-drug-development-meetings

[2] "Externally-Led Patient-Focused Drug Development Meetings." U.S. Food and Drug Administration, FDA, 29 July 2022, https://www.fda.gov/industry/prescription-drug-user-fee-amendments/externally-led-patient-focused-drug-development-meetings

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, including comparative research and therapeutic advance. The agency should then solicit feedback about its relevance to the needs, and outcomes and preferences of patients. CMS should also solicit other patient sources from patients and people with disabilities that may have their own resources for collecting data.

- CMS should solicit input from ***diverse*** communities, in order to gain information about the differences among subpopulations and their needs, outcomes, and preferences.
- CMS should provide patients and people with disabilities the ***resources needed for effective engagement***.
  - Resources may include providing financial assistance to facilitate participation in meetings and roundtables, making meetings accessible to people with disabilities, providing informational materials in accessible formats, funding surveys and other forms of real-world evidence generation, and/or allowing an extended amount of time for input and comments.
  - This recommendation is consistent with best practices supporting engagement, particularly supporting the engagement of those historically not engaged, as consistently reflected in the work of PCORI.[3]
- CMS should ***seek input on topics that are relevant to people with disabilities, patients, and caregivers,*** and should clearly describe these topics to these 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, ideally during the deliberation process before a final decision is made.
- CMS should ***ensure that information gathered during public comment periods and meetings is reflected in the final guidance*** that CMS publishes in advance of the first year of negotiations, advancing the principle of transparency that is supported across organizations.

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[3] 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.

- 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.
    - Organizations such as the Partnership to Improve Patient Care,[4] the National Council on Disability (NCD),[5] and the Disability Rights Education and Defense Fund (DREDF)[6] have identified restricted access implications experienced in countries relying on methods for assessing value that fail to capture the real-world value to patients.

**We urge CMS to Explicitly Recognize, Without Exception, the Existing Statute Barring Use of QALYs and Similar Measures, Consistent with Current Law and Recommendations of the National Council on Disability Against Reliance on Cost-Effectiveness.**

The initial CMS guidance recognized the agency's authorization to consider evidence about the selected drug, including whether the selected drug represents a therapeutic advance, its alternatives, comparative effectiveness and effects on specific subpopulations, and extent to which unmet medical needs are addressed. This reflects the IRA's focus on driving significant discounts in drug prices through the use of comparative clinical effectiveness research and cost data vs. one-size-fits-all cost-effectiveness analyses, consistent with the concerns of the NCD[7, 8] and other disability rights organizations.[9, 10]

CMS acknowledged that the agency may not use evidence from comparative clinical effectiveness research in a manner that treats extending the life of an individual who is elderly, disabled or terminally ill as of lower value than extending the life of an individual who is younger, nondisabled, or not terminally ill. However, the initial CMS guidance did not reference

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[4] Partnership to Improve Patient Care, PIPC, http://www.pipcpatients.org/international.html

[5] National Council on Disability. (November 16, 2019). Quality-Adjusted Life Years and the Devaluation of Life with Disability. https://ncd.gov/sites/default/files/NCD_Quality_Adjusted_Life_Report_508.pdf

[6] DREDF, ICER Analyses Based on the QALY Violate Disability Nondiscrimination Law , September 21, 2021 at https://dredf.org/wp-content/uploads/2021/09/ICER-Analyses-Based-on-the-QALY-Violate-Disability-Nondiscrimination-Law-9-17-2021.pd

[7] The NCD recommended that Congress, "Avoid creating provisions of any bill that would require the agency with management and oversight responsibilities (such as, for example, HHS) to cover only the most cost-effective drugs and treatments, or to require the agency to impose restrictions on less cost-effective treatments." https://ncd.gov/sites/default/files/NCD_Quality_Adjusted_Life_Report_508.pdf

[8] The NCD recommended Medicaid guidance, "The guidance should specifically discuss how these authorities apply to benefits and reimbursement decisions, and that payment decisions should not rely on cost-effectiveness research or reports that are developed using QALYs." https://ncd.gov/sites/default/files/NCD_Quality_Adjusted_Life_Report_508.pdf

[9] Joint letter from advocates to Oregon HERC, "Most cost-effectiveness analyses rely on data from randomized clinical trials (RCTs) and health utility preference weighting surveys, data sources that primarily rely on inputs from non-disabled, white, Caucasian populations. This systematically biases available therapies to favor covering those that are effective for white people to the detriment of covering treatments effective for people of color and people with disabilities." http://www.pipcpatients.org/uploads/1/2/9/0/12902828/herc_letter.pdf

[10] Joint letter to CMS, October 23, 2022, "More broadly, we also support the NCD recommendation that federal programs, including Medicaid, should not rely on cost-effectiveness research or reports that gather input from the public on health preferences that do not include the input of people with disabilities and chronic illnesses."

the Affordable Care Act (ACA) which specifically bars the use of the QALY and includes the language, "The Secretary shall not utilize such an adjusted life year (or such a similar measure) as a threshold to determine coverage, reimbursement, or incentive programs under title XVIII."[11]

We deeply appreciate the statement made by Secretary Becerra on March 29, 2023, reaffirming that CMS will not use QALYs or similar measures, and look forward to the agency strengthening its guidance to reaffirm this.[12] We urge CMS to use language in its final guidance clarifying that existing law bars the use of QALYs and similar measures, not just QALYs as used in the context of life extension, and to state explicitly that, as directed in the IRA, it will rely on the factors of comparative clinical effectiveness outlined in section 1194(e)(2).

At a recent hearing in the House Energy and Commerce Committee, Ranking Member Frank Pallone, a primary author of the Inflation Reduction Act's health care provisions, stated that the Congress had passed a landmark law allowing for Medicare Drug Price Negotiation "while also explicitly prohibiting the use of QALYs in this process."[13] The ACA passed in 2010 and barred Medicare from using QALYs and similar metrics throughout Medicare, including the drug negotiation process. The IRA went a step further, ensuring that no evidence would be considered that valued life extension for older adults, people with disabilities, and people at the end of life as less than their counterparts, which Ranking Member Pallone and others have recognized to include QALYs.[14]

Therefore, we urge CMS to provide clarity that its drug negotiation process will be grounded in evaluation of comparative clinical effectiveness and patient-centered health outcomes and not use or consider QALYs or other cost-effectiveness standards that frequently discriminate against subgroups and devalue the needs and preferences of patients. This includes biased non-QALY measures such as the Equal Value of Life Years 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 shortcomings in accounting for quality-of-life improvements.[15] The NCD and DREDF have each analyzed the QALY and the evLYG to conclude neither are suitable measures for assessing treatments.

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[11] House of Representatives, Congress. 42 U.S.C. 1320e - Comparative clinical effectiveness research. U.S. Government Publishing Office, https://www.govinfo.gov/app/details/USCODE-2010-title42/USCODE-2010-title42-chap7-subchapXI-partD-sec1320e

[12] "Health Subcommittee Hearing: 'Fiscal Year 2024 Department of Health and Human Services Budget.'" YouTube, 29 March 2023, https://youtu.be/OPMG5OU0I6c.

[13] "Health Subcommittee Legislative Hearing (Lives Worth Living)." YouTube, 1 Feb. 2023, https://www.youtube.com/watch?v=lZE_DVqg6dk.

[14] Ranking Member Anna Eshoo stated, "Democrats included a ban on QALYs in Medicare and the Affordable Care Act in 2010. Last year, Democrats further clarified that QALYs could not be used as part of Medicare's prescription drug price negotiations in the IRA." "Full Committee Markup of 19 Bills (Part 2)," 24 March 2023.

[15] "Cost-Effectiveness, the QALY, and the Evlyg." ICER, Institute for Clinical and Economic Review, 28 Mar. 2023, https://icer.org/our-approach/methods-process/cost-effectiveness-the-qaly-and-the-evlyg/

**Recommendation:**

- CMS should ***clarify in guidance and/or regulations that it will not use or consider QALYs or similar measures in any way***.
    - This recommendation is consistent with ACA's statutory ban on the use of QALYs and similar measures 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.
- With regard to CMS solicitation of information on other specific measures that discriminate, ***CMS should avoid consideration of any evidence that is informed by QALYs or similar measures such as the evLYG[16] [17] or Disability Adjusted Life Years (DALYs).[18,19]***

**Consideration of Non-QALY Evidence in Reports Using QALYs**

While we appreciate CMS's assurance that it will not consider QALYs, we are concerned that the guidance leaves the door open to submission of QALY-based analysis within other clinical or cost-effectiveness assessments. We urge transparency in how these assessments are ultimately used by the agency.

It is important to understand that most of the components that make up the calculation of QALY estimates may also be used in a particular study's assessment of comparative clinical

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[16] The NCD described the eLYG in its report as, "There are other challenges to the evLYG that indicate that it is not a suitable alternative to the QALY. First, as evidenced by the assessment of Spinraza, denial of coverage is possible under the QALY/evLYG system, even where a drug would provide significant clinical benefit, including life extension. Second, the QALY/evLYG system still relies on health utility weights to measure quality of life improvements, despite the fact that such measures are typically derived from survey data and do not account for the complexity of the preferences and experiences of people with disabilities. Third, the QALY/evLYG system affords no opportunity to account for clinical knowledge not reflected in the research literature, a significant concern articulated in Chapter 1. Finally, even within the narrow emphasis on life extension, ICER provides no guidance to payers as to which reimbursement level to prioritize—the one derived from the QALY or the one derived from the evLYG." https://ncd.gov/sites/default/files/NCD_Quality_Adjusted_Life_Report_508.pdf

[17] DREDF concluded the following about evLYG, "Thus, adding the evLYG is not a solution; it merely forces payers to choose between one measure that undervalues life extension (the QALY) and one that affords no value to quality of life improvements (the evLYG). Neither account for both the full value of life-extension and the value of quality-of-life improvement." https://dredf.org/wp-content/uploads/2021/09/ICER-Analyses-Based-on-the-QALY-Violate-Disability-Nondiscrimination-Law-9-17-2021.pdf

[18] Coelho, Tony. "PCORI Comments on Value Letter." Received by Dr. Nakela Cook, 3 Mar. 2023. http://www.pipcpatients.org/uploads/1/2/9/0/12902828/pcori_comments_on_value.pdf

[19] Grosse, Scott D et al. "Disability and disability-adjusted life years: not the same." Public health reports (Washington, D.C. : 1974) vol. 124,2 (2009): 197-202. doi:10.1177/003335490912400206

effectiveness and therefore could be subject to the same biases inherent in the QALY totals themselves. Simply cherry-picking the components of these QALY estimates that are included a study of comparative clinical effectiveness is not an effective route to avoiding their biases.

Instead, we urge CMS to identify with greater detail and transparency the acceptable input data variables to be taken from comparative clinical effectiveness research, in order to ensure that the methods used will not result in bias against older adults, people with disabilities, and people at the end of life. For example, CMS should recognize that the use of value or utility weights in comparative clinical effectiveness research may also be used in the QALY calculation and therefore also subject to bias and validity challenges.[20] These weights are often constructed by a very small subgroup of a country's population[21] despite purporting to represent all.[22] Yet, there is considerable empirical evidence that treatments impact people differently and that society strongly disagrees with treating all conditions, disease states, and patient types with the same priority.[23,24]

The QALY can introduce bias into a study of a treatment's effectiveness in several ways. For example, life expectancy estimates for the population being treated may be calculated from an older population or from a population that has co-existing conditions or disabilities, thereby creating weights for the potential life year gains that could accrue to a successfully treated individual that give a biased view of life-years gained. Another example is the quality of life (QOL) part of the equation - the source data for the weights that turn life years into quality-adjusted life years. We are concerned that the patient-reported outcome measures (PROs) in the commonly used EuroQoL instrument (EQ-5D) do not meet the FDA's definition:

> *PRO instrument item generation is incomplete without a range of patients with the condition of interest to represent appropriate variations in severity and in population characteristics such as age or sex.* [25]

The EQ-5D is the most commonly used PRO within QALY calculations, yet it relies upon weightings constructed by populations unfamiliar with the conditions being evaluated and therefore does not have the accuracy that is obtained by consulting with patients. Recent studies have provided strong evidence to suggest that there is a public bias against people with

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[20] Smith S, Cano S, Browne J. "Patient reported outcome measurement: drawbacks of existing methods". bmj. 2019 Feb 27;364:l844.
[21] McClimans L, Browne JP. "Quality of life is a process not an outcome. Theoretical medicine and bioethics". 2012 Aug 1;33(4):279-92.
[22] Broome J. "Fairness Versus Doing the Most Good". The Hastings Center Report. 1994 Jul 1;24(4):36-9.
[23] Weinstein MC. "A QALY is a QALY is a QALY—or is it?" Journal of Health Economics. July 1988 289-291.
[24] Whitehead SJ, Ali S. "Health outcomes in economic evaluation: the QALY and utilities". British medical bulletin. 2010 Dec 1;96(1):5-21.
[25] US Food and Drug Administration "*Guidance for Industry: Patient-Reported Outcome Measures: Use in Medical Product Development to Support Labeling Claims".* 2009. [2020-07-15].

disabilities.[26] Criticism of the inherent bias of the EQ-5D is widespread and growing.[27,28] It is also widely critiqued for failing to represent any consensus about the value of health states, as surveys of the general public reveal enormous heterogeneity (i.e., disagreement) within surveyed populations.[29]

Selective use of QALYs or selective use of the components of data inputs that make up QALY calculations in studies of comparative clinical effectiveness raise many of the same dangers as the blanket use of QALYs for measuring the therapeutic benefit or "value" of a drug to a patient or to society. The biases that CMS emphasizes that it needs to avoid are built into the methodological construction of QALYs at multiple levels. Attempts by CMS to pick their way around these biases by selectively choosing components of QALY estimates where convenient would have significant risks for bias and discrimination.

**Recommendation:**

- CMS should clarify in the final guidance that ***evidence relying on the same biased or discriminatory inputs, particularly the value sets or weights used to measure life extension or quality of life, will not be relied on*** as evidence for the factors of therapeutic benefit that CMS is authorized to consider in section 1194(e)(2).

**Consideration of Comparative Clinical Effectiveness Research and Appropriate Comparators**

We appreciate that CMS clearly states in its guidance its intent to consider "health outcomes, intermediate outcomes, surrogate endpoints, patient-reported outcomes, and patient experience when reviewing the clinical benefit of the selected drug and its therapeutic alternative(s)." As directed by current law, this includes a bar on any use of the QALY. We strongly urge CMS to directly engage affected stakeholders – the patients, people with disabilities and clinicians with practicing experience in the condition being treated – as the experts in determining the therapeutic benefit of treatments based on outcomes that are valued by patients.

**Recommendations:**

- ***CMS should clearly define comparative clinical effectiveness research*** in a manner consistent with the existing definition in the ACA.

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[26] HJ;, Chaudhry. "Expanding Licensure Portability and Access to Care: Lessons Learned during Covid-19." Health Affairs (Project Hope), U.S. National Library of Medicine, https://pubmed.ncbi.nlm.nih.gov/35914196/

[27] Cubi-Molla P, Shah K, Burström K. "Experience-Based Values: A Framework for Classifying Different Types of Experience in Health Valuation Research". *Patient.* 2018 Jun;11(3):253–270.

[28] Helgesson G, Ernstsson O, Åström M, Burström K. "Whom should we ask? A systematic literature review of the arguments regarding the most accurate source of information for valuation of health states". *Qual Life Res.* 2020 Jul;29(6):1465–1482

[29] Bansback N, Tsuchiya A, Brazier J, Anis A. "Canadian valuation of EQ-5D health states: preliminary value set and considerations for future valuation studies". *PLoS One*. 2012;7:e31115.

- 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…" and makes it clear that such research does not involve cost comparisons or cost-effectiveness.
- In determining what comparative clinical 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.[30] We do not recommend that the initial offer rely solely on the price of a therapeutic alternative, but instead reflect the negotiated drug's therapeutic benefit.

**Therapeutic Advance and Unmet Need**

We appreciate that CMS specifically stated its intention to review real-world evidence. Data generated by registries and other sources of real-world data, particularly for subpopulations such as people with disabilities, should be treated as highly relevant to the factors listed in section 1194(e)(2) as they provide current evidence of the experience of patients that may not yet be reflected in other research literature or clinical trial data. When developing its offer for MFPs, CMS should ensure it is prioritizing feedback from patients, people with disabilities, and clinicians with practicing experience with the condition, as well as assessments of therapeutic benefit, thereby considering value through the lens of how patients and people with disabilities experience and value their health care. Doing so will require a strong commitment to engagement.[31]

**Recommendations:**

- CMS should determine whether a treatment reflects a ***therapeutic advance*** based not only on the clinical trial data but also on 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

[30] PIPC, "The German Health Care System and its Impact on Patient Access – Lessons for the U.S., http://www.pipcpatients.org/uploads/1/2/9/0/12902828/germany_draft_2022_9-21_edited_clean.pdf
[31] Smith, Theo. "Real-World Evidence Classroom." National Health Council, 28 Feb. 2023, https://nationalhealthcouncil.org/additional-resources/real-world-evidence-classroom/

improving their quality of life with treatment, a theme consistent in calls for improved patient engagement in research and decision-making.[32,33]

- CMS should specifically call for studies related to therapeutic advancements that reflect the diversity of the patients being treated.[34,35]

- 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.[36]

  - 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 by 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 Set a High Bar for the Quality of Evidence to be Considered.**

CMS stated its intent to consider the "source, rigor of the study methodology, current relevance to the selected drug and its therapeutic alternative(s), whether the study has been through peer review, study limitations, degree of certainty of conclusions, risk of bias, study time horizons, generalizability, study population, and relevance to the negotiation factors listed

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[32] 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.

[33] NCD recommended, "HHS should consider including explicitly recruiting people with disabilities and chronic illnesses as members of committees and working groups formed to develop effective healthcare reform and strategies for lowering the cost of prescription drugs." https://ncd.gov/sites/default/files/NCD_Quality_Adjusted_Life_Report_508.pdf

[34] Wartman , Gretchen C, et al. Aligning Health Technology Assessment with Efforts to Advance Health Equity. Partnership to Improve Patient Care, http://www.pipcpatients.org/uploads/1/2/9/0/12902828/pipc_white_paper_-_measuring_value_in_medicine_-_uses_and_misuses_of_the_qaly.pdf

[35] 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

[36] House of Representatives, Congress. 42 U.S.C. 1320e - Comparative clinical effectiveness research. U.S. Government Publishing Office, , https://www.govinfo.gov/app/details/USCODE-2010-title42/USCODE-2010-title42-chap7-subchapXI-partD-sec1320e

in section 1194(e)(2) of the Act to ensure the integrity of the contributing data within the negotiation process." CMS has also stated its intent to incorporate real-world evidence into its considerations. We urge CMS to prioritize research that is rigorous, as well as real-world feedback from patients, people with disabilities, and practicing clinicians. Randomized clinical trials and studies relying on the existing literature to make conclusions about the effectiveness of drugs should themselves be peer reviewed and rigorous. It is also important to recognize that real-world evidence from the lived experience of patients, people with disabilities, and clinicians may be observational but is nonetheless also relevant to understanding the impact of treatments that may not have been subject to recent rigorous clinical trials. It will be important for CMS to have high standards that drive the rigorous study of therapeutic benefits in a manner that captures the diversity of people on treatment, the differences among subpopulations, and a focus on outcomes that are valued by patients as communicated to CMS by patients, people with disabilities, and practicing clinicians.

**Recommendations:**

- 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 that are biased or discriminatory.***
    - 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.[37,38]
    - To determine what evidence meets standards for quality and patient-centeredness, the agency ***should look to the organizations representing affected patients and people with disabilities as well as the clinical experts*** among practicing physicians and providers, as they would be most familiar with the usefulness of the evidence base for making decisions and its potentially inherent biases.
    - As previously stated, CMS should ***prioritize evidence that is patient-centered*** and captures value for patients, caregivers, and persons with disabilities.

**Conclusion**

We appreciate 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

[37] The Patient Voice in Value - National Health Council. National Health Council, https://nationalhealthcouncil.org/wp-content/uploads/2020/11/20160328-NHC-Value-Model-Rubric-final.pdf

[38] "Landscape Review and Summary of Patient and Stakeholder Perspectives on Value in Health and Health Care." PCORI, Patient-Centered Outcomes Research Institute, 2 Sept. 2022, https://www.pcori.org/resources/landscape-review-and-summary-patient-and-stakeholder-perspectives-value-health-and-health-care

obligations to beneficiaries, it will be critically important for CMS to be thoughtful in how it assesses therapeutic benefit to affected patients. CMS must ensure that patients and people with disabilities are granted a seat at the table and a clear and robust path to engagement throughout the process.

Sincerely,

Access Ready
Alliance for Aging Research
Alliance for Patient Access
Allies for Independence
ALS Association
American Association of People with Disabilities
American Association on Health and Disability
American Behcet's Disease Association (ABDA)
Asthma and Allergy Foundation of America
Bazelon Center for Mental Health Law
Cancer Support Community
Cancer*Care*
Caring Ambassadors Program
Center for Autism and Related Disorders
Center for Independence of the Disabled, NY
Coalition of State Rheumatology Organizations
Coalition of Texans with Disabilities
Color of Crohn's and Chronic Illness (COCCI)
Cutaneous Lymphoma Foundation
Cystic Fibrosis Research Institute
Davis Phinney Foundation for Parkinson's
Derma Care Access Network
Disability Rights Education and Defense Fund
Disability Rights Oregon
Epilepsy Alliance America
Epilepsy Foundation
Global Liver Institute
Healthy Men Inc.
Hereditary Neuropathy Foundation
ICAN, International Cancer Advocacy Network
Independent Women's Forum
Infusion Access Foundation
Lakeshore Foundation
Lupus and Allied Diseases Association, Inc.
Lupus Foundation of America
MLD Foundation
Multiple Sclerosis Foundation

National Association of Councils on Developmental Disabilities
National Association of Nutrition and Aging Services Programs
National Disability Rights Network (NDRN)
National Down Syndrome Congress
National Down Syndrome Society
National Oncology State Network
New Jersey Association of Mental Health and Addiction Agencies, Inc.
Partnership to Advance Cardiovascular Health
Partnership to Improve Patient Care
Patients Rising Now
RetireSafe
Rosie Bartel
Spondylitis Association of America
The Bonnell Foundation: Living with Cystic Fibrosis
The Coelho Center for Disability Law, Policy and Innovation
The Headache & Migraine Policy Forum
The Hepatitis C Mentor and Support Group-HCMSG
TSC Alliance
United Spinal Association