

July 2, 2024

Meena Seshamani, M.D., Ph.D.
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 2124401850

Dear Deputy Administrator Seshamani:

Thank you for this opportunity to comment on the draft guidance for the second cycle of the Medicare Drug Price Negotiation Program. Since passage of the Inflation Reduction Act, we have worked collaboratively as organizations representing patients and people with disabilities to amplify the perspectives of those with lived experience in the implementation of the Medicare Drug Price Negotiation Program. Our comments will focus on the agency's process for engaging patients in its decisions, including determinations related to a treatment's clinical effectiveness, unmet need and therapeutic alternatives, as well as the agency's use of value assessments.

While we appreciate that the agency is considering certain recommendations from patients and people with disabilities for improving its engagement, we are concerned that the new guidance does not put forward a concrete plan or process for developing predictable, targeted, and specific tactics for engaging patients and people with disabilities. We are also concerned that the guidance does not capture the limitations on use of quality-adjusted life years (QALYs) and similar measures explicitly described in the Affordable Care Act. Therefore, we are pleased to share the following recommendations:

- CMS should avoid **one-size fits all value metrics**.
- CMS should develop a formalized process to ensure **continuous, robust engagement** of patients and people with disabilities at multiple levels.
- Using patient insights, CMS should **clearly communicate how it intends to use the input it receives**, and how that input is reflected in the final negotiated prices.
- CMS should solicit input from **diverse communities** to ensure representation of the diversity of the patients and communities affected by the topic.
- CMS should ensure that opportunities for patient engagement are **accessible**.
- To gauge both successes and challenges, CMS should establish a structured process for **continuous review and assessment** of its engagement strategy.

CMS should avoid one-size-fits-all value metrics.

It is now widely recognized that traditional methods and metrics of value assessment such as the QALY have significant shortcomings. This has led to well-intentioned development of other measures and approaches that developers assert to be nondiscriminatory and more

patient-centered. However, each approach comes with tradeoffs, need for improvement, and inherent methodological weaknesses. No patient is average, and no measure of value should assume so.

Prior law, including the Affordable Care Act, bars use of QALYs and similar measures.

CMS made a strong, positive statement of its commitment to “learning from, collaborating with, and engaging the public, including patients, consumer advocates, health and data experts, and pharmaceutical supply chain entities in the policy-making process.” The agency also expressed support for collecting real-world data and engaging patients related to its work to identify therapeutic alternatives. Yet, we are concerned that the new guidance states that CMS will “review cost-effectiveness measures used in studies relevant to a selected drug to determine whether the measure used is permitted in accordance with section 1194(e)(2), as well as with section 1182(e) of Title XI of the Act.”

The guidance narrowly references the IRA’s statutory language, stating that the Medicare Drug Price Negotiation Program will not use “information that treats extending the life of individuals in these populations as of lower value,” leaving out language in the Affordable Care Act (ACA) barring similar measures that “discounts the value of a life because of an individual’s disability.” The aim of this language was not to spur an effort to find loopholes that allow the government to use a single approach. As discussed on the Senate floor, the spirit of the provision was to protect vulnerable patients and people with disabilities from policies that “set national practice standards or coverage restrictions” and ensure research used to make decisions is focused on clinical outcomes.¹

Also, CMS cannot assume that a value assessment does not discriminate simply because it does not use QALYs. The recent final rules governing Section 504 of the Rehabilitation Act², a law passed in 1973, and Section 1557 of the ACA³ both acknowledge the potential for value assessments to discriminate. The agency interpreted the final section 504 rules as “broader than section 1182 of the Affordable Care Act, because it prohibits practices prohibited by section 1182 (where they are used to deny or afford an unequal opportunity to qualified individuals with disabilities with respect to the eligibility or referral for, or provision or withdrawal of an aid, benefit, or service) and prohibits other instances of discriminatory value assessment.”

The data used to value health care may be discriminatory or fail to represent real-world experiences of patients and people with disabilities.

We appreciate that agency’s interpretation of Section 504 that “discounting the value of quality of life on the basis of disability for purposes of denying or limiting medical

¹ Colloquy by Senators Baucus, Enzi, Conrad, Hatch, Carper and Menendez. “Comparative Effectiveness Research Funds.” Congressional Record 155:24 (February 6, 2009) p. S. 1796.

² HHS, “Nondiscrimination on the Basis of Disability in Programs or Activities Receiving Federal Financial Assistance,” May 9, 2024, <https://www.federalregister.gov/documents/2024/05/09/2024-09237/nondiscrimination-on-the-basis-of-disability-in-programs-or-activities-receiving-federal-financial>

³ CMS, “Nondiscrimination in Health Programs and Activities,” May 6, 2024, <https://www.federalregister.gov/documents/2024/05/06/2024-08711/nondiscrimination-in-health-programs-and-activities>

treatment to a qualified individual with a disability would likely violate § 84.56.” The agency also stated, “Methods of utility weight generation are subject to section 504 when they are used in a way that discriminates. They are subject to § 84.57 and other provisions within the rule, such as § 84.56’s prohibition of discrimination based on biases or stereotypes about a patient’s disability, among others.”

Therefore, we urge CMS to not only comply with current law, but also to consider whether the evidence used in its decision-making was developed in a manner that reliably represents the population of patients and people with disabilities impacted. Value assessments are only as good as the data used in their development. Therefore, we urge the agency to consider the following factors:

- **Health utilities:** Also known as Health State Utility Value (HSUV), they mark the health-related quality of life (HrQOL) of a patient with a specific disease. A numeric valuation is applied to a health state based on preference of being in that state relative to perfect health, assigning a number between 0 and 1 to various conditions a person’s health could be in (often called “health states” in which 0=death and 1=optimal health). They are typically derived from surveys asking how much, on average, someone prefers one health state compared to another. Health states typically represent degree of impairment (not the disability or condition) such as active disease, response, remission, or mild, moderate and severe. Shortcomings include:
 - Survey data relies on average perspectives of quality of life in a health state, which are biased, inaccurate and almost never replicable. For example, there is significant research on the bias against disability among the public⁴ and among providers⁵.
 - The identified health states are typically not disease or condition specific, often surveying health from lens of mild, moderate or severe (such as the EQ-5D⁶) and only accounting for health improvements that move between these broad states. Only large health improvements, i.e. HrQOL, count.
 - Health utilities typically give a lower value to people living below optimal health. For example, extending the life for person living at a .5 is worth half of a person at a 1.
- **Disability weights:** Disability weights quantify health losses relating to non-fatal outcomes, expressed as years lived with disability (YLD). They typically have a value between 0 (equivalent to full health) and 1 (equivalent to death). For example, living 10 years with a 10 percent reduction in HRQoL is a disability weight of 0.10 – equal to losing one full year of good health (e.g., by dying one year before the life

⁴ Ari Ne’eman et. al., “Identifying And Exploring Bias In Public Opinion On Scarce Resource Allocation During The COVID-19 Pandemic,” *Health Affairs* Vol. 41 No. 10 (October, 2022), <https://www.healthaffairs.org/doi/10.1377/hlthaff.2022.00504>

⁵ Lisa I. Iezzoni et. al., “Physicians’ Perceptions of People With Disability and Their Health Care,” *Health Affairs* Vol. 40 No. 2 (February, 2021), <https://www.healthaffairs.org/doi/full/10.1377/hlthaff.2020.01452>

⁶ EUROQOL, “EQ-5D-5L,” <https://euroqol.org/information-and-support/euroqol-instruments/eq-5d-5l/>

expectancy). Severity of condition (morbidity) and its death rate (mortality) are expressed as the number of healthy life years lost. Shortcomings include:

- Disability weights are elicited by surveys, often of participants that do not have experience in the studied health state. Surveys are subject to bias against disability.
- Disability weights from different studies are often not comparable, coming from different countries or populations with differing perceptions of disease and disability.
- Assuming same weights to different aspects of quality of life as representative of all people risks being applicable to none.⁷ Triathletes may highly weigh physical function. Academics may weigh mental acuity.
- **Health outcomes data:** Cost effectiveness analysis requires data on health outcomes to measure cost of gaining health. A product's "value" combines clinical effectiveness (impact of intervention on select health outcomes) and economic value (impact of intervention on healthcare resource use and costs). Shortcomings include:
 - Patient-centered outcomes and societal value are often ignored. For example, methods may not incorporate data on economic or social consequences such as loss of ability to work or caregiver effects.
 - Reliance on average estimates based on generic survey data obscures important differences in clinical needs and preferences, particularly complex diseases and those from underrepresented communities.⁸
- **Health equity:** Cost effectiveness analyses and value assessment are intended to maximize health care efficiency. Historically, they have not explicitly incorporated equity concerns related to race, ethnicity, or socioeconomic factors, nor implicit bias or structural inequities within healthcare systems, disparities in access to healthcare services and treatments, or social determinants of health.⁹
- **Real-World Implications:** New methodologies for cost effectiveness analysis are abundant but untested. While recognition of flaws inherent in historic methods for assessing treatment value is driving innovation, literature on almost every method underscores need for extensive detailed data on patients' risk profiles, co-existing conditions, and other relevant factors currently lacking and challenging to obtain. Investment in data is needed.

Every value assessment measure has tradeoffs.

There has been longstanding protection against use of discriminatory value assessment tools in statute. Therefore, we are concerned that CMS' draft guidance explicitly expressed

⁷ Anirban Basu & David Meltzer, "Value of Information on Preference Heterogeneity and Individualized Care," *Medical Decision Making* Vol. 27 No. 2 (March-April 2027), <https://pubmed.ncbi.nlm.nih.gov/17409362/>

⁸ Michael J DiStefano et. al., "Alternative approaches to measuring value: an update on innovative methods in the context of the United States Medicare drug price negotiation program," *Expert Review of Pharmacoeconomics & Outcomes Research* Vol. 24 No. 2 (February 2024), <https://pubmed.ncbi.nlm.nih.gov/37961908/>

⁹ No Patient Left Behind, "The Value of Medicines," <https://www.nopatientleftbehind.org/value-of-medicines>

interest in using alternative approaches as part of drug price negotiations. Every cost effectiveness measure has tradeoffs between conditions advantaged and disadvantaged:

- **Quality-adjusted life year (QALY):** Less value to life-extending treatments among patients whose baseline health-related quality of life is low, particularly people living with disabilities. More value to treatments achieving maximum quality of life.
- **Equal value of life year gained (evLYG):** Less value to treatments improving quality of life in extended life years. Same value as QALYs for treatments that do not extend life years regardless of quality-of-life improvements. More value to treatments extending life years.
- **Generalized Cost Effectiveness Analysis (GCEA):** Less value to treatments for common conditions to manage symptoms. More value to treatments for severe and disabling conditions.
- **Generalized risk-adjusted cost effectiveness (GRACE):** Less value to treatments for common conditions to manage symptoms. More value to treatments for severe and disabling conditions.
- **Disability adjusted life year (DALY):** Less value to treatments for people with disabilities due to focus on life years lost. More value to conditions leading to an early death without treatment.
- **Health years in total (HYT):** Less value to treatments that improve quality of life without increasing life expectancy. More value to treatments that extend life.
- **Life years gained (LYG):** Less value to treatments for patients with fewer years left to live (e.g., older adults or those with disabling conditions) and for largely non-fatal conditions (e.g., blindness, depression, rheumatoid arthritis). More value to treatments extending life.

Recommendation: We urge CMS to avoid use of one-size-fits-all value metrics, like the QALY or evLYG, as part of its decision-making, consistent with current Medicare law and regulations governing nondiscrimination. CMS should also identify and be transparent about the types and sources of research, data, and assessments considered in its decision-making process. In addition, CMS should ensure it and other entities are exercising adequate oversight over the Medicare Drug Price Negotiation Program to ensure decisions do not rely on data from studies relying on one-size fits all metrics, like the QALY or evLYG.

Engaging Patients and People with Disabilities

We urge the Centers for Medicare & Medicaid Services (CMS) to create a systematic engagement process that goes beyond written comment periods and ad hoc listening sessions. Drawing on robust frameworks from leading organizations including PCORI, National Health Council (NHC), the PATIENTS Program at the University of Maryland, the Innovation and Value Initiative (IVI), and AcademyHealth, we are pleased to provide the following recommendations through which CMS would prioritize authentically involving patients and people with disabilities in agency decisions. We urge CMS to incorporate these best practices to foster meaningful dialogue with patients, caregivers, and people with

disabilities across the agency. The insights from their lived experience will allow CMS to ensure advancement of policies and practices that improve health care value and patient outcomes.

Our recommendations for enhancing CMS' patient engagement strategies are grounded in the expertise of organizations dedicated to improving health care value through meaningful engagement with patients and individuals with disabilities. These organizations have developed substantial recommendations to foster and guide patient engagement across the health care sector, emphasizing the crucial role of meaningful and authentic patient and caregiver engagement in research processes.

In response to CMS' 2023 listening sessions on the Medicare Drug Price Negotiation Program, NHC convened a roundtable discussion to provide a platform for the patient community to share their experience engaging with the agency.¹⁰ Stakeholders outlined valuable insights gleaned from these sessions, which can contribute to shaping CMS' broader patient engagement strategies. The PATIENTS Program at the University of Maryland School of Pharmacy adopted a similar approach by hosting a Town Hall, bringing together stakeholders to gather insights and recommendations.¹¹ Their aim was to ensure that patient perspectives are being represented in the agency's decision-making.

Furthermore, the PCORI-developed Foundational Expectations for Partnerships¹² and IVI's Economic Impacts Framework¹³ also informed our recommendations. PCORI's six expectations serve as a framework to guide meaningful, effective, and sustainable engagement to advance patient-centered comparative clinical effectiveness research (CER). Meanwhile, IVI's framework, along with the principles used to develop it, encourages partnerships between patients, caregivers, and researchers to broaden the understanding and measurement of the six main economic impacts for patients.

CMS should work with an advisory group of experts from organizations representing people with chronic conditions and disabilities to develop a formalized process to ensure continuous, robust engagement of patients and people with disabilities at multiple levels.

¹⁰ NHC, "Amplifying the Patient Voice: Roundtable and Recommendations on CMS Patient Engagement," published March 2024, <https://nationalhealthcouncil.org/wp-content/uploads/2024/03/Amplifying-the-Patient-Voice-Roundtable-and-Recommendations-on-CMS-Patient-Engagement.pdf>

¹¹ The PATIENTS Program at the University of Maryland School of Pharmacy, "PATIENTS Professors Town Hall: Recommendations for the CMS Drug Price Negotiation Program Final Report," published July 12, 2023, <https://www.pharmacy.umaryland.edu/media/SOP/wwwpharmacyumarylandedu/programs/PATIENTS/pdf/Patient-driven-recommendations-for-the-Medicare-Drug-Price-Negotiation-Program.pdf>

¹² PCORI, "Engagement in Research: Foundational Expectations for Partnerships," updated February 2024, <https://www.pcori.org/sites/default/files/PCORI-Engagement-in-Research-Foundational-Expectations-for-Partnerships.pdf>

¹³ IVI and AcademyHealth, "A Research Framework to Understand the Full Range of Economic Impacts on Patients and Caregivers," published May 2023, https://thevalueinitiative.org/wp-content/uploads/2023/06/05-2023-Economic-Impacts-Framework-Report_FINAL.pdf

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 crucial. As part of NHC's vision for improving CMS' patient engagement over the next five years, one of three key improvements proposed is inclusion of patient perspectives at every stage of the decision-making process. To achieve this objective, both NHC and the PATIENTS Program urge CMS to establish partnerships with the patient community and formalize a process to create multiple touchpoints with people experiencing the disease or illness being studied. This aligns with PCORI's foundational expectations for partnerships, which emphasizes the importance of initiating touchpoints early, even during planning stages of a study.

Additionally, IVI highlights that continuous partnerships provide valuable context from individuals' lived experiences to shape research priorities and NHC recommends CMS develop methods for incorporating this patient experience data into its program implementation. The experts participating in the advisory group should include those with experience engaging patients and people with disabilities throughout the life cycle of chronic conditions and disabilities to elicit information about the range of burdens and outcomes that matter most to them, as well as the differences among subpopulations.

Recommendation: Based on this strong consensus and alignment of goals, we recommend that CMS develop a formalized engagement process in consultation with engaged partners in the patient and disability communities that have expertise engaging people with lived experience related to their experiences with treatment. This process should not only ensure that the agency is actively engaging early and often with patient stakeholders but also guarantee ongoing engagement, fostering sustainable partnerships and building trustworthy relationships for future endeavors.

Using patient insights, CMS should clearly communicate how it intends to use the input it receives, and how that input is reflected in the final negotiated prices.

Although CMS has asked stakeholders to go through the intensive process of submitting data pertaining to selected drugs, and has made listening sessions available to them, CMS has not explained how input will be used by CMS or will inform CMS' eventual conclusions. While the process for obtaining this information is critical, equally important is how it is being used.

This issue was highlighted during the NHC's roundtable, where numerous stakeholders expressed feeling underprepared by CMS for the 2023 listening sessions, which limited their ability to meaningfully participate. They suggested that CMS could have better communicated the purpose of the information it is seeking, and how it is being used in determining prices for selected drugs. Based on this feedback, NHC recommends CMS enhance its clarity and communication about the intent of its listening sessions — a recommendation that we would apply more broadly to the agency's holistic engagement.

Similarly, the PATIENTS Program's Town Hall echoed these concerns, leading to their recommendation for CMS to provide more information to the patient community throughout the process. They emphasize trust-building through transparency, advocating that patients should understand the agency's decision-making processes and how their input is utilized. They specifically recommend the agency develop a process to share how stakeholder feedback guides decision-making. Patients and people with disabilities, as well as the organizations representing people with the chronic conditions and disabilities being reviewed, will dedicate the time and resources to being engaged partners if they know how their input makes a meaningful difference.

Recommendation: We encourage a cyclical approach, wherein patient engagement helps CMS communicate how it intends to use the information submitted by stakeholders on selected drugs and therapeutic alternatives. It is critical that this information is communicated to stakeholders to ensure they are prepared to provide appropriate feedback at listening sessions and have advance notice to gather and submit useful information throughout the process. CMS should be very explicit and transparent about the information it is seeking from patients and people with disabilities and how it will influence decisions.

CMS should solicit input from diverse communities to ensure representation of the diversity of the patients and communities affected by the topic.

The CMS Framework for Health Equity seeks to further advance health equity, expand coverage, and improve health outcomes.¹⁴ Additionally, the Inflation Reduction Act requires consideration of the differences among subpopulations. Therefore, it is crucial for the agency to formalize an engagement process that prioritizes feedback from diverse communities.

For example, PCORI places significant emphasis on the importance of diversity in patient engagement, particularly ensuring that research partnerships reflect diverse patients and communities affected by the topic. They explain diversity is essential to adequately address the needs of the targeted population, especially those with perspectives historically excluded from research.

NHC's roundtable on CMS' Medicare Drug Price Negotiation Program listening sessions highlighted concerns about the lack of racial and ethnic diversity among speakers and the inadequate accommodations for speakers with disabilities. To enhance the diversity of future patient engagement endeavors, NHC recommends that CMS collaborate with the Office of Minority Health and engage with minority-led patient advocacy groups to promote broader participant diversity.

Recommendation: We concur with the necessity of ensuring that health care research represents the affected population and encourage CMS to take a proactive approach in

¹⁴ CMS Office of Minority Health, "CMS Framework for Health Equity 2022-2032," published July 2022, <https://www.cms.gov/media/529636>

including diverse perspectives in patient engagement efforts. In addition to engaging the Office of Minority Health and minority-led patient advocacy groups, proactive engagement with PCORI and the National Institute on Minority Health and Health Disparities (NIMHD) may be useful to identify research priorities that capture diverse perspectives.

CMS should ensure that opportunities for patient engagement are accessible.

IVI and PCORI emphasize the significance of allocating dedicated funds and resources to support and compensate patient engagement. We concur with this perspective and recommend CMS take responsibility for ensuring the accessibility of their patient engagement opportunities. Patient and disability advocates have echoed these sentiments, urging CMS to allocate resources such as financial assistance, accessible materials, disability-friendly meeting arrangements, and extended input and comment periods.

The PATIENTS Program calls for accessible materials, emphasizing the use of plain language and health literacy principles to ensure patient understanding and inclusivity. They also advocate for diverse engagement approaches, recognizing that online-only methods may not be accessible to everyone. Notably, NHC recommends that Congress provide this support, along with funding and oversight, to strengthen CMS' engagement efforts.

Additionally, NHC recommends that CMS enhance its own accessibility. Communication with executive branch agencies can often be challenging due to bureaucracy and the need for institutional knowledge to communicate effectively. Streamlining the process for initiating dialogue, such as by creating an ombudsman or a clearly identified point of contact, is essential for effective engagement.

***Recommendation:** CMS should create an ombudsman for engagement of stakeholders from the patient and disability communities, dedicate funds and resources to support and compensate patient engagement, and ensure accessibility through use of plain language materials and by providing opportunities for engagement through written comments, in-person meetings and online events. We call attention to the recent regulations from the U.S. Department of Justice governing digital accessibility for people with disabilities and urge CMS' focus on compliance.*

To gauge both successes and challenges, CMS should establish a structured process for continuous review and assessment of its stakeholder engagement strategy.

PCORI's final expectation for patient engagement underscores the importance of gathering input and feedback throughout projects to pinpoint areas of success and areas for improvement, enabling adjustments in future engagement strategies. PCORI emphasizes that continuous learning is essential for enhancing engagement strategies, allowing researchers to assess whether engagement is effective, equitable, and as intended. The PATIENTS Program echoes PCORI's expectation, advocating for a third-party evaluation of

patient and stakeholder engagement to ensure transparency and accountability. Similarly, IVI advocates for integration of health equity throughout research initiatives, ensuring equitable design and implementation.

Recommendation: CMS should commit to continuous learning, refining its patient engagement strategy and promoting health equity as part of a structured assessment of what works and what does not work, in collaboration with engaged patients and people with disabilities.

Conclusion

We urge CMS to finalize guidance that not only assures patients and people with disabilities that its implementation of the program will be aligned with current law governing the use of value assessment, but also provides concrete steps the agency will take to facilitate meaningful engagement of patients and people with disabilities and rely on high quality sources of evidence. We appreciate CMS' consideration of our recommendations, offering a holistic approach to improving patient engagement across the agency. Embracing these recommendations will not only strengthen CMS' relationship with stakeholders but also pave the way for more effective and equitable health care delivery, ultimately benefiting patients and the health care system.

Sincerely,

Alliance for Aging Research
Alliance for Patient Access
ALS Association
American Association of Kidney Patients
American Association on Health and Disability
Autistic People of Color Fund
Autistic Women & Nonbinary Network
Biomarker Collaborative
Bone Health and Osteoporosis Foundation
Brain Injury Association of America
Buscher Consulting
Cancer Support Community
CancerCare
Caregiver Action Network
Caring Ambassadors
Cystic Fibrosis Research Institute
Davis Phinney Foundation for Parkinson's
Diabetes Leadership Council
Diabetes Patient Advocacy Coalition
Disability Rights California
Disability Rights Oregon

Epilepsy Alliance America
Epilepsy Foundation
Exon 20 Group
Familia Unida Living with MS
Family Heart Foundation
FORCE: Facing Our Risk of Cancer Empowered
Genetic Alliance and PXE International
Global Liver Institute
GO2 for Lung Cancer
Health Hats
HealthHIV
Heart Valve Voice US
Hypertrophic Cardiomyopathy Association
ICAN, International Cancer Advocacy Network
Johns Hopkins Disability Health Research Center
Lakeshore Foundation
Lupus and Allied Diseases Association, Inc.
MET Crusaders
MLD Foundation
Multiple Sclerosis Foundation
National Association of Councils on Developmental Disabilities
National Coalition for LGBTQ
National Disability Rights Network (NDRN)
NHMH - No Health without Mental Health
Partnership to Fight Chronic Disease
Partnership to Improve Patient Care
PD-L1 Amplifieds
RASopathies Network
Rosie Bartel
The Coelho Center for Disability Law, Policy and Innovation
The Headache and Migraine Policy Forum
The Hepatitis C Mentor and Support Group-HCMSG
Tourette Association of America
TSC Alliance