

December 23, 2020

Honorable Alex Azar
Secretary
U.S. Department of Health and Human Services
200 Independence Avenue SW
Washington, DC 20201

Re: CMS–5528–IFC

Dear Secretary Azar:

The undersigned organizations – representing patients, people with disabilities, caregivers, older Americans, providers, veterans, and others – are writing to urge the immediate, full withdrawal of the Most Favored Nation rule. We are deeply disturbed that the Administration would seek to misuse statute to impose a sweeping, unilateral change in national policy that threatens significant harm to the most vulnerable among us. We support the agency’s goal of lowering drug prices for all Americans, but it is critical that it is done in ways that work for all Americans. The MFN rule ignores the boundaries of statute established during the creation of the Center for Medicare and Medicaid Innovation that were put in place to protect patients, would import discriminatory standards for policymaking used in MFN nations, and lead directly to lack of access to needed treatments for many Americans. We are concerned that this rule will:

- Rely on cost-effectiveness assessment and the discriminatory Quality-Adjusted Life Year (QALY), which violates current statute that includes safeguards against the use of the QALY and similar metrics in Medicare.
- Lead to lack of and delayed access to needed treatments for Americans, as we currently see in the countries referenced in the rule.
- Lead to discrimination that is in direct conflict with American civil rights and disability policy by importing policies that rely on the underpinning that people with disabilities and seniors are less valuable and less worth treating than “healthy” people.
- Cause an undue disruption to the health care system, leading to immediate access issues to patients in already underserved areas due to the rule being implemented as a massive, mandatory, nationwide demonstration.

We urge CMMI to put in place safeguards to ensure that patients and people with disabilities are at the center of decision-making, including appropriate comment periods. We encourage the administration to work with our communities to develop patient-centered alternatives that

do not discriminate against the nation's most vulnerable and that recognize the inherent value of every person.

CMS Must Reject Use of Discriminatory QALY based Cost-Effectiveness Standards and Honor the Safeguards Against Their Misuse in Medicare

Under the new MFN model, Medicare would assign a new, lower reimbursement rate to providers for complex medications administered by physicians in their offices to patients with Medicare Part B. This rate would be set based on the lowest price paid in comparator countries, defined as OECD countries with GDP per capita of at least 60 percent of the U.S. GDP per capita.¹ Comparators include countries like the United Kingdom, Canada, Australia, and the Netherlands.² As you know, many of the reference countries make reimbursement and coverage decisions using on cost-effectiveness assessments that rely on the Quality-Adjusted Life Year (QALY) metric.³ QALY-based assessments assign a financial value to health improvements and outcomes. When applied to health care decision-making, the results can mean that some patients; particularly those with disabilities and chronic illnesses, and seniors; are deemed not worth the cost to treat.

We are concerned that, in adopting this model, CMS would undermine key protections against discrimination for patients and people with disabilities. Other experts agree with this assessment. In a recent report, the National Council on Disability (NCD), an independent federal agency, made the recommendation that "CMS should refrain from pursuing means of reducing Medicare and Medicaid prescription drug costs that attempt to model US pricing after the pricing in other countries, which may heavily rely on QALYs and often deny people with disabilities access to needed care." NCD's rationale for this argument is that consideration of the QALY in public programs would be contrary to United States civil rights and disability policy.⁴

The United States has a thirty-year, bipartisan track record of opposing the use of the QALY and other discriminatory metrics and putting appropriate safeguards in place to mitigate its use. We are concerned that MFN violates these protections for vulnerable communities.

¹ United States, Department of Health and Human Services, Centers for Medicare and Medicaid Services, "Medicare Program; Most Favored Nation (MFN) Model," Interim Final Rule with Comment," November 27, 2020. Accessed December 1, 2020.

² "GDP and Spending - Gross Domestic Product (GDP) - OECD Data." The OECD, 2019, data.oecd.org/gdp/gross-domestic-product-gdp.htm.

³ World Health Organization, The Health and Environment Linkages Initiative, "Cost-effectiveness analysis for health interventions," Accessed December 1, 2020

⁴ 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.

The Affordable Care Act directly states that the Secretary of Health and Human Services has no authority to deny coverage of items or services “solely on the basis of comparative effectiveness research” nor to use such research “in a manner 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, nondisabled, or not terminally ill.”⁵ Additionally, legislation specifically prohibits the development or use of a “dollars-per-quality adjusted life year (or similar measure that discounts the value of a life because of an individual’s disability) as a threshold to establish what type of health care is cost effective or recommended.” It also states, “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” (Medicare).”⁶ The MFN model appears to directly violate these critical and intentionally crafted safeguards.

The opposition to the QALY far pre-dates the ACA. Section 504 of the Rehabilitation Act ensures that people with disabilities will not be “excluded from participation in, be denied the benefits of, or otherwise be subjected to discrimination,” under any program offered by any Executive Agency, including Medicare.⁷ Title II of the Americans with Disabilities Act (ADA) extended this protection to programs and services offered by state and local governments.⁸ Based on this, in 1992, the George H.W. Bush Administration established that it would be a violation of the ADA for state Medicaid programs to rely on cost-effectiveness standards, as this could lead to discrimination against people with disabilities.⁹

Yet, MFN violates these safeguards, as well as clear precedent, by basing reimbursement decisions on countries that rely on QALY-based cost-effectiveness analyses.

QALY-Based Cost-Effectiveness Thresholds Will Lead to Lack of Access to Needed Treatments for Americans

The implications of discrimination against people with disabilities and chronic illnesses plays out clearly in MFN comparator countries that rely on cost-effectiveness analyses to determine coverage and reimbursement of prescription drugs. In these countries, cost-effectiveness assessments are used to restrict citizens’ access to needed treatments. For example, the National Institute of Health and Care Excellence (NICE) conducts cost-effectiveness analyses to determine what treatments and drugs will be covered by Britain and Wales’ National Health System (NHS). NICE’s reports are known to restrict patients’ access to care, particularly among

⁵ 42 USC Sec 1320e, 2017. Accessed November 30, 2020.

⁶ 42 USC Sec 1320e, 2017. Accessed November 30, 2020.

⁷ 29 USC Sec 794, 2017. Accessed November 30, 2020.

⁸ 42 USC Sec 12131, 2017. Accessed November 30, 2020.

⁹ Sullivan, Louis. (September 1, 1992). Oregon Health Plan is Unfair to the Disabled. *The New York Times*.

individuals with complex conditions.¹⁰ A 2018 Avalere Health study found that of over 329 health technology assessments (HTA) of cancer drugs created by governmental agencies between 2013 and 2017, NICE recommended access restrictions for nearly 70 percent and rejected 22 percent of the cancer drugs it assessed.¹¹

The same pattern exists across countries that rely on similar assessments to determine coverage and reimbursement. In Australia, 27 of the 55 medicines launched globally to treat cancer between 2012 and 2016 were not available in 2017;¹² and, in Canada, 38% of medicines to treat orphan conditions were rejected for coverage between 2002 and 2014.¹³ Even when medicines are available, there are frequently significant restrictions placed on medicine use that further limits access even after they have been prescribed by a physician. These restrictions lead to harmful delays in access to care for patients, and in some instances, patients may be required to get “sicker” before qualifying to access treatment, leading to irredeemable losses to their health. For example, Laura Stevens, a fourteen-year-old patient with Cystic Fibrosis, was forced to maintain a medically-induced 20% loss of lung function for six weeks before being granted access to Orkambi, a drug which had been available to patients in the U.S. for four years without comparable restrictions. Her doctors feared that this requirement would mean the medication was less effective once she received it, and that she would progress to the point where the only treatment option was a lung transplant.¹⁴

As cited by the National Council on Disability in their report,¹⁵ these access restrictions and delays have the effect of worse health outcomes for people in countries that rely on cost-effectiveness assessments. Survival rates for some types of cancer, like lung cancer, are higher in the U.S. than abroad.¹⁶ One recent study looking at non-small cell lung cancer found that if the actual access conditions in the U.S. between 2006 and 2017 were replaced with access conditions in five ex-U.S. comparator countries, the aggregate survival gains for U.S. patients

¹⁰ Sarah Long, “Squashed Dreams and Rare Breeds: Ableism and the Arbiters of Life and Death,” *Disability and Society* 30 (2015): 1118–22.

¹¹ Kathy Hugues and Nikita Jeswani, “HTA Oncology Recommendations Have Grown More Restrictive Over Time,” Avalere, June 1, 2018, <https://avalere.com/insights/htas-recommendations-for-oncology-have-grown-more-restrictive-over-time>.

¹² IQVIA. *Global Oncology Report*, 2018.

¹³ S Mardiguian, M Stefanidou, et al. Trends and key decision drivers for rejecting an orphan drug submission across five different HTA agencies. (2014).

¹⁴ Stevens, Sandy. “I Am a Canadian. Don’t Follow Our Lead on Drug Pricing.” Cystic Fibrosis Research, Inc., 2020, cfri.org/covid-19-advocacy/white-pink-and-yellow-blister-packs-163944/.

¹⁵ 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.

¹⁶ Ziba Kashef, “Disparities Found in Lung Cancer Care, Survival in U.S. Versus England,” *YaleNews* (blog), May 1, 2018, <https://news.yale.edu/2018/05/01/disparities-found-lung-cancer-care-survival-us-versus-england>

would have been cut in half.¹⁷ Since the NCD report, Avalere studied the availability of breast cancer treatments in England, determining that in 2017 and 2018, more than half (56%) of Medicare patients with breast cancer taking a medicine covered by Medicare Part B had received a therapy that was not routinely covered by England's National Health Service (NHS) or its Cancer Drug Fund (CDF).¹⁸

NDC recently recommended the Administration abandon international referencing pricing policies, stating "there has been increasing interest by the Federal Government in reducing the cost of health care by modeling parts of its national health insurance programs after the healthcare systems of other countries, such as the United Kingdom. Several of these countries utilize QALYs to make benefits and coverage decisions. The coverage denials and loss of access to care faced by people with disabilities in these countries illustrate what might happen if the United States made a similar choice."¹⁹

Importing Foreign Pricing Models Has Concerning Moral and Ethical Implications

There is widespread opposition to QALY-based cost-effectiveness thresholds being used in health care decision making because of its many ethical and methodological flaws.^{20,21} The QALY methodology uses numeric 'utilities' to quantify the value of different health states. The utility assigned to a given hypothetical state of health is based upon the preferences of the general public as measured by large, country-specific surveys. The highest possible utility for a health state is 1, representing perfect health. Zero represents death. Thus, the QALY assumes that time spent in some states is more desirable than others. For example, paraplegia is identified by some QALY systems at approximately 0.5, implying that the lives of people with paraplegia are worth approximately half the lives of individuals without.²² Some QALY systems

¹⁷ Wayne Su, "Comparing Health Outcome Differences Due to Drug Access: A Model in Non-Small Cell Lung Cancer," IHS Markit, December 13, 2018, https://cdn.ihs.com/www/prot/pdf/0119/IHSM_NSCLC%20HTA%20model%20white%20paper_18Jan2019r.pdf.

¹⁸ M Sullivan, M Adelsberg, et. al, "Medicare Covers More Part B Therapies for Breast Cancer than NHS England," Avalere, December 9, 2020, <https://avalere.com/insights/medicare-covers-more-part-b-therapies-for-breast-cancer-than-nhs-england>

¹⁹ 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.

²⁰ Knapp M, Mangalore R: "The trouble with QALYs...". *Epidemiologia e psichiatria sociale* 2007, 16(4):289-293.

²¹ Brazier JE, Rowen D, Lloyd A, Karimi M. Future directions in valuing benefits for estimating QALYs: is time up for the EQ-5D?. *Value in Health*. 2019 Jan 1;22(1):62-8.

²²Haagsma JA, Polinder S, Cassini A, Colzani E, Havelaar AH. Review of disability weight studies: comparison of methodological choices and values. *Popul Health Metr*. 2014;12:20. Published 2014 Aug 23. doi:10.1186/s12963-014-0020-2

have even gone so far to assign health states, like severe ALS, negative utilities, implying there are health states worse than death.²³

When QALYs are applied to assessing the value of a treatment, they will inherently find that treatments designed to treat younger, healthier populations have “higher value” and will undervalue treatments designed to treat older, chronically ill, and disabled populations. Consequently, the QALY will then find “less value” in treatments that maintain the current quality-of-life or provide incremental improvements over treatments that can “cure” a patient. This means that treatments that provide incremental quality of life improvements for chronic illnesses or disabilities are found to be low value, when the indicated populations will often make clear that incremental improvements are of great value to them. For example, a recent cost-effectiveness assessment²⁴ gave the utility score of 0.21 for “early non-ambulatory” Duchenne muscular dystrophy. A treatment that was able to extend the life of a person in this health state without large quality of life improvements was only credited for a fraction of the life years extended, whereas people with conditions that are “curable” have a larger percentage of value attributed to their extended life years.

This type of methodology entirely ignores the fact that a person living with a disability or chronic illness may be just as satisfied with their life as a “healthy” individual and should be granted the same access to treatment.²⁵ Valuing individuals with “perfect” health more than those who are disabled or chronically ill is mired with ethical issues. It is also entirely counterintuitive to America’s Constitution and values prioritizing equality of all citizens. Our public policies should advance the goal of equal access to care for all Americans. A prime example of this is the Emergency Medical Treatment and Labor Act (EMTALA), a federal law that requires anyone coming to an emergency department to be stabilized and treated regardless of their ability to pay.²⁶ Currently the COVID-19 pandemic has opened the nation’s eyes to an unequal health care system in which communities of color are being disproportionately impacted by the pandemic. The public policy response should be to look for solutions to remedy these disparities, not entrench them, and drive health equity.²⁷

²³ Ackerman, S.J., Sullivan, E.M., Beusterien, K.M. et al. Cost Effectiveness of Recombinant Human Insulin-Like Growth Factor I Therapy in Patients with ALS. *Pharmacoeconomics* 15, 179–195 (1999). <https://doi.org/10.2165/00019053-199915020-00006>

²⁴ https://icer-review.org/wp-content/uploads/2018/12/ICER_DMD-Final-Report_081519-1.pdf Accessed December 1, 2020

²⁵ Pettitt, D., Raza, S., Naughton, B., Roscoe, A., Ramakrishnan, A., Davies, B., . . . Brindley, D. (2016). The Limitations of QALY: A Literature Review. *The Journal of Stem Cell Research & Therapy*, 6(4).

²⁶ Centers for Medicare & Medicaid Services. (2012, March 26). Emergency Medical Treatment & Labor Act (EMTALA). Retrieved 2018, from <https://www.cms.gov/regulations-and-guidance/legislation/emtala/>

²⁷ The Times Editorial Board (2020) ‘Editorial: Who do we save from coronavirus and who do we let die? Take wealth, race and disability out of that brutal equation’. *The Los Angeles Times*, 25 April.

Rather than moving us toward a more equitable system, importing QALY-based pricing from abroad would deepen and entrench disparities to care faced by vulnerable communities, which is counterintuitive to our moral and ethical obligation to treat all humans as equals.

CMMI’s MFN “Demonstration” is a Mandatory Policy Change that Imports Discriminatory Measures on a National Scale into the United States Health Care System

It is essential that, in a quest to save money, CMS does not harm the populations Medicaid and Medicare are designed to serve: people with disabilities, patients, seniors, and other vulnerable populations. CMS proposes to implement the MFN rule as a sprawling “demonstration” under the statute that created the Center for Medicare and Medicaid Innovation (CMMI). CMMI was specifically designed to test new methods of health care delivery on a small scale, providing the opportunity to evaluate their efficacy and determine if they lead to adverse effects – such as lack of patient access – without forcing these potentially detrimental effects on the entire health care system.

MFN is a mandatory, nationwide demonstration that unilaterally seeks to impose a radical change in policy that threatens the health of millions of Medicare patients the livelihoods of thousands of health care professionals. The roll out of the MFN rule as a nationwide, mandatory model means all providers will be forced into the model, which will translate into many of them ceasing to see Medicare patients, as they will receive a lower reimbursement rate for seeing these patients immediately, leading directly to lack of access for patients.

As some providers either stop seeing Medicare patients or stop offering the treatments captured by this rule, this will force patients see either forego care or switch to a potentially less effective treatment. These impacts will be felt particularly poignantly by communities that already have less access to high-quality health care, including those who live in rural areas, people of color, seniors, and those with disabilities who may not be able to travel long distances to receive care. This will be detrimental, as timely and sustained access to needed medications is critical for many chronically ill and disabled people to experience a higher quality of life and integrate and engage with their families and communities. It would be deeply troubling to impose such a radical change under such a rushed process under any circumstances; to do it in the midst of a fight against a deadly pandemic is unconscionable.

Restricted access is not a theoretical concern; the MFN rule acknowledges outright that the new rule will lead to reduced access for some patients.

Since the rule will reduce the price Medicare pays for medications but will not adjust how providers are compensated for seeing Medicare patients, providers will see a steep cut in their payments. As a result of this, the Office of the Chief Actuary (OACT) foresees that many providers will stop seeing Medicare patients. The rule acknowledges that this will lead to a portion of patients no longer having access to needed treatments and estimated that by 2027,

19% of current prescriptions will have access barriers. The rule states, “while there are significant savings as a result of this model, a portion of the savings is attributable to beneficiaries not accessing their drugs through the Medicare benefit, along with the associated lost utilization.”²⁸

We firmly believe that CMS should not pursue policies that limit patients’ access to needed treatments in an effort to lower government spending on health care.

MFN is a sweeping rule that stretches well beyond CMMI’s mandate on conducting targeted, and measurable demonstrations.

CMMI Needs to Institute Safeguards that Ensure Patients and People with Disabilities are at the Center of Decision Making

We were hopeful when the administration released the “New Direction for CMMI” document in 2017, which highlighted a “new focus on patient-centered care.” However, we are disappointed that the agency seems to have discarded this strategy and has instead advanced a nationwide, mandatory demonstration that is inherently discriminatory without appropriate comment periods. The abandonment of standard procedure makes it more difficult for patients and people with disabilities to provide input on this policy that will have detrimental impacts for our communities. We urge the administration to listen to patient and disability experts who have long advocated for some basic safeguards in CMMI to ensure that models do not endanger patient access to healthcare providers and medically necessary treatments, creating unnecessary barriers for vulnerable patients. Advocates have consistently urged three concrete steps: (1) Establish the “patient-centeredness criteria” mandated under Section 1115A of the Affordable Care Act, which requires evaluation of alternative payment models against patient-centeredness criteria; (2) convene a patient and consumer advisory panels for each of the CMMI models under development as well as those currently being implemented; and (3) define “informed decision-making” as a core criterion of patient-centeredness and a goal of each alternative payment model.²⁹

In addition to these patient-centered safeguards, new proposals should not seek to waive protections against cost-effectiveness standards currently in statute. We encourage the inclusion of specific language in future proposals that explicitly indicate the administration’s intent to abide by all current protections against the QALY and cost-effectiveness assessments.

²⁸ United States, Department of Health and Human Services, Centers for Medicare and Medicaid Services, “Medicare Program; Most Favored Nation (MFN) Model,” Interim Final Rule with Comment,” November 27, 2020. Accessed December 1, 2020.

²⁹ Partnership to Improve Patient Care, “PIPC Submits Joint Letter to CMMI Emphasizing Patient-Centered Payment Models,” Letter, August 2, 2018. Accessed December 5, 2018.

As We Look to Address Health Care Costs in the United States, Patients and People with Disabilities Must be at the Center of the Discussion Around Value in Health Care

As people with disabilities and chronic conditions that rely heavily on health care services, our communities agree that we need to look for constructive solutions to lower the cost of all health care. We are disappointed, though, that these policy discussions have largely omitted representatives of the patient and disability communities. It is essential that in our path toward solutions, patients and people with disabilities are engaged in the earliest stages of policy development and discussion to ensure policy solutions meet their needs and do not lead to discrimination or health care rationing that would limit their access to needed treatments.

As we look to build a more patient-centered model, we would suggest following the blueprint set forth by the Patient-Centered Outcomes Research Institute (PCORI). PCORI conducts comparative clinical-effectiveness research and is statutorily required to include patients in every step of their research, from beginning to end. PCORI's aim is to answer the question of which treatment is best for whom. This gets to the point of providing "high-value" care for each individual and can provide valuable information to patients and providers as they are making care choices together. PCORI is also banned from conducting cost-effectiveness assessments or using the QALY or similar metrics due to their discriminatory nature.³⁰ It has a new mandate to collect cost outcomes data without using a cost effectiveness metric such as QALYs.³¹

We are hopeful that PCORI's work to advance both patient-centered comparative effectiveness research and patient-reported outcomes will be able to illuminate the complex relationship between economic impacts, benefits, and harms impact treatment decisions and treatment adherence.

We wish to partner with the Administration and Congress to advance alternative payment models that put patients and people with disabilities at the center of health care decision making. In doing so, we must maintain our commitment to nondiscrimination and abide by the safeguards against the use of the QALY and similar metrics in current statute. Looking abroad it is very clear that the most vulnerable suffer when subjected to cost-effectiveness standards to determine coverage and reimbursement. We should not import these discriminatory policies designed to ration care, which would hurt our nation's most vulnerable.

In conclusion, the MFN model is not in the best interest of America and would disproportionately harm seniors, patients, people with disabilities, people of color and other vulnerable communities. For these populations, access to the care they need in a timely manner is critical and directly translates into longer and higher quality lives. The MFN rule would jeopardize their ability to access the care needed to achieve their individual optimal

³⁰ "Primer: PCORI Background, Funding Streams, and Reauthorization." Partnership to Improve Patient Care, www.pipcpatients.org/blog/primer-pcori-background-funding-streams-and-reauthorization.

³¹ INSERT CITE

health outcomes. We urge the administration to abide by current statute, which forbids the use of QALY and similar metrics in public programs and abandon the MFN model. We encourage the administration to work directly with patients and people with disabilities to determine an appropriate way to lower health care costs that does not undermine their access to care.

We, the undersigned organizations, urge the Administration to abandon the MFN rule that would import cost-effectiveness standards to the U.S. and undermine key protections for patients, people with disabilities, veterans and seniors. Your response may be directed to Tony Coelho, Chairman of the Partnership to Improve Patient Care, 100 M St SE, Suite 750, Washington, DC 20003 or tony@pipcpatients.org.

Sincerely,