Draft – Letter on CMS IRA Guidance

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Centers for Medicare & Medicaid Services
U.S. Department of Health and Human Services
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Attn: PO Box 8016

Re: Medicare Drug Price Negotiation Program: Initial Memorandum, Implementation of Sections 1191 – 1198 of the Social Security Act for Initial Price Applicability Year 2026

Submitted via email: IRARebateandNegotiation@cms.hss.gov

Dear Administrator Seshamani:

The Partnership to Fight Chronic Disease and the XX undersigned organizations, representing patients, caregivers, providers, and other stakeholders, appreciate the opportunity to share comments, concerns, and opportunities for improving the Medicare Drug Price Negotiation Program: Initial Memorandum.

The Inflation Reduction Act (IRA) of 2022 involves sweeping changes to Medicare including the new Medicare Drug Price Negotiation Program. Given the wide-ranging implications for Medicare beneficiaries today and in the future and the vulnerability of the individuals affected, care to identify and avoid unintended consequences is paramount. These changes also do not occur in a vacuum but are made more complex by challenges with workforce shortages and other access issues that adversely affect many and risk worsening existing health disparities. We urge the Centers for Medicare & Medicaid Services (CMS) to remain vigilant and avoid unintended consequences given the significant changes implementation of the drug negotiation program represents.

Development and implementation of Medicare Drug Price Negotiation Program should accommodate meaningful engagement opportunities for beneficiaries and caregivers.

Given the significance of the changes involved, CMS should seek and allow for more significant and meaningful beneficiary, caregiver, patient, and provider engagement. However, the proposed process and timelines described in the IRA guidance significantly limit those opportunities. When comments are allowed, they involve extremely short timelines for a response. In the guidance, CMS notes that it seeks to include "patient experience" and "factors

that are of importance to a person". Additionally, CMS seeks to include evidence, including the consideration of real-world evidence, from "Medicare populations, including on individuals with qualifying disabilities, patients with end-stage renal disease (ESRD), and Medicare-aged populations, as particularly important."[1] To do so meaningfully, however, allowing sufficient opportunity to analyze the requests, gather evidence to respond, and draft a response takes time.

Given the time, staff, and expertise needed to respond in a timely manner, the short 30-day timeframes for comment will disenfranchise many—particularly those who are already underrepresented and under-resourced. Providing greater transparency in the process and building in more opportunities for engagement, including opportunities to shape data collection, analysis, policy development and ultimately implementation, should be a priority, not just a motion to collect responses to proposed policies. The importance of doing things "right now" should not surpass the importance of doing things "right". Seeking input on this guidance document instead of allowing for more formal engagement from experts outside of CMS through a more typical proposed rulemaking and comment period, for example, means less input on the program from stakeholders. Further, the 91-page guidance limits areas open for comment and offers a tight timeframe within which the public may comment. These actions are indicative of emphasizing implementation speed over beneficiary impact and public trust.

We urge CMS to consider the proposed process and identify opportunities for public comment and feedback that will not only facilitate implementation of this new program, but more importantly assist CMS in avoiding preventable adverse consequences. For example, though this letter identifies several areas where patient-centric perspectives would enhance the proposed guidance, **the fact that the 91-page guidance does not once mention the health disparities or health equity challenges within Medicare and potential effects of this program is a gross oversight**. That would not have occurred had CMS engaged with patients and beneficiaries earlier in the process.

We are also concerned that the guidance from CMS fails to acknowledge the role of caregivers in supporting patients and the importance of their perspective in the value of treatment they receive. We urge CMS to account for factors that caregivers view as important to them and engage caregivers in the process established in the Drug Price Negotiation Program.

CMS too narrowly defines "unmet medical need."

Section 1194(e)(2) of the IRA directs CMS to consider evidence of "unmet medical need" of the drug subject to price negotiations and any therapeutic alternatives: "The extent to which the selected drug and the therapeutic alternative to the drug address unmet medical needs for a condition for which treatment or diagnosis is **not addressed adequately by available therapy**." (Emphasis added). In the guidance, CMS states that it "intends to define [unmet medical need] as treating a disease or condition in cases where very limited or no other treatment options

1 Center for Medicare, Medicare Drug Price Negotiation Program: Initial Memorandum. March 15, 2023.

exist." Adequacy, however, is a much different concept than "very limited or no other treatment options." Adequacy in terms of unmet medical need should be defined more broadly given the heterogeneity of the populations which Medicare serves, the commonality of comorbidities (76% of beneficiaries have three or more chronic conditions), and the significant health disparities that factor into patient need and preference considerations.

The heterogeneity of populations served by Medicare, their needs, and treatment effects should be primary considerations for unmet medical need. Elsewhere in the guidance, CMS notes that information about heterogeneity of treatment affects and the population which CMS plans to consider but ignores "individuals with disabilities, the elderly, individuals who are terminally ill, children, and other patient populations represented among Medicare beneficiaries." Similarly, the guidance notes that in consideration of clinical benefits, CMS will consider potential risks, harms, or side effects, including "any unique scenarios or considerations related to clinical benefit, safety, and patient experience." CMS should not ignore those same considerations and the diversity of needs among Medicare beneficiaries as a significant consideration within unmet medical need.

A more robust, patient-centered definition and approach to evaluating unmet medical need is available in the authorizing statute for the Patient-Centered Outcomes Research Institute (PCORI) where federal law requires consideration of the "needs, outcomes and preferences" of patients.[2] Unmet medical need should incorporate consideration of both the needs and preferences of people living with one or more chronic conditions who may value a treatment with fewer side effects and contraindications. People, particularly those living with disabilities or with limited transportation options or health care access (e.g., in rural areas or care deserts), may need and prefer modes of administration that do not require traveling and involve less frequent administration. A narrow definition of unmet need may also further lead to the undervaluing of communities of color who may have pre-existing health deficits due to numerous inequities including the negative consequences of social determinants of health[3] and racism,[4] including reduced access to care,[5] reduced quality of care,[6] and higher prevalence of disease and disease-related mortality.[7]

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2 42 USC Sec 1320e(d)(1)(A)

3 National Snapshots of Social Determinants of Health. HealthyPeople.gov. Available at: https://www.healthypeople.gov/2020/topics-objectives/topic/social-determinants-of-health/national-snapshot

4 Boyd RW, Lindo EG, Weeks LD, McLemore M. On Racism: A New Standard for Publishing on Racial Health Inequities. Health Affairs. 2 July 2020. Available at: https://www.healthaffairs.org/do/10.1377/hblog20200630.939347/full/

5 Artiga S and Orgera K. Key Facts on Health and Health Care by Race and Ethnicity. Kaiser Family Foundation. 12 Nov 2019. Available at: https://www.kff.org/report-section/key-facts-on-health-and-health-care-by-race-and-ethnicity-introduction/

6 2019 National Healthcare Quality and Disparities Report. Content last reviewed June 2021. Agency for Healthcare Research and Quality, Rockville, MD.https://www.ahrq.gov/research/findings/nhqrdr/nhqdr19/index.html

7 Minority Population Profiles. Office of Minority Health. Available at: https://www.minorityhealth.hhs.gov/omh/browse.aspx?lvl=2&lvlid=26

There is also an important opportunity for considering unmet medical need as being more encompassing of patient needs and realities in evaluating the adequacy of existing therapeutic options. In FDA's Guidance for Industry on Expedited Programs for Serious Conditions—Drugs and Biologics, the FDA defines unmet medical need as a "condition whose treatment or diagnosis is not addressed adequately by available therapy."[8] FDA further describes a new treatment as addressing an unmet medical need if it, "has an improved effect on a serious outcome(s) of the condition compared with available therapy."[9] Other considerations include having "an improved effect on a serious outcome(d) of the condition compared with available therapy," "has an effect on a serious outcome of the condition in patients who are unable to tolerate or failed to respond to available therapy," or "provides safety and efficacy comparable to those of available therapy but has a documented benefit, such as improved compliance, that is expected to lead to an improvement in serious outcomes." None of these factors are captured in CMS's currently proposed definition of "unmet medical need".

Also, reliance on averages to define unmet medical need misses the needs of subpopulations for whom therapeutic options are more limited because of their health status or considerations of social determinants of health. This is especially relevant for millions managing multiple chronic conditions. For example, research provides evidence of racial and ethnic health disparities in outcomes and prevalence of chronic illness among Medicare beneficiaries. Many report poorer health status, higher rates of ED visits and hospitalizations, but fewer doctor's visits.[10] All of these realities factor into consideration of choice and success of prescription drug regimens, adherence, and outcomes. Failure to consider these factors in unmet medical need may further exacerbate existing health disparities and outcomes for Medicare beneficiaries.

By articulating a clear and patient centered definition of "unmet medical need" CMS has an opportunity to create standards and send signals for the types of medical advances that will support the needs of patients for years to come.

The Quality-Adjusted Life Year (QALY) has no place in the guidance given its inherent bias against older adults and people living with disabilities.

We appreciate CMS's recognition of the problems with QALYs and pledge to not consider QALYs outside of clinical effectiveness as noted in the IRA Guidance. For a program designed to primarily serve older adults and people living with qualifying disabilities, however, QALYs and evidence based on QALYs should not be a factor for consideration at all. As noted in the seminal report by the National Council on Disabilities, the QALY discriminates against these populations[11] and pledges to limit their use do not fully eliminate this reality. The QALY undervalues interventions intended for populations with shorter life spans, which include many

8 https://www.fda.gov/files/drugs/published/Expedited-Programs-for-Serious-Conditions-Drugs-and-Biologics.pdf
9 Ibid.
10 https://www.kff.org/medicare/report/racial-and-ethnic-health-inequities-and-medicare/#:~:text=Among%20Medicare%20beneficiaries%2C%20people%20of,have%20higher%20rates%20of%20hospital
11 https://ncd.gov/sites/default/files/NCD_Quality_Adjusted_Life_Report_508.pdf

of the communities for which CMS has expressed a particular interest: older populations and people living with disabilities, as well as people of color. We strongly encourage CMS to adopt the prohibition of reliance on the QALY in Medicare as required as part of the Affordable Care Act and avoid using either metric in evaluating clinical effectiveness or factoring these metrics into developing a fair maximum price.

CMS should protect patient access beyond requiring coverage.

Coverage does not equate to access. Utilization management techniques including, "fail first" or non-clinical step therapy, and prior authorization erect significant barriers to access for patients. We strongly encourage CMS to monitor benefit design and address potential barriers to access in addition to requiring coverage for drugs subject to the drug pricing program.

We appreciate the complexities involved in implementing this significant shift in Medicare and financing of prescription drug coverage. We also appreciate the opportunity to provide comments we hope will aid implementation in ways that protect and enhance beneficiary access to the medicines they need to maintain and enhance their health. We stand ready to assist in that regard and urge CMS to re-evaluate the proposed process for evaluating drugs and determining pricing to allow for additional, meaningful public input and beneficiary engagement in the process.

Signed,
Partnership to Fight Chronic Disease (PFCD)