National Pharmaceutical Council

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Ms. Lara Strawbridge
Deputy Director for Policy, Medicare Drug Rebate and Negotiations
Centers for Medicare & Medicaid Services
U.S. Department of Health and Human Services
7500 Security Boulevard
Baltimore, MD 21244-8016

Submitted Electronically via: http://www.regulations.gov

RE: CMS–10847 Information Collection Request for Negotiation Data Elements under Sections 11001 and 11002 of the Inflation Reduction Act

Dear Deputy Director Strawbridge:

The National Pharmaceutical Council (NPC) appreciates the opportunity to submit comments regarding the Centers for Medicare & Medicaid Services (CMS) Notice, *CMS–10847 Information Collection Request for Negotiation Data Elements under Sections 11001 and 11002 of the Inflation Reduction Act* (ICR or the ICR).

NPC is a health policy research organization dedicated to the advancement of good evidence and science and to fostering an environment in the United States that supports medical innovation. We have rich experience conducting research and disseminating information about the critical issues of evidence, innovation and the value of medicines for patients. Our research helps inform important healthcare policy debates and supports the achievement of the best patient outcomes.

NPC's research and that of others have found that public policies that reduce the incentives to invest in research and development result in less innovation, fewer treatment options, and lower life expectancy.[1] The Inflation Reduction Act (IRA) creates a new price-setting mechanism that will change the economic incentives for bringing new medicines to market, and evidence

[1] Ciarametaro M and Buelt L. Assessing the effects of biopharmaceutical price regulation on innovation. 2022. https://www.npcnow.org/resources/assessing-effects-biopharmaceutical-price-regulation-innovation; Thomas A. Abbott & John A. Vernon, 2007. "The cost of US pharmaceutical price regulation: a financial simulation model of R&D decisions," Managerial and Decision Economics, John Wiley & Sons, Ltd., vol. 28(4-5), pages 293-306; Leonard D. Schaeffer Center for Health Policy & Economics. Annual Report 2020. https://healthpolicy.usc.edu/wp-content/uploads/2021/03/Schaeffer-Center-2020-Annual-Report.pdf

suggests manufacturers are already responding to those incentives.[2] The importance of implementing the price-setting provisions of the IRA in a manner that accurately values medicines and maintains patient access cannot be overstated. This new process forces manufacturers to accept CMS's final price, face an unreasonable excise tax, or exit the market – all of which threaten the development of, and patient access to, new treatments or cures.

We appreciated the opportunity to provide input on the *Medicare Drug Price Negotiation Program: Initial Memorandum, Implementation of Sections 1191 – 1198 of the Social Security Act for Initial Price Applicability Year 2026, and Solicitation of Comments* (Guidance or the Guidance). We further appreciate the opportunity to comment on the ICR and encourage CMS to use discretion in designing the Negotiation Data Elements information collection in a way that neither inappropriately burdens manufacturers nor constrains the ability of stakeholders to fully communicate relevant information in the context of a transparent drug evaluation process. Specifically, NPC makes the following recommendations:

1. Increase Transparency and Flexibility around CMS's Process.
2. Reduce Manufacturer Burden While Removing Inappropriate Constraints.
3. Clarify and Expand Patient-Centered Data Elements on the Value of Treatments.
    a. Specifically recognize the voice of caregivers and patient advocacy organizations (Question 39).
    b. Encourage respondents to provide rationale for their choice of therapeutic alternatives (Questions 40-43).
    c. Collect clarifying information on how submitted evidence has been separated from QALYs (Questions 40-43).
    d. Expand outcomes to consider surrounding Therapeutic Impact and Comparative Effectiveness (Question 41).
    e. Expand definition of unmet need (Question 43).
    f. Expand opportunities for patients and caregivers to describe preferences and priorities.

<u>Increase Transparency and Flexibility around CMS's Process.</u>

This form, even in combination with the Guidance, provides limited insight into how CMS will evaluate drugs, or the factors considered during the price-setting process. With more information on the decision-making framework, respondents can better structure their data responses to include relevant information for CMS's evaluation. More robust information about the evaluation criteria also builds trust in the price-setting process and would improve the

[2] Grogan J. (2022) The Inflation Reduction Act Is Already Killing Potential Cures. WSJ. https://www.wsj.com/articles/the-inflation-reduction-act-killing-potential-cures-pharmaceutical-companies-treatment-patients-drugs-prescriptions-ira-manufacturers-11667508291 Longo, N. (2023). WTAS: Inflation Reduction Act already impacting R&D decisions. PhRMA. Available at: https://catalyst.phrma.org/wtas-inflation-reduction-act-already-impacting-rd-decisions; Powaleny, Andrew. (2023). IRA Impacts: Cancer treatment research and development. PhRMA. Available at: https://catalyst.phrma.org/ira-impacts-cancer-treatment-research-and-development; Longo, N. (2023). WTAS: Inflation Reduction Act already impacting R&D decisions. PhRMA. Available at: https://catalyst.phrma.org/wtas-inflation-reduction-act-already-impacting-rd-decisions; IRA survey: Biotechs bracing for impact. Biocentury. March 16, 2023.

utility of data submitted. We therefore strongly encourage CMS to specify how the data elements will be used in its price-setting process. We further encourage CMS to consider revisions to the Negotiation Data Elements questions in the context of creating a transparent, robust, and replicable framework based in scientific principles for their drug evaluation process, enabling manufacturers and other stakeholders to submit data that will meaningfully inform the drug evaluation process. Because public submission comes with a cost of sorting through and identifying studies that are both high quality and relevant, we recommend CMS make public the procedures by which evidence is identified and included in its assessments. Transparent standards informed by accepted rubrics for evaluating the quality of studies based on diverse data sources, including clinical trials, patient registries, and other real-world data, will promote the use of methodologically rigorous evidence in the evaluation process. While we encourage transparency in evidentiary standards and evaluation processes to build credibility and trust in the process, we also note the utmost importance of maintaining confidentiality of proprietary information. We encourage CMS to state clearly how proprietary data will be stored, accessed, and protected to ensure confidentiality.

CMS's framework for drug evaluation and the Negotiation Data Elements questions would further benefit from flexibility that promotes a collaborative approach to information exchange. Stakeholders should be given opportunities to provide comprehensive evidence on drug value, including information on non-statutory factors and new evidence as it becomes available. Additionally, flexibility in the Data Negotiation Element questions would better accommodate variable approaches across the industry to characterizing data elements, including development programs, which may lead to inappropriate comparisons and assessments. NPC urges CMS to provide greater flexibility in the ability of broad stakeholders to communicate evidence and meaningfully engage with the evaluation process. Constraining diverse stakeholders to a rigid, one-size-fits-all approach to providing evidence limits the robustness of stakeholder engagement and the evaluation process.

Reduce Burden While Removing Inappropriate Constraints.

In its implementation of the IRA, NPC urges CMS to focus on clinical benefits and cost offsets when comparing treatments and determining value, and not to reduce the preliminary price by information unrelated to the value of a treatment (e.g., cost-recovery, remaining exclusivity, etc.). We recognize that under the IRA statute [1194(e)(1)], the Secretary shall consider manufacturer-specific information during the price-setting process, including research and development costs and their recoupment, market data for the drug, unit costs of production and distribution, and Federal financial support. However, the ICR questions soliciting manufacturer-specific data are misaligned with a drug evaluation process focused on the value of treatments. Further, many questions introduce considerable response burden. To respond to the in-depth and specific questions on research and development costs, unit costs, and market data with both accuracy and clarity requires more time and space than is allotted through the current ICR. For example, historical development costs for products approved over seven years ago may easily date back two decades. Given our recommendation that CMS should not reduce the preliminary price by information unrelated to the value of a treatment to US patients and

health systems, as well as the logistical burdens and feasibility concerns surrounding the questions in Sections C (Research and Development), D (Unit Costs of Production and Distribution), and G (Market Data, Revenue, and Sales Volume Data), we encourage CMS to limit the *required* submission of elements in these sections.

While many required Negotiation Data Elements questions, particularly those outlined in the IRA statute in 1194(e)(1), are unduly burdensome, others, notably those in Section H that relate to the elements in the IRA statute in 1194(e)(2), inappropriately constrain stakeholders' ability to fully communicate information relevant to the drug evaluation process. Principles of good comparative effectiveness research – as well as the practices and policies of other payers and regulators - include robust stakeholder engagement. Given their vast knowledge of their products and therapeutic areas, pharmaceutical manufacturers and their pharmacoeconomic researchers are critically important sources of information on the value of treatments for payer decision-making. The ability of stakeholders to communicate relevant information should not be constrained by arbitrary and limited word counts. For example, patient-centered unmet medical needs are multifaceted and may reflect patient preferences, heterogeneity in response to existing treatment options, and improvements in benefits not captured in conventional measures of health gain (e.g., caregiver benefit, equity, and patient dignity).[3] Despite this, Question 43: Addressing Unmet Medical Needs in the ICR restricts respondents to only 1000 words. We urge CMS to eliminate restrictive word counts for information collection responses. At a minimum, we encourage CMS to take the time needed to build an informed and rigorous process and methodology, including eliminating these word counts, for initial price applicability year (IPAY) 2026. As information will develop and change over the course of the negotiation process, particularly for IPAY 2026, manufacturers should be given opportunities to supplement their initial responses and have due consideration.

Clarify and Expand Patient-Centered Data Elements on the Value of Treatments.

The ICR ostensibly provides opportunities for manufacturers, clinicians, and patients to provide evidence about the selected drug and alternative treatments. However, the questions, definitions, and word limits in Section H: Evidence About Alternative Treatments limit stakeholders' ability to communicate critical information about the impact and value of treatments. NPC encourages CMS to specifically seek and incorporate feedback throughout the evaluation process from key stakeholders, including patients, caregivers, clinicians, and manufacturers. Regarding the ICR, we urge CMS to:

a. *Specifically recognize the voice of caregivers and patient advocacy organizations (Question 39).* In Section H: Evidence About Alternative Treatments, respondents may check the relevant box which best describes the person completing the form. Options include "representative of a trade association or patient advocacy

[3] Synnott PG, Voehler D, Enright DE, Kowal S, Ollendorf DA. The Value of New: Consideration of Product Novelty in Health Technology Assessments of Pharmaceuticals. Appl Health Econ Health Policy. 2023 Mar;21(2):305-314. doi: 10.1007/s40258-022-00779-0. Epub 2022 Dec 19. PMID: 36529826

organization," and "a patient who has experience taking this drug." Caregivers' views of the benefits of drugs are essential to understanding the full range of clinical and patient-centered outcomes.[4] Caregiver costs and burden are meaningful outcomes that are often poorly captured in existing data sources. We encourage CMS to explicitly recognize the important voice of caregivers by including caregivers as a category of respondent in Question 39. Additionally, we encourage CMS to recognize the unique role of patient advocacy organizations in promoting the priorities and voice of patients by creating a separate category of respondent for these organizations.

b. *Encourage respondents to provide rationale for their choice of therapeutic alternatives (Questions 40-43).* Questions throughout Section H: Evidence About Alternative Treatments solicit information about the selected drug and its therapeutic alternative(s) but does not explicitly ask for feedback on the selection of therapeutic alternatives. NPC recommends that the choice of comparators be driven by clinical appropriateness, informed by current treatment practices among a relevant patient population, and selected from potential comparators with the same treatment modality and class, rather than be dictated by cost, other concerns, or implicit goals. We encourage CMS to prioritize reducing bias in treatment comparisons by identifying therapeutic alternatives from potential comparators with the same treatment modality, class, and mechanism of action and limiting the choice of therapeutic alternative to drugs and biologics with FDA-approved indications. A rapid, multi-stakeholder scoping process beginning immediately after the selected drugs are announced would enable dialogue with manufacturers and other key stakeholders to identify appropriate therapeutic alternatives prior to the public submission process.

c. *Collect clarifying information on how submitted evidence has been separated from QALYs (Questions 40-43).* The ICR includes language surrounding the exclusion of evidence that uses a metric "such as QALYs in a life-extension context" but specifies that in instances where a study has "separated this use of QALYs from other evidence in the report (e.g., clinical effectiveness, risks, harms, etc.) that is relevant to the factors" considered during the price-setting process, "CMS will consider such separate evidence." NPC is mindful of the prohibition against the use of QALYs and encourages CMS to consider patient-reported outcomes that are complete, comprehensive, and fit for purpose, as opposed to limited, utility-based approaches including the QALY and other potentially discriminatory measures. Accordingly, we encourage CMS to specifically ask for information on how submitted evidence has been separated from QALYs.

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

d. *Expand outcomes to consider surrounding Therapeutic Impact and Comparative Effectiveness (Question 41).* There are a multitude of specific benefits that constitute the value of a drug, including societal benefits such as patient and caregiver indirect costs, scientific spillover, limiting the fear and risk of contagion for infectious diseases, increasing health equity, and cost offsets. In the CMS Framework for Health Equity 2022-2032, the Agency notes that the framework "challenges [CMS] to incorporate health equity and efforts to address health disparities as a foundational element across all [CMS] work, in every program, across every community."[5] We urge CMS to recognize the importance of increasing health equity as a benefit that constitutes the value of a drug, aligned with the Agency's goal of incorporating health equity in all CMS programs. The information solicited by Question 41 focuses data on a selected drug's therapeutic impact on "health outcomes, surrogate endpoints, intermediate outcomes, patient-reported outcomes, and patient experience." Given the importance of societal value and cost offsets, we encourage CMS to specifically recognize these outcomes in Question 41, including, but not limited to: patient and caregiver indirect costs, scientific spillover, limiting the fear and risk of contagion for infectious diseases, increasing health equity, and cost offsets.

e. *Expand definition of unmet need (Question 43).* The definition of unmet need provided alongside Question 43 is substantially narrower than definitions of unmet need found in the peer-reviewed literature and promulgated by the FDA as well as international agencies.[6,7] We encouraged CMS to expand their definition of unmet medical need in their Guidance to include a multifaceted definition informed by patient and provider perspectives. We similarly urge CMS to update the definition of Unmet Medical Need in the Data Negotiation Elements Questions.

f. *Expand opportunities for patients and caregivers to describe preferences and priorities.* Despite the importance of patient engagement during IRA implementation,[8] the ICR provides limited opportunities for patients and caregivers to describe critical elements of the value of drugs, including:
    - the preferences and priorities that inform shared decision-making between appropriate treatment options;
    - definitions of the benefits that are most important to patients;
    - selection of measures to quantify benefits; and,

[5]Centers for Medicare & Medicaid Services. CMS Framework for Health Equity 2022-2032. https://www.cms.gov/files/document/cms-framework-health-equity-2022.pdf

[6] Vreman RA, Heikkinen I, Schuurman A, et al. Unmet Medical Need: An Introduction to Definitions and Stakeholder Perceptions. *Value in Health*. 2019;22(11):1275-1282. doi:10.1016/j.jval.2019.07.007;

[7] Synnott PG, Voehler D, Enright DE, Kowal S, Ollendorf DA. The Value of New: Consideration of Product Novelty in Health Technology Assessments of Pharmaceuticals. Appl Health Econ Health Policy. 2023 Mar;21(2):305-314. doi: 10.1007/s40258-022-00779-0. Epub 2022 Dec 19. PMID: 36529826

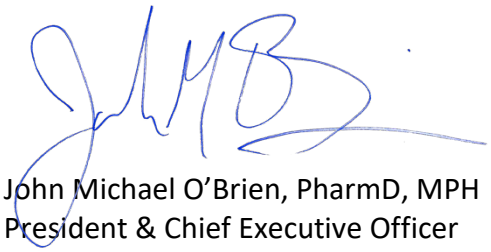
[8] "Patient Engagement & Experience Data: Missing Ingredients For CMS' Successful IRA Implementation", Health Affairs Forefront, May 16, 2023. DOI: 10.1377/forefront.20230515.743661

- patient preference regarding the benefits and risks of a product, its available dosage forms, and innovative delivery systems.

Patient and caregiver engagement is further constrained by the limited word counts and a lack of patient-friendly language. The lack of patient-friendly language creates concerns about the ability of CMS to capture the voices of underrepresented and historically marginalized populations as well as patients with limited health literacy. While this form must be revised to facilitate patient contributions, our recommendation only underscores the need for more robust opportunities for patient and caregiver engagement throughout the evaluation process as we described in our response to the initial Guidance.

The National Pharmaceutical Council appreciates the opportunity to submit comments in response to this ICR and looks forward to ongoing opportunities to engage with CMS as it implements the Medicare Drug Price Negotiation Program. Please contact me at john.obrien@npcnow.org or (202) 827-2080 if we may provide any additional information.

Sincerely,



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