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 initial *CMS–10847 Information Collection Request for Negotiation Data Elements under Sections 11001 and 11002 of the Inflation Reduction Act* and thank CMS for incorporating aspects of stakeholder feedback. However, we remain concerned that CMS' approach in designing the Negotiation Data Elements information collection continues to inappropriately burden manufacturers and 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 to promote meaningful information exchange.
2. Acknowledge manufacturer burden while removing inappropriate constraints.
3. Expand upon the changes introduced for the patient-centered data elements on the value of treatments.
    a. Expand opportunities for patients and caregivers to describe preferences, priorities, and unmet need (Question 31).
    b. Expand measures of Therapeutic Impact and Comparative Effectiveness (Questions 28-29).
    c. Require respondents to provide rationale for their choice of therapeutic alternatives (Questions 27-31).

Increase transparency and flexibility to promote meaningful information exchange.

The revised form, even in combination with the revised Guidance, provides limited insight into how CMS will evaluate drugs, or the factors considered during the price-setting process. As a result, respondents will be challenged to understand how they can best structure their responses to include relevant information for CMS's evaluation. We continue to strongly encourage CMS to specify how the data elements will be used in its price-setting process, including how CMS will weight or prioritize these elements, and work towards creating a transparent, robust, and replicable framework based on scientific principles for their drug evaluation process. Doing so will better equip manufacturers and other stakeholders to submit data that will meaningfully inform the drug evaluation process.

[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.

CMS's overall approach to drug evaluation and the specific Negotiation Data Elements questions would further benefit from flexibility that promotes a collaborative approach to information exchange. We appreciate that the revised Guidance includes an additional CMS-manufacturer meeting to provide manufacturers an opportunity to "present the data elements submission and share new information on the section 1194(e)(2) factors" with CMS. We urge CMS to provide additional details on that meeting as expeditiously as possible (e.g., when in "Fall 2023" it will occur, the duration of the meeting) and also make every effort to share the confidential report of stakeholder feedback with manufacturers of selected drugs well in advance of the Fall 2023 meeting so that manufacturers are adequately prepared. We encourage CMS to provide further flexibility in the submission of new evidence as it becomes available throughout the price-setting process. Additionally, flexibility in the Data Negotiation Element questions would better accommodate heterogeneity in manufacturer documentation and accounting practices for 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, with a particular focus on ensuring patients, caregivers, and providers can easily communicate important data in ways most accessible to them. Constraining diverse stakeholders to providing evidence with a rigid, one-size-fits-all approach limits the robustness of stakeholder engagement, reduces transparency, and impairs the evaluation process.

Acknowledge 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, we remain concerned that the ICR questions soliciting manufacturer-specific data overly emphasize cost-recovery and do not support nor prioritize 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 the current ICR allows. 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 continue to encourage CMS to limit the *required* submission of elements in these sections to those specifically relevant to determining a *Medicare*-only price in the least burdensome way.

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 I 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. We are disappointed that CMS did almost nothing in its revised Negotiation Data Elements form to address the form's arbitrary and limited word counts.

For example, Question 28 includes questions on the therapeutic impact of each indication for the selected drug and therapeutic alternatives. We appreciate clarification in the revised Negotiation Data Elements form that CMS will accept up to 50 citations and 10 tables, charts, or graphs on this question though note that CMS did not provide additional clarity on whether text included within these visual elements will be considered as necessary contextual components. However, the 3,000-word limit remains woefully inadequate for meaningful information exchange between manufacturers and CMS. Consider, for example, the response to Question 28 for a drug selected for price-setting with three indications, where the 3,000-word limit is divided evenly between the three indications. In 1,000 words (roughly four paragraphs of this paragraph's length), a manufacturer must convey information on: therapeutic impact, clinical benefits, and risks of the indication; the patient experience; an explanation of why each outcome for each indication was chosen; the extent to which the drug represents a therapeutic advance for that indication; and any differences in the safety profile of the selected drug and its therapeutic alternatives. The inadequacy of this word count is further underscored by the lack of CMS-manufacturer engagement between drug selection and submission of the Negotiation Data Elements form that would otherwise help manufacturers to respond concisely and best inform meaningful exchange with CMS. NPC urges CMS to eliminate word counts that stand in the way of an evidence-based evaluation of drugs, at a minimum, 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.

Expand upon the changes introduced for the patient-centered data elements on the value of treatments.

The ICR is ostensibly an opportunity for manufacturers, clinicians, and patients to provide evidence about the selected drug and alternative treatments. However, the questions, definitions, and word limits in Section I: Evidence About Alternative Treatments limit stakeholders' ability to communicate critical information about the impact and value of treatments. NPC encourages CMS to actively 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. *Expand opportunities for patients and caregivers to describe preferences, priorities, and unmet need (Question 31).* Patient engagement is critical during IRA

implementation.[3] We appreciate that the addition of patient listening sessions to the revised Guidance took a first step towards capturing the voice of patients and their families, as did the addition of patient experience questions on the revised ICR form and the option for respondents to identify themselves as caregivers. However, the ICR form's new questions on patient and caregiver experience fall short in providing an avenue 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;
- patient preference regarding the benefits and risks of a product, its available dosage forms, and innovative delivery systems; and
- patient perspectives on the ways that a treatment addresses unmet medical needs.

Furthermore, the patient and caregiver questions in the revised ICR form relate only to the patient's experience, not that of the caregiver. While we acknowledge this approach aligns with the revised Guidance's statement that the caregiver perspective will be considered when there is a direct impact on the patient, doing so deviates from key principles for drug evaluation recommending that clinical benefits be broadly defined to include outcomes not only for patients but also for other relevant parties, including family and society.[4] Accordingly, we encourage CMS provide an opportunity for caregivers to communicate experiences with a drug that impacted their own health and wellbeing beyond that of the person they care for.

NPC continues to be concerned that patient and caregiver engagement is further constrained by the limited word count. The word count restriction, combined with the omission of questions soliciting patient perspectives on preferences, priorities, and unmet need, underscores the need for robust opportunities for patient and caregiver engagement throughout the evaluation process as we described in our response to the initial Guidance. We encourage CMS to both broaden their definition of unmet need to be considered throughout the entire lifecycle of the selected drug and expand upon their Questions on Patient and Caregiver Experience in Question 31 and utilize methodologically robust approaches to capturing patient voices, including those of historically marginalized and underrepresented populations, in their patient listening sessions.

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

[4] Drummond M, Schwartz JS, Jansson B, Luce BR, Neumann BR, Seibert U, Sullivan SD. Key Principles for the Improved Conduct of Health Technology Assessments for Resource Allocation Decisions. International Journal of Technology Assessment in Health Care. 2008. 24:3:250.

b. *Expand measures of Therapeutic Impact and Comparative Effectiveness (Questions 28-29).* 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. We appreciate that CMS took a first step towards incorporating health equity into the revised Guidance and in the ICR and continue to encourage CMS to specifically recognize the additional societal outcomes listed above in addition to novel value elements such as adherence, convenience, independence, and productivity/wages[5] in Question 28. Notably, the question on health equity was added to Question 29: Comparative Effectiveness on Specific Populations without any accompanying increase in word limit for the response to the Question. This example further illustrates our concerns for the way in which arbitrary and restrictive word counts limit the ability of stakeholders to communicate meaningful evidence on the multifaceted impact of drugs.

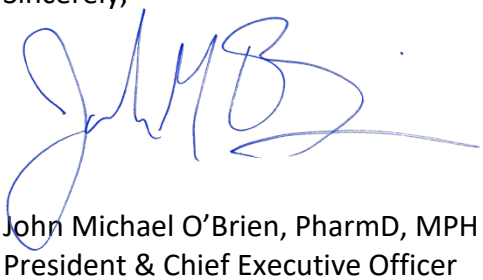
c. *Encourage respondents to provide rationale for their choice of therapeutic alternatives (Questions 27-31).* Questions throughout Section I: Evidence About Alternative Treatments solicit information about the selected drug and its therapeutic alternative(s) but does not explicitly ask stakeholders for the rationale for the chosen therapeutic alternatives. Encouraging stakeholders to submit this information alongside other submitted evidence will clearly help inform CMS's choice of therapeutic alternative. 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 appreciate revisions to the Guidance and ICR indicating that CMS seek to identify therapeutic alternatives within the same drug class as the selected drug based on properties such as chemical class, therapeutic class, or mechanism of action but remain concerned by the potential lack of transparency in the selection of therapeutic alternatives. NPC strongly cautions that that choice of therapeutic alternative should not range beyond the class of each selected drug. Therapeutic alternatives with a different modality, class or mechanism of action conflict with best practices for comparative effectiveness

[5] Lakdawalla DN, Doshi JA, Garrison LP Jr, Phelps CE, Basu A, Danzon PM. Defining Elements of Value in Health Care-A Health Economics Approach: An ISPOR Special Task Force Report [3]. Value Health. 2018 Feb;21(2):131-139. doi: 10.1016/j.jval.2017.12.007.

research[6] and introduce bias[7] that will very likely lead to invalid comparisons in terms of clinical and economic benefits to patients and health systems.

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,



John Michael O'Brien, PharmD, MPH
President & Chief Executive Officer

[6] Jaime Caro J, Eddy DM, Kan H, Kaltz C, Patel B, Eldessouki R, Briggs AH; ISPOR-AMCP-NPC Modeling CER Task Forces. Questionnaire to assess relevance and credibility of modeling studies for informing health care decision making: an ISPORAMCP-NPC Good Practice Task Force report. Value Health. 2014 Mar;17(2):174-82.; Sanders GD, Neumann PJ, Basu A, Brock DW, Feeny D, Krahn M, Kuntz KM, Meltzer DO, Owens DK, Prosser LA, Salomon JA, Sculpher MJ, Trikalinos TA, Russell LB, Siegel JE, Ganiats TG. Recommendations for Conduct, Methodological Practices, and Reporting of Cost-effectiveness Analyses: Second Panel on Cost-Effectiveness in Health and Medicine. JAMA. 2016 Sep 13;316(10):1093-103.

[7] AHRQ. Developing a Protocol for Observational Comparative Effectiveness Research: A User's Guide. Content last reviewed March 2021. Effective Health Care Program, Agency for Healthcare Research and Quality, Rockville, MD. https://effectivehealthcare.ahrq.gov/products/observational-cer-protocol