National Pharmaceutical Council®
1717 Pennsylvania Avenue, NW, Suite 800, Washington, DC 20006 Phone: 202.827.2100 Web: www.npcnow.org

September 3, 2024

The Honorable Meena Seshamani, M.D., Ph.D.
CMS Deputy Administrator and Director, Center for Medicare
Centers for Medicare & Medicaid Services
U.S. Department of Health and Human Services
7500 Security Boulevard Baltimore, MD 21244
Submitted Electronically via regulations.gov

RE: Negotiation Data Elements and Drug Price Negotiation Process for Initial Price Applicability Year 2027 under Sections 11001 and 11002 (CMS-10849)

Dear Deputy Administrator Seshamani:

The National Pharmaceutical Council (NPC) appreciates the opportunity to submit comments regarding the Centers for Medicare & Medicaid Services (CMS) Information Collection Request (ICR) *Negotiation Data Elements and Drug Price Negotiation Process for Initial Price Applicability Year 2027 under Sections 11001 and 11002 (CMS-10849).* 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 in the most efficient way possible.

There is robust evidence demonstrating the value of biopharmaceuticals on public health, including associated improvements in life expectancy,[1] reductions in total healthcare costs,[2] and reductions in other poor health outcomes.[3] The biopharmaceutical industry invests over $276 billion in research and development annually,[4] yet our research shows that the Inflation Reduction Act (IRA) may reduce new treatments and indications.[5] The IRA creates a new price-setting mechanism that will change the economic incentives for bringing new medicines to market, and evidence shows

[1] Buxbaum JD, Chernew ME, Fendrick AM, Cutler DM. Contributions Of Public Health, Pharmaceuticals, And Other Medical Care To US Life Expectancy Changes, 1990-2015. Health Aff (Millwood). 2020 Sep;39(9):1546-1556. doi: 10.1377/hlthaff.2020.00284. PMID: 32897792.
[2] Roebuck MC, Liberman JN, Gemmill-Toyama M, Brennan TA. Medication adherence leads to lower health care use and costs despite increased drug spending. Health Aff (Millwood). 2011 Jan;30(1):91-9. doi: 10.1377/hlthaff.2009.1087. PMID: 21209444.
[3] Ho PM, Bryson CL, Rumsfeld JS. Medication adherence: its importance in cardiovascular outcomes. Circulation. 2009 Jun 16;119(23):3028-35. doi: 10.1161/CIRCULATIONAHA.108.768986. PMID: 19528344.
[4] Chandra A, Drum J, Daly M, et al. Comprehensive Measurement of Biopharmaceutical R&D Investment. Nature Reviews Drug Discovery. August 2024. https://www.nature.com/articles/d41573-024-00131-2
[5] Patterson J, Motyka J, O'Brien JM. Unintended Consequences of the Inflation Reduction Act: Clinical Development Toward Subsequent Indications *Am J Manag Care. 2024;30(2):82-86. https://doi.org/10.37765/ajmc.2024.89495*

manufacturers are already responding to those incentives.[6] There are growing concerns about the potential unintended consequences of the IRA and the Medicare Drug Price Negotiation Program. NPC research highlights that these consequences will likely include delay of access to new medicines, and fewer diseases getting additional approved treatment options.[7,8]

In accordance with the Paperwork Reduction Act (PRA), NPC's comments aim to ensure that CMS is accurately assessing the burden of data collection in the ICR form. Furthermore, we aim to ensure that CMS is clearly communicating the utility of collected data to increase efficiency among the stakeholders involved in the ICR evidence collection and reporting process.

Our comments on the ICR for IPAY 2027 are below:

I. Administrative Burden and Transparency
- A. Reducing Administrative burden for patients and manufacturers
- B. Increasing Transparency and Revising Timelines between the Evidence Collection and Review

II. Section I: Evidence about Alternative Treatments
- A. Data on Therapeutic Alternatives
- B. Patient-Specific Data Elements
- C. Treatment Costs and Offsets
- D. Unmet Medical Need

III. Drug Price Negotiation Process ICR Form
- A. Opportunities for meaningful in-person CMS-manufacturer engagement

[6] 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; IRA survey: Biotechs bracing for impact. Biocentury. March 16, 2023. Slabdokin, Greg. IRA Drives Pfizer's Decision to Focus on Biologics, Not Small Molecules. BioSpace. March 4, 2024. Available at: https://www.biospace.com/article/ira-drives-pfizer-s-decision-to-focus-on-biologics-not-small-molecules/. US IRA May Weigh on Long-Term Global Pharma Growth. FitchRatings. September 2023. https://www.fitchratings.com/research/corporate-finance/us-ira-may-weigh-on-long-term-global-pharma-growth-22-09-2023.

[7] Patterson J, Motyka J, O'Brien JM. Unintended Consequences of the Inflation Reduction Act: Clinical Development Toward Subsequent Indications *Am J Manag Care. 2024;30(2):82-86. https://doi.org/10.37765/ajmc.2024.89495*

[8] O'Brien J, Motyka J, Patterson JA. How The IRA Could Delay Pharmaceutical Launches, Reduce Indications, And Chill Evidence Generation, Health Affairs Forefront. November 2023. DOI: 10.1377/forefront.20231101.123865

I. Administrative Burden and Transparency

A. Reducing Administrative burden for Patients and Manufacturers

Many stakeholders are closely watching CMS's IRA implementation process. The price-setting process is being studied not just by manufacturers, but by the broader patient advocacy, health policy, and pharmacoeconomic communities.[9,10] The credibility of CMS's process will be judged by the agency's use of good evidence and appropriate methods in a transparent and patient-centered process.

We are concerned with the administrative burden placed on patients to complete the ICR form. The ICR form includes 10 sections with 64 specific questions, many of which have multiple sub-questions. In particular, Section I of the ICR provides one of the two current opportunities for patients to provide critical evidence on the effectiveness of the selected medicines in comparison to therapeutic alternatives. However, for patients to provide comments to CMS on Section I, there needs to be a sufficient comment period timeline. We are concerned patients will not have sufficient time to complete the ICR form given the one-month timeline between the date that CMS will announce the selected drugs for IPAY 2027 (February 1st) and the deadline to submit the ICR comments (March 1st). A 30-day comment period will be particularly burdensome for small and/or low-resourced patient groups, who lack full-time dedicated staff to devote all their resources to respond to CMS.

For IPAY 2026, CMS received 106 submissions from individuals and organizations on Section 1194(e)(2), with 55 percent of these submissions from organizations. CMS is estimating that it will receive approximately three times as many public submissions for IPAY 2027, estimated at 150 submissions from individual respondents and 175 from organizations.[11] We ask CMS to publicly release information on how many individuals and organizations respond to the ICR for IPAY 2027, and to stratify data based on respondent type, including patients and patient advocacy organizations. Sharing this information will help CMS and the public understand who CMS receives feedback from, and to address gaps in respondent types if there is limited feedback from respondent types. In particular, CMS should monitor how many responses it receives from patients, as it is critical that the patient voice is heard within the price-setting process.

[9] O'Brien JM and Hansen J. Section 50 of the Inflation Reduction Act Drug Price Negotiation Program: Considerations for the Centers for Medicare Medicaid Services, Manufacturers, and the Health Economics and Outcomes Research Community. Value in Health. 2023; 26 (12).
[10] Tollen L. Is It Working? Evaluating The First Round of Medicare Drug Price Negotiations. Health Affairs. August 2024. Available at: https://www.healthaffairs.org/doi/full/10.1377/hlthaff.2024.00994.
[11] Centers for Medicare and Medicaid Services. Supporting Statement – Part A. Negotiation Data Elements and Drug Price Negotiation Process for Initial Price Applicability Year 2027 under Sections 11001 and 11002 of the Inflation Reduction Act (IRA) Information Collection Request (ICR) (CMS-10849, OMB 0938-1452). July 2, 2024. Available at: https://www.cms.gov/regulations-and-guidance/legislation/paperworkreductionactof1995/pra-listing/cms-10849

To decrease burden on patients, we recommend that patients should have greater flexibility and autonomy to better engage with CMS on evidence about alternative treatments. This recommendation could be accomplished by implementing the following:

- Increase the modalities for patients and caregivers to submit data (e.g., video submissions, focus groups, and one-on-one interviews), outside of the ICR form and patient-focused listening sessions.
- Increase the ICR comment period for patients and caregivers to submit data on Section I to at least 60 days.
- Monitor the amount and breadth of information submitted from patients and caregivers in the 2024 ICR form. *Decreased input from patients and caregivers could signal that the method of collection is burdensome for patients and/or CMS's goals and approaches are not targeted enough.*

Manufacturers also face administrative burdens to complete the ICR form. Manufacturers are required to submit the manufacturer-specific and other evidence within the ICR form one month after their drugs are selected for negotiation. CMS estimates that it should take each manufacturer 500 hours[12] to collect evidence for the ICR form, which we suspect to be an underestimate. We are concerned that CMS has not provided sufficient clarity on certain definitions in Section I of the ICR form, such that the minimal amount of data is requested and collected from manufacturers. For example, definitions of therapeutic advance, and comparative effectiveness are not adequately defined.

We suggest that CMS should provide clarity on the definitions relevant to Section I of the ICR form. If the definitions of key terms remain ambiguous, then manufacturers will not have sufficient information to efficiently submit the ICR form.

The following definitions for key terms should be clarified:

- Therapeutic advance: US government organizations, such as the Food and Drug Administration, have a definition of unmet medical need. We recommend that CMS continue to clarify the definition of therapeutic advances, including signals such as:
    - Representation of a significant impact among a socially or economically vulnerable population, which is not evident among non-vulnerable populations; or
    - Patient-focused improvements in the symptoms or health outcomes associated with a disease (e.g., reduction of symptoms, ability to perform daily functions); or
    - Improvements on a validated clinical outcome assessment, for the disease state
- Comparative effectiveness: The IPAY2027 ICR states that "relevant comparative evidence may include, but is not limited to: head-to-head randomized controlled trials, pragmatic clinical trials, network meta analyses, observation studies, and real-world evidence." We recommend that CMS provide direction on which type of comparative evidence carries the most weight and their

[12] Centers for Medicare and Medicaid Services. Supporting Statement – Part A. Negotiation Data Elements and Drug Price Negotiation Process for Initial Price Applicability Year 2027 under Sections 11001 and 11002 of the Inflation Reduction Act (IRA) Information Collection Request (ICR) (CMS-10849, OMB 0938-1452). July 2, 2024. Available at: https://www.cms.gov/regulations-and-guidance/legislation/paperworkreductionactof1995/pra-listing/cms-10849

order of preference. We suggest that CMS release the framework for evaluation of comparative effectiveness research and the other metrics for evaluating the quality of data on drugs and their alternatives. We also suggest that CMS release clear approaches for the evaluation of regulatory and other health outcomes measures; such that the highly evaluated evidence is prioritized by manufacturers and other stakeholders. Lastly, we recommend that CMS shed light on how comparative effectiveness evidence will be evaluated across different potentially suitable clinical comparators.

- Cost of Therapeutic Alternatives: We recognize that the statute contemplates that CMS will collect information regarding "the costs of . . . existing therapeutic alternatives." However, we do not believe it is appropriate to consider these costs for the purposes of identifying therapeutic alternatives. Instead, we recommend that CMS clarify that the sole function of collecting this information is to identify the starting point for the negotiation process, which CMS has proposed will begin with the price of the selected drug's therapeutic alternatives.
- Therapeutic Alternatives: As stated in our comments to CMS on the IPAY 2027 Draft Guidance, we encourage CMS to incorporate the following during the selection of therapeutic alternatives:
    - Publicly communicate proposed therapeutic alternatives and solicit feedback from manufacturers, clinicians with specific expertise in the treating the disease, patients and caregivers, and other stakeholders before proceeding with comparative effectiveness analyses that inform the initial offer.
    - Ensure guidelines used in identifying therapeutic alternatives are up-to-date and incorporate the latest evidence.[13]
    - Include patient preferences and priorities that inform shared decision-making between appropriate treatment options.[14]
    - Invite manufacturers of the selected drug to proactively present clinical information focused on the relative clinical benefit of their products compared to therapeutic alternatives during the process of comparator selection and give manufacturers the opportunity to respond to CMS's choices of therapeutic alternatives. Early manufacturer communication is also consistent with practices employed by state Medicaid agencies, other federal agencies and commercial payers.
    - Seek input from clinicians with specific expertise in treating the indication of the selected drug to define appropriate therapeutic alternatives among Medicare patient sub-populations, including patients with multiple comorbidities and varying levels of disease severity. There is a long history of multiple stakeholders working together to develop clinical guidelines, including NIH's National Center for Advancing Translational Sciences.[15]

[13] National Health Council. A Dialogue on Patient-Centered Value Assessment: Overcoming Barriers to Amplify the Patient Voice. December 2018. Available from: https://www.nationalhealthcouncil.org/dialogue-patient-centered-value-assessmentovercoming-barriers-amplify-patient-voice

[14] Schmidt T, Valuck T, Riposo J, et al. Impact of Shared Decision-Making and Patient Decision Aids on Health Care Cost and Utilization in the US: A Systematic Review. J Clin Pathways. 2022;8(8):33-43. doi:10.25270/jcp.2022.12.0

[15] Shekelle PG, Woolf SH, Eccles M, Grimshaw J. Clinical guidelines: developing guidelines. BMJ. 1999 Feb 27;318(7183):593-6.; NIH National Center for Advancing Translational Sciences. Toolkit for Creating Clinical Care Guidelines: https://toolkit.ncats.nih.gov/module/after-fda-approval/creating-clinical-care-guidelines/guideline-development-process/

- Consider the use of comparative effectiveness studies and real-world evidence to support the selection of therapeutic alternative.

In addition to clarifying the definitions in Section I, we also recommend that CMS aim to decrease the administrative burden on manufacturers by implementing the following:

- Collect evidence from manufacturers on the approximate time-period required to collect evidence for the submission of the ICR form and adjust the comment period accordingly. For example, if the manufacturer reported time to collect data for the ICR is double the expected time, then the comment period timeline should be correspondingly doubled.
- Reduce the request for evidence that is publicly available (e.g., peer-reviewed papers). For example, CMS could provide manufacturers with a report of publicly available literature on the effectiveness of the drugs – presented in a table format at the time of drug selection. Manufacturers could review the information for any missing information and provide that information to CMS in the ICR form.
- Shift back to word-count (instead of character-counts) for manufacturer and other stakeholder relevant data, which can decrease administrative burden.

B. Increasing Transparency and Revising Timelines between the Evidence Collection and Review

We are concerned that CMS's lack of clarity on the influence of patient-derived and other data into the Medicare DPNP for IPAY 2027 will result in the collection of data that ultimately has decreased utility for the agency. This is partly because CMS is not required to release an explanation of the MFPs for the selected drugs for IPAY 2026 until the date that the subsequent year's ICR evidence is due (i.e., March 1, 2025).[16] Therefore, stakeholders will need to submit information on the ICR form without understanding what CMS valued in the price-setting process for IPAY 2026. The lack of clarity on the evidence around the information that most influenced CMS's price setting process – at the time when the ICR data are due for the next cycle of selected drugs – limits patients', researchers', and others' ability to leverage insights from the first cycle of the price-setting process as it enters its second cycle.

We also suggest the following:

- **We recommend that CMS increase the transparency of the evidence that informed the MFPs for IPAY 2026 by releasing the explanation of the MFPs at least one month prior to the date that the ICR for 2027 is due.** In addition, we encourage CMS' explanation of the IPAY 2026 MFPs include a complete and transparent methodology – revealing how each domain of collected data informed MFPs. A clear methodology framework of the inner

[16] Medicare Drug Price Negotiation Program: Draft Guidance, Implementation of Sections 1191 – 1198 of the Social Security Act for Initial Price Applicability Year 2027 and Manufacturer Effectuation of the Maximum Fair Price (MFP) in 2026 and 2027. May 2024. Centers for Medicare & Medicaid Services. https://www.cms.gov/files/document/medicare-drug-price-negotiation-draft-guidance-ipay-2027-and-manufacturer-effectuation-mfp-2026-2027.pdf

workings of the MFPs will help to reduce administrative burden for stakeholders and increase transparency of this novel program.

- **We recommend that CMS continue to outline safeguards around the protection of confidential information submitted during the ICR process.** The ICR form requests highly confidential information from manufacturers and other stakeholders. We support CMS's inclusion of questions 28 and 64, which allows manufacturers and other stakeholders to clarify which data that should be withheld by CMS under the Freedom of Information Act exemptions. However, we request that CMS provide greater clarity on the safeguards of submitted, confidential data.

II. Section I: Evidence about Alternative Treatments

A. Data on Therapeutic Alternatives

In any assessment of the relative clinical or economic benefits of a drug, the choice of the comparator is a fundamental driver in the outcomes and validity of the assessment with significant implications for patients, payers, and prescribers. We are concerned that CMS has neither provided further information on the literature review informing the selection of therapeutic alternatives nor publicly released more information on the specific outcomes that will be of greatest interest to CMS for developing MFPs. More clarity and guidance are needed to reduce unnecessary administrative burden. **We recommend the following regarding evidence about therapeutic alternatives:**

- Publicly release detailed information on evidence surrounding the selection of the therapeutic alternatives concurrently with CMS's public release of the names of the drugs selected for price setting. In the current process, manufacturers and other stakeholders are required to submit the ICR form, without details on the selection of the therapeutic alternative. We recommend that CMS publicly release the selection of potential therapeutic alternatives that the agency will consider at the time of release of the selected drugs for the MDPNP. CMS should release the list of "potential" therapeutic alternatives and the search strategy opportunity for public comment on the selection and strategy at least one month before the ICR form is due. Increasing transparency in the selection of the therapeutic alternatives will aid in reducing the collection of data of therapeutic alternatives that are not of interest to the agency.
- Shift definition of therapeutic alternative back to the IPAY 2026 guidance definition. CMS changed the definition of a therapeutic alternative in the IPAY 2027 guidance from the IPAY 2026 guidance. The former guidance stated that the therapeutic alternative may refer to "a subset of the most clinically comparable therapeutic alternatives." The new definition of a therapeutic alternative is a change in the wrong direction, away from what is most clinically appropriate. The selection of a less-costly therapeutic alternative that is "clinically comparable" but not in the subset of "most clinically comparable" and lacks the safety, efficacy, and other clinical benefits of a selected drug – solely to lower the initial starting point of the price-setting process – fails to recognize the value of modern treatments and threatens to reverse the incentives that currently encourage innovation and access. We recommend that CMS return to the IPAY 2026 definition of a therapeutic alternative to support clinical decision making. Given

that researchers and others have a limited character count for the submission, we recommend that CMS be more specific about the selection of the therapeutic alternative(s).

B. Patient-Specific Data Elements

CMS has made significant revisions to collected data from the patient perspective. We support CMS's efforts to better solicit patient-centered data. NPC and others have also emphasized the need for CMS to prioritize diversity and a multi-modal approach in outreach at all phases of the DPNP implementation.[17,18] Robust engagement with underrepresented communities through outreach and ongoing dialogue is needed to promote an equity-focused implementation process.[19] While CMS included several questions about the demographics of the patient populations, CMS should allow patients and caregivers to include further demographic and socioeconomic data, if they prefer to share such information with CMS. For example, patients can select their geographic region, but information about the rurality of a patient's residence or gender is not requested. Our research shows that a patient's medication expenses vary across geographies and race/ethnicity.[20]

As CMS considers how to improve upon its patient engagement strategy through revisions in the ICR, CMS should seek feedback from patients, caregivers, and providers about ways to engage patients to complete the ICR forms, including the character-count limits. CMS should also be transparent about the number of comments received from patients on the CMS website.

As such, we recommend that CMS prioritize the following in collecting patient-specific data elements:

- Move towards best practices for patient engagement throughout the DPNP process, such as those developed by NPC and ISPOR. [21,22]
- Increase planned engagement strategies with underrepresented groups with the entire DPNP process, including outreach for submission of evidence for the ICR form.
- Increase the collection of demographic data about patients, as consented to by patients.
- Shift back to word-count (instead of character-counts) for patient relevant data, which can decrease administrative burden.

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[17] National Organization for Rare Disorders. NORD Recommendations: Future Medicare Drug Price Negotiation Program Patient and Provider Listening Sessions [Internet]. 2024.

[18] Miller M, van Geertruyden S, Saxton MC, Savage CY, Weir D, Werner S. A summit on amplifying voices of patients, caregivers, and people with disabilities in Inflation Reduction Act drug price negotiations. J Manag Care Spec Pharm. 2024 Mar 1;30(3):247-251. doi: 10.18553/jmcp.2024.23278. Epub 2024 Jan 30. PMID: 38289281; PMCID: PMC10906444.

[19] The Center for Innovation & Value Research (formerly Innovation and Value Initiative). Comments on the draft guidance for implementation of the Medicare Drug Price Negotiation Program (DPNP) for initial price applicability year 2027 and manufacturer effectuation of the maximum fair price (MFP) in 2026 and 2027. Available at: https://valueresearch.org/the-center-formerly-ivi-provides-comments-on-cms-drug-price-negotiation-program/

[20] Wagner TD, Sahu M, Beauchamp M, et al. Variation in per Beneficiary Prescription Utilization and Spending by Race/Ethnicity in Medicare and Medicaid Insurance Claims. ISPOR 2024 Conference. Available at: https://www.npcnow.org/resources/variation-beneficiary-prescription-utilization-and-spending-raceethnicity-medicare-and

[21] Harrington RL, Hanna ML, Oehrlein EM, Camp R, Wheeler R, Cooblall C, et al. Defining Patient Engagement in Research: Results of a Systematic Review and Analysis: Report of the ISPOR Patient-Centered Special Interest Group. Value in Health. 2020; 23 (6). Available at: https://www.sciencedirect.com/science/article/pii/S1098301520301418

[22] Guiding Practices for Patient-Centered Value Assessment (2024). National Pharmaceutical Council. Jan 2024.

- Clarify that submitted data on a patient's "affiliation" with a manufacturer (i.e., Question 29) will not detract from CMS's evaluation of the patient-centered information. We are concerned that requesting data from patients and other stakeholders around an "affiliation" with a manufacturer may decrease the number of meaningful responses from stakeholders, who interact with manufacturers for a number of reasons.

C. Treatment Costs and Offsets

NPC appreciates that CMS requests evidence related to healthcare resource utilization and usage patterns. Reviewing data related to healthcare resource utilization and usage, with consideration of evidence-based medicine, will provide insight into the economic benefits of selected drugs and their impacts on patient health. However, it remains unclear how CMS will use this information, the methods it will employ to analyze it, and how it will inform their evaluations, and transparency on these points is necessary to evaluate whether this evidence will be used appropriately.

In our comments on the DPNP IPAY 2027 draft guidance, we encouraged CMS to also include comprehensive assessments of the economic benefits of selected drugs, in addition to the costs of the treatments themselves. **We also recommend that the utilization of data on treatment costs and offsets be transparent in the ICR form.**[23] Treatments may have up-front costs that lead to long-term improvements in patient health. Those improvements may yield "cost offsets," or savings due to reduction in healthcare resource needs, such as reduced hospitalizations, or societal gains (e.g., improved productivity, reduction in caregiver burden). The full value of treatment can only be assessed by including both the treatment costs and other associated cost offsets it may produce, while also including clinical benefits of drugs without discretely quantifiable impacts on costs (e.g., improvements in the overall care of the patient). Only considering the treatment costs but not the potential cost offsets would lead to an incomplete assessment of value.

D. Unmet Medical Need

NPC believes assessments of unmet medical need should include a multifaceted definition informed by the patient perspective, as guided by peer-reviewed literature.[24] The ICR asks, participants several questions about unmet medical need, including: "For the condition(s) treated by [the selected drug], describe the extent to which [the selected drug] currently represents (or does not represent) a therapeutic advance as compared to its therapeutic alternative(s)."[25] NPC is concerned that a lack of transparency surrounding what specific factors CMS will consider related to unmet need will result in an approach that is too narrow.

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[23] National Pharmaceutical Council. Guiding Practices for Patient-Centered Value Assessment. 2024. Washington, DC. Available at: https://www.npcnow.org/sites/default/files/2024-01/2024%20Guiding%20Practices%20for%20PatientCentered%20Value%20Assessment%20January.pdf

[24] Levine AA. Kowal S. Chambers J. Unmet Medical Need Under the IRA. Health Affairs Forefront. July 2024. 10.1377/forefront.20240729.713230

[25] Negotiation Data Elements and Drug Price Negotiation Process for Initial Price Applicability Year 2027 under Sections 11001 and 11002 of the Inflation Reduction Act (IRA) Information Collection Request (ICR) Forms (CMS-10849, OMB 0938-1452). Centers for Medicare & Medicaid Services. July 2024. Available at: https://www.cms.gov/regulations-and-guidance/legislation/paperworkreductionactof1995/pra-listing/cms-10849

Rigorous methods can be used to elicit consensus from clinician experts and have been used to identify unmet medical needs to achieve optimal treatment goals throughout the natural history of a disease.[26] These methods have identified patient-centered unmet needs, including patient quality of life, poor adherence, severe stages of a disease that are hard to treat, and patient preferred routes of administration.[27] A determination of unmet medical need should encompass all of these factors and more. A recent survey of over 300 patients aged 65 and older in the US asked patients their perspectives on CMS's definition of unmet medical need in the 2023 guidance of the Medicare DPNP.[28] The study reports that patients believe the "accurate definition of unmet medical need is far broader, more engaging of patients, and more nuanced than the definition CMS has proposed [in 2023]."

The FDA's definition of unmet need, as outlined in its guidance for expedited programs, includes improved efficacy, reduced toxicity and/or potential drug-drug interactions, and improvements in other benefits such as adherence.[29] Notably, the FDA definition of unmet need also highlights conditions for which there is significant heterogeneity in response to existing treatment options. Patients may respond differently to available treatment options due to pharmacologic differences, genetic risk, or social determinants of health, creating unmet need despite existing treatments.[30]

NPC requests CMS clarify and provide greater specificity in the definitions and utilization of unmet medical need for MFP, including the following:

- Broaden the definition of unmet medical need to encompass meeting a public health need and/or health outcomes important to patients, such as: quality of life, time off of work, and caregiver outcomes.
- Specify and publicly report the submitted data that CMS considers to be evidence of meeting a medical unmet need.
- Provide the public and manufacturers with selected products, detailed evidence on the factors considered in determining if a product meets/or does not meet an unmet medical need.

III. Drug Price Negotiation Process ICR Form

A. Provide opportunities for meaningful CMS-manufacturer engagement during the counteroffer process

[26] Danese S, Allez M, Van Bodegraven AA, et al. Unmet Medical Needs in Ulcerative Colitis: An Expert Group Consensus. Digestive Diseases. 2019;37(4):266-283. doi:10.1159/000496739

[27] Danese S, Allez M, Van Bodegraven AA, et al. Unmet Medical Needs in Ulcerative Colitis: An Expert Group Consensus. Digestive Diseases. 2019;37(4):266-283. doi:10.1159/000496739

[28] DeMattis C, Karmo M, Gawuga C. Defining "Unmet Medical Need" in the Inflation Reduction Act for the Maximum Fair Price: Reflecting on Patient Input. Partnership to Fight Chronic Diseases. 2023. Available at: https://www.fightchronicdisease.org/unmet-medical-need

[29] Food and Drug Administration. Guidance for Industry Expedited Programs for Serious Conditions – Drugs and Biologics. U.S. Department of Health and Human Services. May 2014. Silver Spring, MD. Available at: https://www.fda.gov/files/drugs/published/Expedited-Programs-for-Serious-Conditions-Drugs-and-Biologics.pdf

[30] National Pharmaceutical Council. The Myth of Average: Why Individual Patient Differences Matter. 2022. Washington, DC. Available at: https://www.npcnow.org/sites/default/files/2022-01/The_Myth_of_Average_01.2022.pdf

In the IPAY 2027 DPNP Draft Guidance, CMS sought feedback on whether three meetings between the manufacturer of a selected drug and CMS are necessary, and whether it would be preferable to have an additional written offer in lieu of one or more meetings. This ICR notes that up to three in-person, virtual, or hybrid negotiation meetings may occur if the Primary Manufacturer's written counteroffer is not accepted by CMS.

NPC continues to urge CMS to provide significant opportunities for engagement between CMS and manufacturers during the counteroffer process. Additional written offers, the potential for additional in-person meetings, and clear communication surrounding next steps will enhance the "negotiation" process.

Conclusion

The National Pharmaceutical Council appreciates the opportunity to submit comments in response to this ICR and looks forward to additional opportunities to engage with CMS as it implements the second cycle of 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.

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