IVI INNOVATION AND VALUE INITIATIVE

April 14, 2023

Meena Seshamani, M.D., Ph.D.
CMS Deputy Administrator and Director of the Center for Medicare
Centers for Medicare & Medicaid Services
7500 Security Boulevard
Baltimore, Maryland 21244-1850

Dear Dr. Seshamani,

The Innovation and Value Initiative (IVI) appreciates the opportunity to provide comments to the Centers for Medicare and Medicaid Services (CMS) on the initial guidance for implementation of the Medicare Drug Price Negotiation Program (Medicare DPNP).

IVI is a 501(c)3, non-profit research organization committed to advancing the science, practice, and use of health technology assessment (HTA) in health care. Founded in 2017, the organization includes members from the research, patient, payer/purchaser, clinician and innovator stakeholder communities. IVI's work emphasizes collaboration and exploration of new solutions in pursuit of a U.S. learning healthcare system supported by patient-centered HTA and focused on high-quality, efficient, innovative, and equitable care for all people and communities. We believe this is only possible with a fundamental shift to resource allocation, coverage, and access-related decision-making that aims to maximize value for all stakeholders—particularly patients and other covered individuals.

Our work is guided by our Principles for Value Assessment.[1] These principles apply not only to the narrow context of HTA but are the foundation of a patient-centered and equitable health system based on value to all stakeholders. The implementation by CMS of the Medicare DPNP should be grounded in these principles, the foremost among them being patient-centricity, transparency, equity, and vigorous methods enhancement.

IVI recognizes that the legislation includes specific guidelines and places limitations on implementation of the DPNP, and we commend CMS for its efforts to develop thoughtful and thorough guidance under considerable time constraints. Examining the initial

[1] Full description of our Principles for Value Assessment in the U.S. available at: https://thevalueinitiative.org/principles-for-value-assessment-in-the-us/

guidance for the DPNP through the lens of IVI's core principles raises a number of concerns with both immediate and long-term implications, including:

- The guidance fails to incorporate multiple elements needed to advance health equity, deliver value to patients, and influence the evolution of health research and healthcare delivery to support value-based decision making.
- The processes for comment, evidence collection, and assessment of included drugs raise a number of immediate concerns.

We appreciate the opportunity to offer comments and suggestions in the following areas:

Focus of DPNP should be on maximizing value, not simply minimizing prices

To the extent allowed by existing legislation, the DPNP should emphasize understanding and maximization of value—as opposed to simply minimizing product prices—as a primary objective of the program and orient program guidelines around this objective.

As the largest health insurer in the U.S., Medicare policy or programmatic changes have the potential to shape the structure of the U.S. health system at all levels. As such, CMS implementation of the relevant sections of the Inflation Reduction Act (IRA)—and particularly related to the DPNP—presents an opportunity to establish an inclusive and forward-thinking approach that emphasizes negotiations and decision-making based on maximizing value to Medicare enrollees, the Medicare system, and American society, rather than simply on minimizing unit prices.

The initial guidance for the Medicare DPNP, reflecting the emphasis in sections 11001 and 11002 of the IRA on price, outline a process constructed around the primary objective of negotiations to reduce prices for high-cost drugs and minimize costs to Medicare. Price does not equate to value. Rather, price (and costs as a result) is one of multiple components of value. This narrow focus on price as opposed to more comprehensive assessments of value risks perpetuation of decisions that shape patients' care based on budget concerns rather than value.

Value is multi-faceted, and CMS should endeavor to include a broader set of outcomes and other elements influencing value for Medicare and its enrollees when considering any benchmark price for negotiations. Data elements required by CMS per section 50.1 of the initial guidance are not sufficient to determine a price based on the value of the drug. Additional elements of value that reflect impacts on different stakeholders should be considered. For example, patient-level elements may include financial impacts of care, burden on family caregivers, and patient goals for treatment.[2] As a public

[2] See for example:

program, broader societal elements are also important to consider. Some examples include incentives for innovation, impacts on health equity, and insurance value.[3]

Implementation of the Medicare DPNP will establish a formal approach and framework that sets a precedent for both CMS and private sector approaches to price negotiations and decisions about coverage and access. We strongly encourage CMS to incorporate broader concepts of value into assessments and negotiations to the greatest extent possible, and to clearly articulate these considerations in the final guidance.

Health equity must clearly inform all aspects of DPNP guidelines

Equity implications of all aspects of the program should be systematically considered as part of DPNP activities, and strategies to support equity (by including members of underrepresented or "specific" populations as formal partners in the process, for example) should be incorporated wherever possible.

Health equity is the first pillar of the Centers for Medicare & Medicaid Services' (CMS) Strategic Plan,[4] which states:

> *CMS is working to advance health equity by designing, implementing, and operationalizing policies and programs that support health for all people served by our programs by incorporating the perspective of lived experiences and integrate safety net providers and community-based organizations into our programs.*

Regarding the Center for Medicare (CM), it goes on to state:

> *As one of the largest payers in our health care system, CM is catalyzing delivery system transformation by issuing policies that advance equity across all Medicare programs and activities … and consistently engaging people with Medicare throughout the policy process.*

Wilson, M., Thavorn, K., Hawrysh, T. et al. Engaging Patients and Caregivers in an Early Health Economic Evaluation: Discerning Treatment Value Based on Lived Experience. PharmacoEconomics. 2022;40:1119–1130. https://doi.org/10.1007/s40273-022-01180-4

dosReis, S., Butler, B., Caicedo, J. *et al.* Stakeholder-Engaged Derivation of Patient-Informed Value Elements. The Patient. 2020; 13:611–621. https://doi.org/10.1007/s40271-020-00433-8

[3] Lakdawalla DN, Doshi JA, Garrison LP, 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 in Health. 2018;21(2):131-139.

[4] Source: CMS Strategic Plan Fact Sheet. Pillar: Health Equity. https://www.cms.gov/files/document/health-equity-fact-sheet.pdf

In the initial guidance neither the word "equity" nor related terms appear in the text. As a highly visible and novel program within Medicare, it is imperative that the DPNP reflect the objectives and practices outlined in the CMS strategic plan.

IVI recently completed Phase 1 of its Health Equity Initiative, a multi-stakeholder-driven process that aims to identify actionable changes to HTA processes, methods, and communication that acknowledge and resolve existing health disparities in research and healthcare decision-making.[5] Many of the key themes arising through this ongoing process have implications for the Medicare DPNP, for example:

- **Fundamental change**: Incremental changes ("tinkering around the edges") are insufficient to address the systemic issues impacting equity
- **Accountability**: Change will not occur without accountability for and to all stakeholders
- **Meaningful engagement**: All actors—and especially underrepresented communities—must be meaningfully engaged throughout the process, including decision-making.
- **Data and methods**: Incomplete evidence and imperfect methods are not an excuse to continue practices that perpetuate bias or inequities. A "learning laboratory" approach is needed for testing methods (especially mixed quantitative/qualitative) and identifying evidence gaps.
- **Transparency:** Transparency is a necessary condition for both trust and accountability—particularly as an antidote to lack of trust of marginalized communities for researchers, providers, and decision-makers—and critical to the advancement of health equity.

Many of these findings, which largely align with the priorities and planned actions outlined in the CMS strategic plan, have direct application to the Medicare DPNP. In all aspects of planning and execution of the Medicare DPNP, health equity should remain a principal concern and the lens through which decisions are made, with consideration at minimum of:

- **Implications of external factors for equity**—underrepresentation of specific groups in data, for example.
- **Alignment of practices and guidelines with equity goals**—ensuring DPNP process reflects actions outlined in CMS strategic plan and recommendations from IVI's work. For example, engaging members of underrepresented communities as collaborators.

[5] More information available at: https://thevalueinitiative.org/health-equity-initiative/

- **Downstream equity implications of DPNP processes**—deliberate consideration of potential equity impacts now and in the future, including implications for access and affordability, and potential influences on the private market.

IVI strongly recommends revision of the DPNP guidance to align with CMS's strategy, positioning health equity at the forefront by clearly outlining specific actions including:

- **Process for evaluating potential** bias and equity implications of evidence.
- **Creation of enrollee advisory groups**, including patients and other members of diverse communities with relevant lived experience, with explicit opportunities to participate in specified stages of negotiations.
- **Calling for and incentivizing evidence generation and data collection** with underrepresented populations.

Systematic approaches to elevating patient voices are needed

Affected beneficiaries (e.g., current patients), their families and caregivers, and organizations that represent them must be actively involved in DPNP processes with a meaningful voice in decision making, and specific measures to ensure patient experience and other inputs are elevated in CMS's evaluation of candidate drugs must be explicitly outlined in the guidance.

IVI appreciates the steps taken in the initial guidance to provide opportunities for patients, their families and caregivers, and organizations that represent them to provide input or submit evidence to support DPNP negotiations. We are concerned that the initial guidance fails to include mechanisms for patient engagement beyond evidence submissions and does not include guidelines or processes to ensure evidence from patient experience, qualitative or quantitative, is incorporated alongside other evidence such as clinical trial data.

Formal mechanisms for patient engagement in negotiations should be included to provide essential guidance and context from lived experience, such as a treatment-specific advisory group formed for each drug following its selection for negotiation. Mechanisms for inclusion of patient stakeholder voices are also a key tool for advancing health equity. Inclusion of members from diverse populations and backgrounds to serve on patient advisory groups provides members of historically underrepresented communities an important opportunity to have a voice in the process.

Engaging patients in dialogue or soliciting input from patients and other affected populations is necessary but not sufficient, however. It is critical that CMS explicitly include measures throughout the DPNP processes to ensure patients have equal standing as stakeholders and participants in decision making. In the case of a patient advisory body, guidelines should outline the topics within the group's scope, including

how input and recommendations will be documented. Most importantly, the guidelines should identify specific points in DPNP processes where advisory groups have complete or partial decision authority and provide a detailed outline of these processes.

Similar steps are required to ensure that evidence submitted on patient experience, patient preferences, and similar topics is included in negotiations, especially in the context of determining unmet need, degree of therapeutic advance, and comparison to alternative therapies. Such evidence is frequently dismissed or discounted in similar contexts due to perceived lack of rigor relative to other evidence or lack of experience incorporating qualitative data. DPNP guidelines should specify measures to ensure consistent inclusion of patient experience data.

Measures include:

- Inclusion of patient researchers in CMS evaluation teams.
- Explicit frameworks for evaluation of evidence and weighting in reviews.
- Guidelines for mixed-method approaches designed to account for qualitative evidence.

Clarity and specificity in methods and overall approach are needed

While a "qualitative approach" to evaluating evidence on a given drug provides CMS staff with needed flexibility, greater transparency into this process is needed.

As a research organization grappling daily with the challenges and complexity of comparing medical technologies in the context of HTA, IVI recognizes the need for flexibility in CMS's approach (as outlined in sections 50 and 60) when evaluating evidence, conducting analyses, and evaluating therapies in negotiations. To ensure transparency, predictability, and consistency, however, a flexible and "qualitative approach" should be conducted within a methodological framework describing types of data and evidence to be used, guidelines for evaluating and prioritizing evidence, procedures for engagement with external parties, methodological guidelines (especially for mixed-method approaches), and other relevant subjects. Articulating these considerations will provide consistent guidance to CMS teams working in varied therapeutic areas, increase the relevance and usability of evidence submitted to CMS, and provide the transparency necessary to ensure both rigor and accountability.

As part of this descriptive framework, the initial guidance should include greater detail on a number of methodological subjects, including:

- **Therapeutic advance**: Provide clear definition and outline the methods for measurement and evaluation

- **Clinical benefit**: Provide more details on how "clinical benefit" will be translated into "adjusting the starting point" for negotiations. For example, adjustment amount and how patient input will contribute to this definition
- **Unmet need**: Provide clear definition, including steps for consideration of health equity, and outline the methods for measurement and evaluation
- **"Other factors" to be considered**: Further define what "other factors" CMS would consider. These should include patient lived experience as well as additional elements of value, including both patient-level elements, for example non-clinical and economic impacts, caregiver burden, and patient preferences. and elements of value from the societal perspective, including scientific spillovers, impacts on innovation, real-option value, insurance value[6]
- **Modeling and analysis**: Describe whether and in what ways simulation modeling, decision analyses, or other approaches may be used for therapy evaluation

Finally, the potential discriminatory effects of the quality-adjusted life year (QALY) are well-documented, but it remains the most widely accepted methodological tool for incorporating impacts on quality of life alongside life extension. The guidance currently underscores the risks of discrimination without recognizing this methodological need, describing ongoing efforts to identify other potentially discriminatory metrics for exclusion from CMS consideration. The guidance fails to recognize both the methodological role of the QALY and the importance of developing alternative methodologies. CMS should revise the guidance to reflect these considerations, encourage research to advance alternative metrics, and outline a process for considering use of new metrics in DPNP negotiations. The guidance also states that CMS will consider evidence from studies where the QALY evidence is "clearly separated," but the meaning and intent of this statement are unclear. Greater clarity is needed to prevent confusion on the part of researchers and stakeholders interested in submitting evidence, and to ensure evidence submitted is aligned with CMS expectations.

DPNP guidance must be informed by forward-looking strategic considerations

In an environment where availability of evidence and methodologies lags behind the needs of comprehensive value assessments, CMS must endeavor to spur innovations that advance the field, for example, by calling for data on relative treatment affects across patient subpopulations and encouraging exploration of novel methods to capture quality-of-life outcomes as an alternative to QALYs.

IVI is founded on the belief that a patient-centric, value-driven healthcare system is possible with the collaboration, inclusion, and investment of all stakeholders. CMS has a

[6] See footnotes 2 and 3 for relevant references.

unique opportunity to move that vision forward in its implementation of the Medicare DPNP.

We call on CMS to embrace this opportunity. The steps implemented to engage and include diverse populations of patients in negotiation processes can set a higher standard for private health plans. Demonstrating mixed-method approaches to evaluation may drive increased incorporation of patients' lived experience, while the outcomes and other factors considered in the process will shape both the priorities of the research community and the evidence it produces.

We appreciate the opportunity to provide input on this important issue. Please do not hesitate to contact me or Mark Linthicum, Director of Policy, at mark.linthicum@thevalueinitiative.org for further discussion.

Sincerely,

Jason Spangler, MD, MPH, FACPM
Chief Executive Officer
Innovation and Value Initiative