A summit on amplifying voices of patients, caregivers, and people with disabilities in Inflation Reduction Act drug price negotiations

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Plain language summary

The Inflation Reduction Act (IRA) changes how Medicare pays for some prescription drugs. For the first time, the Centers for Medicare & Medicaid Services (CMS) can ask drug companies for lower prices. What is important to patients should be included in a drug's value. CMS should use data about what patients value when defining a drug's "clinical benefit." CMS should also create an ongoing process to include voices of patients and providers in valuing drugs.

Implications for managed care pharmacy

This research provides an analysis of the possible unintended consequences of the IRA and a framework of patient engagement with CMS to mitigate concerns surrounding the implementation of the Medicare Drug Price Negotiation Program. This framework, centering on a 2-way dialogue of patient and caregiver experience data and feedback, should be used by CMS to create a transparent, patient-centered, and successful implementation of the Medicare Drug Price Negotiation Program under the IRA.

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J Manag Care Spec Pharm.
2024;30(3):247-51

ABSTRACT

On September 18, 2023, Cancer Support Community convened patient and caregiver advocates, health care providers, policy experts, and health care innovators and thought leaders for a roundtable discussion on the need to ensure that patients, people with disabilities, and caregivers have a voice in defining "clinical benefit" for the purpose of Medicare Part D drug price negotiations and future health care policies that impact patients. The meeting featured presentations from Lara Strawbridge, Deputy Director for Policy at the Medicare Drug Rebate and Negotiations Group in the Center for Medicare, regulatory expert, Dr Monique Nolan, Counsel at Arnold and Porter, LLP, and 3 panel discussions:

- IRA Implementation—What Matters to Patients, a discussion of policies expected to impact patients and caregivers who are likely to rely heavily on high-cost drugs or biologics to treat cancer or other chronic illnesses, as well as the future development of novel therapies;
- The Science of Measuring Patient Experience, a discussion of current science of measuring patient experience and how it should be incorporated into the definition of clinical benefit; and
- Developing an Infrastructure for External Feedback, a discussion of actions and goals for patient engagement, advocacy opportunities, and how to best coordinate such efforts.

This article represents an analysis of relevant resources as well as highlights from these sessions and subsequent discussions. It also outlines principles for engaging patient and provider advocacy organizations, whether in policy, media, or online discussions, surrounding the implementation of the Medicare Drug Price Negotiation Program.

Impact of the IRA on Patients

The Inflation Reduction Act of 2022 (IRA) includes substantive changes to the ways the Medicare program pays for beneficiaries' prescription drugs. Some changes, like "cap" and "smoothing" policies, are important improvements for patients. However, policy allowing the federal government to negotiate drug prices for covered beneficiaries has been met with some concern and skepticism. As pharmaceutical companies voice concerns about decreasing revenue impacting their investments in research and development, patient advocacy organizations are left to worry about future availability of new and innovative treatment options,

investments in treating rare diseases, and economic forces limiting access to safe, effective, and preferred treatment options. There is even concern that payors will use negotiated prices as benchmarks for spending to justify increasing utilization management practices.

Oversight of these potential unintended consequences will be essential. It is imperative that the Centers for Medicare & Medicaid Services (CMS) articulate how it will benchmark and assess implementation and monitor its impact on insurance premiums, industry investments, and development of new therapies. The patient community cannot afford to wait for regulators to notice that they are struggling with the impacts of the program before taking corrective action. The agency must establish clear processes to monitor the impacts of IRA drug price negotiations, regularly solicit patient feedback, and be nimble enough to respond quickly to any ill effects.

One way that CMS can ensure patients benefit from the IRA is to ensure that patient voices are included in all phases of implementation. CMS has the opportunity to define "clinical benefit" for the purposes of drug price negotiations such that it recognizes and elevates the science of patient engagement to recognize true health care value. It makes sense that the needs of patients and caregivers will change over time, and it makes sense that 2 individuals might experience the same events in entirely different ways. Policies should not dictate that patient feedback is static and standardized. Instead, CMS must use this opportunity to build a new system that acknowledges and responds to diverse and evolving patient experiences. Patients must have the opportunity to share their care preferences, impacts on quality of life, and what they value about their treatment throughout the process of defining clinical benefit and value in the price negotiation process.

KEY RESEARCH AND ANALYSIS

Regulators implementing the IRA should not dismiss the concerns about research and development expressed by patients but instead implement an engagement process that allows for identifying potential unintended consequences as quickly as possible and taking immediate corrective action as they come to light. Patients and organizations representing the interests of patient and disability groups may be at the forefront of identifying where expected innovation slows or stops. CMS should be listening to and working with patients and people with disabilities to determine whether there is a causal relationship between IRA policies and certain negative externalities affecting access to care in the coming years and taking steps to address them.

For example, evidence suggests that the IRA may reduce biologics and pharmaceutical companies' revenue, which may narrow future research and development (R&D) investments. A 2019 peer-reviewed article notes that "The impact of reduced R&D would, in turn, depend on the degree to which lower profits and R&D investments would stymie the development of high-value, innovative new products or simply decrease the output of low-value duplicative products."[1] A model discussed in a University of Chicago issue brief estimates that the IRA price negotiations will cause a $663 billion reduction in private R&D spending in oncology between 2022 and 2039. This could lead to 135 fewer new cancer drug approvals, illustrating the need to monitor the effect on drug development and accessibility of innovative therapies.[2]

Reports have also suggested that the IRA orphan drug carve-out disincentivizes pharmaceutical companies from conducting research into secondary indications, as multiple indications would cause the drug to lose orphan status, making it eligible for price negotiation.[3] According to one white paper's estimates, any extra indication would have to produce a minimum of a 40% increase in revenue to offset the losses due to price negotiation.[3] Greater than 70% of additional indication approvals occur more than 7 years after the initial approval, leaving only 2 years of patent life before price negotiations occur, as the IRA reduced the effective patent life of small-molecule drugs to 9 years.[4] This effect translates to 79 fewer small-molecule drugs, 188 indications, and 116 million life-years lost over 20 years.[5] Remarkably, despite this reduced patent protection, this issue brief suggests that, because of the IRA, patients may be less likely to see generic drugs enter certain marketplaces—meaning that drug price negotiation could actually increase prescription drug prices in the long term.[5]

Alternatively, Shepherd suggests in a 2017 publication that in contrast to just imposing price controls, which will likely lead to reduced innovation, promoting competition would lower pharmaceutical prices and drug spending without such negative effects.[6] "Competition" may refer to competing prices, in relation to generic equivalents, as well as competition between drugs with the same overall survival benefits with different side-effect profiles and treatment pathways. Even when 2 drugs have the same efficacy, the ability to choose a different side-effect profile can be extremely meaningful to patients' quality of life. Inhibiting R&D, particularly in developing new drugs and finding additional indications for approved drugs, could limit the available treatment options and decrease the ability for patients and caregivers to include their preferences in treatment decisions.

Finally, some patient advocates are concerned that IRA implementation is likely to increase in utilization management practices by insurers, creating barriers for patients to prescribed treatment and care. The changes

to Part D benefits increase insurers' liability from 15% of costs during the catastrophic phase in 2023 to up to 60% in 2025.[7] This may cause payers to mitigate their increased costs using utilization management techniques, such as step therapy. Medicaid Part D plans may narrow access to certain medicines to reduce their costs further, likely leading to "nonmedical switching" for stable older patients, potentially resulting in a suboptimal alternative for their specific needs.[8]

It is important to note that some studies and models cited in this analysis are industry-sponsored and non–peer-reviewed sources, including white papers and issue briefs. Although they offer valuable perspectives and data, their potential bias toward the interests of sponsoring organizations should be considered when interpreting the findings.

Patient Engagement

Patient advocates have long engaged with policymakers, regulators, and other stakeholders on a variety of issues that impact access to critical treatments. As a result of those efforts, there are existing frameworks for engagement, such as those written by research institutes and the US Food and Drug Administration (FDA), cited at the end of the article, that can serve as models for CMS related to the implementation of the Medicare Drug Price Negotiation Program (MDPNP).

The June 30, 2023 guidance from CMS stated that CMS will be holding "patient-focused listening sessions in Fall 2023 for patients and other interested parties to share patient-focused input on therapeutic alternatives and other section 1194(e)(2) data regarding selected drugs."[9] Although well intentioned, CMS' recent patient-focused listening sessions surrounding the MDPNP did not adequately represent a broad and diverse range of patient and caregiver perspectives, highlighting the need for continued opportunities for patients to share their care preferences, impacts on quality of life, and what they value about specific treatment plans as implementation of the MDPNP continues. The recommendations discussed below, as well as those outlined in our "Principals of Engagement," would increase patient representation, transparency, and access to information, creating a patient engagement feedback mechanism that gives way to successful and patient-centered implementation of the MDPNP under the IRA.

First, CMS should facilitate a 2-way dialogue throughout the listening session process, offering a concrete list of areas on which they are seeking feedback, including disease- or drug-specific questions, prior to the listening session. For example, CMS should share potential therapeutic alternatives and ask for patient and provider perspectives to ensure optimal feedback. To the extent feasible, CMS should also share data sources under consideration prior to the listening session so patients are able to provide feedback on the patient-centricity and relevance of the source.

Additionally, CMS must ensure staff included in meetings are decision-makers within the agency and in a position to engage in conversations with patient participants that translate into meaningful considerations of a drug's therapeutic benefit and unmet need. CMS should also publish transcripts of the listening session to the public. Lastly, following the first year of the negotiated price being enacted, CMS should hold an additional listening session for patients and providers to share their experiences and learn from CMS about its impact on affordability, access, and availability of treatments.

PRINCIPLES OF ENGAGEMENT FOR CMS

To effectively implement the MDPNP, it is crucial to actively engage patient advocacy organizations, patients, and caregivers in a structured and meaningful manner throughout the process. To do so, CMS must work to include broad diversity in outreach to patients and people with disabilities to ensure that the MDPNP supports all patient populations and does not threaten treatment access. Additionally, the implementation process must consider the groups and populations that have not already engaged in defining patient-focused clinical benefit and impact of the MDPNP process and determine how best to activate those individuals to avoid exacerbating existing health equity challenges. Sufficiently engaging patients in the definition of clinical benefit means prioritizing evaluations around endpoints, patient-reported outcomes, patient experience data including impact on quality of life, and preferences that matter most to patients living with cancer and other complex conditions. This includes both qualitative and quantitative measures such as clinical endpoints, patient preference data/models, patient-reported outcomes, and social impacts.

To optimize the implementation of the MDPNP, it is imperative to establish a robust infrastructure dedicated to educating the patient community and facilitating meaningful feedback that prioritizes patient definitions of value, including feedback on the evidence being considered by CMS and whether it reflects patient experiences and preferred outcomes. Additionally, patient education includes hiring patient navigators to provide information to patients about the impact of these policies and to receive feedback from patients, with an explicit goal to identify any changes in utilization management practices as a result of IRA implementation.

Lastly, developing a system of data collection and evaluation is critical to monitoring the impact and success of the MDPNP. CMS must work to collect and

incorporate meaningful data and real-world evidence that amplifies patient values and input within the MDPNP implementation process, including patient-reported outcomes, patient experience data, impact to quality of life, and models that capture the dynamic and varied preferences of patients. Additionally, CMS should collect and report specifically on access challenges facing patients as a result of the IRA to allow for continuous improvement of the MDPNP process and lessen these unintended consequences of this process on patients. With these data, there must be a monitoring and evaluation platform and reporting framework surrounding the MDPNP and its impacts on patients to support continuous improvement in ongoing implementation and expansion.

Other resources on effective patient engagement and patient-focused implementation processes include the following:

Patient-Centered Outcomes Research Institute (PCORI). Best Practices in Engaging Stakeholders. Accessed October 30, 2023. https://research-teams.pcori.org/stakeholders#Planning%20for%20Collaboration

- Guidance for the PCORI Engagement Rubric that provides guidance on methods for engaging stakeholder partners throughout each phase of a research study.
  PCORI. Advisory Panel on Patient Engagement. March 28, 2023. Accessed October 30, 2023. https://www.pcori.org/about/pcoris-advisory-panels/advisory-panel-patient-engagement
- PCORI's Advisory Panel on Patient Engagement that outlines "Equity and Inclusion Guiding Engagement Principles," providing guidance on how to set diversity, equity, and inclusion at the center of health research partnerships.
  National Health Council. Patient Engagement, Capturing and Including the Patient Voice. Accessed October 30, 2023. https://nationalhealthcouncil.org/issue/patient-engagement/
- Compilation of sources seeking to identify and educate best practices for integrating the patient voice into the conduct of research and health care decision-making.
  FDA. CDER Patient-Focused Drug Development. November 1, 2023. Accessed November 1, 2023. https://www.fda.gov/drugs/development-approval-process-drugs/cder-patient-focused-drug-development
- Sources outlining FDA's systematic approach to help ensure that patients experiences, perspectives, needs, and priorities are captured and meaningfully incorporated into drug development and evaluation.
  FDA. FDA Patient Engagement Overview. September 14, 2020. Accessed October 30, 2023. https://www.fda.gov/patients/learn-about-fda-patient-engagement/fda-patient-engagement-overview
- Overview of FDA's Patient Engagement initiatives, outlining the purpose, frequency, participants, topics, and more.

Conclusions

Patients living with cancer and other complex conditions, alongside their caregivers, families, and loved ones, have unique stories and experiences to share regarding their journey and what matters most to them. CMS must continue to engage patient and caregiver communities to ensure that their perspectives are meaningfully incorporated into the MDPNP implementation process, especially in defining clinical benefit.

ACKNOWLEDGMENTS

The authors acknowledge Gilead, Amgen, Sanofi, Bristol Myers Squibb, and AbbVie for their support of the September 18 event. The recommendations derived from these proceedings represent the input of summit participants and not the event sponsors. Special thanks to Rational 360 for their help with the event and materials.

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