August 29, 2024

The Honorable Chiquita Brooks-LaSure
Administrator
Centers for Medicare & Medicaid Services
Department of Health and Human Services
200 Independence Avenue SW
Washington, DC 20201

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

Dear Administrator Brooks-LaSure and Deputy Administrator Seshamani:

On behalf of the more than 30 million Americans living with one of the over 10,000 known rare diseases, the National Organization for Rare Disorders (NORD) thanks the Centers for Medicare and Medicaid Services (CMS) for the opportunity to comment on the Negotiation Data Elements and Drug Price Negotiation Process for Initial Price Applicability Year 2027 under Sections 11001 and 11002 (CMS-10849). Millions of Medicare beneficiaries are living with a rare disease, and many struggle with high out-of-pocket prescription drug costs.[1] Implementation of the Medicare Drug Price Negotiation Program (MDPNP) will have a significant impact on our rare disease community, and we are encouraged by the continued solicitation of further ways to better include affected communities in the information collection process.

NORD is a unique federation of non-profits and health organizations dedicated to improving the health and well-being of people living with rare diseases. NORD was founded more than 40 years ago, after the passage of the Orphan Drug Act (ODA), to formalize the coalition of patient advocacy groups that were instrumental in passing that landmark law. Our mission has always been, and continues to be, to improve the health and well-being of people with rare diseases by driving advances in care, research, and policy.

We greatly appreciated CMS' efforts to engage patients as part of the 2026 MDPNP implementation. NORD recognizes the time pressure under which CMS established last year's ICR and patient listening sessions, and we value the extensive efforts to incorporate patient perspectives. Furthermore, NORD is grateful for the various opportunities we have had to share our expertise in patient engagement with CMS and has collaborated with other stakeholders and

[1] *Prescription Drug Affordability among Medicare Beneficiaries*. HHS- ASPE Office of Health Policy. (19 January, 2022). https://aspe.hhs.gov/sites/default/files/documents/485edf2a2d4870f88a456df61c8ff471/prescription-drug-affordability.pdf

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patient advocacy groups to provide detailed recommendations to CMS to ensure the perspectives of the patient community are appropriately represented in the listening sessions.[2]

In seeking to improve collection of patient perspectives for future iterations of the MDPNP, we and others have identified a number of aspects related to last year's processes that were barriers or challenges that might have made it prohibitively difficult for some patients and caregivers to participate.[3,4] Our concerns included: premature closing of the written submission portal for patients; limited speaking time for patients and interactions with CMS staff in the listening sessions; and limited transparency into how the patients responses would be used. Though encouraged by CMS' repeated commitments to learn from prior years, improve the program for future years and to increase accessibility of both the listening sessions and the ICR to patients, we are disheartened to see a number of promised elements are not explicitly addressed in this ICR (or currently otherwise available to the public).[5,6]

For example, the May 3rd CMS draft guidance on implementation of the MDPNP for Initial Pay Applicability Year 2027 stated the "ICR will incorporate lessons learned pertaining to the collection process, question format, and content received from respondents for initial price applicability year 2026." Though we recognize that an additional ICR with a 30-day comment period is set to come out later this year, we have not seen any specific discussion of lessons learned, or meaningful changes to the questions, format, or content in this iteration of the ICR in response to last year's challenges that would improve the process for patients, caregivers, or healthcare providers.

Similarly, in the July 2nd email announcing the ICR, CMS referenced plans for a "publicly available web link" for members of the public to submit evidence about therapeutic alternatives.[7] We agree with CMS that the ICR submission process for manufacturers (through an HPMS link) is neither patient friendly nor an appropriate pathway for the voluntary submission of patient experience data and we support CMS's plans to create a separate, stand-alone portal.[8] Unfortunately, we have not seen a link for the general public to provide relevant information. In

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[2] *Amplifying the Patient Voice: Roundtable and Recommendations on CMS Patient Engagement.* National Health Council (NHC). (March, 2024). https://nationalhealthcouncil.org/wp-content/uploads/2024/03/Amplifying-the-Patient-Voice-Roundtable-and-Recommendations-on-CMS-Patient-Engagement.pdf

[3] *Amplifying the Patient Voice: Roundtable and Recommendations on CMS Patient Engagement.* National Health Council (NHC). (March, 2024). https://nationalhealthcouncil.org/wp-content/uploads/2024/03/Amplifying-the-Patient-Voice-Roundtable-and-Recommendations-on-CMS-Patient-Engagement.pdf

[4] https://rarediseases.org/wp-content/uploads/2024/07/NORD-Comments-MDPNP-IPAY-2027_F.pdf

[5] *Draft Guidance on the Medicare Drug Price Negotiation Program.* CMS- Center for Medicare. (May 3, 2024). https://www.cms.gov/files/document/medicare-drug-price-negotiation-draft-guidance-ipay-2027-and-manufacturer-effectuation-mfp-2026-2027.pdf

[6] *Medicare Drug Price Negotiation Program: Negotiation Data Elements and Drug Price Negotiation Process Initial Information Collection Request Published for Comment.* CMS- Center for Medicare. (July 2, 2024).

[7] *Medicare Drug Price Negotiation Program: Negotiation Data Elements and Drug Price Negotiation Process Initial Information Collection Request Published for Comment.* CMS- Center for Medicare. (July 2, 2024).

[8] Ibid

our comments on the IPAY 2027 guidance, we emphasized the importance of beginning the public solicitation process early and partnering with trusted stakeholders to ensure the public facing portals are sufficiently approachable. Without timely access and testing with the population using the portal, we are concerned that the public submission process will continue to stumble. Therefore, we encourage CMS to describe in detail the data elements and questions for the public submission and to stand up the publicly available web link as soon as possible.

Recognizing their crucial role in assessing the impacts of the MDPNP, we are pleased to provide the following specific recommendations for the successful engagement of patients, their caregivers and healthcare providers[9] through listening sessions and the Information Collection Request (ICR) for the 2027 MDPNP:

1. Decouple and simplify the collection of patient experience data through this ICR or the subsequent ICR relevant to the 2027 MDPNP
2. Leverage the ICR and externally-led patient-listening sessions to complement data collection efforts
3. Pilot-test the questions and engage patient engagement experts as well other relevant government, academic, and private sector experts at every step of the data collection process

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1. Follow through on decoupling and simplifying the collection of patient experience data in this ICR or the subsequent ICR relevant to the 2027 MDPNP

The primary purpose of this specific ICR is to facilitate the mandatory collection of manufacturer data, guided by statutory data elements, rigid processes, and tight timelines. The collection of patient experience data is both qualitatively and quantitatively very different from this primary purpose as collecting patient experience data is neither subject to statutory data elements nor does it have to follow the very tight timelines for manufacturer-provided data that would be virtually impossible for most patients to navigate. The type of data elements collected are also different, as evident from the ICR – with the manufacturer data mostly quantitative and clearly defined, capturing highly concrete issues such as a drug's annual sales volume, unit price of production, or patents and exclusivities.

In contrast, the patient-reported data is by design significantly more qualitative and much less precisely defined, capturing issues such as the extent to which a drug provides a meaningful advantage over an alternative therapy, or the extent to which an unmet medical need is not adequately addressed by available therapies. In fact, even the key audience for the patient reported data elements is significantly different from the manufacturers, and is likely to include patients and families, health care providers, academic researchers, and other relevant

[9] *Draft Guidance on the Medicare Drug Price Negotiation Program.* Ibid.

stakeholders. Additionally, the number of individual potential respondents is exponentially higher than for the manufacturer data. As a result, the ICR is unlikely to be an effective tool for capturing patient-reported data and we strongly support CMS's intention to create a separate, stand-alone process.

NORD continues to be concerned CMS' plans to largely rely on this ICR for voluntary data submissions by the public will be unsuccessful. As proposed, the data collection will occur on very short timelines, without meaningful data standardization, using complicated forms written at too advanced reading levels and depending on hard-to-navigate processes that are neither intuitive nor patient-friendly. Based on experiences with last year, NORD is specifically concerned that patients will either not become aware of the data collection effort in time, or struggle to navigate the complex submission process. The extent to which individual data submissions will be confidential and protected from disclosure will be confusing to patients, and we worry the burden for patients not familiar with a process that was developed for manufacturers may be significantly higher than estimated, in particular for patients who may navigate additional challenges such as language barriers, visual impairments, or lack of (broadband) internet access. In addition, the required attestations are worded in a way that will likely discourage many patients from submitting data, and to the extent patients will feel compelled to submit data containing Personal Identifiable Information (PII) and Personal Health Information (PHI), the data collection raises privacy concerns.

Moreover, NORD foresees challenges in aggregating and analyzing individual patient and provider experience data submitted through this process; the data will be collected without a sampling frame and likely not representative while the collection method essentially makes it impossible to determine or account for such inherent biases in the data. In addition, the lack of standardized questions and scientific rigor will likely render this data largely anecdotal as opposed to data collected following appropriate qualitative and/or quantitative research methodologies to collect this information in a scientifically rigorous and reproducible manner as is currently done with data collected through the FDA's patient-focused drug development meetings or patient surveys. FDA's Guidance "Patient-Focused Drug Development: Collecting Comprehensive and Representative Input"[10] for instance, provides detailed and tangible guidance on operationalizing and standardizing data collection and data management in a way that works for the rare disease patient community.

To achieve these goals, NORD urges CMS to:

- **Decouple the collection of patient-reported data from the ICR.** As outlined above, the collection of patient data has virtually nothing in common with the mandatory submission of manufacturer data. Decouple the collection of this important patient data from a process that was never meant to collect this type of data - or to engage this number and diversity of respondents.

[10] FDA GFI: Patient-Focused Drug Development: Collecting Comprehensive and Representative Input; available at https://www.fda.gov/media/139088/download; accessed 4/2023

- **Simplify and streamline the data submission process for patients, caregivers, and providers so that it is workable and does not provide undue barriers to providing the requested information**. Decoupling the process from manufacturer provided data will allow CMS to create a data collection process that is designed to be patient-centered, with input and guidance from patients at every step of the process. This should include pre-testing the forms, attestations, and instructions with representatives of the relevant community to ensure they are clearly understood and easy to navigate, including by individuals with visual and other impairments. Because this data submission is voluntary and not subject to the statutory data submission timeline for mandatory manufacturer-provided data, CMS should work with the patient community to establish feasible timelines that will be workable for the community. Other concerns, such as ensuring the respondents are in fact patients, caregivers, or families afflicted by the disease and report their own experiences and perspectives, will require careful consideration, in close collaboration and with guidance from the patient community. FDA listening sessions, patient-focused drug development meetings, and other FDA-led initiatives routinely navigate these challenges and collect meaningful patient experience data in ways that work for rare disease patients and families and can serve as a valuable guide and resource for CMS, including all applicable attestations and data protections.

- **Clarify now what information the agency is seeking from patients and in what format to allow data standardization and aggregation.** The short time period outlined for the negotiation process makes it imperative to provide detailed instructions as early as possible, before the negotiation period begins, to facilitate and streamline the collection and submission of meaningful data from a patient perspective. Clarifying the key data elements in sufficient granularity ahead of time will also empower patient advocacy groups and other important stakeholders to proactively collect and collate relevant information in a way that is scientifically rigorous and representative of the relevant patient community.

2. Intentionally leverage the ICR and externally-led patient-listening sessions to complement data collection efforts and engage a maximum number of patients, caregivers, and healthcare providers

NORD thanks CMS for recognizing the unique and nuanced value drugs can bring to specific subsets of the patient population, including rare disease patients who often have few or no therapeutic options. NORD commends CMS' efforts to consider data on clinical benefit, therapeutic alternatives, and unmet medical need in the negotiation process. The agency's stated objective to assess value in an indication-specific manner including some off-label uses, is critical to CMS understanding the complex tradeoffs and unmet needs that exist within the rare disease patient community. Moreover, we are encouraged that CMS has explicitly recognized the value of patient experience data, including its nuances, and the expectation that not all patients

are necessarily sharing the same views and experiences. For instance, the science of patient engagement has long recognized that patient experience data may reflect differences depending on disease progression or a patient's cultural, geographic, and socio-economic background. While we are grateful CMS recognizes the value of patient experience data, we strongly encourage CMS to expand the opportunities and strengthen the processes for providing such input.

The external data CMS staff plan to rely on in the negotiation often does not exist for most rare diseases, creating an added burden for CMS and the affected community to collect this data. CMS plans to supplement the data submitted by the public through this ICR with relevant published data, relying on such data being readily available to CMS staff through literature searches. Unfortunately, it is a recognized challenge that for many rare diseases, data relevant to determine a negotiated product's clinical benefit, therapeutic alternatives, or unmet medical need often does not currently exist in peer-reviewed journals or consensus treatment guidelines. FDA's Voice of the Patient (VOIP) reports, which are trying to fill this void, are playing an increasingly important role in patient-focused drug development and frequently collect meaningful information on how patients evaluate therapeutic alternatives or characterize the unmet need and clinical benefit of alternatives. However, these data are not indexed in a way that would clearly find them in a traditional literature search. In addition to ensuring CMS considers all relevant data collected as part of the FDA approval process in the negotiation process, patient and provider engagement will be critical to ensure CMS is aware of and able to leverage all available data. This is particularly important for rare diseases because the lack of disease-specific International Classification of Disease (ICD-10) codes for most rare diseases makes strategies relying on existing real-world data (RWD) from sources such as electronic health records (EHRs) or medical claims data largely infeasible for many rare diseases.

CMS will have to collect data on treatment alternatives, clinical benefit, and unmet medical need for rare diseases *de novo,* including from patients, caregivers, and providers. In fact, patients and caregivers have key insights on issues such as determining the value of a therapy and how it compares to potential alternate treatment options. For instance, rare disease patients are often uniquely positioned to share the challenges associated with unmet medical needs - when there are no or very few options available to treat their condition - and the benefits to themselves, their families, and the community from a safe and effective therapy. Patient experience data will be particularly important given CMS' desire to evaluate price on an indication-specific level including certain off-label uses, which are common in the rare disease space albeit notoriously hard to study.[11] Because published data to assess these specific uses remain scarce, patients and providers are often the best experts from which to elicit such information for the rare disease community.

[11] Fung A, Yue X, Wigle PR, Guo JJ. Off-label medication use in rare pediatric diseases in the United States. Intractable Rare Dis Res. 2021 Nov;10(4):238-245. doi: 10.5582/irdr.2021.01104. PMID: 34877235; PMCID: PMC8630459.

To achieve these goals, NORD urges CMS to:

- **Partner with key stakeholders on externally-led patient listening sessions specific to selected drugs to collect representative data to inform CMS' initial offer for a negotiated price.** In planning these sessions, CMS should use FDA patient listening sessions as a roadmap and work closely with the impacted patient communities to develop a representative and meaningful data collection effort. For instance, while we appreciate CMS intends to only focus on pharmaceutical alternatives and to primarily consider alternatives in the same drug class, we recognize non-pharmaceutical options such as surgery are often the only viable alternative for our patient populations and that therapeutic alternatives in other drug classes and with other mechanisms of actions may be the most appropriate alternatives for some of our patients. Engaging the patient community in planning the listening session will help ensure that these alternatives are appropriately considered. Having external groups take a leadership role can also help address both CMS staffing shortages and concerns about administrative and logistical issues (e.g., compliance with administrative and legal requirements for federal data collection).

- **Patient listening sessions will likely be most effective if they focus on one negotiated drug and one (or potentially multiple closely related) uses or indications.** This may require prioritization among drugs and indications that will be part of the negotiation program and should be guided by considerations such as to what extent the patient listening session will generate unique data to close key data gaps and to what extent the generated data is likely to materially impact the price negotiation. Transparency and engagement of the stakeholder community in this decision-making will be key to success. In fact, pre-meeting community surveys and enrollment strategies such as snowball sampling, when used appropriately, can be effective in helping to ensure the listening sessions will truly reflect the affected community.

- **Other considerations include issues such as:** ensuring appropriate representation and diversity of perspective among the meeting participants; identifying and prioritizing questions for meeting participants ahead of time to provide time to prepare; carefully designing and pre-testing questions with consideration for well-established heuristics and cognitive biases (e.g., anchoring and adjustment, bandwagon effect, availability); and developing tools and approaches to capture the meeting outcomes in a way that is scientifically valid and allows participants to review the summary. Here again, FDA's experience with patient listening sessions and patient-focused drug development meetings will be able to provide valuable lessons learned.

- **Include consistent and granular summaries of the data and assumptions on which each negotiation was based, including patient experience data.** Recognizing CMS has until March 1, 2027 to release information on how negotiated prices were determined, we

urge CMS to report, as soon as possible, a detailed and standardized summary of the data relied upon in the negotiation process including the therapeutic alternatives, clinical benefit, off-label use, and unmet need for each indication and the data sources relied upon. CMS should further break out the use of patient experience data and patient-reported outcomes; list data identified by CMS through literature searches and guideline review as well as primary data, such as claims, EHR, or other real-world evidence (RWE), generated and collated by CMS. This level of transparency will be key to create consistency and trust in the negotiation process now and for subsequent rounds. Clearly breaking out the use of different data will also motivate the creation of valuable patient experience data for future negotiation years. In fact, much of the data for rare diseases collected through this process will be unique and useful beyond this specific negotiation process.

3. Pilot-test the questions and engage patient engagement experts as well other relevant government, academic, and private sector experts at every step of the data collection process

As CMS works to integrate patient perspectives in the MDPNP, the agency can draw upon a rich set of existing data, relevant scientific knowledge, and experience. For instance, considerable deliberation and research has gone into defining and measuring key concepts such as unmet medical need or therapeutic advantage.[12] Rather than reinventing these concepts, CMS can draw upon decades of practice in the FDA space to streamline and fast track the process. Similarly, the science of patient engagement has made tremendous progress in the past decade. The academic literature is full of scientific studies seeking to identify best practices, develop tools to streamline the process, and capture the value of patient engagement. In fact, a 2014 systematic review of patient engagement in research identified 142 studies that met the inclusion criteria[13] – and hundreds more studies have been published in the decade since. FDA has made leaps in developing patient engagement best practices and tools that are largely applicable across FDA's product centers and through every step of the product life cycle.

CMS itself has a long history of successfully engaging patients and families. Tools such as CMS' Person and Family engagement strategy[14] have been instrumental in empowering patients and families to be meaningful partners in the design, delivery, and evaluation of their care. NORD also brings a wealth of experience engaging patients in various parts of the drug development and reimbursement space, and a range of other non-profit and academic institutions from the Patient-Centered Outcomes Research Institute (PCORI) and the Milken Institute's FasterCures Center to the Medical Device Innovation Consortium (MDIC) to a range of more disease-

[12] https://www.fda.gov/files/drugs/published/Expedited-Programs-for-Serious-Conditions-Drugs-and-Biologics.pdf
[13] https://bmchealthservres.biomedcentral.com/articles/10.1186/1472-6963-14-89
[14] https://www.cms.gov/Medicare/Quality-Initiatives-Patient-AssessmentInstruments/QualityInitiativesGenInfo/Person-and-Family-Engagement

specific patient groups and many, many, others will have meaningful advice to offer. Relying on this wealth of experience and tried-and-true best practices, concepts and approaches will prove helpful in ensuring that patients will be meaningfully engaged in this data collection effort – but the right experts will have to be at the table when the data collection strategy for patient experience data is developed, implemented, and assessed.

To achieve these goals, NORD urges CMS to:

- **Engage with FDA patient engagement experts and other relevant government, private and non-profit sector experts.** This will help lay the foundation for a resilient and sustainable patient engagement system to rigorously engage patients and leverage the best practices and approaches to maximize the efficiency and chance of success.

In addition to these ICR-specific recommendations, we would like to re-iterate our recommendations regarding the structure, format and content of the listening sessions:

Make the solicitation and consultation process with patients, caregivers, and health care providers more transparent, predictable, and inclusive and streamline the process to build and refine year-over-year capacity.

NORD appreciated that the patient and health care provider listening sessions for the 2026 MDPNP were livestreamed and available for the public to view. Our recommendations are based on learnings from these sessions, as well as our extensive patient engagement experience and informed by a review of the relevant literature.[15] These recommendations are intended to be complementary to recommendations provided previously, including in a recent National Health Council (NHC) white paper to which NORD was honored to contribute.[16]

Our recommendations to strengthen the solicitation and consultation processes are primarily informed by three main findings with the 2026 MDPNP listening sessions:

1. The format of the listening sessions inadvertently left out some important voices in our community (*e.g., because the public format was uncomfortable for many patients; because of language, logistical, and technology barriers; because many patients were not aware of the listening sessions; because the ICR closed before the listening sessions and patients had no opportunity to submit written comments after the listening session, and because of questions about who was eligible to participate*).

[15] *Three Ways to Improve the Patient-Focused Listening Sessions In The Medicare Drug Price Negotiation Program.* Vandigo et. Al. Health Affairs (24 June, 2024). https://www.healthaffairs.org/content/forefront/three-ways-improve-patient-focused-listening-sessions-medicare-drug-price-negotiation

[16] *Amplifying the Patient Voice: Roundtable and Recommendations on CMS Patient Engagement.* National Health Council. (24 March, 2024). https://nationalhealthcouncil.org/wp-content/uploads/2024/03/Amplifying-the-Patient-Voice-Roundtable-and-Recommendations-on-CMS-Patient-Engagement.pdf

2. Patient listening sessions provided limited data to directly inform the negotiation process and maximum fair price calculation (*e.g., because the 3-minute speaking slots were very short; because patient, caregivers, and health care providers lacked guidance on what insights would be most informative; and because the ridged session format prevented dialogue or clarifying questions*).

3. Patient listening sessions lacked standardization and were very heterogenous, generating inconsistent and widely varying outputs even for products in the same therapeutic area (*e.g., because listening sessions were organized by product rather than indication; included variable mixes of patients, caregivers, and providers; and because they lacked a standard set of questions*).

To ensure the listening sessions can help inform CMS about the true value of the selected therapies to the patient community and other select stakeholders, NORD is pleased to offer specific recommendations around key priorities:

1. Start preparing for the listening sessions ahead of time; be transparent and standardize the outreach and engagement processes; maximize patient engagement including from historically underserved and other harder to engage communities; build long-term relationships, capacity and support in communities that are likely impacted in this and future plan years; and smooth out agency activity and workload on patient engagement over the plan year.

a. Identify therapeutic areas that are likely impacted by the selected drugs (e.g., oncology, lung, cardiovascular, diabetes); proactively begin outreach activities to these communities now; intentionally engage harder-to-reach communities; and with a goal of building long-term partnerships.

One of the most crucial elements of a successful and inclusive public participation campaign is to begin early; partnering with trusted community voices, proactively messaging important timelines, and explaining the information to be gathered (and why) as early as possible is vital to broader participation. While we commend CMS for implementing last year's iteration of the listening sessions on a tight timeline, the reality is that limited runway in advance of the listening sessions resulted in suboptimal patient and provider representation.

Although we recognize the logistical challenges CMS faces regarding proactive patient engagement, we believe this is a largely solvable problem. By the nature of the diseases that are prevalent in the Medicare population, and considering long-standing Medicare spending patterns, it appears almost certain that a limited number of therapeutic areas, including for instance oncology, lung, cardiovascular, and diabetes and related comorbidities, will likely be disproportionately represented amongst the selected products in the 2027 MDPNP as well as in

future plan years.[17] CMS should proactively engage now with key stakeholder groups representing patients impacted by these diseases, and develop these relationships as long-term engagements to leverage in this year as well as future plan years.

Starting now and building out the engagement over time will allow CMS to engage a broader spectrum of diverse stakeholder groups, and to create sustainable, trusting, and fruitful partnerships over time. Moreover, approaching patient engagement by therapeutic area, rather than product, may lead to more diverse stakeholder engagement; for instance, while a given product may not be used by a specific patient group (e.g., because of label restrictions), that patient group may have valuable insights for this and future plan years. In addition, early and sustained partnerships with patient groups can have additional downstream benefits, such as helping to increase written comments and more robust participation in focus group sessions as the community builds capacity and individuals develop levels of familiarity and comfort with the process.

To ensure representation from patients, advocates, providers, and industry leaders from across the country, we encourage CMS to utilize their regional offices and ties to local communities to ensure appropriate patient engagement across different geographic regions. One effective way to do this is through in-person meetings; this would ideally include in-person outreach and education (e.g., at regional patient summits or health care provider meetings) and in-person listening sessions (e.g., at regional offices). While we recognize engaging individuals living in rural areas poses particular challenges, regional education and outreach will allow for richer, and more inclusive engagement than focusing outreach primarily nationally or on those located in, or able to travel to, the DC metro area. This is another area where year-over-year capacity building will be particularly valuable.

b. Develop educational and patient engagement materials that can be leveraged across products, therapeutic areas, and plan years; refine and revise these materials with input from the stakeholder community; and begin publicizing the listening sessions as early as possible BEFORE the selected drug list for negotiation is released.

CMS should begin developing and deploying educational materials and tools now to facilitate effective patient engagement in the drug price negotiation and refine and revise them with input from trusted partners (e.g., patient groups or providers with vested interest in the patient populations utilizing the likely selected therapies). This should include outreach materials in languages other than English, and particular care should be given to ensure these materials are linguistically and culturally appropriate. These activities can and should start long before the announcement of the MDPNP 2027 selected products and build on learnings and successes year over year. Because these materials can be reused in future plan years, we urge CMS to create a feedback process that can be used to refine and revise these materials over time.

[17] *Drugs likely subject to Medicare negotiation, 2026-2028.* Dickson, Sean and Hernandez, Inmaculada. National Library of Medicine. https://www.ncbi.nlm.nih.gov/pmc/articles/PMC10387900/

We encourage CMS to be as specific as possible in the materials about the logistics of the sessions to maximize transparency and give stakeholders a clear understanding of expectations. This transparency is vital to building trust and will mean more participants may be inclined to share their information and provide more meaningful responses. Specifically, in the lead up to the public listening sessions, we encourage CMS to be transparent with participants about how their data will be used and if / how they will be identified. Moreover, CMS should clarify how information from different population subgroups may be considered; for instance, patients who were formerly on a therapy may have inherently different experiences than the patients who are currently on it, and different patient populations may have different therapeutic alternatives available.

Information about how CMS intends to handle real or perceived conflicts of interest will be equally important. The lack of standardized processes or the required disclosure of professional or personal affiliations with interest groups led to inconsistent conflict of interest interpretation and implementation last year, which threatens to undermine trust in the process. We strongly recommend implementing a standardized mandatory disclosure process for professional or personal affiliations as a prerequisite for session participation.

Moreover, while CMS may not be able to release the names of the selected drugs until February 1, 2027, the agency can and should proactively set dates, times, structures, and locations (virtual and/or in person) for each listening session, focus group, or other engagement opportunity (preferably by therapeutic area). Scheduling these sessions early will make it easier for patients, caregivers, and providers to participate, and provide community partners more time to advertise the sessions and prepare their communities for the sessions. CMS should publicize the date and format (including speaker type) for the public engagement sessions even BEFORE the drug negotiation list is published. We encourage CMS to publish whether the sessions will include indication specific reviews, and if so, which of the sessions will be reserved for less common indications (including rare diseases).

A common challenge in the rare disease space is small patient populations. In addition, many rare disease patients experience several comorbidities which can make it harder to travel or rearrange pre-planned health care appointments. Announcing which sessions will be reserved for less common indications will make it easier for rare disease communities to plan, maximizing the chance of robust participation. This will allow for tailored outreach based on the therapeutic area and speaker type and allow umbrella organizations and other key stakeholders to begin socialization of the sessions as early as possible to maximize awareness.

2. Reconsider the session format; provide more options to meet patients where they are; include opportunities for patient engagement that protect patients' privacy and make it easier for all relevant patient populations to engage; better integrate the written and verbal opportunities for feedback and make the written process easier to navigate.

Following the success of the first year, we hope CMS will develop a process to continue to identify incremental improvements for future years. To ensure success of the program in future years, we encourage CMS to create a variety of virtual and in-person engagement opportunities, including smaller focus group style sessions targeted at both patients and caregivers and health care providers (we recommend separate focus groups for health care providers and for patients/caregivers); provide opportunities for more meaningful engagement between CMS staff and participants during the listening session; and provide opportunities for anonymous or closed-door engagement to lower the bar to participation for patients or caregivers who do not feel comfortable sharing their information with the public; provide opportunities for engagement specifically for patients or caregivers whose primary language is not English and those that need other types of accommodations (including opportunities for asynchronous input for those in our community who cannot take off time from work or school to participate during the scheduled times).

a. Streamline the public comment opportunities; provide opportunities for audio-only participation and for patients whose primary language is not English (e.g., Spanish-language listening sessions or real-time translation services); work with the patient advocacy groups and other key stakeholders to prepare patients better for the sessions; and continue to refine and revise the format for the listening sessions year over year.

As last years' experience clearly showed, not all patients feel comfortable sharing highly personal information about their disease or other aspects of their daily life on camera in publicly recorded settings. Furthermore, providing English-only engagement opportunities threatens to leave out important parts of the community. Establishing a system where participants can provide responses that will be deidentified and/or aggregated before being publicly posted has been shown to improve the quality of responses.[18] We urge CMS to continue to work with the affected communities to provide options that meet their needs.

b. Simplify and better integrate the written and verbal comment process to provide patients with a range of options to engage and share feedback without having to engage publicly.

After last year's data submission process, we are pleased to see there will be additional opportunities to strengthen written public comment. To ensure the public data submission process is captured in a meaningful way, we encourage CMS to increase timelines for participation, standardize the data capture process, and increase accessibility for patients with lower literacy comprehension and/or who need other accommodations to navigate the process (e.g., because of chronic diseases or physical or mental disabilities). Specifically, in our opinion, last year's public written comment process was terminated prematurely by closing it before the listening session. By failing to leave the written comment process open throughout the duration of the listening sessions, patients were forced to comply with tight timelines and opportunities

[18] *How Transparency Affects Survey Responses.* Connors, et. Al. Public Opinion Quarterly. (18 June 2019). https://academic.oup.com/poq/article/83/S1/185/5520299

for engagement were missed. Although we recognize that CMS was given a herculean task to accomplish within a short period of time, the short process was a significant barrier to participation for many patients, together with the complexity of navigating the process.

For this upcoming year, we recommend clearly publishing the timeline for public participation well in advance of the opening, alongside the questions that will be asked during the submission process. To our point on transparency above as well, we encourage CMS to share how the written submission will be considered differently than or in addition to the oral participation.

Moreover, we urge CMS to simplify and streamline the data submission process. Last year's data submission process included a complex series of mandatory forms with complicated and potentially concerning language utilizing terms that were not patient friendly. We encourage CMS to use short, simple forms at no greater than an eighth grade reading level to ensure language comprehension is less of a barrier. We view the written submission as a vital opportunity to supplement and complement the other engagement methods, including the collection of information from patient groups who may have difficulty (or wish not to) participating in oral sessions, such as individuals who speak English as a second language, or those who are impacted by audio-visual or physical challenges. All forms should be read with this in mind, and we strongly urge CMS to make the forms available in languages other than English.

To better understand who leverages the written process for future years we encourage CMS to collect voluntary demographic information from participants and/or to collect some of this information from stakeholder partners as appropriate. Moreover, we recommend streamlining the data collection process and prioritizing the information that is most important to CMS. Specifically, NORD recommends prioritizing the collection of plain-language information on:

- Demographic information, such as age, gender, race/ ethnicity, zip code
- Diagnosis and time since diagnosis
- Degree of disease progression
- If the information is provided by a patient or a caregiver
- What therapies the patient uses to manage their disease and for how long
- If the patient has tried other therapies in the past
- Degree of disease progression on treatment
- Most significant challenges in accessing medications
- How the patient feels and functions on the disease, and what symptoms remain unaddressed
- Challenges patient experienced associated with switching from one therapy to another
- What therapeutic alternatives the patient may have considered or may consider

It is also important for CMS to be clear about how written and oral submissions will be analyzed. For a variety of reasons, some patients may prefer submitting a written statement over participating in a live session. CMS may also not be able to find representatives for each of the

indications that a selected product covers and the written responses may provide meaningful ways to substantiate and expand upon the data collected in the listening sessions. However, without clarification on how patient and stakeholder submission will be analyzed, we are concerned that components of the patient populations that are more difficult to survey may fall through the cracks during the negotiation process, and that the written submission form will not be used to its maximum extent. Certain types of patients, such as those with psychiatric conditions, cognitive limitations, and sight deficiencies, are often particularly difficult to include in surveys; specific, intentional efforts will be required to allow for meaningful inclusion of these populations.[19]

Additionally, we are concerned that without clarification of how the oral and written submissions are processed, patients could feel that submitting written comments would be a less valuable contribution. Establishing a system where participants are assured that their (deidentified) responses will be publicly posted has been shown to improve the quality of responses.[20] Even if exact weights for each of the types of responses relative to other factors cannot be shared or may vary by drug and indication, simply sharing the types of analysis used (i.e. quantitative vs. qualitative), will be helpful in how patients may structure their responses to be maximally beneficial.

c. Provide opportunities for more direct interaction with CMS through focus-group sessions in addition to the public listening sessions; this will allow the agency to ask clarifying questions and better understand varied patient perspectives on the most influential aspects of the MDPNP calculations including nuanced thinking around appropriate therapeutic alternatives (in particular in therapeutic areas like oncology or immunology where switching among products may have significant and hard-to predict impacts on long-term patient outcomes).

In our prior experience hosting patient listening sessions, NORD has found smaller focus-group listening sessions to be most effective to gain granular and nuanced input. These closed-door sessions make it more comfortable for patients to share personal details about their disease and how it impacts their daily life. We recommend sessions to be limited to five to 10 participants and set between 60 and 90 minutes. Each session should be limited to patients, providers, or caregivers, depending on the focus of the specific session – and may be further tailored (e.g., by geographic area, population subgroup, or to explore specific questions such as patients' experience switching across therapeutic alternatives).

Maintaining independence of each of the sessions and limiting them to a single stakeholder type will allow participants to develop a greater level of trust, both with one another and with the moderator, and help guard against issues like halo and bandwagon effects. Including different stakeholder types risks changing the power dynamic, where some participants feel their

[19] *Barriers to Participation in a Patient Satisfaction Survey: Who Are We Missing?* Gayet-Ageron, et. Al. National Library of Medicine. (26 October, 2011). https://www.ncbi.nlm.nih.gov/pmc/articles/PMC3202588/

[20] *How Transparency Affects Survey Responses.* Connors, et. Al. Public Opinion Quarterly. (18 June 2019). https://academic.oup.com/poq/article/83/S1/185/5520299

commentary is less worthy than others, or may become more deferential, rather than all participants viewing each other as equals. In addition, we recommend each focus group to be facilitated by a skilled facilitator knowledgeable in appropriately handling group dynamics in scientifically rigorous ways.

After participants have been selected for each of the respective sessions, we encourage CMS to proactively communicate expectations and solicit requests for accommodations. Some individuals may require additional time to process the questions in advance; sending around what each of the participants will be asked is helpful in ensuring all are able to respond on time and feel comfortable doing so. We also encourage CMS to ensure the participants understand what expectations for timing are, and to help stakeholders navigate the timekeeping requirements. While the first year of the listening sessions successfully kept the conversation within the confines of time requirements, the abrupt cut off while patients were telling their stories and no response permitted from CMS staff was suboptimal. Informing participants of the time limits and setting expectations for types of follow-up questions from CMS staff will be crucial in improving the quality of responses from participants moving forward.

We also encourage CMS to consider protecting participant privacy by exclusively releasing a redacted transcript after the conclusion of these focus group sessions. Potential participants may feel dissuaded from taking part in the sessions, or not feel comfortable fully participating in the session, if their identifiable information were to be released to the general public. As we saw in the first sessions, some patients are willing to share sensitive information, and we commend the patients who were willing to share their stories. To encourage participants to share their perspective, however, and to provide more granular responses with the nuance necessary to ascertain the true value of the selected products, extending privacy protections is crucial.

3. Develop a standardized set of questions that are most relevant to CMS; develop a process to tailor these questions to each given therapeutic area, product, or patient group as needed; and focus on the key insights CMS needs most to inform the MDPNP; partner with key stakeholders to optimize the phrasing of these questions for clarity and consistency and explain how this data informs the negotiation process.

We urge CMS to introduce more structure into the sessions compared to last year. While last year's sessions included some general guidelines for how participants should respond to questions, we encourage much more specificity to standardize the feedback the agency receives and ensure the agency can utilize participant answers.

a. Determine which data elements are most meaningful to CMS (e.g., therapeutic alternatives, remaining unmet medical need), both in general and specific to each therapeutic area; prioritize the written and verbal data collection for these critical data elements, and partner with relevant patient groups and other stakeholders to educate the patient community and collect the most meaningful input on these negotiation factors.

Not all data elements that can be informed by patients, caregivers, and health care providers will be equally important to CMS or have the same impact on the negotiation or maximum fair price calculation. Given the large number of products and indications CMS must consider, we strongly urge CMS to prioritize what insights will be most impactful and to be clear and transparent in communication and education to targeted stakeholders. We encourage CMS to clearly communicate how patient experience data informed the drug price negotiation and the final offer for each negotiated product.

b. Standardize the data collection efforts to ensure robustness and comparability across products and plan years while providing for sufficient flexibility to address the unique aspects of each product, therapeutic area, or patient population.

We encourage CMS to consider consistently asking questions specific to three thematic areas: 1. how the patient feels, functions and survives (on the treatment, an alternative treatment, or without any therapy); 2. cost and access; and 3. therapeutic alternatives. We recognize that these questions cannot be meaningfully answered in three minutes and appreciate CMS' flexibility to reconsider the session format. We also recognize that some of these questions may vary in pertinence based on therapeutic area, patient population, or other factors, and we encourage CMS to work with the relevant stakeholder community to prioritize and refine these questions as needed; however, we believe that this is a useful starting point for CMS' listening sessions.

Thematic area 1: How the patient feels, functions and survives:

- How does this disease impact you?
- How has your disease changed since you have been using this treatment?
- How long have you been using this treatment?
- What side effects have you been experiencing with this treatment?
- What formulation do you use for this treatment (if applicable)?
- Does this formulation best fit your needs?
- Rank the importance of the different characteristics of the treatment?
- What symptoms remain unresolved, and how is this impacting your day-to-day life?
- How does this product impact your social and emotional well-being?
- What would it mean to your daily life to no longer have access to the therapy?

Thematic area 2: *Cost and access:*

- Do you find this medication to be affordable? What does affordable mean to you?
- How much do you pay out of pocket annually for this medication?
- Did your insurance company make you try any other medications before agreeing to pay for this medication? If so, how many alternative medications were you required to try?
- Has this medication caused you any financial problems?
- Have you ever skipped a dose of this medication because you could not afford it?

- Have you ever skipped a dose of another medication because this medication was too expensive?
- What would cause you to stop taking this medication?
- Does your insurance company require prior approval before you fill this medication?

Thematic area 3: Therapeutic alternatives:

- What would you consider a therapeutic alternative to your current therapy? What characteristics make it a therapeutic alternative?
- Have you tried using any other medication to treat your condition? What has been your experience?
- How have you felt or functioned on the other therapy, and how does that differ from how you feel or function on the current therapy? Has that changed over time?
- Why did you switch / stop using that medication? Or why have you not tried other therapeutic options?
- How effective do you feel this other medication was compared to the medication you are using now?
- How does the price of your other medication compare to the medication you are using now?
- How were the side effects of the other medication compared to the medication you are using now?
- Did you find the other product(s) easier or harder to use than your current medication?
- What was your experience switching from one product to another?
- Would you consider switching products? Why or why not?

To further refine these questions, we encourage CMS to work with the impacted patient communities, as well as FDA and other stakeholders who have conducted successful patient engagement sessions to identify strategies best able to accomplish the goals of the patient listening sessions. FDA's Voice of the Patient Sessions are a crucial component of FDA's Patient Focused Drug Development (PFDD) sessions. To date, over 200 sessions have been completed on a wide variety of conditions, including both rare and non-rare conditions, and may be a strong resource to supplement listening sessions and focus groups, particularly for rare conditions where participants may be more challenging to source.

Part of FDA's success with the Voice of the Patient Sessions derives from individual modifications made to each session reflective of each of the diseases under consideration. We encourage CMS to individualize each of the patient listening sessions towards both the indications under consideration and the population involved in the sessions. Tailoring individual indications under consideration while adhering to a common structure will allow patients to speak directly to their own experience and provide valuable feedback on the product's value for patients in specific situations.

We urge CMS to further refine and revise these questions with input from key stakeholders for future plan years and to establish a process to consistently learn from and revise these questions with each subsequent plan year. We are particularly concerned about how CMS will select therapeutic alternatives for consideration. As CMS considers how to best identify therapeutic alternatives, it is crucial to solicit information from patients, caregivers, health care providers, and other key stakeholders on when, and when not, certain therapies can be identified as an alternative. We also want to raise two areas of concern regarding information gathering on therapeutic alternatives: prohibitions on medical switching and off-label use of products.

As the listening sessions last year clearly showed, for many diseases, particularly immune conditions and cancers, the use of a treatment can have significant impacts on the effectiveness of other treatments, because of, for instance, emerging tumor resistance or a secondary loss of response due to antibody formation to the drug.[21,22] This raises concerns about the prospect of therapeutic alternatives. Although there may be alternatives available on the market, if they are not available to the patient for medical reasons, they should be given consideration independent of therapeutic alternatives for other indications.

Due to the lack of treatment options available for so many rare diseases, both providers and patients frequently rely on prescription medications without an FDA-approved indication for their condition on the label, known as off-label use. Physicians frequently rely on clinical compendia to make decisions about whether treatment options would be appropriate for their patients. Off-label use accounts for up to one third of all prescriptions, and up to 97% in certain populations.[23] We recognize that CMS has indicated their intent to conduct literature reviews on therapeutic alternatives for each of the selected products in the past; to ensure all relevant therapeutic alternatives, we strongly recommend CMS consider including off-label uses of products

We thank CMS again for the opportunity to comment on this information collection request and look forward to working with CMS to ensure rare disease patients can fully participate in and benefit from the Medicare Drug Price Negotiation Program. For questions related to this letter, please contact Karin Hoelzer, Director of Policy and Regulatory Affairs at KHoelzer@rarediseases.org or Mason Barrett, Policy Analyst at MBarrett@rarediseases.org.

Sincerely,

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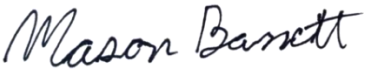
[21] *What to Do When Biologic Agents Are Not Working in Inflammatory Bowel Disease Patients.* Dalal, et. Al. National Library of Medicine. (October, 2015). https://www.ncbi.nlm.nih.gov/pmc/articles/PMC4849518/

[22] *Clone Wars: Quantitatively Understanding Cancer Drug Resistance.* Yates, et. Al. JCO Clinical Cancer Informatics. (28 October, 2020). https://ascopubs.org/doi/10.1200/CCI.20.00089

[23] *Off-Label Use vs Off-Label Marketing of Drugs.* Van Norman, Gail. National Library of Medicine. (27 February, 2023). https://www.ncbi.nlm.nih.gov/pmc/articles/PMC9998554/#:~:text=Off%2Dlabel%20use%20of%20drugs%20is%20common%2C%20constituting%20up%20to,off%2Dlabel%20use%20of%20drugs.



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