December 23rd, 2024

The Honorable Chiquita Brooks-LaSure
Administrator
Centers for Medicare & Medicaid Services
U.S. 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

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

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 individuals 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.

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 patient advocacy groups to provide detailed recommendations to CMS to ensure the perspectives of the patient community are appropriately represented in the listening sessions and the written submission

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

portal.[2] In our response to the July Information Collection Request (ICR) on the same topic, we identified three recommendations to improve the successful collection of information from patients, their caregivers, and health care providers:

1. Decoupling and simplifying the collection of patient experience data relevant to the 2027 MDPNP;
2. Leveraging the ICR and externally-led patient-listening sessions to complement data collection efforts; and
3. Pilot-testing the questions and engage patient engagement experts as well relevant government, academic, and private sector experts at every step of the data collection process.

NORD was pleased to see several of our recommendations incorporated into this iteration of the ICR, including a somewhat simplified process for submitting information, specific questions identified for patient response, and patient-friendly wording of suggestions. NORD believes the increased accessibility of the proposed documents will be crucial in identifying the experiences of users of the selected products.

NORD commends CMS' efforts to consider data on clinical benefit, therapeutic alternatives, and unmet medical need in the negotiation process and to incorporate relevant patient and provider perspectives. The Agency's stated objective to assess value in an indication-specific manner, including some off-label uses, is critical for CMS to fully understand and account for the complex treatment trade-offs and unmet needs that exist within the rare disease patient community. 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 and we are appreciative of CMS' efforts to seek comment from a variety of stakeholders from diverse backgrounds.

To supplement our prior comments, we offer the following recommendations specific to the Negotiation Data Elements and Drug Price Negotiation Process for Initial Price Applicability Year (IPAY) 2027:

1. Decouple the patient experience data section from the manufacturer-required input.
2. Simplify the access interface and reduce barriers to entry for patients who wish to submit information.
3. Conduct proactive outreach to pilot-test the questions and engage patient engagement experts, as well as relevant government, academic, and private sector experts at every step of the data collection process.

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[2] See: https://rarediseases.org/wp-content/uploads/2024/08/NORD-2024-ICR-on-Negotiation-Data-Elements-for-2027.pdf

Recommendation 1: Decouple the patient experience data section from the manufacturer-required input.

NORD strongly reiterates its recommendation CMS decouple the solicitation of patient experience data from the manufacturer-submitted information. While we are encouraged by CMS' efforts to simplify and clarify the patient experience specific information solicited, we remain concerned that the ICR, as currently structured, presents confusing and unnecessary barriers to participation. As we have noted previously, the collection of patient experience data is unique from the information collection process for manufacturers. The collection of patient experience data is neither subject to statutory data elements nor does it have to follow the strict timelines for manufacturer-provided data that prove to be difficult for most patients to navigate.

Decoupling the collection of patient data will allow for several improvements in patient access. First, it would allow for an extended timeline for data collection, beyond that of the statutorily mandated manufacturer information collection. A significant issue that we, and many others, identified as a part of the first round of the drug price negotiation process was the short timeline under which patients were required to provide information. This included the closing of the written submission portal before the conclusion of the listening sessions, which could have prevented willing patients from submitting information.

Currently, information in the ICR is required to be submitted no later than March 1, 2025. As the next set of selected drugs is not required to be announced until February 1, 2025, patients will potentially have a maximum of 29 days to respond to the proposed questions, assuming the written information collection is released at the same time as the selected drug list. This proposed timeline is woefully insufficient for achieving a representative sample of the utilizers of the selected drugs and does not allow for adequate time to gather input on how the selected drugs impact one's community, communicate with various stakeholders, and draft thoughtful written comments.

Second, decoupling the collection of patient data could allow for a dramatically simplified interface. While NORD appreciates the proposed disclaimers that individuals are not required to respond to every question, we remain concerned that the cumbersome questionnaire would discourage patients from submitting information, ultimately not garnering important data from the community. As currently proposed, the collection of patient experience data would begin on question 36 and run to question 42, with sub-questions underneath each major question header. Per NORD's estimation, the questionnaire would be 30 pages long, which could prove to be difficult for patients living with rare diseases and those with accessibility issues to digest. While we understand that when the questionnaire is published online it may appear shorter in length based on respondent answers, the length of the document regardless may be intimidating. Additionally, with the public-provided input beginning on question 36 (and all other publicly available questions beginning on question 29), we are concerned that patients may believe that they are missing a significant number of questions and avoid submitting.

Third, the instructions provided at the outset of the questionnaire are lengthy and written in language that is inaccessible to many patients. If patients cannot understand the instructions, they are less likely to be willing to provide information. NORD finds that providing patient-facing communications at no greater than an 8th grade reading level is best for ensuring understanding. Decoupling the patient experience data section from the rest of the questionnaire would allow for section-specific instructions tailored toward the respondents most likely to participate in each section.

To achieve the maximum impact from decoupling, we recommend the following:

- **Extend the timeline for accepting written submissions for non-statutorily mandated information indefinitely.**
    - An extended timeline for submission of information would increase the opportunity for patients to submit information. At the very least, keeping the written submission process open through the conclusion of the patient roundtables would be an improvement over last year's process.

- **Include section-specific questionnaire links in a publicly accessible location.**
    - We appreciate CMS' proposal to create a public-facing link on the CMS website, rather than having patients attempt to navigate directly to the Health Plan Management System (HPMS) portal. To ease access to the specific questions, we encourage CMS to create section-specific links to guide patients directly to the questions most relevant to them.

Recommendation 2: Simplify the access interface and reduce barriers to entry for patients who wish to submit information.

We appreciate the efforts CMS has made to improve accessibility for the written submission process. NORD is supportive of the creation of a distinct link from the CMS HPMS platform, patient-friendly language in questions 36-42, and specific questions asking about affordability and feel/function impact on patient lives. However, to be inclusive of the largest number of potential respondents, we recommend simplifying the steps for submitting information through the questionnaire and broadening the questions to be more inclusive of individuals with rare diseases. As proposed, the ICR includes several unnecessary barriers that could be resolved to promote participation in the written submission process amongst the largest number of stakeholders. Resolution of these barriers is paramount to ensuring patients can participate fully in the data submission process and will allow for a more fulsome and informed negotiation process. Currently as proposed, CMS will require participants to:

1. Click on the link to begin the written submission process.
2. Enter an email and submit the form.
3. Navigate to a follow-up email from CMS.
4. Follow the instructions contained in the email to begin the written submission.

These steps are unnecessarily complex and a barrier to participation, particularly for those who are not technologically sophisticated, have visual impairments, are utilizers of screen readers, or have cognitive impairments. Additionally, as CMS already proposes to require respondents to provide their email address as a part of question 28 "Respondent Information," the proposed process is duplicative. We understand that CMS likely has a need to certify that the respondents are indeed human, and that the system will not be overwhelmed by bad actors attempting to inappropriately influence the data submission. However, alternative methods, such as "invisible" CAPTCHA (which tracks user behaviors and prevents automated responses, or reCAPTCHA (which works well with screen readers), might prove to be more accessible.

As mentioned above, we are concerned about the complexity of the proposed questionnaire. If the ICR is unable to be decoupled from the mandatory manufacturer response, we strongly encourage CMS to identify solutions to limit the volume of the questions available and the volume of the instructions provided. Though we are appreciative of CMS' efforts to allow all stakeholders to respond to all available questions, the result is an overly expansive and ultimately unapproachable questionnaire.

To limit the volume of questions available, we recommend two options. First, a link that would take the respondent directly to the section most pertinent to them would help to reduce confusion. The link could be available directly on the CMS website, accompanied by a short write-up of what link is most relevant to what population. Second, after the user submits their response to question 28 indicating the description that is most relevant to them, they could be taken directly to the set of questions paired with the descriptor. Either of these steps could help cut down on confusion related to which set of questions a participant should respond to. To limit the volume of the instructions available, we encourage CMS to generate section-specific instructions, with language targeted at the potential respondents. For example, the language used to inform academic researchers on the types of information solicited and prohibited would likely be different than that used for patients and caregivers.

We are appreciative of CMS' proposal to provide a set of definitions for words that patients and caregivers may be unfamiliar with. To ensure patients and caregivers maximally benefit from the definitions, we encourage their maximal integration into the interface. While a set of definitions at the outset of the questionnaire is helpful, having the ability to highlight over a key unknown word or phrase (such as "indication" or "off-label use") and bring up the definition immediately would provide significant benefit. Alternatively, CMS could consider reposting the definitions at the start of each section, as it may be challenging for respondents to flip back and forth between the questions they are answering and the list of definitions at the outset.

Finally, we again encourage CMS to release information on how both the written submissions and the listening sessions impacted the IPAY 2026 negotiation process as soon as possible. As proposed, the release of information on how the testimony and data submitted by the public is set on the day that the written submission portal closes. We strongly encourage CMS to consider

releasing the justification for the negotiated prices prior to the conclusion of the solicitation of public input to allow respondents to understand what type of information is most relevant for CMS.

Expanding the breadth of the proposed questions to be inclusive of all potential respondents, including patients with rare diseases and non-traditional backgrounds, is crucial to ensuring the success of the written submission process. We are encouraged by CMS' proposed questions designed to solicit clinical outcome assessments, such as how a patient feels and functions on the medication and the inclusion of solicitation around off-label use for each of the selected products. However, while many of these questions work well for patients with more common diseases, those with rare diseases are more likely to struggle with a variety of different issues.

For example, many of the questions assume that the patient may have an alternative therapy available to treat their condition. For rare disease patients, however, there frequently are no pharmaceutical alternatives as over 95% of rare diseases lack a single FDA-approved treatment. As such, we encourage CMS to consider soliciting information on alternatives, such as surgeries or procedures, as well as to the extent that the selected drug helps to ameliorate an otherwise unmet medical need.

Further, we continue to urge CMS to monitor formulary placement for each of the negotiated products. While the negotiated products may carry a more affordable list price, we are concerned that plans could retaliate because of lower rebate amounts and place the negotiated products on formulary tiers that make the products more inaccessible to patients. To establish a baseline, we encourage you to add a question about current access and utilization management. This type of question could be phrased, "What barriers to access for your medication do you, or did you have? Were you required to try another, less expensive, therapy before being prescribed your current therapy? What is the co-pay for your therapy now? What formulary tier is your therapy on?"

Finally, NORD encourages CMS to consider how best to achieve parity in the types of questions asked and answers solicited between the roundtables and the written submission process. We believe that establishing a comparator between the roundtables with live participants and asynchronous responses provided in written format will be crucial in determining the wrap-around value of the selected therapy. To accomplish this goal, the questions asked in the roundtable sessions will need to be substantially similar to those asked as a part of this ICR.

To increase the accessibility of the written submission process, NORD urges CMS to:

- **Limit the number of steps required for a patient to access the written submission form.**
    - The more limited the access requirements, the better.

- **Limit the volume of patient-facing questions and simplify the patient interface.**

- Find ways to simplify the patient interface when they access the document. These may include section-specific links, automatically taking the respondent to the section that is most relevant to them based on selected criteria, and generating section-specific instructions.

- **Ensure that questions work for all patients, not just those with more common diseases.**
  - Consider tailoring questions to account for patients with rare diseases to include therapies beyond just pharmaceuticals. Also, consider monitoring formulary placement and access requirements for patients.

Recommendation 3: Pilot-test the questions and engage patient engagement experts as well as 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.[3] 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. 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 – 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.[4]

CMS itself has a long history of successfully engaging patients and families. Tools such as CMS' Person and Family engagement strategy have been instrumental in empowering patients and families to be meaningful partners in the design, delivery, and evaluation of their care.[5] 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

[3] Guidance for Industry: Expedited Programs for Serious Conditions - Drugs and Biologics. FDA Center for Drug Evaluation and Research and Center for Biologic Evaluation and Research. (2014, May). https://www.fda.gov/files/drugs/published/Expedited-Programs-for-Serious-Conditions-Drugs-and-Biologics.pdf

[4] Patient Engagement in Research: A Systematic Review. Domecq, et. Al. BMC Health Services Research. (2014, February). https://bmchealthservres.biomedcentral.com/articles/10.1186/1472-6963-14-89

[5] Quality Measurement and Quality Improvement. CMS Center for Medicare. (2024, September). https://www.cms.gov/Medicare/Quality-Initiatives-PatientAssessmentInstruments/QualityInitiativesGenInfo/Person-and-Family-Engagement

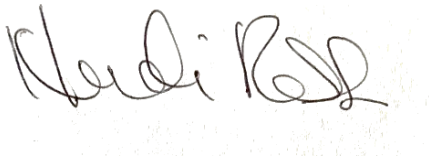
Patient-Centered Outcomes Research Institute (PCORI) and the Milken Institute's FasterCures Center to the Medical Device Innovation Consortium (MDIC) to a range of more disease-specific patient groups and many others will have meaningful advice to offer. Relying on this wealth of experience and tried-and-true 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 relevant government, private and non-profit sector experts**. This will help lay the foundation for a resilient and sustainable system to rigorously engage patients and leverage the best practices and approaches to maximize efficiency and the chance of success.

We thank CMS for the opportunity to comment on this ICR. With any questions, please contact Heidi Ross, Vice President of Policy and Regulatory Affairs, at hross@rarediseases.org or Mason Barrett, Policy Analyst, at mbarrett@rarediseases.org.

Sincerely,



Heidi Ross, MPH
Vice President, Policy and Regulatory Affairs
National Organization for Rare Disorders

Policy Analyst
National Organization for Rare Disorders