January 22, 2024

NORD Recommendations:
Future Medicare Drug Price Negotiation Program Patient and Provider Listening Sessions

NORD is grateful for CMS' commitment to bring the patient voice into the Medicare Drug Price Negotiation Program (MDPNP) and continuing to revise and refine the patient listening sessions. The recommendations outlined within this document are based on NORD's years of experience supporting patient focused drug development at the FDA, observations from the first year's set of MDPNP listening sessions and our previous discussions with CMS on this issue. A quick-reference summary of NORD's key opportunities and specific recommendations for future improvements can be found at the end of this document in Table 1.

NORD believes the first year of the patient listening sessions were a success. Standing up ten separate listening sessions for each selected drug under a very constrained timeline is a significant accomplishment. Moreover, NORD believes that the insights gained from patient testimonials will provide value in the determination of a fair price for each of the selected products. Given the strong foundation of the first year, NORD hopes that some incremental changes coupled with the best practices and lessons learned from this first year can be incorporated into future patient listening sessions to further optimize the experience for patients and support CMS in obtaining the data necessary to fulfill its statutory mandate.

Opportunity 1: Engage key patient organizations now and create patient friendly education materials

NORD recognizes that statutorily mandated timelines in the negotiation process present limitations to the extent of pre-work that CMS can do in advance of the listening sessions. However, early preparation is crucial to ensuring diverse representation of patient perspectives and appropriate understanding of what types of insights are most useful to the agency is crucial to continued success of the listening sessions. Recognizing CMS is months away from announcing the 15 drugs that will be selected for the second year of the program, there are a limited number of diseases and conditions likely to be represented among the drugs with high Medicare Part D spend. **NORD urges CMS to begin outreach to relevant umbrella patient groups now.**

Pre-work with umbrella patient groups that address disease areas across a wide scope provides a number of advantages. Early engagement with patient groups that cover disease areas such as

oncology, diabetes, and cardiology, all of which are likely to have a product selected in upcoming years, can help to ensure the perspective of the patient community is effectively incorporated in the listening session development process. Patient groups can be a sounding board for the development of disease specific education materials and outreach strategies, as patients across disease areas tend to benefit most from targeted outreach and engagement from trusted partners. Even if the patient group engaged does not represent a population of selected patients for this year specifically, learnings can be applied to future years of the MDPNP.

Soliciting participation from both traditional patient groups and more informal patient communities may provide the largest constituency from which to receive input. Outreach should entail a multi-modal approach, utilizing popular platforms such as WhatsApp, which NORD has found to be successful at reaching the middle aged and/or immigrant Latinx community members, and Facebook, which can be useful in connecting with informal patient groups.

- Where applicable, review FDA's condition-specific meeting reports related to patient experience to help identify relevant organizations, fill data/information gaps and inform the patient listening sessions.

- Utilize NORD's patient organization resources to help identify organizations for targeted outreach. We currently have more than 340 members, and our website has a searchable database with links to nearly 1,000 non-member rare (and non-rare) disease patient advocacy organizations.

Please continue to consider NORD a resource to help spread awareness and promote patient listening sessions with our relevant members and other relevant patient advocacy organizations.

Opportunity 2: Intentionally capture the diversity of patient perspectives

While undoubtedly the first year of the listening sessions provided valuable insight into patient perspectives about unmet medical needs, therapeutic alternatives, and the value of the selected therapy, NORD urges CMS to further strengthen efforts to intentionally increase diversity among patient perspectives as first year participants represented a relatively homogenous fraction of the impacted patient community.

NORD recommends using the registration process as a tool to intentionally enhance diversity in the patient perspectives represented. Ideally, the following information related to a patient or caregivers would be collected at registration:

- Demographic information (e.g., age, gender, race/ethnicity, zip code) – clarify that CMS is seeking both Medicare and non-Medicare eligible patient population representation
- Diagnosis
- Time since diagnosis

- What drugs they use for their disease, and since when
- Degree of disease progression
- Biggest challenges in accessing medication

NORD highly encourages the agency to place particular emphasis on engaging patients from historically underserved communities by working with organizations and health care providers that serve these communities. To foster trust within the patient community, consider ways that would allow patients the opportunity to participate 'anonymously', or have their identity limited to the staff members with whom they engage. Where applicable, ensure patients know their information will be de-identified and understand exactly how their data will be used. Also, be mindful of the unique needs and communication preferences of different patients and leverage trusted community messengers and multiple channels. For instance, we recommend:

- Promotional and registration materials be in English and Spanish (at minimum) to support non-English speaker patient and caregiver participation.
- Tailor the reading level to applicable patient communities. Based on prior experience, we recommend creating materials at or below an 8th grade reading level.
- Ensuring accessibility for patients with lack of broadband access, audio-visual and other physical or cognitive challenges through provision of a 1-800 number.

To increase provider participation, be explicit in soliciting their feedback and actively engage the provider networks that typically serve the affected patient communities. Provider networks are a great source of outreach for patients served by products with rare indications and may help solicit the valuable opinions of experts who work with the selected product frequently.

- NORD operates a Rare Disease Centers of Excellence network, with 40 academic medical centers and children's hospitals across the country that NORD can tap into to promote the listening sessions with providers, patients and caregivers.

Finally, NORD is concerned that the lack of a mandatory conflict of interest disclosure during last year's sign-up period may erode public trust in the data provided during the listening sessions and create real or perceived conflicts of interest. During the first year of the listening sessions, attention had been called to potential non-disclosed conflicts of interest. Moving forward, we recommend **providing a clear definition and mandatory reporting of conflicts of interest as an integral part of the registration process**, and to work with the patient communities to ensure the conflict of interest policies are appropriate and clearly understood by all participants.

Opportunity 3: Educate participants about what insights are most useful to CMS and utilize their experiences in year 1 of the program to make subsequent improvements

Moving forward, CMS should enhance understanding among the patient and provider community about what information from the listening sessions will be most useful in the

negotiation process, and how this information will be incorporated into the offers. The inaugural year of the program limited understanding of participants, which coupled with the limited allotted speaking time, resulted in non-standardized responses that we anticipate will be difficult to compare side by side and more challenging to consistently incorporate into the negotiation process.

The first year of the listening sessions included broad avenues through which patients could provide testimony. **Further specifying up front the questions that the agency finds most useful will help to narrow the scope of testimony and provide actionable information in the negotiation process**, as well as demonstrating a commitment to the patient community that they have been heard. Moreover, creating a safe and trusting environment, by setting clear and realistic expectations about participation in the listening sessions will be critical to ensure effective engagement of different parts of the patient community.

As mentioned above, describing the rules of engagement (e.g., how the data will be used and be specific as to whom the data will be shared with, to what extent individuals will be identified (or re-identifiable), what types of insights are most useful for the agency, how patients can expect to engage with each other and CMS before, during, and after the meeting, etc.) ahead of time will help to set realistic expectations.

To generate opportunities for the most meaningful engagement, CMS should consider transitioning to a more interactive session format such as focus groups. A more interactive session offers bi-directional benefit. For the agency, the opportunity to ask follow-up questions will allow for further clarification of aspects of testimony that are most valuable to the negotiation process, as well as helping to encourage a more standardized response format. For the patients, a dialogue-based format demonstrates the commitment of the agency to listening to patient voices and helps engender trust within the broader patient community, hopefully encouraging participation amongst broader segments of the impacted population in future iterations. While the number of participants in a more interactive session may be limited due to time constraints, the quality and granularity of the testimony and interaction can offer significant advantages.

If an interactive session is not feasible, NORD strongly recommends expanding the time for patients to testify and CMS being explicit in what information is most valuable for the agency in the negotiation process. While we understand that each of the sessions is intended to be operated under tight timelines, three minutes of testimony is insufficient to capture the breadth of an individual's experience with a selected product, in particular without very clear and tangible guidance for the type of information needed. Increased clarity on the types of insights the agency seeks to gain from the listening sessions, and providing adequate time to make these points, will result in more actionable responses and increased quality of engagement for both the agency and the patients offering testimony.

Based on NORD's experience with conducting a considerable number of patient listening sessions with FDA under our MOU, we saw that a critical mass was 6-8 patients for a 90-minute synchronous listening session. With these parameters, CMS will be able to obtain granular data from patients while allowing for a diversity of opinions. Alternatively, FDA has successfully used a 5-minute time limit for public listening sessions on narrowly defined questions. There clearly is an inverse relationship between the complexity of the question and the necessary time commitment. **NORD strongly urges CMS not to go below 5 minutes per patient**, and caution that more time may be needed to gain sufficient granularity in the responses.

CMS should also consider offering selected patients an opportunity to record their answers to the questions in advance, which can help ensure time allocations are adhered to, allow for subtitles to be added, and make participation potentially more equitable. Additional support for the patients to navigate the process may be needed, although organizations such as NORD can help patients overcome any of the technical challenges.

Finally, NORD recommends soliciting feedback from first year listening session observers and participants and fostering continued engagement in the negotiation process by patients and providers. This can be accomplished by CMS staff:

- Explicitly outlining the findings from CMS' patient listening sessions and how they were factored into the initial and subsequent offers. This will foster continued patient engagement in the process and be an important signal to the patient community that they are the central focus of the MDPNP.
- Surveying participants from this past year to identify what went well and where improvements could be made.
- Previewing plans to revise and refine patient listening sessions for MDPNP years 2 and later with community partners.

Opportunity 4: Make the written data submission process a truly complementary avenue for patients and providers to submit patient experience data to CMS

While the opportunity for patients and providers to provide written submissions is helpful in providing data supplemental to the live listening sessions, NORD is concerned that the process from the first year was complicated, lacked transparency, and closed before the conclusion of the listening sessions and therefore did not allow sufficient time for patients to react to or supplement the points raised during the listening sessions.

Ideally, a separate submission form for providers and patients, open to both participants of the listening sessions and non-participants, increases the opportunity for more directly tailored responses. In the event separate forms are not possible, creating a simplified written submission form focused on patient and provider experience, alongside providing accessibility focused

opportunities for patients with a lack of broadband access, audio-visual, and other physical and cognitive challenges will allow a larger subset of the impacted patient population to participate.

Additionally, NORD encourages the agency to keep the written submission form open throughout at least the duration of the listening sessions. Closing the form prematurely cuts off avenues for patients who participate in the listening sessions to submit vital supplemental information they may have forgotten about, or were unable to provide, during the live session. Moreover, patients who attended the listening sessions may be motivated to complement (or contradict) the statements from the listening sessions based on their lived experience. Keeping the submission form open may also inspire others to submit their testimonial, even if they were unable to participate in the live listening session.

Finally, **clearly demonstrating how the agency has used, and will use the submitted answers in the negotiation process and treat the identifying information of participants generates greater trust amongst the patient community.**

Table 1: Quick-reference summary of key opportunities and specific recommendations for future improvements

Opportunity	Specific Recommendations
1. Enhance patient engagement	• Engage relevant patient and provider groups now • Leverage multi-modal outreach strategy to meet patients via avenues that are most convenient for them
2. Increase diversity in patient testimonials	• Use the registration process to identify participants with diverse backgrounds and experiences • Conduct outreach to historically underserved patient communities through intentional partnerships • Define and mandate conflict of interest reporting
3. Increase usefulness of the provided data	• Transition to a dialogue-based listening session • Expand time for patients to speak • Be explicit in what information would be most useful the agency
4. Make the data submission process complementary to the listening sessions	• Shorten and simplify the written submission form • Keeping the written submission form open throughout the duration of the listening sessions

	• Be transparent about how patient data will be used (including specific examples from the first round of negotiations)

Again, NORD appreciates the ongoing dialogue with CMS officials on the listening sessions and the opportunity to provide recommendations on this important aspect of the Medicare Drug Price Negotiation Program. We look forward to continuing to work with CMS to ensure rare disease patients can fully participate in and benefit from the MDPNP. For questions related to this letter, please contact Mason Barrett, Policy Analyst at MBarrett@rarediseases.org, Karin Hoelzer, Director of Policy of Regulatory Affairs at KHoelzer@rarediseases.org, or Heidi Ross, Vice President of Policy and Regulatory Affairs at HRoss@rarediseases.org.

Sincerely,

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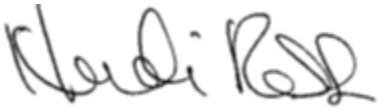

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