June 20, 2023

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

RE: CMS-10849

Dear Administrator Brooks-LaSure,

On behalf of the more than 25 million Americans living with one or more of the over 7,000 known rare diseases, the National Organization for Rare Disorders (NORD) thanks the Centers for Medicare and Medicaid Services (CMS) for their extensive engagement with the rare disease community around implementation of the Inflation Reduction Act (IRA). NORD appreciates this opportunity to provide comments on the information collection request (ICR) for the counteroffer component of the draft guidance 'Medicare Drug Price Negotiation Program: Initial Memorandum, Implementation of Sections 1191- 1198 of the Social Security Act for Initial Price Applicability Year 2026, and Solicitation of Comments,' hereafter referred to as the "Negotiation Program" guidance.

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 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 appreciates CMS' willingness in the Negotiation Program guidance (Section 30.1.1) to consider additional actions to "best support orphan drug development" and is pleased to submit the following comments to help CMS make good on its commitment to the rare disease community.[1] These comments are intended to supplement the April 14th comment letter submitted by NORD. As discussed in NORD's April 14th comments (linked here), successful implementation of the Negotiation Program hinges on CMS:

1. Ensuring rare disease patients have meaningful opportunities to submit patient experience data and provide valuable insights given the unique value of the negotiated products to rare disease patients, and the scarcity of published data specific to rare diseases;

[1] Meena Seshamani, Memorandum to Interested Parties: Medicare Drug Price Negotiation Program: Initial Memorandum, Implementation of Sections 1191 – 1198 of the Social Security Act for Initial Price Applicability Year 2026, and Solicitation of Comments, March 15, 2023, at 10- 11.

1779 MASSACHUSETTS AVENUE NW, SUITE 500
WASHINGTON, DC 20036
T 202-588-5700 ▪ F 202-588-5701

55 KENOSIA AVENUE
DANBURY, CT 06810
T 203-744-0100 ▪ F 203-263-9938

1900 CROWN COLONY DRIVE, SUITE 310
QUINCY, MA 02169
T 617-249-7300 ▪ F 617-249-7301

rarediseases.org ▪ orphan@rarediseases.org

2. Ensuring rare disease patients have access to the negotiated therapies through appropriate formulary placement and limited or no utilization management barriers;

3. Further clarity around the implementation of the orphan drug exclusion to ensure orphan products remain excluded from negotiation until the product is FDA-approved for a second disease; and

4. Tracking the impact of IRA implementation immediately.

NORD thanks the agency for recognizing, in Section 60 of the draft guidance, the value that orphan drugs can provide to specific parts of a negotiated product's patient population.[2] NORD also appreciates CMS' objective to assess value in an indication specific manner, allowing the full range of patient values for a product to be captured. However, as outlined in our April 14th comments, NORD is concerned that CMS' proposed approach to the Negotiation Program would not allow patients to submit patient experience data, including missing, but critical, information on patient perspectives regarding unmet needs, clinical benefit, and therapeutic alternatives, in a meaningful manner.

Specific to the counteroffer process, NORD urges CMS to ensure rare disease patient perspectives are appropriately considered in the process by:

a. **Ensuring CMS has access to the relevant, high quality patient experience data as the agency is crafting the initial offer and during every step of the negotiation process; this means ensuring that the data submission process works for the impacted patient communities, proactively collecting patient experience data as needed, and allowing for meaningful patient comments during every step of the negotiation process. Specifically, we urge CMS to:**

- Make it easier for patients to participate in the data submission process. Increased accessibility measures could include interfacing with leaders of the appropriate disease specific community, accommodations for individuals with impairments, and clear instructions with applicability criteria to ensure information is being appropriately and adequately sourced.

- Clarify the information the agency is seeking from patients and the timeline on which the Agency seeks to receive such information to ensure a rigorous, standardized approach to data collection. Given the short timelines for collecting data prior to negotiations, the submission process and expectations should be clarified well in advance so that community leaders may have time to appropriately aggregate patient stories and respond in a meaningful manner.

- Use the FDA's experience with patient listening sessions to organize similar meetings to inform patient voice discussions on negotiated drugs. For example, FDA's Voice of the Patient reports help to capture the patient perspective of a drug by soliciting information about therapeutic alternatives, unmet need, and clinical benefit of alternatives. Patient submissions lacking standardization run the risk of becoming a series of anecdotes, rather than concrete and actionable information. NORD recommends utilizing FDA's "Patient Focused Drug Development: Collecting Comprehensive and

[2] Meena Seshamani, Memorandum to Interested Parties: Medicare Drug Price Negotiation Program: Initial Memorandum, Implementation of Sections 1191 – 1198 of the Social Security Act for Initial Price Applicability Year 2026, and Solicitation of Comments, March 15, 2023, at 10- 11.

Representative Input" guidance as a framework for successful data collection and management practices. By increasing the standardization of submissions, the agency will ensure that patient time spent responding to the comprehensive data submission will be useful in informing the initial offer and counteroffer process for products selected for negotiation.

- Additional submission periods during the negotiation process could also increase the opportunity for the community to submit valuable information. To ensure patient input is given the due consideration it deserves, NORD recommends extending public comment periods and allowing patients to submit additional information during the counteroffer process. As the submission process is voluntary, and not subject to the statutory data submission timeline and contents for manufacturer provided data, CMS should work with the patient community to determine timelines that would be feasible to submit relevant supporting information for both the initial offer and counteroffer processes.

- Furthermore, allowing patient stakeholder comments at subsequent steps of the submission process including during the counteroffer process will allow the patient community to react to CMS' initial proposal and provide supplemental data as appropriate.

b. **Establish a process for engaging the patient community in the counteroffer process.** CMS' proposed approach does not include the patient community in the counteroffer progress at all and, in fact, severely restricts what information manufacturers are allowed to share about the negotiation process, making it almost impossible for the patient community to meaningfully contribute to the counteroffer process. NORD strongly urges CMS to engage patients in the counteroffer process. For instance, patients can, under certain circumstances, join confidential meetings between the Food and Drug Administration and drug sponsors to provide critical insights on patient perspectives during these regulatory meetings. In fact, countless drug development programs have benefited from the inclusion of patients at critical junctures of the regulatory interactions, and NORD urges CMS to work with FDA, patient groups, and manufacturers to develop an analogous process for the counteroffer process.

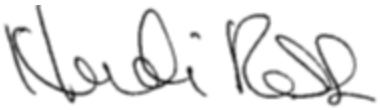
c. **During the negotiation process, clearly and transparently communicate to manufacturers and other key stakeholders how patient experience data was incorporated in developing the initial and subsequent offers.** To support robust, meaningful patient engagement, and to ensure relevant orphan-indication specific data is being used to support the Negotiation Program from the initial offers throughout the negotiation process, NORD recommends all initial and subsequent offers include a detailed outline of the data sources utilized to establish an initial offer including where and how patient experience data was used to arrive at the initial (and subsequent) offer. NORD recognizes that initial (and subsequent) offers may be kept confidential. However, transparency for everyone privy to the offer on the factors and decisions made in establishing an initial offer and counteroffer(s) will allow patients to meaningfully provide insights on specific community perspectives to the negotiation process and increase the efficiency and effectiveness of the negotiation process.

d. **Upon completion of the negotiation process, CMS should provide public, granular summaries of the data and assumptions on which the final offer or negotiated price was based, including what patient experience data was used and how; this will help create consistency and**

trust in the negotiation process. A negotiation process lacking specific and granular summaries of patient value and data sources utilized could be perceived as lacking validity. Additionally, processes to establish patient-specific input and data standardization will allow CMS to specifically articulate the value that negotiated drugs have to the patient community.

We again thank CMS again for the opportunity to comment and look forward to working with CMS to ensure rare disease patients can fully participate in and benefit from the Negotiation Program. For questions related to this letter, please contact Heidi Ross, Vice President of Policy and Regulatory Affairs (HRoss@rarediseases.org) or Karin Hoelzer, Director of Policy and Regulatory Affairs (KHoelzer@rarediseases.org).

Sincerely,



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

Karin Hoelzer, DVM, PhD
Director, Policy and Regulatory Affairs
National Organization for Rare Disorders