



April 3, 2017

Institute for Clinical and Economic Review  
Two Liberty Square  
Ninth Floor  
Boston, MA 02109

**Re: ICER's Patient Participation Guide**

Dear Dr. Pearson:

On behalf of the 30 million Americans with one of the nearly 7,000 known rare diseases, NORD thanks the Institute for Clinical and Economic Review (ICER) for the opportunity to provide comments on the Institute's "Overview of the ICER value framework and proposals for an update for 2017-2018".

NORD is a unique federation of voluntary health organizations dedicated to helping people with rare "orphan" diseases and assisting the organizations that serve them. NORD is committed to the identification, treatment, and cure of rare disorders through programs of education, advocacy, research, and patient services.

We are committed to fostering an ecosystem that encourages the development and accessibility of safe and effective therapies for rare disease patients. We are excited by the advent of value frameworks, and believe that value frameworks, if developed collaboratively and used responsibly, can provide objective analysis for assessing the value of therapeutic interventions.

People with rare diseases have a uniquely important role to play in value framework development. Almost every input into a value framework involves the personal experiences of people with rare diseases and their families. With patient-centricity fueling innovations in rare disease research and drug development, it is critical that any and all value frameworks place the patient at the center of its creation.

It is for these reasons that we are pleased to provide comments on ICER's "Patient Participation Guide". We also commented on ICER's "Overview of the ICER value framework and proposals for an update for 2017-2018" and the September 2016 call for comments on the "Proposed Improvements to its Value Assessment Framework".

Overall, we are pleased with ICER's proposals for patient participation. The opportunities are comprehensive, and ICER is again clearly reiterating its commitment to including patients in the value assessment process. This being said, targeted improvements to ICER's patient participation practices should be adopted to better include patient viewpoints.

First, ensuring patient representation on the independent expert committees is critical to ensuring the deliberations and decisions of these committees are inclusive of patient viewpoints. At the submission of

these comments, it appears only a handful of patients or patient representatives are included in the voting membership of these Committees. Setting standards for patient representation on these committees should be pursued to ensure each includes adequate representation.

Second, the comment periods are still far too short for many patient organizations to participate, particularly rare disease patient organizations. Of NORD's over 260 members, approximately 70 percent have fewer than five full time staff members. With the expanding opportunities for patient organizations to contribute extensive non-RCT data, nuanced analyses of patient subgroups, and more, it is important that organizations have the required time to complete these studies.

Additional time is also needed for stage 3 during which patient organizations could be expected to digest hundreds of pages of analysis and respond within 4 weeks.

Understanding ICER's need for expedient reviews, perhaps ICER can integrate flexible comment period times based upon the patient and patient organization immediate capabilities to respond. In addition, ICER could offer technical and logistical support to patient organizations hoping to contribute.

Third, in addition to comment periods being too short, the allowable length of comment documents is also too short, particularly for patient organizations. If patient organizations wish to submit detailed analysis of patient subgroups or patient experiences, three to five pages is far too short. ICER should explore expanding the allowable length for comments for patient organizations in particular.

Fourth, ICER should not expect all patient organizations in the relevant space to be following ICER's work, and proactively reaching out to ICER to contribute. Instead, ICER should develop a plan to proactively reach out to patient organizations to ensure their participation. ICER could also work with larger patient groups, such as NORD, to assist in the process of finding patient organizations and patients to contribute. The FDA's Patient Representative Program works similarly. If the FDA does not have a patient with a particular background or expertise desired, they reach out to large patient organizations such as NORD to facilitate the search.

Finally, in regards to the public meeting, several improvements can be made to better include patient viewpoints. A five-minute time allotment is appropriate for oral comments, but ICER should give speakers the opportunity to expand on their comments in written form, similar to Congressional committees. ICER should also be more transparent on how it will choose a "patient representative to answer questions". Patients with the same disease can have vastly different experiences, so the choice of patient to participate could be impactful to the proceedings. Choosing one patient amongst various subpopulations may also prove challenging.

In addition, ICER should allow patients to participate from afar, much like the FDA facilitates for Patient-Focused Drug Development meetings. Patients with severe conditions will likely be unable to travel to the meeting to attend in person. By excluding these patients from participating, ICER will skew the viewpoints presented towards the healthier and abler patients. ICER will also skew participation towards individuals with the financial means to miss work and travel to the meeting, thus excluding lower-income patients. By allowing patients to participate in the open public comment period virtually, ICER will alleviate many of these problems.

We thank ICER for the opportunity to comment, and we look forward to working with ICER to accurately and collaboratively assess the values of therapeutic interventions. For questions regarding NORD or the above comments, please contact me at [pmelmeyer@rarediseases.org](mailto:pmelmeyer@rarediseases.org) or (202) 545-3828.

Thank you in advance for your consideration.

Sincerely,

A handwritten signature in black ink, appearing to read 'P. Melmeyer', with a long horizontal flourish extending to the right.

Paul Melmeyer  
Director of Federal Policy