



April 3, 2017

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

Re: ICER Overview of the ICER value framework and proposals for an update for 2017-2018

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 "Overview of the ICER value framework and proposals for an update for 2017-2018". We also commented on ICER's September 2016 call for comments on the "Proposed Improvements to its Value Assessment Framework".

First, we understand that the proposed framework may not apply to ultra-orphan drugs as "there are reasons to consider the possibility of modifications to the evaluation of strength of evidence and value given the unique features of the development and evaluation of ultra-orphan drugs, as well as the size of the potential patient population for such therapies." We are supportive of ICER's nuanced approach to ultra-orphan drugs. However, it is unclear what ICER regards as an ultra-orphan drug. There is no formal definition for ultra-orphan drugs, and we are wary of an arbitrary stratification of rare disease populations.

Moreover, we are quite disappointed to learn that “ICER is working with stakeholders to develop specific guidance on how the ICER value framework methods will be adapted for ultra-orphan drugs”, yet we have not been invited to participate. We are thankful for ICER’s inclusion of our perspective in its prior deliberations, including a September 2016 stakeholder meeting, but are puzzled as to why we are currently excluded from ICER’s evaluation of ultra-orphan drugs.

We hope that ICER further clarifies its intents on examining the valuation of ultra-orphan drugs, and we hope to be included as intimately as feasible in this endeavor.

Improvements in the Value Assessment Framework:

After examining the remainder of the proposed changes, we are pleased to see that many of our recommendations, as well as recommendations from other patient advocacy organizations, are accepted and integrated into ICER’s updated framework. ICER further integrates patient perspectives and participation into the framework development process. Patients may now participate on the policy roundtable at the public meetings. Data collected by patient organizations outside of randomized-controlled trials (RCTs) can be integrated into the review, and are given the appropriate consideration when assessing a therapy’s value. Patient organizations are also fully consulted and collaborated with throughout the value assessment process.

We are also pleased to see further elucidation of the “other benefits or disadvantages” category as well as the “contextual considerations” category. The ten elements described for consideration are exhaustive, and we are confident that the true value of a therapy can be adequately estimated if thorough consideration of each of these categories is included.

Other areas of ICER’s proposed changes are also steps in the right direction. ICER recognizes the heterogeneity of patient populations and potential treatment effects across patients and patient subgroups. By pursuing a nuanced approach, ICER will better capture the true value of a therapy across a patient population.

In section 3.4, ICER proposes to “perform scenario analyses that examine the influence of lower utilities for individuals with chronic severe conditions on cost per QALY findings.” This is incredibly important. Cost-effectiveness studies that use QALYs too often undervalue improved health and wellbeing in patients with particularly severe illnesses. Many rare diseases are incredibly severe, often leading to disabling morbidities and accelerated mortality. ICER’s strategy of seeking patient input and public comment to better align the analysis with the specific disease will be important in correcting for these analytical biases.

Section 3.5 adds an additional important distinction to the analysis by no longer relying on the inaccurate wholesale acquisition cost (wac) and instead estimating the price of a drug following discounts, rebates, and other price concessions.

While we are pleased with the improvements proposed for the framework, we still have concerns with other aspects of the proposal. We also have recommendations for how ICER can improve upon the proposed framework to further benefit rare disease patients.

Budgetary Impact Assessments

We appreciate ICER’s recognition of stakeholder concerns regarding the emphasis on short-term budget effects. To quote from our September 2016 comments,

“We join many in the patient community in finding ICER’s prioritization of short-term budget impacts troubling. It is our understanding that ICER assesses therapies within the short-term budget impact of one-year because this is the window of time in which payers assess their actuarial soundness, set their premiums, and structure their benefit design. While we understand the need to be useful to payers by fitting within their schedule, it should not come at the cost of accurate valuation of therapies.

In addition, using a five-year time window for “long-term budget impacts” will also substantially devalue various therapies. For example, we are on the cusp of a medical breakthrough in gene therapy and gene editing technology. We will likely see within the next decade the availability of cures for previous incurable genetic disorders.

If ICER only values these therapies over a five-year window, ICER will ignore years, perhaps even decades, of vastly improved quality-of-life for these patients. ICER should craft its time windows to specifically reflect the therapy and disease it is treating instead of adopting a one size fits all approach.”

We again understand the need for inclusion of short-term budgetary impact analysis, but we request that longer-term analysis be included in the final report. The benefits of innovative curative therapies, such as gene therapy and gene editing technologies, could be woefully undervalued if budgetary impacts are only assessed through a five-year time frame. We do not believe that longer-term budgetary analysis supplants the one year or five year assessments, but we hope this analysis can be included to afford the reader a fuller picture of the financial value of a therapy.

We, too, believe that eliminating short-term budgetary impact considerations in the report would be a mistake, but we ask that additional financial analysis be offered in order to provide greater benefit to non-payers. Currently, the one-year analysis is most useful to insurers, but perhaps not quite as useful to patients. We hope ICER explores additional ways to broaden the reports’ audiences by including other financial analysis relevant to patients and other stakeholders.

Quantifying “Other Benefits or Disadvantages” and “Contextual Considerations”

We support the attempt to quantify the “other benefits or disadvantages” and “contextual considerations” categories. We understand that many stakeholders support using qualitative measures for these categories, but we are concerned this could leave readers with the impression that these categories do not hold the same importance as the quantified outcomes.

We do believe this proposal can be improved. First, additional explanation and rationale behind this specific system rather than alternatives would be helpful. Second, under section 4.4, the final score developed by the committee will be included in the final report, but with the added caveat that it will only represent one committee’s perspective. We understand the rationale for this distinction, but we ask

that the final report does not de-emphasize the importance of these findings, and instead highlights the equal importance of these valuations compared to the clinical cost per QALY.

In addition, this methodology is rather complex and difficult to envision in practice. Publishing an illustrative example of this tool in practice would be helpful.

Collection of Non-RCT Data

We are thankful for ICER's recognition of the importance of patient-generated data, and with this recognition, we see an opportunity for partnerships between ICER and patient organizations. ICER and its academic colleagues have the expertise on non-RCT generation, and patient organizations have the patients ready and willing to participate.

If ICER is able to forge partnerships with patient organizations for the collection of these data, it will dramatically improve the quality of the assessments. This will more accurately evaluate WTP, quality-of-life improvements, and more.

This could be particularly important for assessments of orphan therapies where the patient organization representing that disease is short-staffed and not as financially endowed as larger patient organizations. These organizations may only have a handful of staff members, and without assistance, may not be able to participate in ICER's processes.

We understand similar collaborations have already taken place with patient organizations. We hope this can be standardized and made available to all patient organizations wishing to participate.

Finally, we have additional comments on patient and patient organization engagement, but we will save those remarks for our comments on the "Patient Participation Guide".

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 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