



September 12, 2016

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

**Re: ICER National Call for Proposed Improvements to its Value Assessment Framework**

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 proposed "Value Framework Assessment 2.0".

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 "Proposed Improvements to its Value Assessment Framework". The below comments are organized to reflect ICER's prioritized areas for improvement.

**1. Methods to integrate patient and clinician perspectives on the value of interventions that might not be adequately reflected in the scientific literature, elements of value intended to fall in the current value framework within "additional benefits or disadvantages" and "contextual considerations"**

We commend ICER for the stated goal of appropriately integrating patient perspectives on the value of interventions. While laudable, we are unconvinced the proposed practices will achieve this goal.

First, due to the inherently small and dispersed patient populations common in rare diseases, there is often a dearth of scientific literature or understanding in the disease. The vast majority of rare diseases are rarely researched or given any attention by the scientific and medical professions. It is for these reasons that if ICER is looking to integrate existing publications for rare diseases into their value assessment, they will likely come up empty.

Even when there is current scientific literature available, it is often outdated, or archaic in its formulation. We are only now starting to conduct scientific and medical research in partnership and collaboration with the patient. For ICER to rely on existing scientific literature without assessing its patient-centricity, ICER will be departing from its stated patient-focused goal.

We understand this may limit ICER's ability to use existing sources, perhaps precluding their use entirely. This underscores even further the importance of partnering with patients and patient organizations in assessing "additional benefits or disadvantages" and "contextual considerations".

In doing so, ICER must not make the same mistakes many others have already made. ICER cannot rely on one or two patients to represent the entire patient population with the disease. ICER cannot rely on one or two researchers or physicians who treat the disease.

ICER must not even rely on one patient organization to collaborate with. People with rare diseases who have a patient organization representing them are actually quite fortunate, as most rare diseases have no representative organization. ICER must consider this, and work to collaborate with the existing networks of patients in place if no established organization exists. But some rare diseases are fortunate to have multiple organizations representing the population. It is critical to include all viewpoints, perspectives, and opinion across the patient, physician, and patient organization landscape.

ICER must also give patient organizations the required amount of time to appropriately participate. It is our understanding that ICER's public comment period for various documents generally are allotted three to four weeks. This is entirely inadequate, particularly for rare disease patient organizations. NORD has over 250 rare disease patient organizations as members. Over 70 percent of our members have fewer than five full-time employees (FTEs) conducting the entirety of the organization's operations. To require these organizations to comment on a lengthy and incredibly esoteric economic analysis in a matter of weeks is absurd.

If ICER is truly committed to collaboration with patients and their organizations, they will recognize the realities rare disease patient organizations operate under.

Finally, we understand that "a comprehensive Patient Participation Manual is under development". It is our understanding that ICER is partnering with one patient representative in the development of this manual. While we thank ICER for the intent of this effort, ICER is committing one of the errors we are concerned about: using one person as the sole representative of the entire patient and patient organization community. It is not enough to request feedback from the patient community on the patient engagement manual once completed. The patient community must be involved in its development from its inception, just as if it was a value framework.

## **2. Incremental cost-effectiveness ratios: appropriate thresholds, best practice in capturing health outcomes through the QALY or other measures**

The use of incremental cost-effectiveness ratios using QALYs has become a standard practice in the health economics and value framework field. However, we are concerned these methods used within ICER's framework could inaccurately assess the therapy's value for the patient population.

Similar to the problems with using existing scientific literature (or lack thereof), the willingness-to-pay (WTP) values used to evaluate quality-of-life improvements or declines, in many, if not most, existing assessments were not developed in coordination with patients. As discussed in ICER's publication titled "Addressing the Myths About ICER and Value Assessment", the "QALY was developed by health economists and doctors..." Notably absent are the patients.

ICER later adds that it "select(s) quality of life scores whenever possible from individuals who have the condition rather than asking people without the condition to judge 'how bad' it would be to have that disease". While this is certainly preferred, it is incredibly unlikely that existing WTP valuations exist for individuals with most rare diseases let alone subpopulations within those diseases. It is not enough to include patient-generated data only when available.

For ICER to craft truly representative incremental cost-effectiveness ratios, they must do several things in collaboration with patients and patient organizations. ICER must survey a representative sample of patients with the disease across the entire disease progression and spectrum on their WTPs for quality-of-life improvements or declines. ICER must also craft a disease-specific and subpopulation-specific assessment of the baseline quality-of-life assessment for each subpopulation. Simply extrapolating existing analyses on the quality-of-life from other diseases or symptom estimates would result in fallacious findings. ICER must also include the WTPs of families and caregivers for each specific subpopulation.

Again, partnering with patients and their organizations is critical to overcoming these hurdles.

## **3. Methods to estimate the market uptake and "potential" short-term budget impact of new interventions as part of judging whether the introduction of a new intervention may raise affordability concerns without heightened medical management, lower prices, or other measures.**

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 it's time windows to specifically reflect the therapy and disease it is treating instead of adopting a one size fits all approach.

**4. Methods to set a threshold for potential short-term budget impact that can serve as a useful “alarm bell” for policymakers to signal consideration of whether affordability may need to be addressed through various measures in order to improve the impact of new interventions on overall health system value.**

Here again we are concerned with ICER's approach. ICER again appears to be prioritizing short-term budgetary impacts through the lens of the insurer. Arbitrary thresholds should not be used for incredibly case-specific analyses. We also agree with the National Health Council in suggesting “ICER move(s) away from the terminology ‘alarm bell,’ which might incite knee-jerk reactions that lead to inappropriate access restrictions and other unintended consequences”.

Overall, we implore ICER to be cognizant of the responsibility they bear in crafting these value frameworks. While ICER's motives may indeed be patient-centric, their work can very easily be used in anti-patient ways. We believe it is ICER's duty to take responsibility for how their analyses can be used, and do everything in its power to responsibly and collaboratively craft an unbiased publication.

We are unsympathetic to defenses of ignorance or time constraints for publishing analyses that misrepresent the value of therapies and lead to limited access to patients. For ICER to succeed, we expect them to carefully, thoughtfully, and collaboratively assess the value of therapies no matter the time and resource investment it takes.

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 [mrinker@rarediseases.org](mailto:mrinker@rarediseases.org) or (202) 588-5700, ext. 102.

Thank you in advance for your consideration.

Sincerely,

A handwritten signature in blue ink, appearing to read "Martha Rinker".

Martha Rinker, J.D.  
Vice President, Public Policy