

Considerations for Submitting Comments to the ICER Assessment of Eteplirsen or Golodirsen: A Tip Sheet for Duchenne Community Members

Background:

Thanks to decades of funding and innovation, our healthcare landscape is rapidly changing. As new therapies emerge into the marketplace, patients, providers, and payers are faced with challenges as to how to discern which therapies are most appropriate for which patients in which combinations and which time. In order to facilitate this decision-making, an attempt to define the ‘value’ of specific treatments is made.

An entire sector exists around this definition of ‘value’ and – in the United States – value assessments for neurological products are led by the Institute for Clinical and Economic Review (ICER). **PPMD has long been engaging with ICER and will work to ensure that our community and all relevant stakeholders are included within this review process.** By ensuring your voice is included in this review – more specifically your experience with eteplirsen or golodirsen – it is our belief that ICER’s conclusions will more accurately reflect our community’s overall experience.

What is ICER?

ICER is an independent and non-partisan research organization that evaluates the clinical and economic value of prescription drugs, medical tests, and other health care delivery innovations by conducting rigorous analyses of clinical data. The ICER assessment includes engagement with key stakeholders at specific time-points including opportunities to submit public comments, as well as an opportunity for those with direct experience with the products to participate in a Public Meeting. The review also includes the collection of clinical data and extensive economic modeling. ICER then develops an evidence report around their findings.

ICER’s drug assessment reports include a full analysis of how well each new drug works, the economic value each treatment represents, and other elements of value that are deemed to be important to patients and their families. The reports use these analyses to establish a “value-based price benchmark” reflecting how each drug should be priced to appropriately reflect long-term improved patient outcomes. The ICER Reports also evaluate the potential short-term budget impact of new drugs to alert policymakers to situations when short-term costs may strain health system budgets and lead to restrictions on patient access.

Community Participation in the ICER Assessment:

One of the criticisms of ICER assessments is that they fail to take real-life patient preferences and socio-economic considerations into account within their modeling. Participation in the **Open Input Period** is a critical way for ICER to gain an understanding of the Duchenne community, the significant unmet medical need within our community, the costs incurred by families, and the values being derived by those utilizing the products ICER's evaluating that may not be evident in existing data. **Your participation in this stage of the assessment is critical.**

Step 1 of the ICER Review — Open Input Period: 12/13 – 1/8

The first part of the ICER Review process has begun and is known as the **Open Input Period**. Extending from **December 13 through January 8**, this is a critical part of the process in which the clinical and patient community is invited to submit information directly to ICER about your direct experiences with the products under review. Please note that it is the testimony of individuals with direct experience with these products that will be most impactful to the process. These submissions will help ICER to gather information on the various benefits derived from the products, as well as the patient's personal experience which they will need to account for throughout their review. In addition to the information below, please visit ICER's website <https://icer-review.org/patient-guide-to-open-input/>.

Below are points to consider including when crafting your comments for ICER:

- 1. Your child's specific mutation.** It is critical that we relay to ICER that the therapies being evaluated have been designed for specific subsets/mutations within our Duchenne community and that not all patients with Duchenne are amenable to treatment with these therapies.
- 2. If your child is on a steroid,** the particular steroid (prednisone, Emflaza, or non-commercial deflazacort) and consistency of dosing regimen is important information.
- 3. The significant impact that Duchenne has on your child's quality of life.** Quality of life impact plays a significant role in the ICER assessment of a product's value. If you observed changes in your child's quality of life due to the use of eteplirsen or golodirsen, that information is important for the reviewers to understand.

Factors to consider may include:

- Impact on ability to participate in school day or school activities.
- Impact on school attendance. Does your child miss fewer days of school since being on the treatment? (Do not include time missed from school for clinical trial participation here.)
- Impact on ability to play with neighbors or friends in the community.
- Stamina or impact on overall level of fatigue.
- Impact on behavior, emotion, or mental health.
- Whether use of any prescribed durable medical equipment has changed since being on the product.

4. The significant impact that Duchenne has on your family's financial considerations. ICER will be conducting 'economic modeling' to understand how these interventions factor in the overall cost of living with Duchenne. To that end, it is critical that considerations around the economic impact on a family when you have a child with Duchenne are well understood by the evaluators. This should take into account everything since the diagnosis of Duchenne. Factors to consider including in your comment include:

- Since your child was diagnosed with Duchenne, have you moved or made modifications to your home due to accessibility?
- Have you or family members left the workplace or made job changes in order to be available for caregiving?
- Have you paid out of pocket for a new vehicle – or made modifications to an existing vehicle - in order accommodate for your child's diagnosis? What financial impact has the increased insurance and gas charges had on your expenses?
- Do you routinely travel for medical appointments or clinical care?
- Do you pay out-of-pocket for caregivers for siblings when you are tending to the medical, clinical trial, or educational needs of your child with Duchenne?

5. The significant impact that Duchenne has on your family's quality of life of family caregivers and siblings. Factors to consider including in your comment include:

- Impact on daily routine and any changes in the level of need for caregiver support. For example: the use of durable medical equipment, changes in independence throughout the day or at night, changes to pulmonary routines, etc.

- Impact that administration of therapy has on family.
- Impact on interactions/participation with siblings or friends.
- Impact on behavior, emotion, or mental health of siblings or other family members.

6. In your own words, any real-life access challenges that have impacted your child's ability to derive ultimate benefit from the product. Any relevant details that might be important for the reviewers to understand around:

- Prescribing challenges.
- Insurance hurdles or delays.
- Disruptions in treatment access.
- Administration challenges that may have changed throughout your experience with the product.

The information collected from this stage of the process will be used to inform the Draft Scoping Document that will be published on January 11.

The ICER Assessment Timeline – Additional Key Engagement Dates for Community Members with Experience with Either Eteplirsen or Golodirsen

Step 2 – Public Comment on Draft Scoping Document: 1/11 – 2/1

On January 11, ICER will post their 'Draft Scoping Document'. This initiates a public comment period that extends through February 1.

We will post more details on commenting on the scoping document after it is released. The comments received will inform the Final Scoping Document, posted February 11.

Step 3 – Draft Evidence Report Posted – Public Comment Period: 5/22- 6/18

The Draft Evidence Report (or 'final draft', if you will) is scheduled to be posted on May 22. This initiates the public comment period on the final draft, with the Revised Evidence Report (reflecting any changes made based on public input) posted on July 11.

Step 4 – Public Meeting, July 25 (more details to come)