

Looming Challenges for ICER in Assessing the Value of Rare Disease Therapies



The Institute for Clinical and Economic Review (ICER) and the Quality Adjusted Life Years (QALY) approach to value assessment is particularly ill-suited to assess the cost effectiveness of orphan and rare disease treatments, which represent a growing sector of the biopharmaceutical marketplace.

Why ICER is Unfit to Evaluate the Value of Rare Disease Treatments

- ✓ **ICER Thresholds are Not Appropriate for Rare Disease Drugs**
- ✓ **The ICER Definition of “Ultra-Rare Disease” is Arbitrary**
- ✓ **Clinical Trial Data is Too Limited to Evaluate the Value of Rare Disease Drugs**
- ✓ **ICER’s “One-Size-Fits-All” Approach Has Failed to Adapt to Precision Medicine**

ICER itself has acknowledged the challenges that exist in evaluating the growing number of drugs for complex and rare diseases and has unsuccessfully attempted to revise its framework to meet the unique challenges associated with this segment of medicine.

Between 2014-2018, none of ICER’s reviews of rare disease drugs resulted in a “high value” rating.

Pioneer Institute discourages policymakers and payers from adopting ICER and other “one-size-fits-all” cost-effectiveness models, especially for those innovative and groundbreaking therapies to treat orphan and rare diseases.

Pioneer Institute, instead, believes patients, their physicians and caregivers, and a variety of other societal factors should determine value of these therapies.



ICER’s continuing negative reviews of rare disease drugs seem to indicate that their goal is not to accommodate the unique contextual challenges of rare disease therapies but simply to push their prices down.

To read the full report, *Looming Challenges for ICER in Assessing the Value of Rare Disease Therapies*, visit pioneerinstitute.org.