

The National Minority Quality Forum is a 501(c)(3) not-for-profit, nonpartisan, independent research and education organization. The Forum’s vision is a research, delivery, and financing system that provides quality and effective health services to the biodiverse population of the United States in the 21st century. The Forum contributes to the national dialogue to ensure health policies are grounded in scientific and clinical evidence and that they place a priority on the quality of care and patient outcomes in all populations.

Two new therapies (crizanlizumab and voxelotor) and one relatively new therapy (L-glutamine) have become available for patients with sickle-cell disease (SCD). ICER undertook an assessment to evaluate “the clinical effectiveness and cost effectiveness of crizanlizumab, voxelotor, and pharmaceutical-grade L-glutamine for patients with SCD.” Although ICER recognized that each of these drugs has novel clinical effectiveness that could reduce pain and suffering and improve quality of life for SCD patients, it did not find that any of them to be a high-value medication according to its cost-effectiveness criteria. This failure to meet ICER’s criteria for high value has little to do with the drugs value to SCD patients. From the perspective of patients and their physicians and caregivers, ICER’s value metrics introduce questionable benchmarks that distort the definition of high medical value as it is commonly understood.

We have reviewed the ICER Draft Report and considered the consequences that our comments might have. ICER—a nongovernmental agency—certainly has a right to its opinion. ICER and

2 Bradt et al., Crizanlizumab, Voxelotor, and L-Glutamine for Sickle Cell Disease, p. 92.
the Forum work from profoundly different principles about the obligations of medicine and how those obligations inform patient care.

The Forum’s values are predicated on two axioms: (1) Every patient should have access to appropriate care, and (2) health systems and their treatment protocols should not elevate a patient’s risk for a poor outcome or a poor quality of life. Summarized: Do no harm. Based on these predicates, we view treatment plans, policies, assessments, or value judgements that deny access to appropriate care as social determinants of health, and we see their negative consequences as reportable events.

ICER operates from a different frame of reference. Fundamental to every one of ICER’s assessments is the application of its benchmark cost thresholds to help it distinguish high-value from low-value care. A medication that is inferior in its medical benefit, but whose cost meets ICER’s long-term money value and short-term affordability valuation could be designated high value compared with a more clinically effective, but more costly, medication. A more costly, but clinically effective, medication could be rated as low value in the ICER assessment. ICER conveniently ignores that clinical effectiveness and lowering of patient risk are inextricably linked, and cost does not mediate that relationship.

ICER is an advocate for health-care systems that regard structural inequalities as acceptable by-products of value assessments that promote the elevation of a patient’s risk for a poor outcome or a poor quality of life in order to avoid exceeding cost thresholds. Working from its financial model, ICER withholds a high-value assessment for any of the three novel SCD treatments because the cost exceeds ICER’s predetermined cost thresholds, providing dubious justification for health systems to limit or deny access to these medications. The Draft Report does not suggest that the withholding agency seek a patient’s consent to this care limitation or suggest the appropriateness of reporting the negative consequences of the policy.

The Forum sees no useful purpose in trying to deconstruct the ICER value assessment to show that flaws, inconsistencies, and inequities disqualify the Draft Report as a useful assessment of the value of these new treatments for SCD. Actually, ICER’s assessment model succeeds at what it is designed to accomplish: to determine whether any of the medications meets its narrow definition of high value by having a market price that does not exceed its benchmark cost threshold.

We disagree with the premise that medical value must be a function of cost. The corollary of that premise is a tiered health-care system where patient risk is deliberately elevated and inequities are commonplace. The US Food and Drug Administration by its approval and ICER by its own evaluation have determined that the three SCD medications are clinically effective. Our root-and-branch rejection of the ICER Draft Report is founded on the operational assumptions that are the report’s underpinning. They are antithetical to our value, fundamental to medicine, which we summarize as do no harm.

ICER and the Forum have different opinions, based on different values; we believe that our values more closely align with what any SCD patient (or any patient, for that matter) would expect of the health-care system. Providers that build their SCD-treatment plans on the ICER Draft Report without informing patients of its inherent limitations not only will violate a trust relationship but also will introduce inequalities and poor health outcomes into their systems while diminishing the quality of life for those who come to them for care.