



February 6, 2024

The Honorable Cathy McMorris Rodgers

Chair

Committee on Energy and Commerce
United States House of Representatives
Washington, DC 20515

The Honorable Jason Smith

Chair

Committee on Ways and Means
United States House of Representatives
Washington, DC 20515

The Honorable Frank Pallone, Jr.

Ranking Member

Committee on Energy and Commerce
United States House of Representatives
Washington, DC 20515

The Honorable Richard Neal

Ranking Member

Committee on Ways and Means
United States of Representatives
Washington, DC. 20515

RE: Support for HR 485 Prohibiting use of Quality Adjusted Life Years (QALY) in federal programs

Dear Chair Rodgers, Chair Smith, Ranking Member Pallone, and Ranking Member Neal,

Haystack Project writes to express its support for the HR 485 prohibition on use of QALY in federal health programs. The disconnect between “value” calculated through traditional value assessment frameworks and the impact a new treatment has on the lives of rare disease patients and their families is a significant challenge for our communities. Prohibiting the use of QALY in decisions impacting federal health care programs is an essential step toward ensuring that our patients’ lives are valued, and their medical needs are appropriately prioritized.

Haystack Project is a 501(c)(3) non-profit organization with a membership of 140+ rare and ultra-rare disease patient advocacy organizations. Our core mission is to evolve health care payment and delivery systems with an eye toward spurring innovation and quality in care toward effective, accessible treatment options for all Americans. We strive to coordinate and focus efforts that highlight and address systemic reimbursement obstacles to patient access in disease states where unmet need is high and treatment delays can be catastrophic.

Treatments for exceedingly rare diseases and rare cancers present unique challenges for value frameworks given the high disease burden, limited treatment options, and potentially dire health consequences for patients if treatment access is delayed or denied due to payer perception of low or questionable value. Similarly, a treatment option could have a high value from a payer or societal perspective yet be associated with an unacceptable side-effect or risk profile, or address outcomes that are not meaningful to patients living with the condition.

Entities that evaluate the clinical effectiveness and economic value of pharmaceuticals and other health care interventions in the US, including the Institute for Clinical and Economic Review (ICER), adopt a payer or societal perspective. Model designs, input selection, and metrics such as QALY were developed to aid payer decisions toward cost effective care, primarily in highly prevalent conditions with multiple treatment options. These frameworks do not incorporate or consider our societal goals and values, i.e., to ensure that all patients, including those with rare and ultra-rare conditions have access to the treatments they need. In particular, the use of QALY subverts those goals by discounting the value of maintaining health and function, reducing pain and disease burden, slowing disease progression, or extending life for disabled and/or elderly individuals.

Haystack strongly believes that patients and their caregivers deserve innovation in health care economics and value assessments that rise to meet the innovations we are seeing in treating diseases that have long been untreatable and incurable. **We have long expressed our concern that QALY limitations and deficiencies are most pronounced when applied to rare and ultra-rare conditions.** A comprehensive study on the use of incremental cost per QALY gained in ultra-rare disorders by Schlander et al., discussed that a growing body of literature considers cost per QALY economic evaluations in ultra-rare diseases as flawed, and likely to set inequitable benchmarks that treatments for ultra-rare diseases cannot meet.¹ Patients in countries with technology assessment approaches that use QALY and rigid willingness-to-pay criteria experience treatment delays, coverage denials, and decreased associated survival rates.

Recent examples of ICER value assessment reports illustrate the dangers that a health care system driven by QALY calculations would impose on rare disease patients.

Spinraza and Zolgensma for Spinal Muscular Atrophy (SMA).

In calculating the value of these treatments in babies and young children suffering from a progressive, fatal condition, ICER noted that “[t]he US health care system cannot sustain paying prices far above traditional cost-effectiveness levels for the growing tide of treatments for ultra rare disorders.” We see this SMA example as providing a clear barometer on the threshold issue of whether QALY-based value frameworks align with our societal goals.

- SMA is a catastrophic disorder with subtypes sufficiently severe to make it unlikely that a baby will survive to age two.
- ICER’s New England CEPAC acknowledged “the remarkable effectiveness and many additional potential benefits and contextual considerations of Spinraza and Zolgensma.”

¹ Schlander M, Garattini S, Holm S, Kolominsky-Rabas P, Nord E, Persson U, Postma M, Richardson J, Simoens S, de Solà Morales O, Tolley K, Toumi M. Incremental cost per quality-adjusted life year gained? The need for alternative methods to evaluate medical interventions for ultra-rare disorders. J Comp Eff Res. 2014 Jul;3(4):399-422. doi: 10.2217/ce.14.34. PMID: 25275236.

- ICER lauded Biogen for its randomized, controlled clinical trial design and its robust enrollment, noting that “their efforts to generate such high-quality evidence sets a standard of excellence which other manufacturers should follow.”
- Despite the catastrophic impact of the disease, and the high quality of evidence demonstrating efficacy, ICER’s QALY-based framework drove a unanimous panel vote that Spinraza until very recently, the only SMA treatment available - represented low long-term value for the money due to its high price.

We believe that it is highly likely that novel approaches to ultra-rare conditions and many rare cancers will similarly fail to clear QALY-driven hurdles.

Luxturna

ICER’s assessment of LUXTURNA (voretigene neparvovec) (VN) underscores the importance of eliminating QALY and its “penalty” on value for treatments in patients who are disabled or have experienced a disease-related loss of function. This treatment to halt progression to blindness delivers substantial value for each gain in vision or reduction in disease progression. The QALY-based calculations resulted in a conclusion that treating individuals over age 15 (who have potentially lost a great deal of vision) to prevent progression to blindness would not be justified, stating that:

- When used to treat individuals at age 15, VN does not meet commonly accepted cost-effectiveness thresholds of \$50,000–\$150,000 per quality-adjusted life year (QALY).
- On average, younger patients with this condition have better baseline vision. Because of this, VN appeared to be more cost-effective for individuals treated at age three, particularly when evaluated from a societal perspective. However, it is not clear how many individuals could be diagnosed and treated at this early age.
- Cost-effectiveness of VN was considered both from a health care system perspective that included only direct medical costs, and from a societal perspective, which also accounted for benefits related to education, greater productivity, reduced caregiver time, and other factors.

It is important to note that ICER’s cost-effectiveness analysis did not quantify or consider patient benefits not captured in the quality adjusted life year (QALY) calculation, including:

- Reduced caregiver burden
- Significant impact on productivity
- The high burden and severity of the condition.

Haystack Project and its member organizations strongly support initiatives, including HR 485, that remove QALY from the decisions that influence access to rare and ultra-rare disease treatments. We urge you and your colleagues to support HR 485.

If you have any questions or would like additional information, please contact me at
Kara.berasi@haystackproject.org

Sincerely,

Alpha-1 Foundation
ALS Association
Born a Hero Research Foundation
CDG CARE
CLL Society
Costello Syndrome Family Network
CSNK21A Foundation
CTNNB1 Connect and Cure
cureCADASIL
Cure VCP Disease
Cutaneous Lymphoma Foundation
Danny's Dose Alliance
Dup15q Alliance
FACES: The National Craniofacial Association
FOD Family Support Group
Galactosemia Foundation
Global Foundation for Peroxisomal Disorders
HealthTree Foundation
ICAN, International Cancer Advocacy Network
Biomarker Collaborative
Exon 20 Group
MET Crusaders
PD-L1 Amplifieds
International Foundation for CDKL5 Research
International Fibrodysplasia Ossificans Progressiva (FOP) Association
Sudden Arrhythmia Death Syndromes (SADS) Foundation

Luka Shai Foundation
MLD Foundation
Myasthenia Gravis Foundation of America
National MPS Society
National Niemann-Pick Disease Foundation, Inc.
National Tay-Sachs and Allied Diseases
Association, Inc. (NTSAD)
NBIA Disorders Association
NBIA Disorders Association
NTM Info & Research
Organic Acidemia Association
Pachyonychia Congenita Project
Phelan-McDermid Syndrome Foundation
Rare And Black
Sophie's Neighborhood
Sturge-Weber Foundation
SYNGAP1 Foundation
Taylor's Tale
The NW Rare Disease Coalition
United Porphyrias Association (UPA)
Usher 1F Collaborative
VHLA