

October 3, 2022

VIA ELECTRONIC DELIVERY

The Honorable Xavier Becerra
Secretary
Department of Health and Human Services
Attention: 1557 NPRM [RIN 0945-AA17]
200 Independence Avenue SW
Washington, DC 20201

**RE: IGT Comments on the Nondiscrimination in Health Programs and Activities Proposed Rule
[RIN 0945-AA17]**

Dear Secretary Becerra:

The Institute for Gene Therapies (IGT or “the Institute”) is pleased to submit comments to the Department of Health and Human Services (HHS or “the Department”) on the Proposed Rule, titled, “Nondiscrimination in Health Programs and Activities” (“Proposed Rule”).¹

IGT was launched in February 2020 to advocate for a modernized regulatory and reimbursement framework that encourages the development of transformative gene therapies and promotes patient access. Through a Corporate Advisory Council, Patient Advocacy Advisory Council, and Scientific, Academic & Medical Council, the Institute represents a wide array of gene therapy manufacturers, patient advocacy groups, and scientific, medical, and academic stakeholders seeking to advance the promise of gene therapies. IGT aims to inform the conversation regarding the value of transformative therapies and advocate for policies and practices to ensure patient access to these treatments. A full list of our members is available at <https://www.gene-therapies.org/advisory-councils>.

Our comments mainly respond to the Department’s request for feedback on the extent, scope, and nature of value assessment methods that discriminate on the grounds prohibited by Section 1557 of the Affordable Care Act (ACA).

Discriminatory Implications of the QALY Indicator

It is well documented in literature that value assessments derived from the quality-adjusted life year (QALY) may lead to unfair discrimination against individuals with less than perfect health. The QALY framework relies on a system of numeric utility to quantify the value of various health states wherein the highest possible utility for a health state is 1, representing “perfect health,” and 0 which is an arbitrary value for death. The central criticism to the use of the QALY framework is that QALYs place greater value on years lived in full health, or on

¹ 87 Fed. Reg. 47,824 (Aug. 4, 2022).

interventions that prevent loss of perfect health while discounting gains in health for individuals with chronic conditions or disabilities. Within the QALY framework, individuals with chronic conditions and disabilities experience a lower maximum baseline in health than their non-disabled counterparts. As a result, a treatment that improves their quality of life may result in fewer QALYs gained than a similar treatment for individuals who are not disabled. These individuals are thus at a serious disadvantage as the framework favors those with greater potential for health.

The QALY was originally developed for use in academic population-level assessments. However, use of QALYs has expanded over time in the United States to determine the economic value of health care interventions for the purposes of guiding coverage and reimbursement decisions, though not without objection. Notably, in 1992, HHS found that Oregon's efforts to use a QALY-based cost-effectiveness standard in the state's Medicaid program violated the Americans with Disabilities Act (ADA) by systematically disadvantaging individuals with pre-existing disabilities.² The ACA also explicitly prohibits the Patient-Centered Outcomes Research Institute (PCORI) from using the cost-per-QALY as a threshold to establish what type of health care is cost effective or recommended.³ The ACA further restricts the use of QALYs by precluding their use as a threshold to determine coverage, reimbursement, or incentive programs in Medicare.⁴ Moreover, the National Council on Disability (NCD), an independent federal agency, has found sufficient evidence of the discriminatory effects of QALYs to warrant concern, including concerns raised by bioethicists, patient rights groups, and disability rights advocates.⁵ Finally, the recently-enacted Inflation Reduction Act (IRA) of 2022 expressly prohibits HHS from considering "evidence from comparative clinical effectiveness research in a manner that treats extending the life of an elderly, disabled, or terminally ill individual as of lower value than extending the life of an individual who is younger, non-disabled, or not terminally ill" when negotiating drug prices under the new Drug Price Negotiation Program.⁶

Other Value Elements That Must Be Given Weight

A separate critique of the QALY framework is that it does not represent how society actually views health or value. QALYs capture only a subset of benefits that may be produced by a health care intervention while ignoring additional considerations of value. IGT is leading the effort to educate policymakers and stakeholders about what constitutes value and identify additional elements of value that warrant consideration in health care interventions. We believe value cannot be limited to one or two elements, such as a QALY or direct medical costs. For patients, families, and society, value must include patient preferences with respect to a treatment, the impact of a treatment on the patient's family and caregivers, a treatment's ability to advance health equity and address unmet needs, and societal impact. In the gene therapy space in particular, a value assessment must include the lifetime impact or durability of a treatment, a treatment's effects (both short- and long-term), and the rarity or severity of disease. It is our unwavering assertion that to the extent that every vital measure or element of value that is not included in a value assessment, such assessment is not complete and must not be used for coverage or payment decisions. To restrict coverage and access to any health intervention based on an incomplete or inaccurate depiction of value amounts to discrimination in violation of federal law.

² Pear R. White House Expected to Back Oregon's Health-Care Rationing, *New York Times* (Mar. 1993).

³ 42 U.S.C. §1320e-1(e).

⁴ *Id.*

⁵ NATIONAL COUNCIL ON DISABILITY, *Quality-adjusted life years and the devaluation of life with disability* (Nov. 2019).

⁶ Inflation Reduction Act (IRA) of 2022, Pub. L. No. 117-169, adding new Social Security Act § 1194(e)(2)(D).

Conclusion

The Institute appreciates the Department's consideration of these comments. IGT would be pleased to continue to serve as a resource on gene therapy issues during this process and answer any questions regarding these comments.

Sincerely,

A handwritten signature in blue ink, appearing to read "John R. Feore, III". The signature is fluid and cursive, with the last name "Feore" being particularly prominent.

John R. Feore, III
Director, Policy and Advocacy
Institute for Gene Therapies