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November 13, 2023

VIA ELECTRONIC DELIVERY

Melanie Fontes Rainer
Director, Office for Civil Rights
Department of Health and Human Services
Attention: Disability NPRM, RIN 0945–AA15
200 Independence Avenue SW
Washington, DC 20201

RE: IGT Comments on Discrimination on the Basis of Disability in Health and Human Service Programs or Activities [HHS–OCR–2023–0013]

Dear Director Rainer:

The Institute for Gene Therapies (IGT or "the institute") appreciates the opportunity to submit these comments to the US Department of Health and Human Services (HHS) Office for Civil Rights (OCR) on the Discrimination on the Basis of Disability in Health and Human Service Programs or Activities proposed rule (the "Proposed Rule").[1] IGT was launched in February of 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 patient advocacy groups, gene therapy manufacturers, and scientific, medical, and academic stakeholders seeking to advance the promise of gene therapies. IGT is devoted to promoting the value of transformative therapies and advocating for policies and practices to ensure patient access to these treatments. Our most vulnerable patients and their families anxiously wait for the life-altering treatments that gene therapies will offer to some of the most debilitating or rare diseases. A full list of our members is available at https://www.gene-therapies.org/about-igt.

IGT commends the HHS OCR for proposing new protections from discrimination that individuals with a disability often face when seeking healthcare services. We support OCR's admission that disability discrimination in the medical treatment context presents a real and continued threat to individuals with disabilities and support the Proposed Rule's clarification of Civil Rights protections. We strongly support prohibiting the use of value assessment methods that place a lower value on life extension for a group of individuals based on disability where such methods are then used to deny or afford an unequal opportunity to an individual with a disability. **IGT lends its full support to the proposed addition of new regulatory text at 45 CFR §§ 84.56-57**.

I. About Gene Therapy

Scientists have been working for decades to deliver on the promise of gene therapy, with a goal of revolutionizing treatment paradigms and replacing life-long chronic therapies with potentially curative therapies

[1] 88 Fed. Reg. 63,392 (Sept. 14, 2023).

for diseases for which limited or no treatment options exist – or where the only available treatment options are burdensome, expensive, painful, and lifelong. Gene therapies are potentially one-time treatments that aim to fix the underlying cause of genetic diseases at the DNA level. The effects are long-lasting – potentially lifelong – and will prompt changes in how we think about the value of treatment, payment models, and the like. The science underpinning gene therapies is highly complex. The development and manufacturing processes for gene therapies are built on a deep understanding of biologic medicines, e.g., vaccines, insulin, and monoclonal antibodies. While gene therapies and biologics share some characteristics, gene therapies are significantly more scientifically complex both in how they are designed for safety and efficacy and in how they are manufactured for scalability and capacity. Gene therapies deliver a functional gene to affected cells throughout the body, so that tissues can begin to produce the protein that is missing due to the patient's disease.

Since August 2022, the Food and Drug Administration (FDA) has approved six gene therapies.[2] Scores of gene therapies are currently in clinical trials in the US; most of which will address rare and ultra-rare diseases, central nervous system diseases, and conditions disproportionately impacting historically disadvantaged populations.[3] In many cases, gene therapies halt but cannot reverse the effects of a disease by addressing the underlying genetic cause. For this reason, any discriminatory delay in, or obstacle to, accessing an approved gene therapy can result in patients continuing to suffer irreversible damage caused by their disease that may otherwise be avoided. As the promise of gene therapy transitions from research to reality, HHS and OCR must ensure that recipients of federal financial assistance do not restrict or block access to these transformative therapies.

II. Discrimination Against People With Disabilities in Medical Treatment

As OCR notes in the Proposed Rule, "Medical literature, government agency reports, and court decisions demonstrate that individuals with disabilities face discrimination at every stage of the medical treatment process."[4] **IGT strongly supports the Proposed Rule's clarification and reiteration that qualified individuals with disabilities may not be discriminated against in the medical treatment context**. We agree with the assertion that "medical treatment" refers to a broad and inclusive catalogue of patient management and care, including "the prescribing, dispensing, or management of medications."[5] When providers practice medicine with ingrained stereotypes about the value and quality of lives of people living with disabilities, medical treatment decisions may lead to inadequate or deficient care.

The promise of gene therapy is quickly becoming a reality and many patients will have the option of replacing life-long chronic therapies with potentially curative therapies for diseases for which limited or no treatment options currently exist or where the only available treatment options are burdensome and lifelong. If an individual with a disability also has a genetic condition that may be treated by a gene therapy, discriminatory medical treatment decisions may preclude these patients from the option of a transformative therapy. A study cited by the Proposed Rule indicates that 82% of doctors thought people with disabilities had a lower quality of life than people without disabilities, even though most people with disabilities manage to derive satisfaction and pleasure from their lives.[6]

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[2] In chronological order, see: (1) https://www.fda.gov/vaccines-blood-biologics/zynteglo; (2) https://www.fda.gov/vaccines-blood-biologics/skysona; (3) https://www.fda.gov/vaccines-blood-biologics/vaccines/hemgenix; (4) https://www.fda.gov/vaccines-blood-biologics/vyjuvek; (5) https://www.fda.gov/vaccines-blood-biologics/tissue-tissue-products/elevidys; and (6) https://www.fda.gov/vaccines-blood-biologics/roctavian.

[3] See ClinicalTrials.gov.

[4] 88 Fed. Reg. at 63395.

[5] Id.

[6] Id. at 63396.

Medical treatment sought by an individual with disabilities is often unrelated to the disability. Given the nature of gene therapies, i.e., treating the root cause of a genetic disease, it is imperative that individuals with disabilities are not treated any differently than those without a disability. IGT supports OCR's enforcement of disability protections in medical treatment decisions under any program or activity that receives federal financial assistance. We fully support the declaration that, "A recipient's failure to provide treatment to an individual with disabilities who meets all qualifications for the medical treatment results in a denial of health care to a person with disabilities and, barring any applicable limitation, constitutes discrimination in violation of section 504 [of the Americans with Disabilities Act]."[7]

IGT appreciates the example provided in the Proposed Rule regarding pharmacological interventions, which are often studied on populations that might not be fully representative of the general patient population. While individuals with disabilities may not have been included in clinical trials, in many cases due to the rare or ultra rare occurrence of the genetic disease being treated, it is unlikely that a disability alone would not render a gene therapy less effective than in the general population. "Physicians have substantial discretion to assess mixed or inconclusive evidence regarding effectiveness according to their own judgment."[8] This discretion is paramount to the shared decision making that is necessarily involved in discussing the pros and cons of administering a gene therapy between a treating physician and patients and their caregivers, and ultimately the decision whether to pursue this course of treatment. Recipients of federal financial assistance must not stand in the way of access via veiled discriminatory medical treatment or coverage decisions.

III. Discrimination Against People With Disabilities in Value Assessment Methods

IGT strongly supports newly proposed 45 CFR §84.57 that prohibits a recipient of federal financial assistance from directly or indirectly using any measure that discounts the value of life extension on the basis of disability to deny or afford unequal opportunity to individuals with disabilities. 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, particularly for individuals with rare diseases. 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. QALYs place greater value on years lived in full health, or on 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 US to determine the economic value of health care interventions for the purposes of guiding coverage and reimbursement decisions, though not without objection. As noted in the Proposed Rule but worth repeating here, 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.[9] The Affordable Care Act (ACA) also explicitly prohibits the Patient-Centered Outcomes Research Institute (PCORI) from using the cost-per-QALY as a

[7] Id. at 63402.
[8] Id. at 63407.
[9] Pear R. White House Expected to Back Oregon's Health-Care Rationing, New York Times (Mar. 1993).

threshold to establish what type of health care is cost effective or recommended.[10] The ACA further restricts the use of QALYs by precluding their use as a threshold to determine coverage, reimbursement, or incentive programs in Medicare.[11] Moreover, the National Council on Disability (NCD), an independent federal agency cited throughout the Proposed Rule, 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.[12] Finally, the Inflation Reduction Act of 2022 (IRA) 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.[13] These regulatory decisions and statutory prohibitions all recognize a simple, irrefutable fact: the QALY metric illegally discriminates against individuals with disabilities in violation of their Civil Rights.

Patients Deserve More than an Arbitrary, Discriminatory Metric

It is unnecessary for recipients of federal financial assistance to base coverage, cost, and other decisions implicating healthcare access on discriminatory value assessment metrics such as the QALY. In fact, the QALY framework does not represent how society views health or value. QALYs capture only a subset of benefits that may be produced by a healthcare intervention while ignoring additional considerations of value. IGT is leading the effort to educate policymakers and stakeholders about what constitutes value. In collaboration with our patient and corporate advisory councils, we identify critical 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 the 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 and its impact on individuals with disabilities. It is our unwavering assertion that to the extent every vital measure or element of value 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. We encourage HHS and OCR to learn more about the value of gene therapies and how they provide a complete understanding of how gene therapies offer immense value to patients, families, the healthcare system, and society.[14]

IV. Conclusion

Gene therapies have the potential to transform how we approach disease in this country, particularly serious conditions with high unmet medical need, and to help reverse decades of health inequities and discrimination against disadvantaged populations including individuals with disabilities. The Institute for Gene Therapies appreciates OCR's efforts to ensure that recipients of federal financial assistance do not discriminate against

[10] 42 U.S.C. §1320e-1(e).
[11] *Id.*
[12] National Council on Disability (NCD), "Quality-Adjusted Life Years and the Devaluation of Life with Disability," (Nov. 6, 2019); available at: https://ncd.gov/sites/default/files/NCD_Quality_Adjusted_Life_Report_508.pdf.
[13] Inflation Reduction Act (IRA) of 2022, Pub. L. No. 117-169, adding new Social Security Act § 1194(e)(2)(D).
[14] See https://www.gene-therapies.org/value-of-gene-therapies.

individuals with disabilities, whether through direct medical treatment decisions or through the use of discriminatory value assessment metrics. IGT welcomes the opportunity to engage with HHS and OCR and we are pleased to serve as a resource on gene therapy issues and answer any questions regarding these comments.

Sincerely,

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