American Society of Gene + Cell Therapy

March 15, 2024

The Honorable Virginia Foxx
Committee on Education and the Workforce
US House of Representatives
2176 Rayburn House Office Building
Washington DC 20515

Dear Chairwoman Foxx,

The American Society of Gene and Cell Therapy (ASGCT) welcomes the opportunity to respond to your request for information on the Employee Retirement Income Security Act (ERISA). ASGCT is a nonprofit professional membership organization comprised of more than 6,200 scientists, physicians, patient advocates, and other professionals. Our members work in a wide range of settings including universities, hospitals, government agencies, foundations, and biotechnology and pharmaceutical companies. Many of our members have spent their careers in this field performing the underlying research that has led to today's robust pipeline of transformative therapies.

A core portion of ASGCT's mission is to advance the discovery and clinical application of genetic and cellular therapies to alleviate human disease. To that end, ASGCT supports policies that foster the adoption of, and patient access to, new therapies, which thereby encourage continued development of these innovative treatments.

If you have questions about any of the information provided below, please contact Margarita Valdez Martínez, Director of Policy and Advocacy, at mvaldez@asgct.org.

Sincerely,

David Barrett, JD
Chief Executive Officer
American Society of Gene & Cell Therapy

American Society of Gene + Cell Therapy

Request for Information
ERISA's 50th Anniversary: Reforms to Increase Affordability and Quality in Employer-Sponsored Health Coverage

Specialty Drug Coverage

The American Society of Gene & Cell Therapy (ASGCT) is a scientific organization dedicated to advancing the discovery, development, and availability of genetic therapies. We have members working across the field: from bench to bedside, in academia and industry, as payers and clinicians. ASGCT therefore has an interest in this RFI, but we are not an industry trade organization or payer specialist. Our responses are therefore limited to the areas within ASGCT's established positions and member experiences.

1. What challenges do employers face in offering coverage of high-cost specialty drugs, and how can those challenges be addressed?

Cell and gene therapies (CGTs) can have a transformative impact on patients' lives. Rather than treating disease symptoms, gene therapy can address the root causes of disease by modifying expression of a patient's genes or by repairing or replacing abnormal genes. Taking into account only medical expenses, independent reviews of gene therapy products have shown that over the course of a patient's lifetime, gene therapies can result in savings over traditional treatment options despite high upfront costs.[1] In addition, there are a number of non-medical factors that also increase gene therapies' value.[2] Patients with chronic genetic illnesses often struggle with serious interruptions of their schooling, jobs, and social lives. Alleviating their symptoms or halting progression can allow them to lead a much more normal life and can also significantly ease caregiver burden.

Anecdotally, ASGCT members report that portability is one of the primary challenges for employer-sponsored coverage of specialty drugs. A single individual can have coverage through multiple plans over their lifetime, as they may move to a new insurance carrier each time their employment changes. That system was not designed to accommodate high-cost, high-value, single-administration therapies like CGTs, in which the cost is front-loaded but the benefits play out over many years. In the traditional employer-sponsored insurance model, payers worry about bearing the cost of treatment without ever getting the chance to recoup the savings.

ASGCT members have noted challenges related to forecasting. Most of the indications for currently approved CGTs are rare, so it can be difficult for smaller employers or health groups to predict whether they will cover someone with a given condition in a plan year. That said, "rare diseases" are not truly "rare." As ASGCT Vice President Dr. Terence Flotte recently shared with the House Energy & Commerce Committee, there are over 10,000 rare diseases.[3] In the US,

[1] Udeze, C. et al. (2022). Projected lifetime economic burden of severe sickle cell disease in the United States [Poster]. *HemaSphere, 6,* 1585-1586. https://journals.lww.com/hemasphere/Fulltext/2022/06003/P1704__PROJECTED_LIFETIME_ECONOMIC_BURDEN_OF.1585.aspx
[2] American Society of Gene & Cell Therapy. (2018). *Addressing the Value of Gene Therapy: Enhancing Patient Access to Transformative Treatments* [White Paper]. https://www.asgct.org/ASGCT/media/about/18GeneEdit_WP_FINAL.pdf
[3] Flotte, T. (2024). *Written Testimony of Terence Flotte, MD, Vice President of the American Society of Gene and Cell Therapy.* https://asgct.org/global/documents/advocacy/testimony-of-terence-flotte-md-on-behalf-of-asgct.aspx

approximately 10% of the population - 30 million people - have a rare disease. Given the robust pipeline of CGTs currently in development, this question is only going to become more acute.[4]

In an attempt to address costs, commercial insurers may turn to a number of strategies including value-based arrangements, risk pooling, and warranty models. We will discuss value-based arrangements further in Question 7. ASGCT does not have a specific position on the latter approaches, though we generally support efforts to explore flexible, innovative solutions to coverage challenges. The Society has previously served as a direct convener for those conversations. At our 2019 Policy Summit we hosted a panel on *Advances in Novel Payment Solutions for Public & Private Payers.*[5] We would be pleased to make a recording of that session available to the Committee.

In 2023, ASGCT assessed Medicaid coverage practices across 16 states and 3 MCOs. The takeaway of this peer reviewed publication, a copy of which is included at the end of this letter, is that that states are not always adhering to federal requirements to provide coverage for products to their "medically accepted" indication. [6] States are narrowing coverage to populations covered in clinical trials, despite broader labeled indications; and many states require additional information or eligibility criteria that are not included in the drug's indication. While this paper specifically assessed Medicaid coverage, we anecdotally know that the identified challenges are present in the commercial insurance market as well - including broad exclusions for gene therapy coverage under some health plans. From the paper:

> "Federal law sets overarching requirements for state Medicaid programs, which mandate coverage of certain medical benefits. However, the bulk of the operational decisions are left at the discretion of each state, including enrollment eligibility, reimbursement methodology, and service coverage. Once approved by the Centers for Medicare and Medicaid Services (CMS), state Medicaid programs may draw down federal funds based on the federal medical assistance percentage (FMAP). Under the Medicaid Drug Rebate Program (MDRP), states that include prescription drug coverage in their Medicaid programs—which all states do—must cover all drugs approved by the Federal Drug Administration (with limited statutory exceptions) according to their 'medically accepted indications,' and in return manufacturers provide rebates on their products to the states, which are then shared between the states and the federal government…
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> Despite general coverage to the labeled indication being mandated by federal law, variations in state plans, policies, and practices have created anecdotal inconsistencies regarding how therapies are covered, which populations they cover, and how quickly coverage is approved for individual patients. This leads to patient access issues, as denials or delays in coverage lead to delays in treatment. These disparities and delays all culminate into an unsustainable public payor system that undermines Medicaid's objectives to improve the care and health of its beneficiaries."

[4] American Society of Gene & Cell Therapy, Citeline. (2024). *Gene, Cell, & RNA Therapy Landscape: Q4 2023 Quarterly Data Report.* https://asgct.org/publications/landscape-report
[5] American Society of Gene & Cell Therapy. (2019). *Policy Summi Agendat: Perspectives on Payment Policies for Gene Therapies.* https://asgct.org/advocacy/policy-summit/policy-summit-archive/2019-policy-summit/payment-policies-november-5-asgct-policy-summit
[6] Allen, J. et al. (2023). Medicaid coverage practices for appvoed gene and cell therapies: Existing barriers and proposed policy solutions. *Molecular Therapy: Methods & Clinical Development, 29,* 513-521. https://asgct.org/ASGCT/media/about/Medicaid-paper.pdf?ext=.pdf

ASGCT members have also expressed concerns with overuse of prior authorization as a utilization management tool for CGTs. For patients with rare genetic diseases, it is imperative that they receive a swift diagnosis and access to treatment as soon as possible. Early diagnosis and treatment are especially important for diseases where damage to organs and other body systems can accumulate over time; damage may be halted or prevented by early intervention but cannot be reversed. Additionally, many pediatric rare diseases have upper age limits for the approved indication. In other cases, such as pediatric blood cancers that may be treatable with CAR T-cell therapies, patients' lifespans are often measured in a matter of months by the time they have failed enough lines of treatment to qualify for a CAR T-cell product. Lengthy treatment delays due to opaque prior authorization processes can lead to significantly worse outcomes for these patients.

This is not just theoretical. ASGCT members have numerous first-hand experiences with payers asking for data that's clinically irrelevant to the treatment at hand; requiring hospitals to go through predictable but inescapable rounds of appeals for each patient prescribed a given CGT product; and in some cases delaying approval to the point that the patient was no longer eligible for the therapy, due to deteriorated health condition, age, or death. While prior authorization has a role to play in the healthcare system, ASGCT believes that requirements must be transparent, consistent, and comprehensible. ASGCT would be pleased to connect Committee members directly with these members so that they can share their experiences. For a more in-depth discussion of our Medicaid paper, and the issues ASGCT is focused on in that space, please see our recent response to the Senate HELP Committee's RFI on Improving Americans' Access to Gene Therapies.[7]

6. What role should the federal government play in assisting employers, drug manufacturers, and other entities to manage risks and to share the costs and savings of employer-sponsored coverage of high-cost specialty drugs?

Again, ASGCT does not specialize in private payer issues. However, our engagement on the public payer side may offer lessons for employer-sponsored insurance.

One of the policy recommendations in our Medicaid white paper is that "CMS should establish a public dashboard tracking coverage policies, denials, complaints, and discrepancies in coverage and reimbursement for each product across states. The information would be useful in quantifying the true scope of the problem and provide a forum to assess claims of overly restricted coverage."[8] In that vein, CMS recently finalized regulations[9] that will require managed care plans and states to report on prior authorization outcomes, which we believe is a positive

[7] American Society of Gene & Cell Therapy. (2024). *Response: Request for Information from Stakeholders on Improving Americans' Access to Gene Therapies.* https://asgct.org/advocacy/policy-statement-landing/2024/rfi-improving-americans-access-to-gene-therapies

[8] Allen, J. et al. (2023). Medicaid coverage practices for appvoed gene and cell therapies: Existing barriers and proposed policy solutions. *Molecular Therapy: Methods & Clinical Development, 29,* 513-521. https://asgct.org/ASGCT/media/about/Medicaid-paper.pdf?ext=.pdf

9 Centers for Medicare and Medicaid Services. (2024). Medicare and Medicaid Programs; Patient Protection and Affordable Care Act; Advancing Interoperability and Improving Prior Authorization Processes for Medicare Advantage Organizations, Medicaid Managed Care Plans, State Medicaid Agencies, Children's Health Insurance Program (CHIP) Agencies and CHIP Managed Care Entities, Issuers of Qualified Health Plans on the Federally-Facilitated Exchanges, Merit-based Incentive Payment System (MIPS) Eligible Clinicians, and Eligible Hospitals and Critical Access Hospitals in the Medicare Promoting Interoperability Program. https://www.federalregister.gov/documents/2024/02/08/2024-00895/medicare-and-medicaid-programs-patient-protection-and-affordable-care-act-advancing-interoperability

step; we encourage CMS to create new reporting standards specific to gene and cell therapies, incorporating factors beyond simple prior authorization. Given the federal regulation of ERISA plans, a parallel effort is important to consider, as transparency and predictability are critical to ensuring the prior authorization process is being utilized appropriately.

7. What barriers exist in ERISA or elsewhere that prevent employers from entering into value-based arrangements with drug manufacturers for coverage of high-cost specialty drugs?

The Centers for Medicare and Medicaid Services (CMS) has enacted regulations that enable value-based, risk-sharing arrangements that tie payment to product performance. ASGCT supports this concept, as such arrangements could provide cost savings to patients and payers, including state Medicaid programs.[9] In addition, doing so redistributes some risk of uncertain outcomes from payers to manufacturers and distributes costs more equitably based on individual patient outcomes. In addition, ASGCT supports allowing VBAs that include a pay-over-time component. Enabling payment models that combine the two concepts would be useful by, for example, allowing payers to make installment payments upon patient attainment of benchmarks of efficacy. Doing so ties a portion of product value to durability over time.

Anecdotally, ASGCT members in the commercialized product space report a range of potential barriers to value-based agreements (VBAs). First, risk-sharing contracts have specific operational and administrative elements required to monitor, track, and adjudicate performance. VBAs can be further limited by government policies such as Medicaid Best Price, Average Sales Price, and Average Manufacturer Price reporting, and the Federal Anti-Kickback Statue, both of which can create uncertainty for manufacturers who might otherwise be interested in pursuing a VBA. For rare therapies, with total nationwide populations of only a few hundred patients, it may be challenging to focus resources towards the development of a VBA with a limited reach. As noted in Question 1, assuming those upfront hurdles are passed, patient portability between insurers can make it logistically difficult to track a patient if they are treated with a therapeutic product subject to a VBA.
There is also the question of what outcomes a given VBA would measure. For some diseases, there are comparatively clear clinical criteria that can be assessed against either a patient's prior health status and/or against the disease's established natural history. Many others, however, are heterogeneous and progress slowly; developing standard metrics to judge all patients on a particular therapy might be impractical in those cases. Predicting the duration of treatment effect is also seen as a challenge. The anticipated durability of gene therapies – from several years up to a lifetime – stands out as one of the major value propositions for CGTs. Numerous long-term follow-up studies for CGTs are underway, and we are now starting to reach major milestones in those assessments. For instance, the first pediatric patient treated with a CAR T-cell therapy reached the ten-year post-treatment mark in 2022.[10] While we have a large body of pre-clinical and early clinical evidence predicting that CGTs can have extremely durable effects, time is needed to confirm the real-world longevity of those products.

[10] Children's Hospital of Philadelphia. (2022). *Emily Whitehead, First Pediatric Patient to Receive CAR T-Cell Therapy, Celebrates Cure 10 Years Later.* https://www.chop.edu/news/emily-whitehead-first-pediatric-patient-receive-car-t-cell-therapy-celebrates-cure-10-years

8. What innovative coverage models are currently in use that address the high cost of specialty drugs?

There have been several recent efforts to foster federally financed coverage and reimbursement mechanisms for new medical products, including CGTs.

Earlier this year the Administration announced the Cell and Gene Therapy (CGT) Access Model.[11] This model will enable the Centers for Medicare and Medicaid Services (CMS) to negotiate outcomes-based agreements with manufacturers. The CGT model is expected to launch in January 2025, and enrollment for states and select territories will be on a rolling basis. This multi-year model will be voluntary for both states and manufacturers. If implemented properly, the CGT Access Model could help Medicaid beneficiaries gain access to potentially life-changing therapies. ASGCT has engaged with CMMI to share the unique nature of CGTs and the realities of many patients' experiences under the current system: that transformative potential is often hindered or blocked altogether by administrative roadblocks.

[11] Centers for Medicare & Medicaid Services. (2024). Cell and Gene Therapy (CGT) Access Model. https://www.cms.gov/priorities/innovation/innovation-models/cgt