March 18, 2024

The Honorable Virginia Foxx
Chairwoman
Committee On Education and Workforce
U.S. House Of Representatives
2176 Rayburn House Office Building
Washington, DC 20515-6100

Dear Congresswoman Foxx:

On behalf of the Alliance for Regenerative Medicine (ARM), I thank you for your interest in expanding affordable coverage and increasing quality and access to care for Americans covered by employer-sponsored care. ARM is the leading international advocacy organization championing the benefits of engineered cell therapies and genetic medicines for patients, healthcare systems, and society. We look forward to providing input on behalf of our diverse membership in response to your recent Request for Information ("RFI") titled "*ERISA's 50th Anniversary: Reforms to Increase Affordability and Quality in Employer-Sponsored Health Coverage.*"

As the global voice of the cell and gene therapy (CGT) sector, ARM represents more than 400 members across 25 countries, including emerging and established biotechnology companies, academic and medical research institutions, and patient organizations. ARM is working to build the future of medicine by convening the sector, facilitating influential exchanges on policies and practices, and gathering and analyzing data. We engage stakeholders across the private and public sector to enable the development of advanced therapies and to modernize healthcare systems so that patients benefit from CGTs. Further, because many of our members range from small to large sized employers who offer employer sponsored health benefits, we share your sentiments in celebrating the upcoming 50th anniversary of the Employee Retirement Income Security Act ("ERISA").

Availability of novel treatments, particularly for complex ailments such as cancer, hemophilia, or sickle cell disease, are expanding rapidly. In recent years, numerous life-changing and often life-saving CGTs have been approved by the Food and Drug Administration (FDA) for some of the most difficult-to-treat conditions that affect both children and adults. These durable, potentially curative therapies\can bring decades or a lifetime of benefits to the seriously or incurably ill.

As of March 2024, there are nearly 1,000 CGT clinical trials ongoing in the US and nearly 1,900 globally to study the future of medicine for the next generation. These novel therapies present new opportunities and challenges for America's healthcare system. CGTs address high unmet medical needs, can be lifesaving; and many have the potential to reduce the need for burdensome and costly chronic care. While traditional pharmaceuticals typically treat the symptoms of diseases for short periods and may need to be administered regularly over a patient's lifetime, CGTs target the root causes of disease, are typically administered in a single or limited number of doses and with durable and potentially curative treatment effects.

1015 18th Street NW, Suite 1102
Washington, DC 20036

www.alliancerm.org
info@alliancerm.org

@alliancerm
@Alliance for Regenerative Medicine

The standard of care for many of the diseases targeted by approved CGTs is relatively expensive. For example, a severe hemophilia B patient requires more than $21 million in lifetime care costs when using the current standard of care.[1] Lifetime healthcare costs for a severe sickle cell disease patient range from $4 to $6 million.[2] A patient with transfusion-dependent thalassemia requires $5.4 million in lifetime costs, on average.[3] Cell and gene therapies, due to their single administration and durable nature, save healthcare system resources in the medium to long term. Additionally, conditions targeted by CGTs are devastating and often deadly. For example, the average life expectancy for rare diseases targeted by approved gene therapies is 40 years, which is half the average U.S. lifespan, and for some rare pediatric diseases, life expectancy is much lower. This illustrates the high unmet medical need that CGTs address, the impact on patients' length and quality of life, and the potential societal benefits of extending life and restoring the ability of caregivers and even patients to return to work and living fuller lives. Thus, responsive and forward-looking reforms are necessary to align the promise of CGTs with the needs of patients and society.

ARM believes that by addressing challenges related to access and payment through strategic planning, collaboration, and innovation, employers can better navigate the complexities of offering coverage for CGTs while ensuring the health and well-being of their employees. Accordingly, ARM looks forward to working with you and the House Committee on Education and Workforce to ensure patients in the U.S. have timely, equitable access to CGTs. We offer the responses below to the questions posed in your RFI:

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

The complex nature of CGTs can make coverage and reimbursement processes challenging for employers to navigate. Determining eligibility criteria, coverage limitations, and negotiating with carriers can impact the ability of an employer to offer a competitive benefits package. Employers must assess the risk of offering coverage for high-cost therapies, considering factors such as employee utilization rates, potential adverse effects, and the overall impact on their healthcare spending. Balancing the need to provide access to innovative treatments with financial sustainability is a critical consideration for many employers. Specialty drugs are often used in orphan diseases, which are conditions that affect fewer than 200,000 people in the United States.[4] A 2021 study of U.S. commercial payer medical policies found that more than two-thirds of health plans restrict coverage of CGTs and are substantially more restrictive in their coverage of CGTs as compared to other orphan products.[5] Patients and healthcare providers

---

[1] Li, Nanixin, et al. "Adult Lifetime Cost of Hemophilia B Management in the US: Payer and Societal Perspectives from a Decision Analytic Model." *Journal of Medical Economics*, U.S. National Library of Medicine, pubmed.ncbi.nlm.nih.gov/33591884/. Accessed 18 Jan. 2024.

[2] Liu, Angus. "Sickle Cell Disease Gene Therapies from Vertex, Bluebird Can Be Cost-Effective at $1.9m: ICER." *Fierce Pharma*, 13 Apr. 2023, http://www.fiercepharma.com/pharma/sickle-cell-disease-gene-therapies-vertex-crispr-bluebird-can-be-cost-effective-19m-icer. Accessed 18 Jan. 2024.

[3] Udeze, C, et al. "PB2339: Projected Lifetime Economic Burden of Transfusion Dependent Beta-Thalassemia in the United States." *HemaSphere,* 23 Jun. 2023, https://www.ncbi.nlm.nih.gov/pmc/articles/PMC9429534/. Accessed 18 Jan. 2024.

[4] 21 Code Federal Regulations Part 316 - Orphan Drugs

[5] Beinfeld MT, Rucker JA, Jenkins NB, de Breed LA, Chambers JD. Variation in Medicaid and commercial coverage of cell and gene therapies. Health Policy Open. 2023 Oct 13;5:100103. doi: 10.1016/j.hpopen.2023.100103. PMID: 38023441; PMCID: PMC10660088.

often encounter challenges related to prior authorization requirements and coverage denials for CGTs, which can result in harmful and sometimes fatal delays.

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?

The federal government has oversight on matters that impact pathways specialty drugs take to gain FDA approval, minimum standards for federally regulated plans and broadly set the precedent for how private markets function. Congress empowered the FDA to be the sole arbiter of establishing the safety and efficacy of drugs and biologics; therefore, payers should resist efforts to duplicate or subvert FDA's regulatory review process for the purposes of determining how to cover or pay for approved products. In recent years there have been efforts to restrict coverage or reduce payment for therapies approved by the FDA through the Accelerated Approval pathway. Such attempts undermine the intent of the Accelerated Approval pathway – to bring treatments to critically ill patients with unmet needs more quickly – and inconsistent coverage and payment policies risk exacerbating health disparities. ARM encourages early engagement and collaboration between the developers of CGTs and payers. This can involve pre-submission discussions to align on coverage and reimbursement considerations.

Positive coverage decisions and adequate reimbursement structures in federal programs have a cascading effect on commercial payers directly impacting access to CGTs. Many commercial payers reference Medicare reimbursement rates as a benchmark (+/-) to build their own case rates. For this reason, it is critical that Medicare's MS-DRG rates fully encompass the value of novel CGTs. Commercial payers also look to Medicare for coverage policy direction; notably many watched CMS' national coverage analysis of CAR-T to inform their coverage policies after the first CAR T-cell therapies were approved. Therefore, federal payers should ensure robust coverage of FDA-approved therapies, as such policies are likely to be emulated by private payors, including ERISA plans.

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?

Appropriately structured VBAs are a critical and market-based approach to addressing payer uncertainty leveraging real-world efficacy that supports the durability and value of these cutting-edge therapies. These agreements are designed to support risk-sharing by aligning financial incentives with the value of a treatment. These arrangements shift some of this risk to the manufacturer by linking payment to the therapy's actual performance in improving patient health or other pre-determined outcomes. Manufacturers are incentivized to continually improve the efficacy and safety of their therapies. The prospect of higher reimbursement tied to better outcomes encourages ongoing monitoring and development efforts to enhance the overall performance of the treatment.

Often, value-based arrangements are predicated on meeting specific clinical or economic endpoints that require ongoing monitoring after administration of the negotiated therapy. Many patients undergoing treatment with CGTs contend with disabilities or possess unique medical requirements. Employee mobility results in patient churn which presents distinct obstacles to effectively implementing Value-Based Agreements (VBAs) for beneficiaries of ERISA plans. These hurdles can detrimentally affect the

continuum of care, the evaluation of treatment outcomes, and the overall efficacy of risk-sharing arrangements. Challenges such as patient disengagement, outcome tracking difficulties, fragmented data systems, intricate attribution models, potential for selection bias, administrative complexities, and privacy concerns are commonly associated with patients seeking eligibility for CGTs. Resolving these issues necessitates collaborative endeavors among healthcare stakeholders, including providers, payers, regulatory entities, and biotechnology companies, to establish standardized data-sharing protocols, bolster interoperability, and devise strategies for monitoring patient outcomes during care and coverage transitions.

Medicaid best price requirements can hinder the use of VBAs in the commercial space. For example, if a manufacturer sought to offer a commercial plan a VBA under which the manufacturer must provide a deep discount in instances where the drug fails to produce a desired outcome, the manufacturer would have to offer that same discount (in the form of a substantial rebate) to all states for al of their Medicaid utilization, regardless of the specific outcomes among actual Medicaid patients. The potential for VBAs to set a product's "best price" creates a significant disincentive to VBA adoption. CMS issued a regulation in 2020 to address this by creating a "Multiple Best Prices" policy, which would be codified by the Medicaid VBP for Patients (MVP) Act (H.R.2666).[6] While uptake has been limited to date, these policies ensure manufacturers do not owe outsized rebates for offering steep discounts, or even full refunds, in the event a product fails to satisfy the applicable outcomes measures under a VBA. The MVP Act similarly ensures that Average Sales Price calculations are not distorted due to discounts offered in good faith under a VBA, avoiding inappropriate pay cuts to Medicare physicians.

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

Payers are utilizing coverage restrictions and utilization management practices to limit access to specialty drugs. Coverage determinations and prior authorizations must be evidence based and developed with input from appropriate medical specialists; however, this is often not the case, particularly for highly specialized CGTs. The medical necessity and appropriateness of a CGT should be determined by the patients' treating clinicians. Lawmakers should continue to explore policies that protect patients from inappropriate coverage denials and unscrupulous utilization management practices that prevent the timely provision of necessary medical care.

Increasingly, in cases involving high-cost drugs, self-funded plans governed by ERISA have turned to Alternative Funding Programs (AFPs). In AFPs, specialty drugs are excluded from the plan's formulary, the patient then technically has "no drug coverage". A third-party vendor helps the patient disguise themselves as "uninsured," and the patient then applies for the manufacturer's patient assistance program funds (which can be used for commercial or public payers). These prescriptions sometimes are mandated to be filled by external specialty pharmacies linked to the payer; however, despite these patients having full prescription drug coverage, the manufacturer ends up paying for the full cost of the prescription. Meanwhile, the plan sponsor incurs no costs for the specialty drug and the third-party vendors retain up to 20% to 25% of a drug's full list price. A 2022 study found that 10% of employers with at least 5,000 employees were using AFPs and another 27% were considering them. This practice

[6] HR 2666. Medicaid VBPs for Patients Act Accessible at: https://www.congress.gov/bill/118th-congress/house-bill/2666/text

leads to the redistribution of need-based funds away from the underinsured patients they were originally developed for.[7] Policymakers should examine the prevalence and impact of these types of arrangements on affordability and access to specialty drugs for patients in need.

*****

We thank you for your interest in reducing barriers for employers seeking to increase access to specialty drugs, including CGTs, for their employees. ARM looks forward to continued engagement with you as the Committee considers legislative options to advance our shared priorities.

Sincerely,

Erica Cischke, MPH

Vice President, Government Affairs

[7] Employer Market Trends Report. Gallagher Research Trends and Insights. (June 2022) Accessible at: https://www.benfieldresearch.com/pdf/2022%20Gallagher%20Research%20&%20Insights_Employer%20Market%20Trends.pdf