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Cell and Gene Therapies in Employment-Based Health Insurance: Financing the High-Cost, High-Impact Future

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AT A GLANCE

Cell and gene therapies (CGTs) are a relatively new frontier in medicine, offering the potential to treat or potentially cure rare diseases by targeting their root causes at the cellular or genetic level. These therapies can dramatically improve a patient's quality of life, but they come with extremely high costs. While only a limited number of CGTs are approved, their use is growing, and more therapies are in the pipeline. As a result, the financial implications for employers and employment-based health plans are likely to increase over time.

In this *Issue Brief*, we use claims data to examine the evolving landscape of CGTs within employment-based health plans. We analyze the prevalence of conditions targeted by CGTs, patterns in therapy utilization, and the broader spending implications for enrollees who use these therapies. Our analysis further highlights the disproportionate concentration of CGT-related costs among a small subset of high-cost claimants and explores emerging strategies that employers and insurers may consider to manage the associated financial risks.

Key Findings:

- **Rising Number of Approvals:** As of 2025, the U.S. Food and Drug Administration (FDA) has approved 48 CGTs, with more in development. A growing number of these therapies are targeting conditions that are more common than traditional rare diseases.
- **Modest Growth in Use:** Use of CGTs remains limited but has shown a gradual increase. In 2018, 7.9 per 100,000 enrollees received a CGT; by 2022, the rate rose to 9.2 per 100,000.
- **Small Share of Spending:** CGT users represent fewer than one-tenth of one percent of the enrollee population but account for approximately one-half of one percent of total spending. This includes both the cost of CGTs and spending on other health services. These proportions have remained relatively stable between 2018 and 2022.
- **Impact on High-Cost Claimants:** Among enrollees in the top 1 percent of total health care spenders, just 0.58 percent used CGTs. However, these individuals accounted for 1.6 percent of spending within that top-spending group. While the share is small, it signals that CGTs can meaningfully impact spending patterns among high-cost claimants.

As CGTs evolve from rare to more common disease applications, their financial footprint within employment-based health plans is likely to grow. Employers are beginning to explore innovative solutions to manage these costs, including stop-loss insurance, carve-out programs, gene therapy reinsurance, value-based payment arrangements, and performance-based contracts.

Understanding the trajectory and implications of CGTs is critical for employers, policymakers, and health plan sponsors. While current utilization and spending remain limited, the pipeline of therapies and their high costs warrant close attention to ensure sustainable access and benefit design in the years ahead.

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Introduction

One of the crucial statistics in health care is the 80/20 rule — approximately 20 percent of the population account for 80 percent of the spending. The statistic applies to the population with employment-based health benefits as well. For example, in 2022, 20 percent of the population accounted for 84 percent of the total spending (Figure 1). Average spending in this group was $30,000, though median spending was $15,000. High users — often referred to as high-cost claimants, the costliest patients, or top spenders in the health system — are commonly individuals with significant, intensive, and complex medical needs. These individuals usually require ongoing or intensive care for chronic conditions. Definitions for high-cost claimants typically rely on annual health care spending thresholds or percentile-based cutoffs, but it can be agreed that a small segment of the population uses a lot more health care than the general population.

One group of individuals requiring ongoing or intensive care for a chronic condition is those who use cell and gene therapies (CGTs). CGTs are two cutting-edge approaches in medicine that aim to treat or potentially cure diseases by altering cells or genetic material within a patient. Very few people will ever receive a CGT. In 2020, 16,244 new patients were treated with a gene therapy (Wong et al., 2023); they accounted for just 0.0049 percent of the population. Yet their substantial price tags — combined with additional costs incurred during the diagnostic, treatment, and post-treatment phases — make employers aware of the financial risks, as 57 percent report that managing those costs is a top priority in their future pharmacy benefits (Belousova, Brown, and Peel, 2025).

This *Issue Brief* uses claims data to examine the current landscape of cell and gene therapies (CGTs), including the prevalence of treatable conditions and overall spending by individuals receiving these therapies. It also analyzes the share of CGT-related spending among users across various cost thresholds. Finally, the paper briefly explores potential strategies employers may consider for financing CGTs moving forward.

Figure 1
Distribution of Health Spending, Among Individuals With Employment-Based Health Coverage, 2022

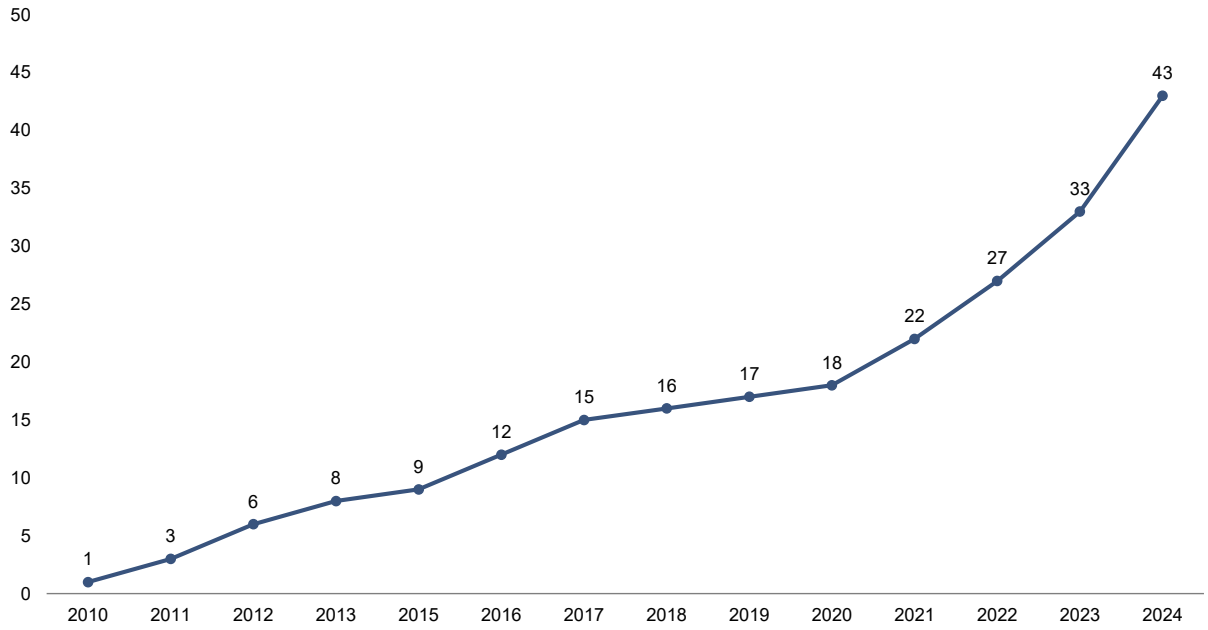
Percentage of Enrollees	Percentage of Spending	Median Spending per Person	Average Spending per Person	Minimum Spending per Person
1%	29%	$150,000	$206,000	$96,000
5%	57%	$51,000	$81,000	$29,000
10%	71%	$29,000	$51,000	$15,000
20%	84%	$15,000	$30,000	$6,000

Source: EBRI estimates using 2022 Merative™ MarketScan® Commercial Database.

Current State of Cell and Gene Therapy

Cell and gene therapies (CGTs) are a relatively new frontier in medicine, offering the potential to treat or potentially cure diseases by targeting their root causes at the cellular or genetic level. Cell therapies work by introducing new or modified cells into the body to restore function or fight disease. Gene therapies involve altering a person's genetics to correct or compensate for faulty genes. The U.S. Food and Drug Administration approved the first CGT in 2010 (Figure 2). By 2025, 48 CGTs had been approved.

Figure 2
Cumulative Number of Cell and Gene Therapy Approvals, 2010–2024



Source: www.fda.gov/vaccines-blood-biologics/cellular-gene-therapy-products/approved-cellular-and-gene-therapy-products

Because of the complex and time-intensive research, the uncertainty in the success of clinical trials, and the individualized or small-batch nature of CGT manufacturing, the cost of developing CGTs is significantly higher than that of traditional pharmaceuticals. Cell therapies typically range between $400,000 and $500,000 per treatment, while many gene therapies are priced over $1 million, and several are priced at $2–3 million (Belousova, Albers, Ryzmo, Peel, and Paik, 2025).

Beyond the cost of the CGT itself, patients often incur substantial health care expenses throughout their health care journey, making CGT users among the highest-cost claimants. A recent report by (Wagner, Sils, and Campbell, 2025) describes this process as a five-stage health care "odyssey" that can span several years. Notably, it can take up to seven years to receive an accurate diagnosis for a rare disease.

One of the five stages in the health care journey of CGT users is the first stage of "Awareness and Diagnosis." Patients' first point of contact is typically a primary care provider. Provider knowledge of rare diseases may be limited, and thus, patients may visit multiple primary care providers and specialists. This process could take months or years, leading to significant use of health care services before a formal diagnosis is made. During this prolonged period — from symptom onset to diagnosis — patients often generate significant health care costs (The Lewin Group, Inc. 2023). And once a diagnosis is made, a patient is not always deemed eligible to receive a CGT.

Although the number of approved CGTs and treated patients remains relatively small, the landscape is evolving. Newer therapies are being developed for broader indications, including more common diseases (Braga, Filho, and Mota, 2022), which could expand the number of eligible patients (Wong et al., 2023). By 2032, 85 new gene therapies are expected to be approved (Phares, Trusheim, Emond, and Pearson, 2024). This expansion has implications for employers and health plans.

As CGT use expands, traditional cost-control mechanisms may prove inadequate. For example, deductibles, long used to manage health plan spending, are unlikely to significantly affect costs for CGT users. Most deductibles fall far below the typical minimum $6,000 in annual spending by high-cost claimants (Fronstin and Roebuck, 2019). The high cost of CGTs ensures that health plans bear the majority of financial responsibility, even when enrollees meet their deductibles.

As more CGTs are developed and approved for a wider range of conditions, employment-based health plans will likely face growing financial pressures. CGT-related spending includes not only the cost of the therapy, but also expenses incurred during pre-diagnosis, administration, and post-treatment monitoring. Given the scale and pace of CGT development (Wong et al., 2023), traditional methods of managing health care costs — such as deductibles, copayments, and coinsurance — may no longer be sufficient to address the financial challenges CGTs pose.

Ultimately, the approval and adoption of CGTs will force payers — particularly employers — to reevaluate how they finance health care. As more patients become eligible and more therapies enter the market, employers will need to adopt new financing strategies to ensure the sustainability of health benefits.

Data and Study Sample

For this study, we analyzed data from the MarketScan® Commercial Database covering the years 2018 to 2022. The database includes detailed enrollment records along with inpatient, outpatient, and pharmacy claims for policyholders and their spouses and dependents. We limited our analysis to individuals under age 65 who maintained continuous enrollment for a full calendar year to avoid inconsistencies associated with partial-year claims. Although individuals may receive CGT across multiple years, we treated each year as a separate cross-sectional observation. The annual sample size ranged from approximately 13 million to 19 million individuals.

Identifying CGT Usage

Of the 48 CGTs approved by FDA to date, 27 had received approval by 2022. However, not all of these therapies were included in our analysis due to a number of limitations. For instance, two CGTs were excluded because they were approved in late 2022, while others could not be identified in the claims data due to the absence of specific billing codes. Additionally, some therapies were no longer available in 2022. Ultimately, we were able to analyze 12 CGTs and eight FDA-approved hematopoietic progenitor cell (HPC) cord-blood therapies in our study.

A key step in our analysis involved identifying individuals in the sample who received a CGT. To do this, we relied on several coding systems commonly used in administrative claims data:

- **Healthcare Common Procedure Coding System (HCSPCS)**: a set of medical codes used to represent health care procedures, supplies, products and services.
- **International Classification of Diseases, 10th Revision Procedure Coding System (ICD-10 PCS):** a system used to classify medical procedures performed in hospitals and inpatient settings.
- **Current Procedural Terminology (CPT):** used to identify medical services and procedures.
- **National Drug Code (NDC):** a unique 10-digit identifier that specifies the labeler, product, and package size of a drug.

We gathered information on relevant codes from multiple sources, including the Centers for Medicare and Medicaid Services (CMS) payment bulletins, individual drug websites, and widely used medical coding resources. Because CGTs are used to treat rare conditions, the number of enrollees receiving any single therapy was small. As a result, our analysis combines cell and gene therapy users into one group.

Calculating Spending From Health Care Claims

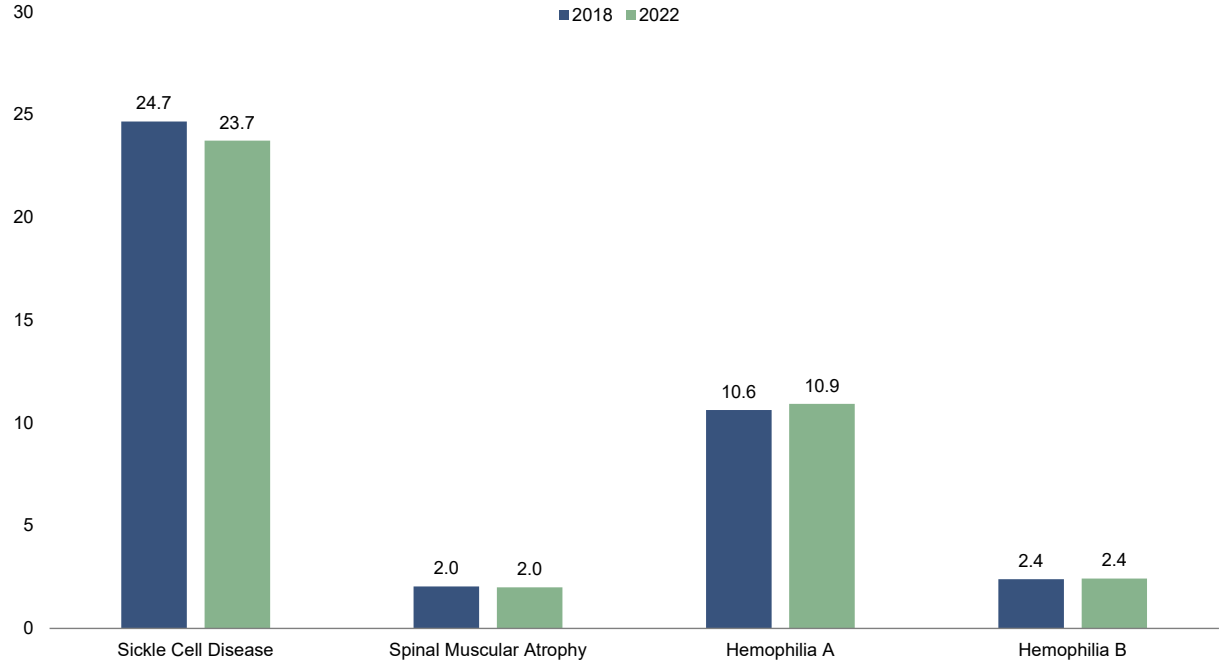
The financial impact of CGTs extends beyond the direct cost of the therapies themselves, encompassing a range of associated expenses, as discussed above. To capture the full scope of these costs, we aggregated total spending for each enrollee. Specifically, we summed the allowed amounts from inpatient, outpatient, and pharmacy claims to calculate annual total spending per enrollee.

Findings

Prevalence of Select Conditions Treated With CGTs

CGTs are used for the treatment of a few rare conditions. The number of patients in the United States for some of these rare conditions is very small, which means that even in a dataset of 13 to 19 million enrollees, we are dealing with small sample sizes. Furthermore, some CGTs have specific requirements for treatment like relapsed or refractory multiple myeloma, which can be challenging to identify in the data. Despite the challenges, we have identified enrollees with rare diseases and can determine the prevalence of a number of conditions, such as sickle cell disease, spinal muscular atrophy, and hemophilia, from the data. Among individuals with employment-based health coverage in 2022, the prevalence of sickle cell disease was 23.7 per 100,000 enrollees, spinal muscular atrophy had 2 cases per 100,000 enrollees, hemophilia A was 10.9 per 100,000 enrollees, and hemophilia B was 2.4 per 100,000 enrollees in the data (Figure 3).

Figure 3
Prevalence of Select Conditions, per 100,000 Enrollees With Employment-Based Health Coverage



Source: EBRI estimates using 2022 Merative™ MarketScan® Commercial Database.

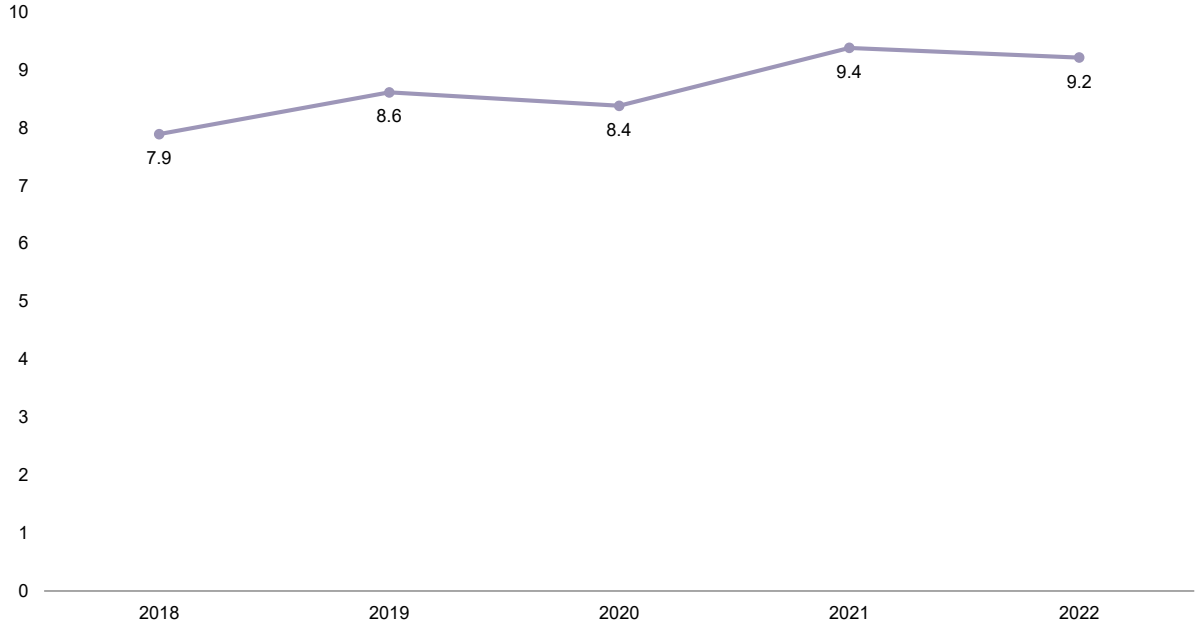
Trends in the Use of CGT

Since CGTs are associated with the treatment of rare diseases, use of CGTs is also rare. The number of CGT users has increased slightly over the years. In 2018, CGT use was 7.9 per 100,000 individuals. It increased to 8.6 per 100,000 individuals in 2019, 8.4 in 2020, and 9.4 in 2021, dropping slightly to 9.2 per 100,000 individuals in 2022 (Figure 4).

After the increase in CGT use from 2018 to 2019, there was a dip in use in 2020. The decline may have been related to the COVID-19 pandemic, which put a significant strain on the health care delivery system. There was a shortage of equipment, and hospital resources like beds and medical ventilators were prioritized for COVID-19 patients. Some CGTs, such as CAR-T cell therapies, require continuous monitoring and evaluation of the patient, which was not possible due to constrained resources. The disruption of activities with travel restrictions, concerns of transmission of COVID-19 to patients, and supply constraints could have been the reason for decreased use of CGTs in 2020 (Qiu, Wang, Liang, Han, and Toumi, 2021).

More generally, as an increasing number of CGTs have been approved, their usage has increased as expected. However, as we have noted earlier, diseases treated by CGTs are very specific and rare. When a new CGT is approved and becomes available, there is a surge in usage due to a pool of patients waiting for treatment. However, as these diseases are rare, once the existing pool of individuals get treated, the annual number of patients receiving the treatment may decrease.

Figure 4
Number of Cell and Gene Therapy Users, per 100,000 Enrollees, 2018–2022



Source: Employee Benefit Research Institute estimates based on administrative enrollment and claims data.

Spending Among Enrollees Who Are High-Cost Claimants

Overall, CGT users accounted for just under 0.1 percent of enrollees, and about 0.5 percent of total spending. This includes spending on CGTs as well as spending on other health care services. This number was in large part unchanged between 2018 and 2022.

Among the overall top 1 percent of spenders, 0.58 percent used CGT (Figure 5). They accounted for 1.6 percent of spending among those in the top 1 percent of spenders. In other words, CGT users still account for a small share of spending among the highest users of health care services.

Figure 5
Spending on Health Care Services, by Use of Gene Therapy, Among Top Spenders, 2022

Spending Tier	Percentage of Enrollees Using Gene Therapy	Mean Spending of Gene Therapy Users	Mean Spending of Enrollees Not Using Gene Therapy	Median Spending of Gene Therapy Users	Median Spending of Enrollees Not Using Gene Therapy	Share of Spending Accounted for by Gene Therapy Users
1%	0.58%	$569,000	$204,000	$468,000	$149,000	1.60%
2–5%	0.04%	$58,000	$50,000	$58,000	$44,000	0.05%
6–10%	0.02%	$21,000	$20,000	$21,000	$20,000	0.02%
11–20%	0.01%	$11,000	$10,000	$10,000	$9,000	0.01%

Source: EBRI estimates using 2022 Merative™ MarketScan® Commercial Database.

Financing CGTs

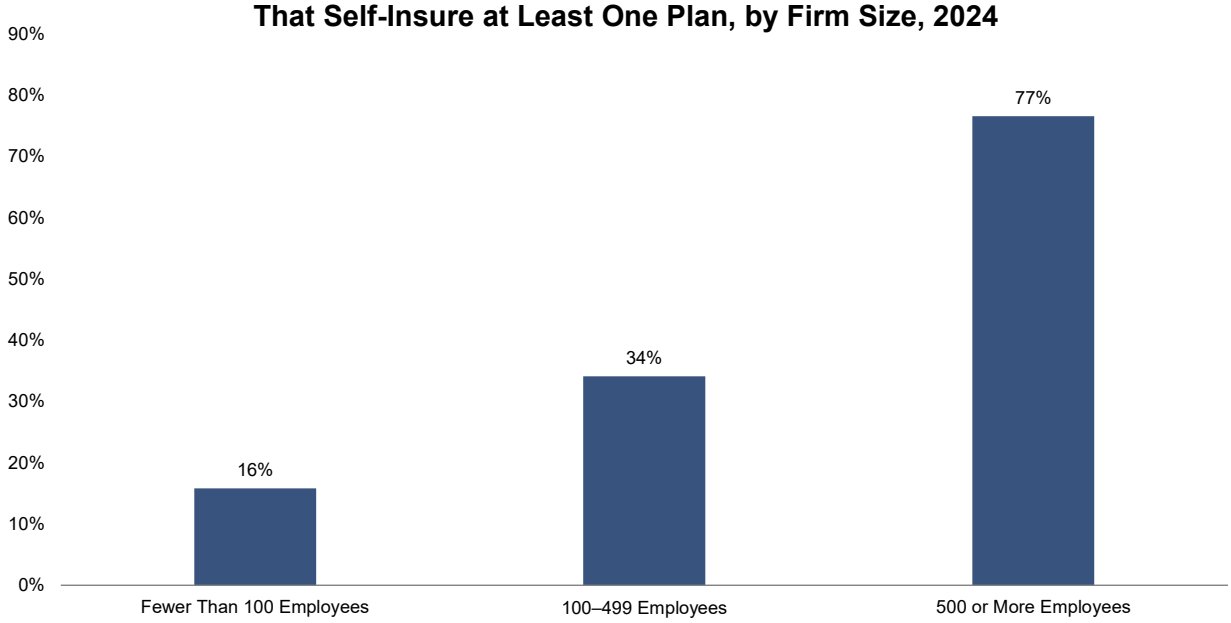
To manage health benefits costs, many employers have adopted high-deductible health plans (HDHPs). As of 2025, HDHPs must have deductibles that are no less than $1,650 for employee-only coverage and $3,300 for family coverage. With their growing prevalence, HDHPs have become a focal point in discussions around employer cost-sharing strategies. However, employers use a variety of cost-sharing mechanisms beyond deductibles to influence employee behavior and manage spending. These include copayments, coinsurance, tiered cost structures for providers and prescription drugs, value-based insurance design, and reference pricing.

Deductibles are typically much lower than the $6,000 or more in annual spending seen among the 20 percent of individuals who account for approximately 80 percent of total health care costs. Moreover, out-of-pocket maximums — which encompass all forms of cost sharing — are relatively insignificant in the context of total spending for high-cost users, particularly those receiving CGTs. As a result, deductibles and other cost-sharing mechanisms often have little to no effect on how these individuals use health care services. Employers seeking to manage the financial impact of enrollees receiving CGTs will need to explore alternative strategies beyond traditional cost-sharing models.

Employer Size Considerations

The financial impact of a CGT claim can vary significantly depending on the size of the employer. Larger employers often self-insure their health plans, meaning they take on the financial risk themselves and directly pay the cost of claims incurred by their covered employees. In contrast, smaller employers are more likely to offer fully insured plans, where they pay premiums to an insurance company that assumes the financial risk. In fact, only 16 percent of private-sector employers with fewer than 100 employees were self-insured in 2023, compared with 74 percent among employers with 500 or more employees (Figure 6). Consequently, CGT claims may have a more immediate and direct budgetary impact on self-insured employers than on those with fully insured plans.

Figure 6
Percentage of Private-Sector Establishments Offering Health Plans That Self-Insure at Least One Plan, by Firm Size, 2024



Source: Fronstin, Paul, "Self-Insured Health Coverage From 1996 to 2024," EBRI Issue Brief, no. 642 (Employee Benefit Research Institute, August 28, 2025). Available at www.ebri.org/health/publications/issue-briefs/content/summary/self-insured-health-coverage-from-1996-to-2024.

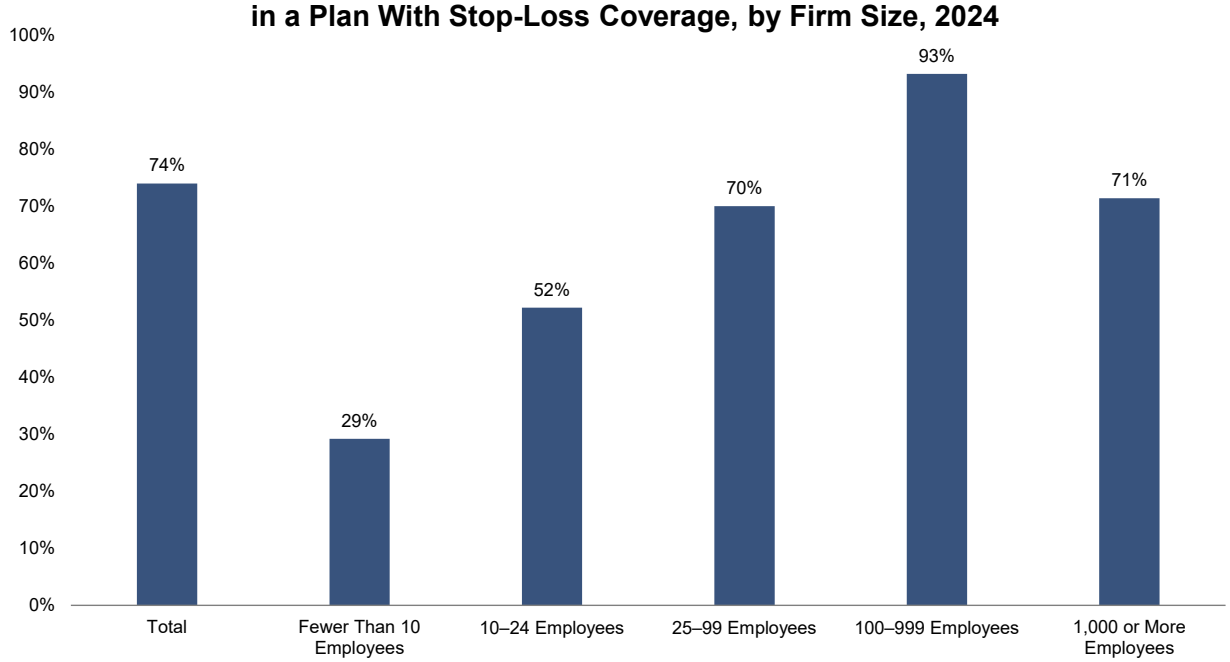
Self-Insurance and Stop-Loss Coverage

Employers that self-insure their health plans often purchase stop-loss insurance to protect *themselves* from unexpectedly large claims. Stop-loss coverage reimburses the employer for claims that exceed predetermined thresholds. Importantly, stop-loss insurance is not medical coverage and does not provide any direct protection or benefits to workers.

Unlike the steady increase in the percentage of employers who self-insured as firm size grew, the prevalence of stop-loss coverage among self-insured employers did not follow a similar pattern, instead varying significantly by firm size. In 2024, employees in firms with 100–999 employees were most likely to be in plans with stop-loss coverage (93 percent). Coverage rates were lower in both directions on the firm-size scale: 70 percent for firms with 25–99 employees, 71 percent for firms with 1,000 or more employees, 52 percent for firms with 10–24 employees, and 29 percent for firms with fewer than 10 employees. The low coverage rates among the smallest firms may reflect a lack of awareness of their stop-loss arrangements, confusion about the terminology, and the affordability and volatility of the stop-loss premium. At the other end, large firms may forgo stop-loss because they can spread the cost of high claims over a large work force (Figure 7).

It should come as no surprise that the largest employers are the least likely to purchase stop-loss coverage, as they benefit most from the law of large numbers — a fundamental principal in insurance. This concepts states that as the size of a population increases, the average outcome becomes more predictable. In the context of health care, large employers with many covered employees can better anticipate overall health care costs and are less affected by the financial shock of a single high-cost claim. By spreading risk across a broad base of enrollees, they can absorb the cost of outlier events, such as claims for CGTs. In contrast, smaller employers, with fewer enrollees to distribute risk, face greater financial volatility from large claims and are therefore more likely to purchase stop-loss coverage as a protective measure.

Figure 7
Among Self-Insured Plans, Percent of Private-Sector Workers Enrolled in a Plan With Stop-Loss Coverage, by Firm Size, 2024



Source: Fronstin, Paul, "Self-Insured Health Coverage From 1996 to 2024," EBRI Issue Brief, no. 642 (Employee Benefit Research Institute, August 28, 2025). Available at www.ebri.org/health/publications/issue-briefs/content/summary/self-insured-health-coverage-from-1996-to-2024.

Illustration: Impact of High-Cost Claimants by Employer Size

To illustrate how a single high-cost claimant can affect employers differently based on their size, consider the following example. In 2023, the average annual premium for employee-only coverage among employers with 1,000 or more employees was approximately $8,300.[1] For an employer with 1,000 covered employees, total health care spending would amount to about $8.3 million. For an employer with 100,000 enrollees, that figure would scale to $830 million.

Now, assume a high-cost claimant — such as an enrollee receiving a CGT — incurs $1 million in health care costs. Based on the prevalence rate of 9.2 CGT users per 100,000 individuals with a rare condition (Figure 4), the large employer with 100,000 enrollees would be expected to have about nine such high-cost claimants (Figure 8). The total cost of these claims would represent roughly 1.1 percent of the employer's overall health care spending. By contrast, the smaller employer with 1,000 employees would be expected to have 0.092 such enrollees — less than one. For the purposes of this illustration, we round up and assume the smaller employer has one high-cost claimant. That $1 million claim would represent 12 percent of total spending.

If both employers were to experience one additional $1 million claimant, the smaller employer would now have two high-cost claimants, whose combined costs would account for 24 percent of total spending. Meanwhile, the large employer would have 10 high-cost claimants accounting for only 1.2 percent of overall spending.

Figure 8
Illustrated Impact of High-Cost Claimants, by Employer Size

Number of Employees	**1,000**	**100,000**
Average Spending per Employee	$8,300	$8,300
Total Spending	$8,300,000	$830,000,000
High-Cost Claimant Experience		
Predicted Number of High-Cost Claimants	0.09	9.2
Actual Number of High-Cost Claimants	1	9
Average Spending per High-Cost Claimant	$1,000,000	$1,000,000
Total Spending on High-Cost Claimants	$1,000,000	$9,000,000
Percent Spent on High-Cost Claimants	12%	1.1%
Impact of Additional High-Cost Claimant		
Actual Number of High-Cost Claimants	2	10
Total Spending on High-Cost Claimants	$2,000,000	$10,000,000
Percent Spent on High-Cost Claimants	24%	1.2%

Source: EBRI estimates using 2022 Merative™ MarketScan® Commercial Database.

This example highlights how health care expenses related to rare, high-cost conditions have a disproportionately larger financial impact on smaller employers. It helps explain why smaller self-insured employers are more likely to purchase stop-loss coverage — to protect themselves from the outsized risk that even a single high-cost claimant can pose.

While stop-loss insurance can help protect self-insured employers from catastrophic claims, it is not a perfect solution. The vast majority of stop-loss carriers are covering CGT claims like other claims (Belousova, Albers, Ryzmo, Peel, and Paik, 2025). However, not all high-cost claims are fully covered under these policies. In particular, stop-loss carriers may limit coverage for specific individuals with known high-cost preexisting conditions through a practice known as "lasering." Under lasering, the policy's deductible is set at or near the expected cost of treating a particular enrollee, effectively excluding or minimizing reimbursement for that individual's care. This technique is often used to manage stop-loss premium costs. Conditions frequently subject to lasering include cancer, end-stage renal disease, premature births, and organ transplants.

Lasers are more commonly applied when a stop-loss policy is newly underwritten and are less frequently used at renewal — particularly if the carrier has developed a claims history with the employer. In addition, since CGTs are intended as one-time treatments, these claims are often not considered as part of an employer's claims history at the time of renewal.

Some stop-loss carriers also offer stand-alone coverage options that provide protection for specific high-cost services, such as CGTs. These policies typically do not use lasers and may offer a more appropriate solution for coverage treatments like CGTs. They may be more appealing to large employers who do purchase stop-loss.

Other Financing Options for Employers

An important consideration when evaluating stop-loss coverage is that it typically applies to all health care costs incurred by an enrollee, not just the cost of the CGT. While stop-loss policies receive significant attention — largely because they are already well-established and familiar tools for larger employers — other financing mechanisms are emerging that specifically target the unique financial challenge posed by CGTs.

As outlined by (Horrow and Kesselheim, 2023), three primary approaches to financing CGTs are emerging: amortization, risk spreading, and performance-based payments.[2]

1. Amortization

Amortization allows employers to spread the cost of a CGT over time, rather than paying the full amount upfront. Payments can be structured evenly over a period of years or front-loaded with smaller payments thereafter. These arrangements can be made directly with the CGT manufacturer or financed through a third-party lender. The goal is to align the cost of the therapy with the period over which its benefits are realized, potentially easing budget strain.

2. Risk Spreading

Risk-spreading models distribute the financial impact of CGTs across multiple entities, much like traditional insurance. Employers could contribute to a shared fund or purchase reinsurance designed specifically for CGT coverage. Contributions would be based on group size and the projected incidence of CGT usage, which becomes more predictable across larger populations. This model is particularly attractive for smaller self-insured employers that lack the scale to absorb rare, high-cost claims on their own.

3. Performance-Based Payments

Also known as value-based purchasing or pricing, this approach ties payments for CGTs to clinical outcomes. Employers, insurers, or pharmacy benefit managers (PBMs) would only pay the full price of a CGT if it proves effective based on pre-defined patient- or population-level metrics. While implementing value-based arrangements broadly remains challenging — as discussed in (Fronstin and Roebuck, 2025) — CGTs may be more conducive to outcome-based pricing because their therapeutic effects are often significant and more measurable than those of other treatments. However, there have been challenges that have limited the effectiveness of performance-based payments (Belousova, Albers, Ryzmo, Peel, and Paik, 2025).

Study Limitations

This study focused on CGTs, which are used to treat rare conditions. As such, several limitations should be noted:

1. Small Sample Size: Due to the rarity of the conditions treated with CGTs, the number of enrollees receiving these therapies in our dataset was very low. In particular, the number of individuals with total spending exceeding $1 million was in the single digits, limiting the robustness of analyses at higher spending thresholds.

2. Potential Undercounting of CGT Use: Some CGT users may have been missed due to incomplete or unavailable procedure and drug coding in the claims data. As a result, certain individuals classified as non-users may in fact have received a CGT.

3. Inability to Analyze Individual Therapies: Because of the limited number of CGT users identified, we did not conduct cost analyses at the level of individual therapies.

These limitations underscore the challenges of studying rare, high-cost interventions in administrative data and highlight the need for improved coding practices and expanded access to comprehensive datasets.

Conclusion

The growing use of CGTs presents both a transformative opportunity in medical treatment and a financial challenge for employment-based health plans. These therapies offer the potential for life-altering or even curative outcomes, particularly for individuals with rare conditions. However, their substantial price tags — combined with additional costs incurred during the diagnostic, treatment, and post-treatment phases — make employers aware of the financial risks, as 57 percent report that managing those costs are a top priority in their future pharmacy benefits (Belousova, Brown, and Peel, 2025).

While the prevalence of rare diseases treated by CGTs remains low, FDA approvals and real-world utilization have steadily increased. Despite their limited use, CGT users may represent a disproportionate share of overall health care spending. Traditional cost-sharing mechanisms such as deductibles and out-of-pocket maximums will have little effect on use of health care services among high-cost claimants, including those receiving CGTs.

Employer size plays a critical role in how financial risk is managed. Larger, self-insured employers benefit from the law of large numbers, allowing them to absorb high-cost claims by distributing expenses across a large enrollee base. In contrast, smaller employers are more financially vulnerable and often depend on stop-loss coverage to mitigate risk. However, stop-loss coverage is an imperfect solution and may exclude coverage for known high-cost individuals through mechanisms like "lasering."

To address the unique financial risks posed by CGTs, additional financing models — such as amortization, risk-spreading mechanisms, and performance-based payment arrangements — are emerging as other options. These strategies may offer more targeted, sustainable ways to support access to CGTs while managing costs. However, in the context of CGTs, these financing strategies may be promising but are in large part unproven. If they fail to prove workable, payers may be left relying on traditional reimbursement approaches to address the costs associated with CGTs.

Future research should continue to explore the full spectrum of health care spending associated with CGT users, including the breakdown of inpatient, outpatient, and pharmacy-related costs. A better understanding of these patterns will be crucial for employers, insurers, and policymakers seeking to balance innovation with financial sustainability.

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Endnotes

[1] See Table I.C.1 in https://meps.ahrq.gov/data_stats/summ_tables/insr/national/series_1/2023/ic23_ia_g.pdf.

[2] See citations in Horrow and Kesselheim (2023) for the references they used.