EMPLOYER SNAPSHOT

MBGH
Midwest Business Group on Health

The World of High-Cost Cell & Gene Therapies (CGTs)

Breakthrough Therapies at a Breaking Point

The Evolution of High-Cost Therapies

Over the past two decades, the pharmaceutical landscape has undergone a dramatic transformation. The era of "pills in a bottle"—dominated by small-molecule drugs designed to treat chronic conditions—has evolved into one defined by innovative biologics, specialty drugs, and most recently, cell and gene therapies (CGTs) targeting rare, orphan, and complex diseases. These groundbreaking treatments hold the promise of one-time, potentially curative outcomes, but they come with unprecedented price tags—often ranging from hundreds of thousands to several millions of dollars per treatment.

Beyond the therapy itself, costs continue to rise due to necessary ancillary services such as hospitalization, specialized medical care, and patient travel.[1] Employers also face indirect costs tied to lost labor and productivity. Collectively, these factors have created serious financial challenges for employers, who are increasingly burdened by catastrophic claims from patients requiring these therapies.

The evolution of high-cost therapies has far outpaced traditional medical and pharmacy benefit models, which were built to support high-volume, population-level treatments for chronic conditions. In contrast, today's therapies require individualized, precision medicine approaches that introduce unprecedented financial instability for employers.

Faced with these challenges, many employers are left with few viable options—and some are choosing not to cover them.

An Overview of Cell & Gene Therapies

CGTs, rare disease treatments, and Orphan Drug Designations (ODDs) are closely related areas within the emerging category of high-cost therapies. These innovations are increasingly being developed to address rare diseases and unmet medical needs—conditions that often lack effective treatment due to small patient populations and the high cost of research and development. CGTs represent a groundbreaking approach that targets the underlying cause of disease, rather than just managing symptoms. Their goal is to improve health outcomes, slow or halt disease progression, and potentially cure disease by repairing or replacing damaged or defective cells or genes.

- **Cell Therapy** involves the transfer of whole, living cells into a patient to replace or repair damaged tissues, or to fight disease with the primary goal of modifying cellular function directly. The cells themselves are the medicine, with typical use cases including oncology, regenerative medicine and autoimmune disorders.
- **Gene Therapy** involves the introduction, removal, or alteration of genetic material (DNA or RNA) within a patient's cells to treat or prevent disease, with the primary goal of modifying the genetic instructions that cause disease. Therapeutic effects come from changes to the cell's genes, leading to altered protein production or function. The genes themselves are the medicine, with typical use cases including genetic diseases, enzyme deficiencies, blood disorders, and some cancers.
- Some treatments combine both — for example, CAR-T therapy is a cell-based gene therapy, since T cells are genetically modified before being reinfused.

Many CGTs qualify for expedited regulatory pathways and orphan drug status, given their potential to transform outcomes for patients with rare or previously untreatable conditions.

The Benefits of CGTs

CGTs offer the potential for curative benefits by directly addressing the underlying genetic or cellular causes of disease, rather than simply managing symptoms. This marks a fundamental shift from traditional treatments, enabling long-term—or even permanent—resolution of conditions once considered incurable. While costs remain a key consideration for employers, it is important to recognize that these therapies can be truly life-changing for members.

Current and Emerging Cost Challenges

The U.S. pipeline for CGTs is robust – see the links to approved therapies and those in the pipeline below.

FDA Approved CGT Products

American Society of Cell & Gene Therapy – CGT Pipeline Report

CGTs are typically administered at specialized centers, often requiring coverage for travel, lodging, and related services—adding significant costs beyond the therapy itself.

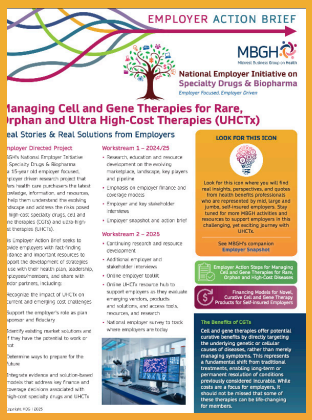
Because most of these therapies face limited or no market competition, there is little to no formulary leverage and few, if any, rebate opportunities. Many are launched by smaller biotechnology companies that lack established contracts with PBMs or carriers,

which can complicate billing and reimbursement processes. When therapies are distributed directly by manufacturers or novel specialty pharmacies, they may be considered out-of-network, offered without negotiated discounts, or even have claims denied—further increasing costs and creating administrative challenges for employers and members alike.

Traditional pharmacy benefit management tools—such as formularies, step therapy, and prior authorization—are largely ineffective in this context due to the low patient volume, high clinical complexity, and the need for coordinated care through specialized centers and ancillary services.[1]

For a Deeper Dive…

See MBGH's companion Employer Action Brief, **Managing Cell & Gene Therapies for Rare, Orphan and High-Cost Therapies** that includes employer insights, along with action steps to support potential financing and coverage models for novel and curative CGTs.



MBGH encourages employers to stay tuned for upcoming educational programs, resources, and real-life employer stories to support their efforts in effectively managing CGTs. These initiatives aim to help employers learn, understand, and address the challenges of these high-cost therapies, enabling them to make informed decisions about financing and coverage. For more information, contact info@mbgh.org.

Employer Cost Impacts

The pipeline for CGTs is robust. This growth will increase both the frequency and financial impact of high-cost claims, prompting employers to reassess benefit designs and risk management strategies. Regulatory changes, including provisions from the Inflation Reduction Act (IRA), will further influence pricing dynamics and market behavior.
Key implications for employers include:

- **Rising Premiums and Stop-Loss Costs:** As high-cost claims become more frequent and severe, stop-loss carriers may raise premiums, tighten underwriting, or impose exclusions—potentially leaving employers exposed.
- **Pressure on Benefit Designs:** Employers may need to adjust benefit structures, coverage policies, and employee communications to balance cost control with patient access.
- **Regulatory and Market Evolution:** The shift from small molecules toward complex cell and gene therapies is accelerating, driven by both scientific innovation and policy changes that influence pharmaceutical investment decisions.[1]

Key Trends and Implications

The U.S. market is posed for significant transformation driven by scientific innovation, evolving regulatory frameworks, and changing payer dynamics.

1. **Continued Growth and Innovation in Specialty and CGTs**

   The pipeline for novel therapies, particularly CGTs, is expanding rapidly. The global pipeline for advanced genetic therapies—including gene, cell, and RNA-based treatments—has surpassed 4,000 therapies in development, spanning from preclinical to pre-registration stages. The FDA is expected to accelerate approvals, shifting the market from traditional small molecule drugs toward biologics and precision medicines. This trend reflects strategic investment decisions by pharmaceutical companies, many of which are reducing research in small molecules to focus on CGTs.[8][9]

2. **Escalating Costs and Complex Financial Implications**

   Beyond the therapy itself, total patient care expenses—including specialized hospital administration, travel, genetic testing, and post-treatment monitoring—can add hundreds of thousands of dollars. This creates substantial financial risk and volatility for payers, particularly employers who may face catastrophic claims from a single patient.[9]

3. **Emerging Payment and Risk Management Models**

   Traditional pharmacy benefit models and utilization management tools are insufficient for low-volume, extremely high-cost therapies. Employers and payers are increasingly exploring innovative financial models to manage risk and ensure patient access.

4. **Regulatory and Policy Influences**

   The Inflation Reduction Act and other federal and state policies are reshaping drug pricing dynamics. While the IRA's Medicare provisions may moderate price growth for existing drugs, new therapies—especially for rare diseases or orphan indications—may launch at higher initial prices. States are also implementing transparency and cost-containment measures, adding further complexity to the market.[10][11]

5. **Market Trends and Cost Drivers**

   Specialty drugs are projected to account for up to 60% of total drug spending by 2025, driven by increased utilization, expanded indications, and the introduction of new high-cost therapies. Oncology and immunology remain the leading growth areas. While biosimilars and generics can provide cost relief, their impact is currently offset by the influx of novel branded specialty therapies.[8][11][12]

6. **Patient Access and Care Coordination Challenges**

   Access to CGTs often requires specialized centers of excellence that provide genetic counseling, coordinated patient management, testing, and therapy delivery. The current fragmented delivery system can increase both financial and emotional stress for patients, highlighting the need for integrated clinical and financial ecosystems to improve outcomes and manage total cost of care.

Emerging Financing Models for Self-Insured Employers

Self-insured employers can better manage the challenges associated with CGT by proactively adopting innovative finance and coverage models to support fiscal sustainability and member access to life-changing treatments. The emerging models below may help address the unique financial dynamics of curative CGTs—particularly their high upfront costs, clinical uncertainty, and unpredictable patient populations. By combining some of these approaches, employers can optimize affordability, manage exposure, and enhance access to these transformative therapies.

FINANCING MODEL	DESCRIPTION	EXAMPLES
Amortization / Installment Payments	Spreading the high upfront costs over a set period through structured payments. This reduces the immediate financial burden, allowing payers to pay in smaller, predictable installments and aligning payments with realized benefits or milestones.	*Example:* A payer agrees to pay $2 million for a therapy over five years in annual installments, contingent on patient outcomes and durability of effect.
Outcomes-Based / Performance-Based Contracts	Payment is linked to clinical outcomes or durability of the therapy, with the manufacturer receiving full payment only if the therapy achieves predefined health benchmarks; otherwise, rebates or refunds are issued. This shares financial risk and incentivizes optimal patient management.	*Example:* A biopharma offers an outcomes-based contract for a sickle cell gene therapy, where reimbursement depends on the patient's response and durability of its benefit over time.
Reinsurance / Stop-Loss Insurance	Payers purchase reinsurance or stop-loss coverage to cap their financial exposure from catastrophic high-cost claims. These policies reimburse or limit costs when expenses exceed a specified threshold, protecting against unpredictable, large claims.	*Example:* A financial services company offers stop-loss products for small and mid-sized employers which covers costs exceeding $2 million, shielding employers from unexpected expenses.
Subscription Models	Payers pay a fixed, predictable fee for access to a portfolio of therapies or for treatment at a specific site, regardless of the number of patients treated. This model simplifies budgeting and spreads risk across multiple treatments or populations.	*Example:* Some regional health systems or Medicaid programs are exploring subscription models for broad access to multiple high-cost therapies, paying a set fee annually for unlimited or capped treatment access.
Warranties / Manufacturer Guarantees	Manufacturers offer warranties that reimburse payers if the therapy does not deliver the promised clinical benefit or durability. This shifts some risk back to the manufacturer and provides payer confidence in the therapy's value.	*Example:* A biotech company offers a warranty for a therapy, refunding a portion of the cost if the patient does not maintain response after a certain period.
Risk Pooling / Multi-Employer Risk Pools	Multiple employers or payers pool their risk to collectively fund high-cost therapies, sharing the financial burden and reducing individual exposure. This approach is especially useful for rare diseases with unpredictable treatment needs.	*Example:* A multi-employer risk pool for rare disease treatments with participating employer's contributing premiums to cover potential costs for members.
Third-Party Contract Negotiation and Management	Engaging specialized vendors or third-party administrators to handle negotiations, data collection, outcome tracking, and payment execution, ensuring transparency and efficiency in complex financing arrangements.	*Example:* Employers contract with a third-party firm to manage outcomes-based contracts, including outcome tracking and rebate processing.
Combined Model (reinsurance, group risk pooling, warranties/guarantees, and level-funding structures)	By combining these four financing models, self-insured employers manage the financial risk of high-cost cell and gene therapies by spreading risk across multiple employers, transfering catastrophic exposure, and offering predictable funding with outcome-based protection.	*Example:* A self-insured employer pays into a pooled program via PEPM/PMPM rates, which reinsures claims for selected therapies while relying on a manufacturer warranty/guarantees to offset costs if a therapy does not meet its clinical goals.

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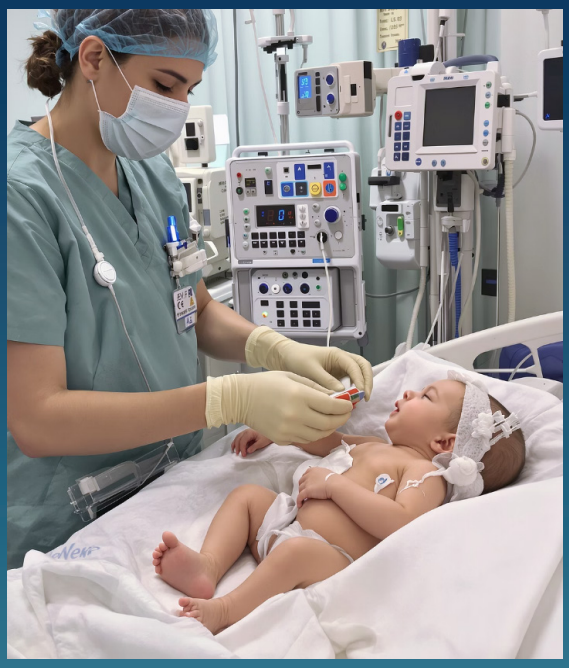
About MBGH

Midwest Business Group on Health (MBGH) is a 501c3 non-profit supporting employers seeking solutions to better manage the high cost of health care and the health and productivity of their covered populations. Founded in 1980, MBGH offers members leading educational programs, employer-directed research projects, purchasing opportunities and community-based activities that increase the value of health care services and the health benefits they offer to members. MBGH serves over 150 companies who provide benefits to over 4 million lives, with employer members spending more than $15 billion on health care each year.

mbgh.org

Real-Life Example for a $2.1 Million, One-time Therapy

Zolgensma, a gene therapy approved for spinal muscular atrophy (SMA), is priced at approximately $2.1 million for a single administration, Zolgensma offers the potential to halt disease progression in infants. For a self-insured employer, the financial exposure from just one case can far exceed annual stop-loss thresholds, underscoring the need for robust risk management strategies[1].



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