ACTION BRIEF

Employer Strategies that Drive Health, Equity and Value

National Alliance of Healthcare Purchaser Coalitions
Driving Health, Equity and Value

AN EMPLOYER ROADMAP TO ADDRESSING CELL AND GENE THERAPIES

ACTION STEPS FOR EMPLOYERS

1. Determine population and pipeline risk.
2. Explore coverage options.
3. Consider non-traditional payment options.
4. Become familiar with new care delivery approaches.
5. Educate and communicate treatment options with employees.

Note: A companion *Action Brief*: "Understanding Cell and Gene Therapies and Its Impact on the Workforce" provides background on this fast-growing field of medicine and how employers can begin to consider developing CGT health coverage policies.

Cell and gene therapy (CGT) breakthroughs are dramatically changing—and saving—lives. Employers are strategizing about how best to provide equitable access to CGTs. The innovative treatments also tend to be costly, often administered in single doses costing between $300,000 and $3.5 million per treatment, which may require employers and other plan sponsors to consider alternative healthcare funding models.

ACTION STEP 1

Determine population and pipeline

CGTs are continuously evolving, introducing exciting new treatment options for employees but creating complexities for plan sponsors assessing population and pipeline. Determining how CGTs may affect a population begins with an analysis of claims data to understand the prevalence of conditions or preconditions that may suggest development of a particular disease.

Many of the diseases that may be treated with CGT are very rare, with a few to perhaps only a dozen affected individuals born each year in the US. The long-term potential savings from curing conditions, such as cancer, blindness, hemophilia and more, has many in the medical community anxiously awaiting approvals. The science of CGT is continuing to advance, and an expanding set of conditions may benefit from these innovations in the future. Therefore, employers should track this space and routinely reassess the potential population eligible for CGT treatment.

Two important determinants of financial risk from the employer's perspective include CGT acquisition costs and real-world effectiveness of the treatment. While CGTs' high fixed costs are a predominant component for many employers, self-insured sponsors also care about cure rates and effectiveness.

A couple of examples include: Hemgenix®, for hemophilia B. It is one of the most expensive gene therapies at

$3.5 million. Based on clinical trial data, the treatment can eliminate the need for repeat treatments for at least eight years, but the drug is anticipated to save the US healthcare system $5–$5.8 million per patient treatment.

The average life expectancy is 52.6 years for a person with sickle cell disease (SCD). The cost of traditional SCD treatment can exceed $1.6 million over the lifetime of a person 64 and younger with the chronic condition. The cost of CGT treatment for SCD is currently $2.2 million.

While the cost of CGT for a specific treatment may be known, cost offset comparisons may be based on variable or individual factors. It can be easier to estimate what the cost would be if the patient had continued the current standard of care including:

- Traditional alternative treatment
- Lost employee productivity due to illness or medical appointments
- Lost productivity due to mental and physical pain and related challenges
- Cost of time off for treatment and recovery
- Short- and long-term disability
- Time off for caregiving

ACTION STEP 2:

Explore coverage options

Employers are exploring ways to cover future costs as part of a larger calculus of which CGTs to cover. Traditionally, healthcare costs and benefits are spread over time. Cell and gene therapies can be front-end loaded, with large upfront costs followed by improved health over a lifetime.

While C&GT advances are truly lifechanging, and in some case lifesaving, they introduce major challenges within the healthcare system that have fueled discussions about they need to pay for these innovations differently.

From, "Cell and Gene Innovative Payment Models in the US – Will they Stick?"

The science ecosystem of CGT is fundamentally different, with treatments being supplanted by innovation in a much shorter time frame. This shortened innovation cycle presents challenges to the pharmaceutical industry, as their ability to recoup investments (including development failures) and fund future innovation is lessened.

Employers and insurers must build a new sustainable finance plans based on the high costs and the new up-front payment paradigm. Different solutions may need to be tailored based on population impact and type of therapy. Eventually, payers may need government support through new risk pools and taxpayer help.

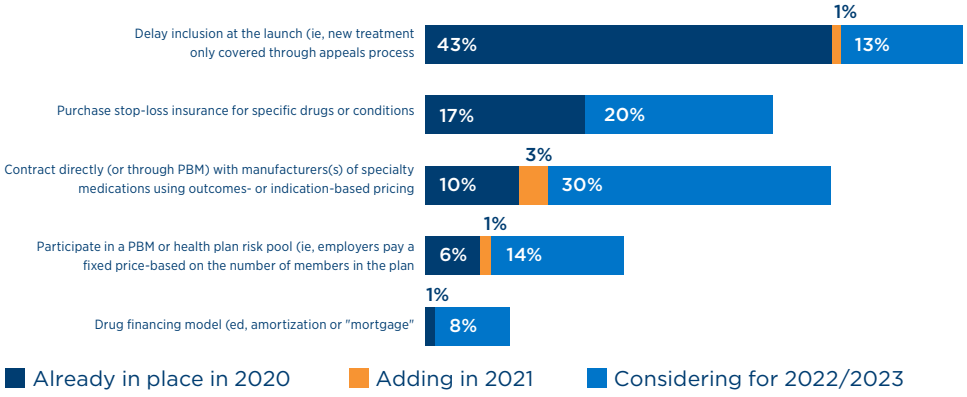
Innovative payment models are more commonly offered for gene therapies given their higher price. Most products demonstrate a health benefit and value case, but challenge legacy healthcare financing models. The government is concerned that private sector payment and contracting options might limit employee access. Elected officials are asking employers to evaluate different contracting or coverage models and what role the government takes.

Innovative payment models and strategic planning can help mitigate financial risks while providing access to life-saving therapies for employees. Here are several approaches employers can consider:

Stop loss

This traditional approach to high-cost claims, whether individual or aggregate, may not be sufficiently practical to employers trying to cover CGTs. Because of the high cost, some insurers may not cover gene therapies or may only cover a single year or limited procedures. Any potential patient carve-out policies put the responsibility for the treatment of any potential complications back on the employer. Because use of CGTs is relatively rare, few stop-loss insurers have used lasers (i.e., blocks against certain CGT coverage). However, some employers already are having necessary discussions with carriers about this issue. A future initiative might be to establish an employer risk pool so large that it can accommodate shifts as CGT-eligible covered lives come and go from companies.

Large employer options for addressing high-cost treatments, 2020-2023



80% of employers are concerned that current strategies to finance high-cost therapies will not be sufficient to contain costs.

Source: Business Group on Health

Installment plans

Some drug manufacturers and insurers are accepting installment payments or annuity payment models for CGTs therapies enabling measured access to these innovative therapies. Paying over time, allows smaller payers and employers to distribute the financial impact over the course of the fiscal year or multiple years. Larger payers may prefer to pay the entire cost up front, receiving a rebate that allows them to tie payments to patient outcomes.

Outcomes-based payments

This model ties payment to patient outcomes, with manufacturers receiving more if the therapy is effective. An example is the CGT Access Model, which is a multi-year voluntary model for states and manufacturers. By increasing access to transformative therapies, this model can potentially help address the historic disparities, poor health outcomes, and low life expectancy associated with SCD. Other conditions might be added to the model over time. Supporting outcomes-based agreements between states and manufacturers lowers prices for states and ties payment to outcomes intended to make it easier for states to pay for cell and gene therapies.

Per member per month (PMPM) assessments

Some insurers are offering protection for CGTs with limitations for an additional per member per month fee. These programs, also known as gene therapy "financial protection programs" are now offered by many large payers and sometimes called "Netflix models" as the subscription provides "unlimited" access to gene therapies for a single fee.

Third-party networks

Third parties with stand-alone provider networks and risk-sharing agreements with pharmaceutical companies are underwriting targeted coverage based on their condition expertise. Employers must manage out-of-network coverage exemptions.

Health Savings Accounts (HSAs) and Flexible Spending Accounts (FSAs)

Encouraging the use of HSAs and FSAs can help employees set aside pre-tax dollars for high-cost treatments, including cell and gene therapies. Employers can contribute to these accounts to further support the workforce. Contribution limits may need to be increased.

Partnerships and consortia

Joining forces with other employers, either directly or through consortia, can increase bargaining power with insurance carriers and manufacturers. This collective approach can lead to better pricing or access to innovative payment models.

Patient assistance programs

Many pharmaceutical companies offer patient assistance programs to help cover the cost of expensive therapies. Employers can facilitate access to these programs for employees, reducing the financial burden on the employee and the employer.

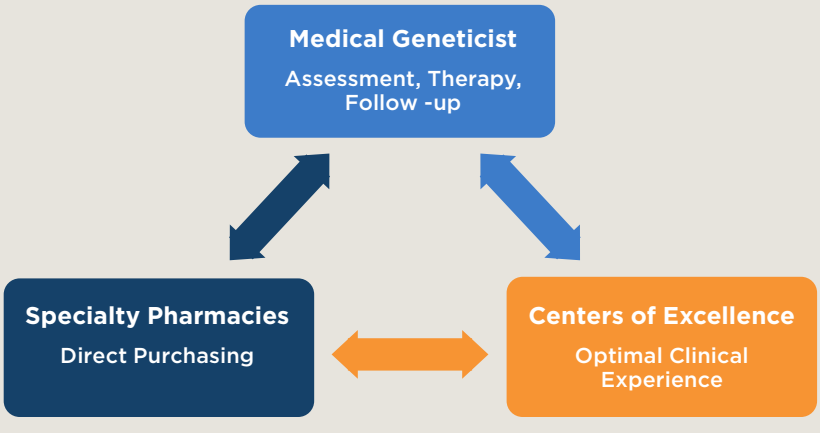
ACTION STEP 3:

Become familiar with new care delivery approaches

Because CGT treatments are new, care providers are only beginning to develop effective care pathways.

When CGT treatments were first developed, delivery was through academic institutions certified by biopharma companies. Over time, organizations such as the Joint Accreditation Committee will establish standardized care for some CGT models. Therapies will become available in broader clinical settings. How care models and pathways evolve may vary by the therapy, its popularity, and the cost model. Some treatments may be best suited for specialty centers of excellence, some for integrated care involving hospitalization, and some for community health centers.

The New Ecosystem for CGT Delivery



Once clear care pathways evolve, three entities in some combination will carry forward the actual hands-on and day-to-day work of CGTs:

1. **Medical Specialists with Genetics Training**
   The role of medical specialists with training in genetic is being redefined by CGTs. With the growth of CGTs, their role is expanding to a more hands-on clinical experience with patients including developing models of care and educational programs and will be involved directly with patients in assessment, therapy administration and follow up.
2. **CGT Centers of Excellence**
   The centers of excellence model for CGT are based on an optimal clinical experience (including complex case management and cell and gene transplants), quality of outcomes, and competitive economic factors. Many centers are affiliated with academic institutions leading CGT research. The American Society of Gene + Cell Therapy tracks development.
3. **Specialty Pharmacies**
   Employers and plan sponsors will contract directly with specialty pharmacies for CGT drugs, rather than purchasing through a pharmacy benefit manager (PBM) for potential savings.

ACTION STEP 5:

Educate and communicate treatment options with employees

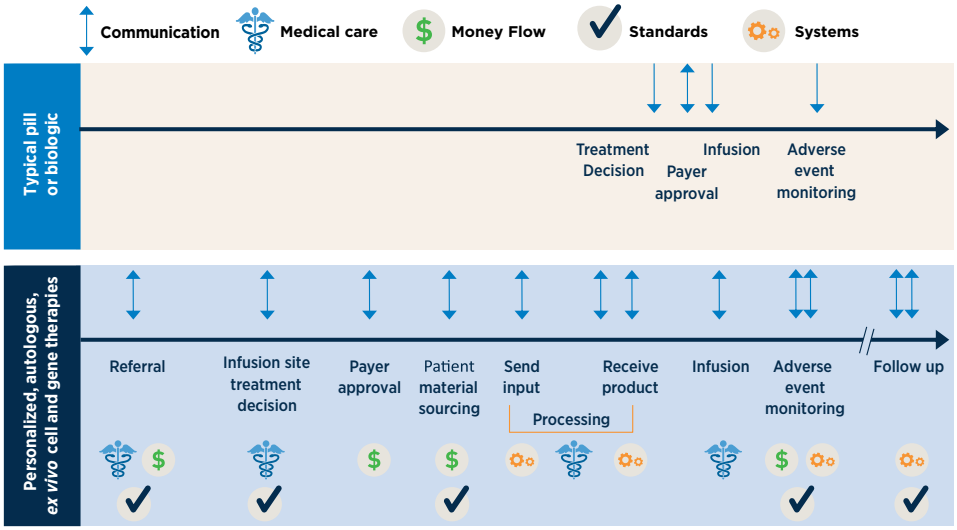
As employers begin to cover CGTs in benefit plans, they will be tasked with implementing educational programs so employees can make informed decisions. Because so few employees are affected, employers will need targeted strategies.

Employers may consider hosting targeted workshops or seminars led by healthcare professionals, manufacturers, researchers, or experts in the field. Employees will want to understand available treatments, how to qualify, what the treatment and recovery

Eight Questions to Ask When Engaging CGT Vendors

1. Which accreditations or certifications does the facility or team possess in CGT?
2. Which CGTs are offered and what are the treatment specifics, such as the mechanism used and expected outcomes of therapies?
3. What safety measures are in place during the administration of therapies? Can the employer document compliance with all regulatory standards and guidelines?
4. What are statistics on efficacy and outcomes, showing success rates and information on the patient experience?
5. How easily accessible are therapies for employees in terms of location and scheduling? Are there telemedicine or remote consultation options for employees in different locations?
6. What are the costs associated with therapies for both the employer and the employees? How are costs covered?
7. What kind of post-treatment support and follow-up care are provided? How are patients monitored and managed for long-term effects or complications?
8. Are educational resources or workshops available to help employees understand therapies better?

CGT: A More Complex Patient Journey



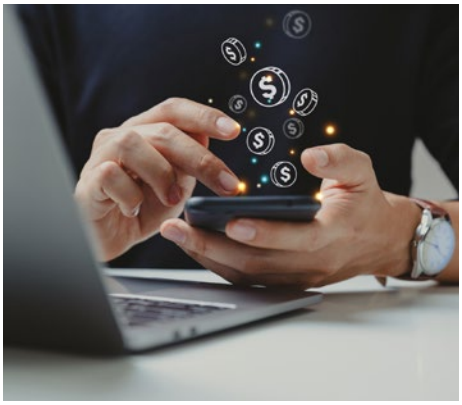
This diagram from Gene Therapy maps the patient journey when receiving personalized (or "precision") medicine such as CGTs, and compares it to the more traditional patient journey involving mass-produced medicine.

is like, and what the treatment costs will be. The patient journey for CGTs (see diagram) is more complex than traditional medicine. Case studies help personalize the information.

Employees living with rare diseases eligible for CGTs but facing a lack of coverage may challenge coverage decisions. Employers need to consult with their legal and medical teams about these decisions and communicate the coverage rationale.

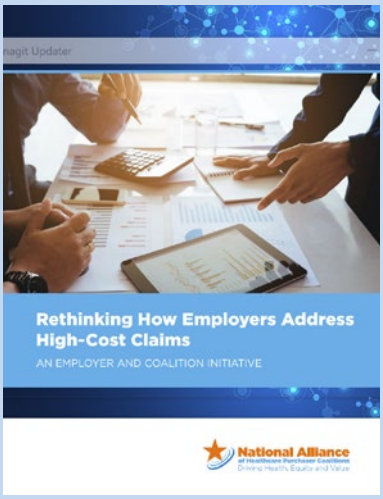
The management of CGT high-cost claims is evolving, and self-insured plan sponsors are proceeding with caution and empathy. The optimism around these lifechanging, and in some cases lifesaving, therapies is balanced with an need to navigate and assess models that can ensure sustainable access for patients.

National Alliance Action Brief: Understanding Cell and Gene Therapies and Coverage Complexities (link coming soon!)



RESOURCES

- FDA list of approved cell and gene therapy products
- "Gene Therapy: Yesterday, Today and Tomorrow," video by the National Organization for Rare Disorders
- "Gene Therapy: Your Questions Answered," video by the National Organization for Rare Disorders
- Gene and Cell Therapy FAQs, American Society of Gene and Cell Therapy.
- Gene therapy resources, Gene Therapy Center, UNC School of Medicine
- National Alliance Action Brief: New Directions to Better Manage High-Cost Claims
- National Alliance Guidebook: Rethinking How Employers Address High-Cost Claims



A National Alliance special report, with recommendations across cancer, prenatal/neonatal care, rare disease and gene therapy and specialty drugs.

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