# GENE AND CELL THERAPY: WHERE COMPASSION AND COST COLLIDE

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As advancements in how individuals consume healthcare have drastically and rapidly improved (i.e., the prevalence of virtual patient care, online pharmacies where treatments can be received and delivered to the home, etc.) face-to-face communication between providers and patients has often been supplanted by virtual interactions that expedite processes. The result is faster and easier access to healthcare.

Improved access is certainly a step forward, but is it enough? For patients diagnosed with a rare or life-threatening condition where the only treatment is difficult to obtain, the answer is probably not.

Availability is a challenge for patients seeking gene and cell therapy as treatment can cost millions to obtain. Cost is a factor and has the potential to impact the availability of treatment for patients. Access and availability are not always in parity when it comes to patient care.

Research shows and common sense would suggest that providing healthcare services for patients to maintain or restore their physical, mental, or emotional well-being is best when the patient care is embedded with empathy and kindness; it greatly enhances the healing process. Compassion is important for healthcare providers, and for employers with self-funded healthcare benefit plans.

Gene and cell therapies are quickly evolving, and employers need to understand the potential impact. Employers face the tall order of exuding empathy while relating to employees contemplating the multi-million-dollar treatment that is gene and cell therapy.

Even the most compassionate of such professionals find it difficult to strike the delicate balance of weighing costs to their respective plans with the natural inclination to provide coverage for potentially lifesaving or life-altering therapy.

Knowledge and a solid game plan for how to manage gene and cell therapy within their plan design, however, will allow employers to confidently focus their energy on the employee facing life-threatening circumstances instead of being blindsided by the economics of the situation. By understanding the nuances and limitations, plan sponsors should be well-positioned to employ a strategy that uniquely balances compassion and cost.

The following discussion highlights some of the plan administration and documentation considerations for employers navigating the evolving gene and cell therapy landscape.

## GENE AND CELL THERAPY BACKGROUND

According to the FDA, gene therapy is a method that modifies a person's genes to treat or cure disease, while the American Society of Gene + Cell Therapy (ASGCT) defines cell therapy as the transfer of live cells into a patient to treat or cure a disease -- i.e., a blood or marrow transplant is a type of cellular therapy.

Undoubtedly, accessibility and attainability are challenges for patients and plan sponsors as they learn more about cell and gene therapies, which has been a very hot topic in recent years. The response to these innovative treatments has been mixed: The medical community is advocating the new treatments for patients hoping to find a cure for their respective rare diseases while health plans remain concerned about potential costs.

In the past couple years, the pipeline of both pending and U.S. Food & Drug Administration (FDA) approved treatments has grown. In fact, in January 2019 FDA Commissioner Scott Gottlieb noted the surge in cell and gene therapy products entering early development and predicted that the FDA would be approving 10 to 20 cell and gene therapy products a year by 2025 based on the assessment of the current pipeline and clinical success rates.

Flash forward to the present day and as of April 2023, the FDA has (according to fda.gov) approved over 25 cell and gene therapy products. Furthermore, according to the Alliance for Regenerative Medicine (ARM), there could be as many as 13 brand new cell or gene therapies approved in the United States, Europe, or both by the end of 2023, and there is a large queue of various clinical trials for gene and cell therapies listed on the ClinicalTrials.gov website.

Why is there so much excitement over these developments? Gene and cell therapies potentially offer a new avenue for treatment in cases where there were otherwise limited options for such rare and life-jeopardizing diseases.

Indeed, some of the FDA approved cell and gene therapy treatments on the market have yielded curative changes with a one-time treatment. Long term metrics and results are still being examined, but as the interest

in and education about these types of treatments increases, employers who sponsor self-funded health plans should take the opportunity to decide how to address such coverage within their plan designs.

## PLAN LANGUAGE CONSIDERATIONS

Many factors should be considered by employers as they navigate this new landscape.

For example, if a treatment is approved for use in Europe, but not in the United States, would such benefit be eligible for coverage under the current plan design (if the individual travels to Europe) or is there an exclusion for foreign travel (i.e., benefits received outside the United States if travel is for the sole purpose of obtaining medical services)?

Even if plans have the strictest language possible for international medical tourism, how does the plan currently address gene and cell therapy? Is there an exclusion? Is there a benefit? Are there limits? Is the plan silent?

This is an important conversation as cutting-edge science and technology continue driving forward the current state of medicine in the United States, and the queue for more FDA approved gene and cell therapies is rapidly growing.

While it's undeniable that these advancements in treatment options can come with an overwhelming price tag, the conversation becomes more nuanced when further questions are considered: What if covering the one-time treatment could potentially cure the condition and offset the need for other expensive ongoing or life-long care? Further, if the plan did cover such benefits would they be reimbursable by the plan's stop loss carrier? Are there alternative payment options?

Plans, however, are still permitted (and should) ensure benefits are covered subject to the appropriate medical management techniques.

For example, plans should have a strong definition and policy for how medical necessity is determined. The plan should also review the definition of experimental and investigational to ensure the language is in line with its expectations. Other plan options may include pre-certification or prior authorization requirements on the benefits.

## COMPLIANCE CONSIDERATIONS

A key question plans may be asking is what they are required to cover. The Affordable Care Act (ACA) prohibits plans from imposing dollar limitations on any of the 10 categories of essential health benefits (EHB). To the extent the plan covers an EHB, no dollar limitations may apply to that benefit. Self-funded plans should have identified a particular state benchmark to which they determine and classify EHBs under the plan design.

In 2022, HHS released the 2023 final payment rules which provide that for plan years beginning on or after January 1, 2023, an EHB plan design should be clinically based, among other factors.

Further, in late 2022, HHS published a request for information (RFI) asking for public comment by January 31, 2023, regarding a variety of topics, but including the description of EHBs and questions involving the barriers to accessing services due to cost, coverage and whether EHBs needed to be updated to account for changes in medical evidence or scientific advancements.

The timing for plan design changes should be considered as well. Plan design modifications should comply with the ACA and ERISA timing requirements and may be best incorporated at plan renewal to mitigate discrimination concerns.

Consequently, coverage and access issues remain pressing issues. Plans should be mindful when it comes to their decision-making surrounding how and whether to extend (or limit) coverage for gene and cell therapy. These are tough choices for employers and plan sponsors, and they need support as they weigh the various factors and considerations to make an educated decision.

## DEFINE A STRATEGY

As gene and cell therapies develop so too are the options for plan sponsors when it comes to potential payment methodologies. Traditional health plans were not structured to handle one-shot, high-cost treatments so alternative options are important to consider.

For example, new reinsurance programs are emerging to offer options for plans hoping to cover gene and cell therapies. Each of these programs is unique and should be reviewed against the existing plan materials to ensure the program aligns with the intentions of the plan sponsor and the terms of the plan document/summary plan description.

Patient support programs where patients receive support for cell and gene therapy may also be available. Unlike traditional drug patient assistance programs, these programs may offer psychosocial, economic, and caregiver assistance to the patients and their families.

The support programs may be individualized to address clinical issues and matters involving logistical and transportation related support, financial support, and nurse navigation support.

Other payment strategies could include multiple year payment plans, reimbursement based on meaningful outcomes or performance-based measures for the treatment, payment model where the price reflects the value, or a methodology that includes a warranty (where a refund may be issued if the treatment fails after a certain time).

In adopting a strategy for coverage (or exclusion, or limited coverage) of gene and cell therapies, plan sponsors should balance the various factors. While there may not be a perfect solution, plans need a strategy that is clearly consistent with plan materials so there is no confusion among plan participants (or between other contracts held by the plan sponsor).

For example, in navigating this evolving area of benefits, plan sponsors should ensure the plan document/summary plan description aligns with their coverage decision; the stop loss policy provisions are free from conflict with the underlying plan materials; review any potential supplement programs that may assist in the coverage of these gene and cell therapy benefits; continually monitor the regulations as state and federal laws regarding price transparency and drug pricing pass to ensure ongoing compliance; and keep abreast of the pipeline of pending and newly approved FDA therapies.

New (and costly) gene and cell therapy treatments are on the horizon. As a result, it will be important for employers to have the appropriate balance between tight controls to ensure patients see the value and benefit of these high-cost treatments. Having a strategy that successfully connects employees and plan members with life-changing treatments and mitigates the financial impact, while demonstrating compassion, is critical. ■

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