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Million dollar drugs have arrived

Key strategies plan sponsors should consider

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As pharmaceutical innovation continues to thrive, novel categories of ultra-high cost drugs called gene and cell therapies are entering the market. These cutting-edge products have the potential to drastically improve or even cure certain serious or terminal conditions. Unlike traditional drugs, most gene and cell therapies are given as one-time treatments, fundamentally changing the natural course of the illness and offering hope to many individuals. However, because of their high development costs and promising clinical outcomes, gene and cell therapies are astronomically expensive, raising significant concerns of access and affordability.

One prominent example is Zolgensma, a gene therapy approved in late 2019 for treatment of a rare, often-fatal neuromuscular disorder in children. Priced at $2.1M per single treatment course, Zolgensma currently holds the title of most expensive drug in the world. Roctavian, an investigational gene therapy for treatment of Hemophilia A, a rare bleeding disorder, is another product gaining recent attention. Scheduled for FDA review in 2022, Roctavian is anticipated to cost $2-3M per treatment course, if approved. Along with the shocking price tags, the difficulty of predicting these one-time claims further compounds the budgeting challenges these drugs present to plan sponsors.

While only a handful of gene and cell therapies are on the market today, the narrative may soon change as the drug pipeline continues to swell. By 2025, the FDA predicts they will be reviewing and approving 10-20 gene and cell therapies per year[1]. In addition to the sheer increase in the number of available products, in time, the gene and cell therapy market will venture beyond targeting only rare conditions and select cancers to targeting common conditions, such as HIV and heart failure.

With these changes on the near horizon, plan sponsors should take action today. Building a sustainable, long-term strategy now will help mitigate plan cost exposure and ensures member access to these therapies in years to come. Realizing that a silver bullet does not exist, plan sponsors should begin by exploring the following spectrum of strategies and regularly engage with their vendors to stay up to date as new solutions emerge:

Understand your plan's current coverage of these products.

Are gene and cell therapies covered under the plan? Do these therapies flow through the most clinically appropriate, cost effective part of the benefit? Confirming these coverage

details with medical and pharmacy vendors and ensuring coverage aligns with the plan's philosophy and goals is the first step in building successful management strategies.

Because gene and cell therapies may be the only treatment choice or a last resort option for a serious or life-threatening disease, excluding these products from coverage may raise legal, ethical, and public relations issues. Accordingly, most plan sponsors consider exclusion of these therapies as an extreme and potentially problematic cost containment strategy and do not currently pursue it. Potential long-term savings of these therapies also plays a role in coverage decisions since these products may actually reduce or eliminate the need for other ongoing costly medical and pharmacy treatments.

These complex products require clinician administration in specially designated healthcare facilities and are therefore typically housed under the medical benefit. As long as the medical carrier has adequate cost and clinical management strategies in place, this approach is appropriate. However, plan sponsors should make sure other gene and cell therapy strategies, such as stop loss coverage and clinical programs, are also in sync with the channel designated for coverage.

Confirm robust utilization management rules are in place.

Gene and cell therapies have very narrow indications and carry the risk of serious side effects. Before treatment begins, healthcare providers should confirm patients meet clinical criteria such as appropriate diagnosis, age, disease severity, and ability to tolerate treatment. In the case of gene therapy, genetic testing should be performed to confirm presence of the specific genetic mutation being targeted. Cell therapies may require testing and pre-treatment to confirm that an individual's immune system can tolerate therapy. Applying precertification requirements to gene and cell therapy claims would ensure improved safety and clinical outcomes for members, while protecting the plan against inappropriate utilization. Plan sponsors should confirm that prior authorization requirements are in place for all gene and cell therapies currently available on the market. Additionally, plan sponsors should check that the criteria for approval does not go beyond the FDA approved indication for use as entities like stop loss carriers may challenge that the use was "experimental," potentially withholding payment.

Ensure therapy access through clinically superior, cost effective treatment facilities.

Gene and cell therapy administration is very complex and requires high-touch clinical support. For this reason, manufacturers restrict administration of these therapies to designated treatment centers that have the operational capabilities and clinical expertise to properly administer these products and ensure positive clinical outcomes. The manufacturers' selection criteria may require adequate access to specialists, geographical proximity to major metropolitan areas, and unique facility capabilities such as in-house cryopreservation, - a technique for freezing live cells. Although manufacturers conduct this rigorous screening, medical carriers may provide an additional layer of oversight by promoting or requiring the use of centers of excellence and appropriate care management protocols.

In addition to taking measures to promote clinical care and outcomes, medical carriers are exploring solutions that seek to control the cost of these therapies within the network. At least one medical carrier has recently created a narrow network for gene and cell therapy drugs; this network promises to lower claim costs for plan sponsors without sacrificing access or quality of care for members. While this offering is brand new and savings data is not yet available, it represents a step in the right direction and demonstrates that medical carriers are starting to evolve their gene and cell therapy programs. Plan sponsors should confirm their medical carrier's long-term network strategy including availability of programs that may enhance clinical care and control claim costs for gene and cell therapies.

Evaluate your stop loss coverage options.

Currently, most traditional stop loss policies cover gene and cell therapy claims, as long as the treatments are covered under the medical/pharmacy plan. That said, as these therapies continue to gain attention, alternative approaches to funding these claims are starting to emerge in the marketplace. At least one stop loss carrier has created a hybrid offering that provides comprehensive stop loss coverage at a high limit, with a separate step-down deductible for two currently approved gene therapy drugs (Zolgensma and Luxturna). Additionally, many medical carriers and PBMs have developed alternative funding arrangements that solely target gene therapy. However, many of these programs have not seen wide spread adoption since the stop loss carriers are already agreeing to cover gene and cell therapy claims under the stop loss policy. Also, these alternative programs currently offer coverage for a limited number of therapies, with low incidence rates and minimal (if any) premium savings available on the stop loss coverage. Additional evaluation will be needed as these programs further develop, to determine how they fit into the existing stop loss framework, and if they offer any unique advantages.

Stay up to date on the development of alternative payment models.

Since long-term efficacy data for gene and cell therapies is limited but the corresponding price tags are staggering, plan sponsors may worry about paying a fortune for treatment that will fail. In light of this reality, the concept of outcomes based reimbursement becomes appealing. As the name implies, this reimbursement model ties the clinical outcomes of therapy to its ultimate cost. While this model is certainly promising, outcomes based agreements for gene and cell therapy are still in their infancy and face significant obstacles, including difficulty of measuring appropriate clinical endpoints, government regulations that indirectly prevent the ability to discount these drugs, as well as data and logistical challenges. Despite this outlook, continued pressure from plan sponsors and other key stakeholders in the healthcare system may eventually change the status quo. With that in mind, plan sponsors should request regular updates from medical carriers on outcomes based reimbursement and other payment models.

Continuously reevaluate your risk and evolve your strategy.

Last but not least, a key pillar to a sponsor's overall strategy is realizing that a plan's propensity for incurring these claims may vary based on its unique member population and the drugs available on the market. As more of these costly treatments gain approval or as the plan's patient population fluctuates, plan sponsors should regularly assess their risk level and update their strategy accordingly. Engage with your Mercer consultants to explore available predictive analytics tools that may help you understand your plan's risk for these ultra-high cost claims.

While gene and cell therapies certainly represent an exciting breakthrough in treatment of serious illnesses, these products create unique, unpresented cost challenges for plan sponsors. As more of these costly therapies enter the market and reshape the drug treatment landscape, key stakeholders in the healthcare system will be pressured to evolve their approach to reimbursement and clinical care. By building a comprehensive strategy today, plan sponsors will be more prepared to mitigate costly gene and cell therapy claims while preserving member access to these life-altering therapies.

Sources:
[1] https://www.fda.gov/news-events/press-announcements/statement-fda-commissioner-scott-gottlieb-md-and-peter-marks-md-phd-director-center-biologics